Ultragenyx Announces Upcoming Data Presentations at American Society of Gene & Cell Therapy 2021 Virtual Annual Meeting

April 27, 2021

NOVATO, Calif., April 27, 2021 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development and commercialization of novel therapies for rare and ultra-rare diseases, today announced that clinical, preclinical and manufacturing data from its investigational gene therapy programs will be presented at the American Society of Gene & Cell Therapy (ASGCT) 24th Annual Meeting, which will be held virtually May 11-14, 2021.

“We continue to advance our proprietary HeLa producer cell line (PCL) manufacturing platform and look forward to sharing recent HeLa 3.0 improvements that further increase productivity and reproducibility of this platform, and continue to drive down AAV production costs,” said Sam Wadsworth, Ph.D., Chief Scientific Officer of Ultragenyx Gene Therapy. “We will also share longer-term data from our Phase 1/2 studies in Ornithine Transcarbamylase (OTC) Deficiency and Glycogen Storage Disease Type Ia (GSDIa).”

Oral Presentations include:

- AAV8 Gene Therapy as a Potential Treatment in Adults with Late-Onset Ornithine Transcarbamylase (OTC) Deficiency: Updated Results from a Phase 1/2 Clinical Trial (Abstract #34)
    - Date/Time: Tuesday, May 11, 6:45-7:00 PM ET
    - Presenter: Cary O. Harding, M.D., Oregon Health & Science University, Portland, OR

- Characterization of rAAV key quality attributes generated from a highly optimized, HeLa 3.0 producer cell line (PCL) production platform (Abstract #169)
    - Date/Time: Thursday, May 13, 6:15-6:30 PM ET
    - Presenter: Nicholas Richards, Ultragenyx Gene Therapy, Cambridge, MA

- AAV8-mediated Liver-directed Gene Therapy as a Potential Therapeutic Option in Adults with Glycogen Storage Disease Type Ia (GSDIa): Updated Results from a Phase 1/2 Clinical Trial (Abstract #261)
    - Date/Time: Friday, May 14, 1:15-1:30 PM ET
    - Presenter: David F. Rodriguez-Buritica, M.D., University of Texas McGovern Medical School, Houston, TX

Digital Poster Presentations, which will be available throughout the conference, include:

- Similar DTX301 (scAAV8OTC) Gene Therapy Outcomes in Female and Male Cynomolgus Macaques (Abstract #734)
    - Presenter: Lili Wang , Ph.D., University of Pennsylvania, Philadelphia, PA

- Prophylactic Prednisolone and Rapamycin Improve hFVIII Gene Therapy in Cynomolgus Macaques by Reducing Plasma Cells (Abstract #733)
    - Presenter: Barbara A. Sullivan, Ph.D., Ultragenyx Gene Therapy, Cambridge, MA

About Ultragenyx Pharmaceutical Inc.
Ultragenyx is a biopharmaceutical company committed to bringing novel products to patients 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 typically no approved therapies treating the underlying disease.

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.

Forward-Looking Statements

Except for the historical information contained herein, the matters set forth in this press release, including statements related to Ultragenyx's expectations and projections regarding its future operating results and financial performance, anticipated cost or expense reductions, the timing, progress and plans for its clinical programs and clinical studies, future regulatory interactions, and the components and timing of regulatory submissions 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, collaboration with third parties, 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 effects from the COVID-19 pandemic on the company's clinical activities, business and operating results, risks related to reliance on third party partners to conduct certain activities on the company's behalf, uncertainty and potential delays related to clinical drug development, smaller than anticipated market opportunities for the company's products and product candidates, manufacturing risks, competition from other therapies or products, and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations, the company's future operating results and financial performance, the timing of clinical trial activities and reporting results from same, and the availability or commercial potential of Ultragenyx's 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 Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 12, 2021, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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