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Ultragenyx Announces Top-Line Results from Phase 3 Study of Ace-ER in GNE Myopathy

Study did not meet its primary endpoint

NOVATO, Calif., Aug. 22, 2017 (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 a Phase 3 study evaluating aceneuramic acid extended release (Ace-ER) in patients with GNE Myopathy (GNEM) did not achieve its primary endpoint of demonstrating a statistically significant difference in the upper extremity muscle strength composite score compared to placebo. The study also did not meet its key secondary endpoints. Adverse events were generally balanced between Ace-ER and placebo and safety was consistent with previously released Ace-ER data. Ultragenyx plans to discontinue further clinical development of Ace-ER.

"We are disappointed by these results, as we had hoped that Ace-ER would offer a new option for GNEM patients. We would like to thank the patients, caregivers, and investigators involved in the Ace-ER development program," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "This outcome does not affect our overall strategy, as the company moves forward with multiple preclinical and clinical programs and regulatory filings."

The Phase 3 Ace-ER study enrolled 89 adults with GNEM able to walk ≥ 200 meters in the six minute walk test. Patients were randomized 1:1 to Ace-ER at a dose of 6g/day or placebo for 48 weeks. The study did not meet the primary endpoint of demonstrating a statistically significant improvement in UEC score (+0.74 kg, $p=0.5387$) for Ace-ER treated patients ($n=45$, -2.25 kg) compared to placebo ($n=43$, -2.99 kg) patients for the change from baseline to 48 weeks. There were three pre-specified key secondary endpoints, including the lower extremity muscle strength composite score as measured by hand-held dynamometry (HHD), physical functioning using the Mobility domain of the GNE Myopathy-functional activity scale (GNEM-FAS), and a measure of muscle strength in knee extensors. The study did not meet any of these key secondary endpoints.

Overall, Ace-ER was well tolerated, with slightly more patients experiencing treatment emergent adverse events and related treatment emergent adverse events. There were three serious adverse events including two on Ace-ER and one on placebo, none of which were considered treatment related. There were no discontinuations due to treatment emergent adverse events, and there have been no deaths in the study.

The company plans to terminate the development program based on these results and will work with investigators and patient groups to make available the valuable natural history data and development tools for the development of other therapies. The company will be working with investigators and patients on a reasonable transition plan for patients still on Ace-ER.

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 plans for its clinical programs and ongoing or additional studies for its

product candidates, 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, such as the regulatory approval process, the timing of our regulatory filings, and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations and the availability or commercial potential of our drug candidates. 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 July 28, 2017, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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