Ultragenyx Announces Upcoming Data Presentations at American Academy of Neurology (AAN) 2024 Annual Meeting

April 12, 2024 12:00 PM EDT

Company will host investor call on April 17 at 8:00 a.m. ET to discuss new data from GTX-102 Phase 1/2 clinical study in patients with Angelman syndrome

NOVATO, Calif., April 12, 2024 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE) today announced that preclinical and clinical data, including new clinical efficacy and safety data from the expansion cohorts in the Phase 1/2 study of GTX-102 for the treatment of Angelman syndrome, will be presented at the 76th Annual American Academy of Neurology Meeting (AAN) being held April 13-18 in Denver and virtually. GTX-102 is an investigational antisense oligonucleotide delivered via intrathecal administration and is designed to target and inhibit expression of *UBE3A* antisense transcript *(UBE3A-AS)*.

Abstracts are available to view online at www.aan.com/events/annual-meeting-abstracts. The GTX-102 emerging science presentation will include data beyond what is currently available online.

AAN 2024 Presentation Details:

Title: Clinical Activity and Safety of GTX-102, an Investigational Antisense Oligonucleotide for the Treatment of Patients With Angelman Syndrome
Format: Oral (abstract #192, presentation #008)
Session: Emerging Science 2
Presenter: Kemi A. Olugemo, M.D., FAAN, Ultragenyx
Date/ Time: Tuesday, April 16, 6:12-6:18 p.m. MDT

Title: Proteomic Profiling of Angelman Syndrome for Disease-Associated Biomarker Discovery
Format: Poster (#8.001)
Session: Child Neurology and Developmental Neurology: Neurogenetics 2
Presenter: Rachael Elizabeth Hawtin, Ph.D., Ultragenyx
Date/ Time: Tuesday, April 16, 5:30-6:30 p.m. MDT

Title: Clinical Characteristics of Creatine Transporter Deficiency (CTD): Final Results of the Vigilan Observational Study
Format: Poster (#8.002)
Session: Child Neurology and Developmental Neurology: Neurogenetics 2
Presenter: Melanie Brandabur, M.D., Ultragenyx
Date/ Time: Tuesday, April 16, 5:30-6:30 p.m. MDT

Conference Call and Webcast Information
Ultragenyx will host a conference call at 8:00 a.m. ET on Wednesday, April 17, 2024, to discuss the new efficacy and safety data from the GTX-102 Phase 1/2 clinical study being presented at the conference.

The live and replayed webcast of the call will be available through the company's website at https://ir.ultragenyx.com/events-presentations. To participate in the live call, please register by clicking on the following link (registration link) and you will be provided with dial-in details.

About Ultragenyx Pharmaceutical Inc.

Ultragenyx is a biopharmaceutical company committed to bringing novel products to patients for the treatment of serious rare and ultrarare 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 and Use of Digital Media

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, business plans and objectives for GTX-102, expectations regarding the tolerability and safety of GTX-102, and future clinical and regulatory developments for GTX-102 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 uncertainty of clinical drug development and unpredictability and lengthy process for obtaining regulatory approvals, the ability of the company to successfully develop GTX-102, the company's ability to achieve its projected development goals in its expected timeframes, risks related to adverse side effects, risks related to reliance on third party partners to conduct certain activities on the company's behalf, 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 (SEC) on February 21, 2024, 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-/).

Contacts
Ultragenyx Pharmaceutical Inc.

Investors
Joshua Higa
+1-415-475-6370
ir@ultragenyx.com

Media
Carolyn Wang
+1-415-225-5050
media@ultragenyx.com