January 12, 2015

# Ultragenyx Announces Intent to File for Conditional Approval in Europe for Sialic Acid Extended-Release Tablets in Hereditary Inclusion Body Myopathy

NOVATO, Calif., Jan. 12, 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 its intent to file a Marketing Authorization Application (MAA) seeking conditional approval from the European Medicines Agency (EMA) for the use of six grams per day of sialic acid extended-release (SA-ER; UX001) tablets in the treatment of hereditary inclusion body myopathy (HIBM; also known as GNE myopathy). SA-ER is designed to replace the deficient sialic acid substrate in patients with HIBM, a rare, progressive muscle-wasting disease. Based on Scientific Advice recently received from the EMA's Committee for Medicinal Products for Human Use (CHMP), the company intends to file an MAA in the second half of 2015 for stabilization of upper extremity muscle strength.

"HIBM is a devastating disease that can lead to severe progressive and irreversible muscle damage," said Sunil Agarwal, M.D., Chief Medical Officer of Ultragenyx. "Based on the Phase 2 data demonstrating slowing of disease progression, we are pursuing conditional approval of SA-ER in the European Union in order to accelerate access to this therapy for patients who otherwise have no approved treatment options."

The EMA may grant conditional marketing authorization when the potential treatment addresses a severely debilitating disease with an unmet medical need, has a positive benefit to risk profile, and the benefits to public health of its immediate availability outweigh the risks inherent in the fact that additional data are still required. Ongoing or new studies must be completed with the objective of confirming that the benefit to risk balance is positive. The approval is renewed on an annual basis until all obligations have been fulfilled, at which point a full approval may be granted.

In order to satisfy the EMA's requirement for additional controlled data, Ultragenyx plans to initiate a global, randomized, double-blind, placebo-controlled Phase 3 study of six grams per day of SA-ER in patients with HIBM in mid-2015. A composite of upper extremity muscle strength (UEC) will be the primary endpoint. Key secondary endpoints include GNE myopathy-functional activity scale (GNEM-FAS) (including patient-reported outcome scores of mobility and upper extremity function), and several measures of lower extremity muscle strength including the lower extremity muscle strength composite (LEC). The US Food and Drug Administration (FDA) has accepted this same Phase 3 study design as pivotal, including the primary endpoint of UEC.

**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 2,000 HIBM patients in the developed world, and there is currently no approved therapy.

**About SA-ER Treatment in Hereditary Inclusion Body Myopathy**

A Phase 2 randomized, double-blind, placebo-controlled study with SA-ER has been completed. The data showed a statistically significant difference in the upper extremity composite of muscle strength at 48 weeks with a higher dose group compared to a lower dose group. SA-ER appeared to be generally safe and well-tolerated with no serious adverse events observed to date. Over an approximate two-year treatment period in the Phase 2 study and long-term extension, SA-ER appeared to slow the progression of upper extremity disease when compared to the 24-week placebo group extrapolated out to two years.

**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, as well as plans for a potential pivotal study and the timing of same, 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.*

CONTACT: Ultragenyx Pharmaceutical Inc.

844-758-7273

For Media, Bee Nguyen

For Investors, Robert Anstey