Contact Ultragenyx Pharmaceutical Inc.
For Media, Bee Nguyen
For Investors, Shalini Sharp
415-483-8800

FOR IMMEDIATE RELEASE:

Ultragenyx Announces In-Licensing of Clinical-Stage Product Triheptanoin for Treatment of Long-Chain Fatty Acid Oxidation Disorders

NOVATO, CA –January 10, 2013— Ultragenyx Pharmaceutical Inc., a biotechnology company focused on the development of treatments for rare and ultra-rare genetic disorders, announced that it has in-licensed rights for triheptanoin, a promising treatment for long-chain fatty acid oxidation disorders (FAOD), from Baylor Research Institute in Dallas, TX. Triheptanoin is intended as a substrate replacement therapy to restore deficient intermediates in the mitochondria and to enable energy metabolism in patients with FAOD and potentially other genetic metabolic disorders.

Fatty acid oxidation disorders are a group of autosomal recessive diseases characterized by metabolic deficiencies in which the body is unable to break down fatty acids into energy. Triheptanoin, also known as UX007, is a specially designed synthetic compound intended to provide patients with medium-length, odd-chain fatty acids that are metabolized to replace intermediate compounds of the tricarboxylic acid (TCA) cycle, a key energy-generating process. Several thousand people are afflicted with long-chain FAOD in the US. FAOD are also now included in newborn screening panels. Approximately 100 patients are diagnosed with the 4 most common long-chain FAOD diseases each year in the US.

In investigator-led research published in scientific journals, triheptanoin has been shown to reduce rhabdomyolysis (muscle rupture), hypoglycemia (low blood sugar), and cardiomyopathy (heart weakness and failure), among other symptoms in a variety of FAOD. More than 100 adults and children affected by FAOD or other energy diseases have been treated to date with triheptanoin, including 22 patients who have remained on treatment for up to 10 years.

“We believe that UX007 has the potential to benefit many patients with FAODs and other diseases of energy metabolism,” said Emil D. Kakkis, MD, PhD, Chief Executive Officer of Ultragenyx. “Recent published data suggest that as many as half of these FAOD patients die from this disease and are in need of better treatment options. This

new program is another example of our focus on transformative treatments for rare diseases."

The company licensed exclusive North American rights to triheptanoin from Baylor Research Institute and has an exclusive option to an exclusive license for the rest of world. The compound derives from the groundbreaking research of Dr. Charles Roe, who discovered the basis for continued difficulties in treating FAOD patients, and demonstrated triheptanoin's potential to better address these problems.

In addition to UX007, Ultragenyx's portfolio includes UX001, a potential substrate replacement therapy for hereditary inclusion body myopathy, a muscle-wasting disease, currently in a Phase 2 trial, and UX003, a potential enzyme replacement therapy for mucopolysaccharidosis type 7 (MPS 7), which will be tested in a Phase 1/2 clinical study later in 2013.

About FAOD

Fatty acid oxidation disorders (FAOD) are a group of autosomal recessive disorders in mitochondrial metabolism of fatty acids critical to the production of energy. The sequential breakdown of long-chain fatty acids into smaller units is required to generate energy in many tissues, particularly during fasting or exercise. This deficiency can lead to hypoglycemia, rhabdomyolysis, cardiomyopathy and muscle pain on exertion, which can be triggered or worsened by intercurrent illnesses, fasting, or exercise. In many cases these patients require hospitalization for treatment, and the overall mortality from long-chain FAOD is about 50%. There is currently no approved drug therapy for FAOD.

About Ultragenyx

Ultragenyx is a privately held, development-stage biotechnology company committed to bringing to market life-transforming therapeutics for patients with rare and ultra-rare metabolic genetic diseases. The company focuses on diseases for which the unmet medical need is high, the biology for treatment is clear, and for which there are no effective treatments.

The company is led by a management team experienced in the development and commercialization of rare disease therapeutics. Ultragenyx' 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.

About Baylor Research Institute

Established in 1984 in Dallas, Texas, Baylor Research Institute (BRI) promotes and supports research to bring innovative treatments from the laboratory workbench to the patient bedside. To achieve this bench-to-bedside concept, BRI focuses on basic science, clinical trials, healthcare effectiveness and quality-of-care research. Today, BRI is conducting more than 800 active research protocols with 250 research investigators, spanning more than 20 medical specialties. BRI has research and development projects in areas ranging from human immunology and orphan metabolic diseases to diabetes, cardiovascular disease and many other unmet medical needs. Its Personalized Medicine arm offers a unique platform for identifying microarray-based fingerprint signatures. The Baylor Health Care System offers its research affiliates unique access to one of the largest patient bases available for research in the US within a single institution. BRI has received full accreditation from AAHRPP. www.baylorhealth.edu/Research/