Ultragenyx to Present GTX-102 Angelman Syndrome Program Update at the ASF Family Conference and Research Symposium

July 24, 2024 12:00 PM EDT

Presentations will include an encore of the positive interim Phase 1/2 results presented in April at the 76th Annual American Academy of Neurology (AAN) Meeting

NOVATO, Calif., July 24, 2024 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE) today announced that the company will share the latest clinical data, regulatory progress and program next steps for GTX-102, its investigational antisense oligonucleotide for Angelman syndrome, on Wednesday, July 24, at the 2024 Angelman Syndrome Foundation (ASF) Family Conference and Research Symposium in Sandusky, Ohio.

“Since our positive interim Phase 1/2 data presentation at AAN in April, we have continued to see the patients in our study develop new skills across multiple domains with no new serious adverse events,” said Eric Crombez, M.D., chief medical officer at Ultragenyx. “We look forward to working with the ASF and the broader community as we prepare to initiate a global randomized study by the end of this year.”

The ASF data presentation will review the previously disclosed Phase 1/2 results presented at AAN and an update on plans to initiate a Phase 3 pivotal trial by the end of the year.

ASF 2024 Presentation Details:

Title: Developing Treatments for Complex Rare Diseases
Session: Industry Introduction with Ultragenyx
Presenter: Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx
Date / Time: Wednesday, July 24, 2024, at 3:10 pm Eastern Time

Title: GTX-102 Angelman Program Update
Session: Industry Overview and Updates
Presenter: Kim Goodspeed, M.D., medical director, global clinical development at Ultragenyx
Date / Time: Wednesday, July 24, 2024, at 3:40 pm Eastern Time

Both presentations will be livestreamed from the ASF website: https://asfconference.org/family-conference/

About GTX-102
GTX-102 is an investigational antisense oligonucleotide delivered via intrathecal administration and designed to target and inhibit expression of UBE3A-AS. Nonclinical studies have shown that GTX-102 reduces levels of UBE3A-AS and reactivates expression of the paternal UBE3A allele in neurons of the central nervous system (CNS). Reactivation of paternal UBE3A expression in animal models of Angelman syndrome has been associated with improvements in some of the neurological symptoms associated with the condition. GTX-102 has been granted Orphan Drug Designation, Rare Pediatric Disease Designation, and Fast Track Designation from the FDA and Orphan Designation and PRIME designation from the EMA.

About Angelman Syndrome
Angelman syndrome is a rare, neurogenetic disorder caused by loss-of-function of the maternally inherited allele of the UBE3A gene. The maternal-specific inheritance pattern of Angelman syndrome is due to genomic imprinting of UBE3A in neurons of the central nervous system (CNS), a naturally occurring phenomenon in which the maternal UBE3A allele is expressed and the paternal UBE3A is not. Silencing of the paternal UBE3A allele is regulated by the UBE3A antisense transcript (UBE3A-AS), the intended target of GTX-102. In almost all cases of Angelman syndrome, the maternal UBE3A allele is either missing or mutated, resulting in limited to no protein expression. This condition is generally not inherited but instead occurs spontaneously. It is estimated to affect approximately 60,000 people in commercially accessible geographies.

Individuals with Angelman syndrome have a lifelong neurodevelopmental disorder including cognitive impairment, motor impairment, balance issues and debilitating seizures. Some individuals with Angelman syndrome are unable to walk and most do not speak. Anxiety and disturbed sleep can be serious challenges in individuals with Angelman syndrome. Although individuals with Angelman syndrome have a normal lifespan, they require continuous care and are unable to live independently. Angelman syndrome is not a degenerative disorder, but the loss of the UBE3A protein expression in neurons results in abnormal communications between neurons. Angelman syndrome is often misdiagnosed as autism or cerebral palsy. There are no currently approved therapies for Angelman syndrome; however, several symptoms of this disorder can be reversed in adult animal models of Angelman syndrome, suggesting that improvement of symptoms can potentially be achieved at any age.

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.

Ultragenyx 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 the clinical benefit, tolerability and safety of GTX-102 and the corresponding impact on patients, the anticipated

dosing of the Phase 2 study for GTX-102 and the timing for initiation of a Phase 3 study for GTX-102 and associated regulatory meetings, 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, the risk that results from earlier studies may not be predictive of future study results, 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 product 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 3, 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-/).

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