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Ultragenyx Announces Presentation of Data From Long-Chain Fatty Acid Oxidation Disorder Patients With Cardiomyopathy Treated With Triheptanoin

Orphan Designation for Three Additional FAOD Subtypes Granted in Europe

NOVATO, Calif., Sept. 1, 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 open-label data from five infants with cardiomyopathy due to long-chain fatty acid oxidation disorders (LC-FAOD) treated with triheptanoin (UX007). Severely affected LC-FAOD patients can present early in life with severe cardiomyopathy, arrhythmia, heart failure, hypoglycemia, hepatic dysfunction, and rhabdomyolysis that can lead to death. The data were presented at the Society for the Study of Inborn Errors of Metabolism (SSIEM) Annual Symposium in Lyon, France.

"Some LC-FAOD patients can progress to severe and life-threatening cardiomyopathy while on standard of care," commented Sunil Agarwal, M.D., Chief Medical Officer of Ultragenyx. "We are encouraged by the preliminary results presented at SSIEM indicating the potential to help these severely affected patients."

Case reports from five infant patients with moderate or severe cardiomyopathy due to LC-FAOD were presented. All patients were detected by newborn screening and managed with standard treatment, including medium-chain triglyceride oil. While on the standard of care, the patients were hospitalized with heart failure that required cardiac support (ventilation, ECMO, vasopressors) and, in some cases, resuscitation. The patients discontinued medium-chain triglyceride oil and then began to receive triheptanoin on an expanded access basis.

All patients demonstrated an improvement in ejection fraction (EF), a measure of cardiac function evaluated by echocardiogram, after treatment with triheptanoin. The improvements in EF began between two days and three weeks following initiation of treatment with triheptanoin and were associated with stabilization of the clinical signs of cardiomyopathy in these patients. Additionally, EF continued to improve or was maintained with further treatment. In patients with known EF values before and after treatment (n=4) the mean EF prior to triheptanoin was 32% (range: 21% to 44%) and after treatment at last assessment was 66% (range: 55% to 71%).

The most common adverse events were gastrointestinal distress, including loose stools. One patient discontinued treatment after approximately 14 weeks due to gastrointestinal symptoms. No other significant tolerance issues or treatment-related adverse events were reported. Four of the patients continue to receive triheptanoin. These data are from an expanded access program and are based on open-label uncontrolled treatment, which limits definitive conclusions about efficacy and safety.

Ultragenyx is conducting a separate Phase 2 study of triheptanoin in patients with LC-FAOD. The musculoskeletal and liver manifestations of the disease represent the most prevalent symptoms in the patients enrolled in the Phase 2 study. Data from the study are expected by the end of 2015.

Orphan Designation Granted in Europe

In addition to the previously granted orphan medicinal product designation of Very Long-Chain Acyl-CoA Dehydrogenase (VLCAD) deficiency, the European Commission has recently granted orphan medicinal product designation for triheptanoin for the treatment of three other subtypes of LC-FAOD: Long-Chain 3-Hydroxyacyl-CoA Dehydrogenase (LCHAD) deficiency, Trifunctional Protein (TFP) deficiency, and Carnitine Palmitoyltransferase II (CPT-II) deficiency. These four subtypes are estimated to represent more than 90% of the patients born with LC-FAOD each year.

The European Commission grants orphan drug status for medicinal products intended to treat diseases or conditions that affect fewer than five in 10,000 people in the European Union. The designation provides certain benefits and incentives in the EU, including protocol assistance, fee reductions, and ten years of market exclusivity once the medicine is on the market.

Ultragenyx already holds orphan drug designation for triheptanoin for the treatment of LC-FAOD from the U.S. Food and Drug Administration (FDA), as well as for the treatment of glucose transporter type-1 deficiency syndrome (Glut1 DS) from both the U.S. FDA and European Commission.

About LC-FAOD and Triheptanoin

LC-FAOD are a group of autosomal recessive genetic disorders characterized by metabolic deficiencies in which the body is unable to convert long-chain fatty acids into energy. The inability to produce energy from fat can lead to severe depletion of glucose in the body, and serious liver, muscle, and heart disease, which can lead to hospitalizations or early death. LC-FAOD are included in newborn screening panels across the U.S. and in certain European countries. LC-FAOD patients are currently treated with the avoidance of fasting, low-fat/high carbohydrate diets, carnitine, and medium-chain triglyceride (MCT) oil, a medical food product. Despite current therapy, many patients have significant metabolic events including hospitalizations and mortality due to LC-FAOD.

Triheptanoin, also known as UX007, is a purified, pharmaceutical-grade, specially designed synthetic triglyceride compound created via a multi-step chemical process. It is an investigational medicine intended to provide patients with medium-length, odd-chain fatty acids that can be metabolized to increase intermediate substrates in the Krebs cycle, a key energy-generating process. Unlike typical even-chain fatty acids, triheptanoin can be converted to new glucose through the Krebs cycle, potentially providing an important added therapeutic effect, particularly when glucose levels are too low.

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 expected timing of release of additional data, 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 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 August 14, 2015, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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