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Ultragenyx Announces Completion of Enrollment in Phase 3 Study of Aceneuramic Acid Extended Release (Ace-ER) in GNE Myopathy

NOVATO, Calif., July 27, 2016 (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 that it has completed patient enrollment in the Phase 3 study of aceneuramic acid extended release (Ace-ER) for the treatment of GNE myopathy. Data from the study are expected in 2017.

"Patients with GNE myopathy typically are diagnosed in their twenties and thirties, are wheelchair-bound within ten to twenty years of diagnosis, and continue to suffer muscle atrophy as they lose strength in the upper-extremity muscles as well. Our pivotal Phase 3 study is intended to evaluate the effect of Ace-ER on this devastating disease," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx.

The Phase 3 global, randomized, double-blind, placebo-controlled clinical study is designed to assess the efficacy and safety of six grams per day of Ace-ER over 48 weeks in 89 patients. The primary endpoint of the study is a comparison between active and placebo treated patients for the change from baseline to 48 weeks in the composite of upper extremity muscle strength as measured by hand-held dynamometry (HHD). Key secondary endpoints include the GNE myopathy-functional activity scale (GNEM-FAS), a disease-specific patient-reported outcome (PRO) that includes measures of mobility and upper-extremity function, a composite of lower extremity muscle strength, and other measures of lower-extremity muscle strength and function. The Phase 3 study design and primary endpoint were based on feedback from the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA).

Ultragenyx is also currently seeking conditional marketing authorization from the EMA for Ace-ER for the treatment of adults with GNE myopathy, based on data from the Phase 2 randomized, double-blind, placebo-controlled study. The Committee for Medicinal Products for Human Use (CHMP) opinion is expected in the second half of 2016, with a final decision expected in the first half of 2017. If conditional approval is granted, the Phase 3 study is expected to be required for confirmatory purposes. If conditional approval is not granted, the Phase 3 study would be required for approval.

About GNE Myopathy

GNE Myopathy is also known as Hereditary Inclusion Body Myopathy (HIBM). GNE Myopathy is a rare, severe, progressive, genetic neuromuscular disease caused by a defect in the biosynthetic pathway for sialic acid, with onset usually occurring in the 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 within 10 to 20 years and ultimately having to rely on others for daily care due to the loss of upper extremity and other muscle function. There are approximately 2,000 GNE Myopathy patients in the developed world, and currently there is no approved therapy.

About Aceneuramic Acid Prolonged Release (Ace-ER) Treatment in GNE Myopathy

Aceneuramic acid prolonged release, also known as aceneuramic acid extended release (Ace-ER) outside of Europe, is being developed as a substrate replacement therapy for the treatment of adult patients with GNE Myopathy who have a genetic defect in sialic acid production. A Phase 1 single and multiple dose study and a Phase 2 randomized, double-blind, placebo-controlled study with Ace-ER have been completed, and the Phase 3 study has completed enrollment and is ongoing.

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 intent to file an MAA and the anticipated timing of such filing and the expected timing of receipt of a decision from the CHMP and the EU on the application pending before from the EMA, as well as the timing of release of data from the pivotal study of aceneuramic acid, 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 (including with respect to the MAA we filed seeking conditional approval from EMA with respect to Ace-ER), whether the Phase 3 results for Ace-ER will in fact confirm or mirror the results from the prior Phase 2 study, and whether the FDA and/or EMA will accept the primary endpoint from the Phase 3 study of Ace-ER. 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 March 10, 2016, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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