Ultragenyx Announces Upcoming Data Presentations at American Society of Gene & Cell Therapy (ASGCT) 2022 Annual Meeting

May 9, 2022

Oral presentations include longer term durability data from DTX301 and DTX401, and manufacturing data on the Pinnacle PCL™ (AAV vector Producer Cell Line) platform and technology

NOVATO, Calif., May 09, 2022 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development and commercialization of novel products 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) 25th Annual Meeting, which will be held both in person and virtually May 16-19, 2022. The company will present new data supporting its gene therapy portfolio and discuss critical topics during a scientific symposium on Accelerated Approval and at the pre-meeting workshops.

Clinical and pre-clinical presentations include:

- Oral presentation: Safety and Efficacy of DTX301 in Adults with Late-Onset Ornithine Transcarbamylase (OTC) Deficiency: A Phase 1/2 Trial (Abstract #463)
    - Date/Time: Tuesday, May 17, 4:15-4:30 PM ET
    - Presenter: Cary Harding, M.D., Oregon Health & Science University

- Oral presentation: Sustained Efficacy and Safety at Week 52 and up to Three Years in Adults with Glycogen Storage Disease Type Ia (GSDIa): Results from a Phase 1/2 Clinical Trial of DTX401, an AAV8-mediated, Liver-directed Gene Therapy (Abstract #1212)
    - Date/Time: Thursday, May 19, 11:00-11:15 AM ET
    - Presenter: Rebecca Riba-Wolman, M.D., University of Connecticut

- Poster presentation: Improving Neuronal Gene Transfer to the Brain After CSF Administration of AAV9 in Juvenile Non-Human Primates (Board No. Tu-143)
    - Date/Time: Tuesday, May 17, 5:30-6:30 PM ET
    - Presenter: Maggie Wright, Ph.D, Ultragenyx

- Poster presentation: Understanding the Educational Needs of United States Physicians Related to Gene Therapy (Board No. Tu-67)
    - Date/Time: Tuesday, May 17, 5:30-6:30 PM ET
    - Presenter: Emily Belcher, CE Outcomes

Manufacturing presentations covering the company's Pinnacle PCL™ (AAV vector Producer Cell Line) platform and technology include:

- Oral presentation: Development and Characterization of Highly Optimized Monoclonal Producer Cell Lines (PCLs) for the Treatment of CDKL5 Deficiency Disorder (CDD) (Abstract #863)
    - Date/Time: Wednesday, May 18, 4:15-4:30 PM ET
    - Presenter: Laurie Tran, MSc, Ultragenyx

- Poster presentation: Development of High Density & High Purity AAV Production Processes (Board No. M-293)
    - Date/Time: Monday, May 16, 5:30-6:30 PM ET
    - Presenter: Jan Panteli, Ph.D, Ultragenyx

- Poster presentation: Development of an *In Vitro* Model for the Evaluation of Adeno-Associated Virus Delivered Microdystrophin Transgenes (Board No. W-56)
    - Date/Time: Wednesday, May 18, 5:30-6:30 PM ET
    - Presenters: Eric Himelman, Ph.D, and Hwan June Kang, Ph.D, both Ultragenyx

In addition to the data presentations, Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx will deliver a presentation at ASGCT's session on "Accelerated Approval for Gene Therapies," taking place on Monday, May 16 from 8:00-9:45 AM ET.

Separately, several members of the team will present during the following pre-meeting workshops on Sunday, May 15:

- Newborn Screening: Toward a System That Keeps Pace with Gene Therapy Advances
    - Time: 8:55-9:45 AM ET
    - Presenter: Erin Frey, Director, State Government Affairs, Ultragenyx
- AAV Vector Integration
    - Time: 2:50-4:50 PM ET
    - Presenter: Sam Wadsworth, Ph.D., Chief Scientific Officer of Ultragenyx Gene Therapy
- What to Expect as a Participant in a Gene Therapy Clinical Trial
    - Time: 1:00-3:00 PM ET
    - Presenter: Heather Lau, M.D., Executive Director, Global Clinical Development, Ultragenyx

About Ultragenyx Pharmaceutical Inc.
Ultragenyx is a biopharmaceutical company committed to bringing novel therapies to patients for the treatment of serious rare and ultra-rare genetic diseases. The company has built a diverse portfolio of approved medicines and treatment candidates aimed at addressing diseases with high unmet medical need and clear biology, 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 and commercial activities and 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, the company's ability to achieve its projected development goals in its expected timeframes, risks and uncertainties related to the regulatory approval process, 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 Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on May 5, 2022, and its subsequent periodic reports filed with the SEC.

In addition to its SEC filings, press releases and public conference calls, Ultragenyx uses its investor relations website and social media outlets to publish important information about the company, including information that may be deemed material to investors, and to comply with its disclosure obligations under Regulation FD. Financial and other information about Ultragenyx is routinely posted and is accessible on Ultragenyx's investor relations website (https://ir.ultragenyx.com/) and LinkedIn website (https://www.linkedin.com/company/ultragenyx-pharmaceutical-inc-/).

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