Contact:

Ultragenyx Pharmaceutical Inc.
Media and Investors
Chanterria McGilbra
415-827-7179

FOR IMMEDIATE RELEASE:

Ultragenyx Announces Series A Financing

$45 million to support development of rare disease therapeutics

NOVATO, CA. - June 20, 2011 - Ultragenyx Pharmaceutical Inc. today announced the closing of a $45 million Series A financing to support the development of rare disease therapeutics. The co-lead investors are TPG Biotech and Fidelity Biosciences, joined by European investor HealthCap, and Pappas Ventures. This funding will advance multiple rare disease product programs in the pipeline, as well as the development of new product candidates and partnerships. The lead product, UX-001, a first-in-class therapy for treatment of Hereditary Inclusion Body Myopathy (HIBM), is expected to enter the clinic in 2011.

"I am pleased and excited to have found a set of investors with the insight to support the development of a new rare disease company with multiple product candidates. They believe in the concept that a tightly focused company of committed rare disease development specialists will be able to create value as well as bring a series of novel, life-changing therapeutics to patients affected by rare and ultra-rare disorders", said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx.

Dr. Kakkis formed and funded Ultragenyx in April 2010, based on his experiences in academia and over 11 years at BioMarin, where three rare disease products were approved during his tenure. The company has received additional funding from John Klock, M.D., and William Aliski, both experienced rare disease company executives. Joining the Ultragenyx Board of Directors are Eran Nadav, Ph.D. (TPG Biotech), Ben Auspitz (Fidelity Biosciences) and Mårten Steen, M.D., Ph.D. (HealthCap).

The company will be located in Novato, CA.

Transforming good science into great medicine
for severe genetic diseases

About Ultragenyx

Ultragenyx™ develops therapeutics for rare diseases, sometimes referred to as Orphan products. Founded in 2010, the company is led by Emil Kakkis, M.D., Ph.D., former CMO of BioMarin Pharmaceutical. Efficient and effective development strategies are essential for success in the rare disease space, in which information may be incomplete or ambiguous, and few development precedents exist. The management team's experience in selecting and developing products with clear mechanisms of action for untreated rare diseases will help Ultragenyx create life-changing therapeutics by leveraging existing yet undeveloped science for these little-known indications.

For more information on Ultragenyx, please visit the company's website at www.ultragenyx.com.