FOR IMMEDIATE RELEASE:

Ultragenyx Announces Presentation of Three Abstracts at the 12th International Congress of Inborn Errors of Metabolism (ICIEM 2013)

NOVATO, CA – August 28, 2013 - Ultragenyx Pharmaceutical Inc., a biotechnology company focused on developing treatments for rare and ultra-rare genetic disorders, today announced three abstracts related to UX007, a substrate replacement therapy intended for the treatment of fatty acid oxidation disorder (FAOD), and UX003, an enzyme replacement therapy intended for the treatment of mucopolysaccharidosis type 7 (MPS 7, Sly syndrome). The abstracts will be presented at the 12th International Congress of Inborn Errors of Metabolism on September 3-6, 2013 in Barcelona, Spain.

• The Impact of Triheptanoin Treatment on the Incidence of Major Medical Events in Patients with Long-Chain Fatty Acid Oxidation Disorders (FAOD), will be an oral presentation by Jerry Vockley, M.D., Ph.D., Professor of Pediatrics, School of Medicine, Professor of Human Genetics, Graduate School of Public Health, Chief of Medical Genetics Children's Hospital of Pittsburgh of UPMC, on September 4, 2013 from 11:00am to 12:00pm CEST. The data will be released subsequent to the presentation.

• Design of an Open-Label Phase 2 Study to Assess Safety and Clinical Effects of UX007 in Subjects with Long-Chain Fatty Acid Oxidation Disorders (FAOD), will be presented as a poster [P300] on September 4th and 5th, from 12:30pm to 2:00pm CEST.

• The Randomized Blind Start Trial: A New Study Design to Assess Clinical Outcomes in Rare, Heterogeneous Patient Populations, will be presented as a poster [P726] on September 4th and 5th from 12:30pm to 2:00pm CEST.

These abstracts will be available at www.ultragenyx.com on September 9th.
About UX003 and UX007

**UX003**
UX003 (recombinant human beta glucuronidase, rhGUS) is under development as an enzyme replacement therapy (ERT) for the treatment of mucopolysaccharidosis type 7 (MPS 7) via intravenous (IV) administration. Based on prior experience with other successful IV ERTs, it is expected that UX003 will be distributed to affected tissues and clear lysosomal storage of glycosaminoglycans (GAGs). Reduction of GAG accumulation in affected tissues is anticipated to reduce the clinical signs and symptoms of the disease.

**UX007**
UX007 (triheptanoin) is a purified form of a specially designed synthetic triglyceride compound and is produced using a GMP-compliant process required to obtain FDA drug approval. UX007 is intended to provide patients with medium-length, odd-chain fatty acids that are metabolized to replace intermediate substrates in fatty acid oxidation downstream of their genetic block in fatty acid metabolism. UX007 is also metabolized to a substrate that is intended to replace deficient intermediates in the TCA cycle, a key energy-generating process, and can also help support gluconeogenesis to produce net glucose, both of which do not happen with Medium Chain Triglycerides comprised of even-chain fatty acids. Together, the substrates produced by UX007 during metabolism are intended to improve energy production in FAOD patients.

**About Ultragenyx**
Ultragenyx is a privately held, clinical-stage biotechnology company committed to bringing to market life-transforming therapeutics for patients with rare and ultra-rare metabolic genetic diseases. Founded in 2010, the company is rapidly building 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 effective treatments.

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](http://www.ultragenyx.com).