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Ultragenyx Announces Interim Data From Phase 2 Extension Study of Sialic Acid Extended-Release at International Congress of the World Muscle Society

NOVATO, Calif., Oct. 13, 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 results from a Phase 2 extension study of sialic acid extended-release (SA-ER, UX001) tablets in patients with hereditary inclusion body myopathy (HIBM; also known as GNE myopathy), a rare, progressive muscle-wasting disease. SA-ER is designed to replace the deficient sialic acid substrate in patients with HIBM. The data were presented at the 19th International Congress of the World Muscle Society (WMS) in Berlin.

"The data from the extension study of SA-ER support our plans to move forward with this program," said Sunil Agarwal, M.D., Chief Medical Officer of Ultragenyx. "While the 12 gram per day dose did not appear to have a clear advantage over the 6 gram per day dose, it does provide additional evidence of activity and safety and we are encouraged to see a potential long-term impact on disease progression in upper extremity muscle strength after approximately two years of treatment."

Patients in the initial Phase 2 study were randomized to receive placebo, 3 grams/day, or 6 grams/day of SA-ER. After 24 weeks, placebo patients crossed over to either 3 grams/day or 6 grams/day, on a blinded basis, for an additional 24 weeks. The 48-week analysis compared change from baseline for the combined groups at 6 grams/day versus 3 grams/day of SA-ER.

The initial Phase 2 data, which were presented at the American Academy of Neurology (AAN) Annual Meeting in April 2014, showed a statistically significant difference in the upper extremity composite (UEC) of muscle strength at 48 weeks with the higher dose group compared to the lower dose group. SA-ER appeared to be safe and well-tolerated with no serious adverse events observed to date. Most adverse events were mild to moderate and most commonly gastrointestinal in nature.

In the first part of the extension study, all 46 patients from the 48-week Phase 2 study crossed over to 6 grams/day for a variable period of time that was on average 24 weeks. In the second part of the extension study, all 46 patients and 13 treatment-naïve patients received 12 grams/day of SA-ER for 24 weeks. The results presented at WMS include the 49 out of 59 patients who had 24 weeks of data at the higher dose. While the 12 grams/day data do not suggest any clinically meaningful advantage over 6 grams/day, the 12 gram data do provide additional data that support clinical activity with SA-ER treatment. The 12 gram daily dose of SA-ER appeared to be generally safe and well tolerated with no drug-related serious adverse events, but the rate of mild to moderate gastrointestinal adverse events did appear to be greater with this dose. Over the entire approximate two-year study, treatment with SA-ER appeared to slow the progression of upper extremity disease when compared to the 24-week placebo group extrapolated out to two years.

Based on the 48-week and extension study data, Ultragenyx intends to discuss with regulatory authorities a potential pivotal study of SA-ER in HIBM patients. The company will also continue to treat patients in the ongoing extension study.

About Hereditary Inclusion Body Myopathy

Hereditary inclusion body myopathy (HIBM) is also known as GNE myopathy. HIBM is a rare, severe, progressive, genetic neuromuscular disease caused by a defect in the biosynthetic pathway for sialic acid, with onset in the late teens or twenties. The body's failure to produce enough sialic acid causes muscles to slowly waste away and can lead to very severe disability, with patients typically becoming wheelchair bound and losing most major muscle function within ten to 20 years from onset. There are approximately 1,200 to 2,000 HIBM patients in the developed world, and there is currently no approved therapy.

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 the potential impact of SA-ER on the progression of HIBM and plans for a potential pivotal 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 August 11, 2014, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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