Ultragenyx Announces Exclusive License to REGENXBIO AAV Vectors to Develop Gene Therapy for CDD (CDKL5 Deficiency Disorder)

October 22, 2018

Ultragenyx CEO to present Keynote Address at the 2018 CDKL5 Forum hosted by the Loulou Foundation

NOVATO, Calif., Oct. 22, 2018 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development of novel products for serious rare and ultra-rare genetic diseases, today announced it has exercised its option with REGENXBIO to develop a gene therapy to treat patients with CDD (CDKL5 Deficiency Disorder) utilizing REGENXBIO's adeno-associated virus (AAV) vectors, including AAV9. CDD is a severe and debilitating neurological disorder that shares many features of Rett Syndrome, though the two disorders are now considered distinct from each other.

"We are looking forward to initiating this program in CDD, a disease with increasing awareness and diagnosis," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "AAV9 has been shown to be effective for gene therapy delivery to the central nervous system, and we believe it is well-suited for this neurological indication."

Emil D. Kakkis, M.D., Ph.D., the company's Chief Executive Officer and President, will present the Keynote Address on Tuesday, October 23 at the 2018 CDKL5 Forum hosted by the Loulou Foundation.

About CDD
CDD is an X-linked genetic disorder that results in seizures that typically begin in the first few months of life and severe intellectual and gross motor impairment. The CDKL5 gene provides instructions for making a protein that is essential for normal brain development, with mutations causing a deficiency in the protein level. CDD is caused by a deficiency in an enzyme that may only be needed in small quantities for the brain to function normally. In the past, CDD was commonly diagnosed as an atypical Rett Syndrome until genetic diagnostics changed this view and improved accurate diagnosis.

About Ultragenyx Pharmaceutical Inc.
Ultragenyx is a biopharmaceutical company committed to bringing to patients novel products for the treatment of serious rare and ultra-rare genetic diseases. The company has built a diverse portfolio of approved therapies and product candidates aimed at addressing diseases with high unmet medical need and clear biology for treatment, 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.

About REGENXBIO Inc.
REGENXBIO is a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. REGENXBIO's NAV Technology Platform, a proprietary adeno-associated virus (AAV) gene delivery platform, consists of exclusive rights to more than 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10. REGENXBIO and its third-party NAV Technology Platform Licensees are applying the NAV Technology Platform in the development of a broad pipeline of candidates in multiple therapeutic areas.

Forward-Looking Statements
Except for the historical information contained herein, the matters set forth in this press release, including statements relating to Ultragenyx's plans for its pre-clinical programs, are forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements involve substantial risks and uncertainties that could cause our clinical development programs, future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, the uncertainties inherent in the clinical drug development process, such as the regulatory approval process, the timing of regulatory filings, and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations and the availability or commercial potential of our products and drug candidates. Ultragenyx undertakes no obligation to update or revise any forward-looking statements. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to the business of Ultragenyx in general, see Ultragenyx's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on August 3, 2018, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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