ultragenyx pharmaceutical

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FOR IMMEDIATE RELEASE:

Ultragenyx Raises $75 Million in Oversubscribed Crossover Financing

NOVATO, CA – December 20, 2012 - Ultragenyx Pharmaceutical Inc., a biotechnology company focused on the development of treatments for rare and ultra-rare genetic disorders, today announced the successful completion of a $75 million Series B round financing led by Adage Capital Partners, LP. Joining Adage as new investors in this financing are mutual funds and separate account clients advised by T. Rowe Price Associates, Inc., Jennison Associates LLC (on behalf of clients), funds and accounts under management by subsidiaries of BlackRock, Inc., Sanofi-Genzyme BioVentures[1], Shire plc and additional blue chip public market funds. Existing investors, TPG Biotech, Fidelity Biosciences, HealthCap and Pappas Ventures also participated in the transaction.

Ultragenyx plans to use the proceeds from the financing primarily to advance development of the company's lead clinical-stage programs, UX001 and UX003, and other undisclosed programs. UX001 is a potential substrate replacement therapy for hereditary inclusion body myopathy currently being investigated in a fully enrolled, randomized placebo-controlled Phase 2 clinical study with results anticipated in 2013. UX003 is a recombinant enzyme replacement therapy intended for the treatment of mucopolysaccharidosis type 7 (MPS 7), which will enter a Phase 1/2 clinical study in MPS 7 patients in 2013. The company intends to continue to expand its pipeline through the potential in-licensing of additional products.

"We welcome the participation of such highly regarded public market and strategic investors in this pivotal financing," said Eran Nadav, PhD, Managing Director at TPG Biotech and Chairman of the Board of Ultragenyx. "Ultragenyx, led by a distinguished team experienced in rare disease drug development, is building a world-class orphan drug company with a rich pipeline of transformative therapeutics."

[1] Sanofi-Genzyme BioVentures is a successor fund to Genzyme Ventures, which has been re-named to reflect an expanded strategic investment focus on areas of interest to both Sanofi and Genzyme.

"We deeply appreciate the support of all our investors, new and existing, and their confidence in our ability to find and efficiently develop compelling new treatments for devastating rare genetic disorders," said Emil D. Kakkis, MD, PhD, Chief Executive Officer and Founder of Ultragenyx. "This financing transaction is critical to expanding our efforts to deliver profound novel therapies that benefit even more rare disease patients."

Cowen and Company served as financial advisor on the financing. Ropes & Gray LLP served as legal advisor.

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.