Ultragenyx Announces Program and Pipeline Updates at Analyst Day Including Interim Data from Ongoing Studies in Osteogenesis Imperfecta (OI), Angelman Syndrome (AS) and Wilson Disease

October 16, 2023

Treatment with setrusumab (UX143) for at least 6 months resulted in 67% reduction in annualized fracture rate in patients with OI in Phase 2/3 *Orbit* study

Quantitative data from the Phase 1/2 study of GTX-102 for AS show clinically meaningful improvements in multiple domains as compared to natural history

4 of 5 patients in lowest-dose cohort of Phase 1/2/3 study of UX701 in Wilson disease are tapering off of chelators and/or zinc therapy, including 2 that are now completely off standard therapy

Ultragenyx Analyst Day live webcast available today at 8:30 a.m. ET

NOVATO, Calif., Oct. 16, 2023 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE) today will provide updates on its development pipeline, including setrusumab (UX143) for osteogenesis imperfecta (OI), GTX-102 for Angelman syndrome (AS), UX701 in Wilson disease and the rest of the company's gene therapy portfolio at an Analyst Day held in New York City and by webcast.

"The data we are presenting today show that these investigational therapies are having meaningful clinical effects on difficult diseases with limited or no approved treatments and are potentially transformative for people living with these diseases if proven safe and effective in Phase 3 studies," said Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx. "We also have one of the largest, late-stage gene therapy pipelines in rare disease with full end-to-end capabilities in-house that uniquely position us to deliver high quality robust commercial manufacturing at scale to support our large pipeline."

Analyst Day Updates

UX143 (setrusumab) monoclonal antibody for Osteogenesis Imperfecta (OI): Interim Phase 2 data from the Phase 2/3 Orbit study show statistically significant decrease in annualized fracture rates following at least 6 months of treatment

- Data presented at the American Society for Bone and Mineral Research 2023 Annual Meeting (ASBMR) show that treatment with setrusumab reduced the annualized fracture rate by 67% and this reduction was associated with continuing large and meaningful improvements in bone mineral density (BMD).
- Setrusumab was generally well tolerated with no drug related serious adverse events (SAEs) reported and no reports of drug-related hypersensitivity.
- The company plans to provide updated Phase 2 data next year.

GTX-102 antisense oligonucleotide for Angelman syndrome: Data from the extension cohorts in the Phase 1/2 study show clinically meaningful improvements in multiple domains

- Quantitative data show improvements across multiple clinical domains compared to natural history data, where available, and clinical changes were associated with quantitative changes in EEG.
- Long term data showed patients who stopped and restarted treatment reacquired previously gained developmental skills when they were re-dosed with the current regimen.
- There have been no additional treatment-related SAEs, including lower extremity weakness, since November 2022.
- Data from the dose expansion cohorts on at least 20 patients who have been on therapy for at least 6 months is anticipated in the first half of 2024.

UX701 AAV gene therapy for Wilson disease: Four of five patients in the lowest-dose cohort of the Phase 1/2/3 Cypress $^{2+}$ study show improvements in tapering standard of care

- Four out of 5 patients in the low-dose Cohort 1 have had reductions in urinary copper and are tapering off of chelators and/or zinc therapy, including 2 of 3 earlier treated patients in the Cohort that are now completely off standard therapy.
- UX701 has been generally well tolerated with no treatment-related SAEs.
- The seamless study is expected to complete dosing of all 3 dose cohorts in Stage 1 at the end of 2023 and these data are expected in the first half of 2024.

Company also provided update on other late-stage gene therapy candidates

- **DTX401 AAV gene therapy for Glycogen Storage Disease Type Ia (GSDIa):** The Phase 3 *GlucoGene* study was fully enrolled in the first

quarter of 2023 and the company plans to provide preliminary data in the first half of 2024.

- **UX111 for Sanfilippo syndrome (MPS IIIA):** The pivotal *Transpher A* study has been fully enrolled and the company plans to meet with the FDA in the fourth quarter of 2023.
- **DTX301 AAV gene therapy for Ornithine Transcarbamylase (OTC) Deficiency:** The Phase 3 *Enh3ance* study is expected to complete enrollment in the first half of 2024.

Analyst Day and Webcast Information

Ultragenyx will host an Analyst Day at 8:30 a.m. ET on Monday, October 16, 2023 to discuss these data and to provide an update on the company's development pipeline. A live video webcast of the program will be available at https://www.webcaster4.com/Webcast/Page/359/49192. An archived version of the remarks will also be available through the Ultragenyx website.

About Ultragenyx
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 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 its future operating results and financial performance, business plans and objectives for UX143, expectations regarding the clinical benefit, tolerability and safety of UX143, future clinical and regulatory developments for UX143, the clinical benefit, tolerability and safety of GTX-102, future clinical and regulatory developments for GTX-102, the clinical benefit, tolerability and safety of UX701, future clinical and regulatory developments for UX701, timing for enrollment, dosing and data for Ultragenyx's investigational therapies and gene therapy candidates and 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 and Mereo BioPharma to successfully develop UX143, 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, the potential for any license or collaboration agreement, including the company's collaboration agreement with Mereo to be terminated, 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 August 4, 2023, 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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