Ultragenyx Announces Positive Topline Data from Ongoing Long-Term Extension Study of UX007 for the Treatment of Long-chain Fatty Acid Oxidation Disorders

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Reductions in Major Clinical Events Sustained
After an Additional 78 Weeks of UX007 Treatment

Additional 20 Patients Naïve to UX007 Also Demonstrated Meaningful Reductions in
Major Clinical Events with Treatment

Company On Track for mid-2019 NDA Submission

NOVATO, Calif., Jan. 22, 2019 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development of novel products for serious rare and ultra-rare genetic diseases, today announced positive topline data from an ongoing long-term extension study of UX007 in patients with long-chain fatty acid oxidation disorder (LC-FAOD), demonstrating sustained reductions in the duration and frequency of major clinical events (MCE) and a long-term safety profile similar to what has previously been seen with UX007.

“Evidence from a larger number of patients and longer treatment period supports that UX007 provides a sustained, clinically meaningful improvement for those with LC-FAOD, which can be a severe and life-threatening disease,” said Camille L. Bedrosian, M.D., Chief Medical Officer of Ultragenyx. “These additional data replicate findings from previous studies, and will form an important component of our NDA submission.”

Long-Term Extension Study Results
A total of 75 patients are enrolled in the long-term safety and efficacy study including 24 patients who were previously enrolled in the company-sponsored Phase 2 study, 20 naïve patients who had not previously been treated with UX007 and 31 patients from expanded access or investigator-sponsored studies. For the Phase 2 study and naïve patients, retrospective medical chart data were collected allowing comparison of the annualized major clinical event and duration rates between pre-UX007 and UX007 treatment periods. Major clinical events reported are primarily comprised of hospitalizations but do include some emergency room visits, and the duration is the number of days in the hospital or emergency room.

Efficacy Results: Patients from Phase 2 Company-Sponsored Study
Patients who previously completed the Phase 2 company-sponsored study and rolled over to the extension study (n=24) have now received treatment for an additional 78 weeks (minimum of 3 years of total UX007 treatment). The median annualized major clinical event and duration rates during the extension treatment period were zero. Over the entire treatment period, patients had a 67 percent reduction in median annualized event rate and a 66 percent reduction in the median annualized duration rate.

UX007 Phase 2 Rollover Patients **All MCEs (n=24)**	**Pre-UX007**	**Phase 2 Tx** **(78 weeks)**	**Extension Tx** **(78 weeks)**
Median Annualized Event Rate (events/yr) [25th, 75th percentile]	1.5 [0.3, 2.7]	0.7 [0.0, 1.3]	0.0 [0.0, 1.3]
Median Annualized Duration Rate (days/yr) [25th, 75th percentile]	5.3 [0.3, 9]	1.7 [0.0, 4.7]	0.0 [0.0, 6.3]

Efficacy Results: Additional 20 Patients Naïve to UX007
Patients who were naïve to UX007 (n=20) at study entry have received up to 78 weeks of treatment. These patients have demonstrated a 70 percent reduction in the median annualized event rate and an 80 percent reduction in the median annualized duration rate.

UX007 Naïve Patients **All MCEs (n=20)** [25th, 75th percentile]	**Pre-UX007** **(78 weeks)**	**Extension Tx (median 63 weeks)** **[26,78]**
Median Annualized Event Rate (events/yr) [25th, 75th percentile]	2.3 [1.0, 3.8]	0.7 [0, 1.9]
Median Annualized Duration Rate (days/yr) [25th, 75th percentile]	10.0 [3.3, 19.0]	2.0 [0, 6.6]

Safety Profile for Extension Study
The safety profile observed in the long-term extension study was consistent with what has been previously observed with UX007. The most common treatment-related adverse events were diarrhea, vomiting, and abdominal pain. One patient discontinued due to a treatment-related adverse event. There were two deaths during the extension study, both deemed to be related to disease progression and not due to treatment with UX007. One of these patients was naïve to UX007 and one was previously in an investigator-sponsored study. Both patients had Trifunctional Protein (TFP) Deficiency type LC-FAOD, a type known to have a high mortality rate, and had experienced severe disease manifestations when initiating UX007 treatment in the extension study.

New Drug Application Submission in Mid-2019
Ultragenyx is on track to submit the New Drug Application (NDA) in mid-2019. The submission will include these data from this long-term efficacy and safety extension study in 75 patients, the Phase 2 study of UX007 in 29 patients, a retrospective medical record review of 20 original compassionate

use patients, data from 70 patients treated through expanded access, and a randomized controlled investigator-sponsored study of 32 patients showing an effect of UX007 on cardiac function.

About LC-FAOD
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. Patients with LC-FAOD 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 management, many patients have significant metabolic events including hospitalizations and mortality due to LC-FAOD.

About UX007
UX007 is a highly purified, pharmaceutical-grade, synthetic, seven-carbon fatty acid triglyceride 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, UX007 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 Pharmaceutical Inc.
Ultragenyx is a biopharmaceutical company committed to bringing to patients novel products for the treatment of serious rare and ultra-rare genetic diseases. The company has built a diverse portfolio of approved therapies and product candidates aimed at addressing diseases with high unmet medical need and clear biology for treatment, 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 related to Ultragenyx's expectations regarding plans for its clinical programs and clinical studies, future regulatory interactions, and the components and timing of regulatory submissions 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 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 products and 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 Ultragenyx in general, see Ultragenyx's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on November 6, 2018, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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