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Ultragenyx and Saint Louis University's Center for World Health and Medicine Announce Research Collaboration to Advance Muscular Dystrophy Treatment

NOVATO, Calif., March 23, 2016 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ:RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, and Saint Louis University's (SLU) Center for World Health and Medicine, today announced that they have entered into three-year sponsored research and option agreements to collaborate on the development of small molecule therapeutics for the potential treatment of Facioscapulohumeral Muscular Dystrophy (FSHD).

Under the terms of the agreements, SLU's Center for World Health and Medicine will identify and conduct preclinical research of select small molecules for the potential treatment of FSHD. Ultragenyx has an exclusive option to an exclusive license to existing and future intellectual property arising from the collaboration.

"By combining our Center's drug development capabilities with the rare disease expertise of Ultragenyx, this collaboration may increase the chances and accelerate the process of delivering an effective therapy to patients with FSHD," said Pete Ruminski, executive director for SLU's Center for World Health and Medicine. "The fact that our own scientist's daughter has FSHD provides extra motivation and passion to our team's efforts to find a therapy to treat her and all those with the disease."

"SLU has already made significant progress in the area of FSHD research, and we are excited to begin a robust collaboration to bring forward what we hope will be the first treatment for this debilitating and progressive disease," said Emil D. Kakkis, MD, PhD, Chief Executive Officer of Ultragenyx.

About Facioscapulohumeral muscular dystrophy (FSHD)
FSHD is a heritable, autosomal dominant muscle disease characterized by progressive weakening and loss of skeletal muscle. FSHD is the third most common type of muscular dystrophy, affecting children and adults of both sexes. There are 15,000 or more people living with FSHD in the United States. The name of the disease relates to the areas of the body that are most affected early on in the disease: the face (facio), the shoulder blade (scapula) and the upper arm (humeral). As the disease progresses it can also affect the legs. Most individuals with FSHD experience some degree of hearing difficulty, and complete hearing loss occurs in some cases.

Most individuals have a normal life span, but symptoms can vary from mild to severely disabling and usually increase in severity over time. In approximately 20 percent of patients, FSHD leads to the loss of mobility. There are currently no FDA-approved treatments for patients with this disease.

About Saint Louis University's Center for World Health and Medicine

The Center for World Health and Medicine at Saint Louis University is dedicated to the discovery and development of new therapies for rare and neglected diseases, many of which afflict children, the poor and the underserved. The Center is comprised of a multi-disciplined team of skilled drug development scientists, formerly employed in the pharmaceutical industry, with extensive expertise in translating discoveries made in basic science laboratories into safe and effective drugs for the patients in need of them. The Center has launched the dancendonate.org social media campaign to support research on FSHD.

Saint Louis University is a Catholic, Jesuit institution that values academic excellence, life-changing research, compassionate health care, and a strong commitment to faith and service. Founded in 1818, the University fosters the intellectual and character development of nearly 13,000 students on campuses in St. Louis and Madrid, Spain. Building on a legacy of nearly 200 years, Saint Louis University continues to move forward with an unwavering commitment to a higher purpose, a greater good.

About Ultragenyx

Ultragenyx is a clinical-stage biopharmaceutical company committed to bringing to market novel products for the treatment

of rare and ultra-rare diseases, with a focus on serious, debilitating genetic diseases. Founded in 2010, the company has rapidly built 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 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.

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