Ultragenyx to Present Setrusumab (UX143) Update at ASBMR 2023 Including New Data from Phase 2/3 Orbit Study in Osteogenesis Imperfecta (OI)

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NOVATO, Calif., Oct. 09, 2023 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE) today announced that data from its ongoing late-stage program evaluating setrusumab (UX143) in osteogenesis imperfecta (OI) will be presented at the American Society for Bone and Mineral Research 2023 Annual Meeting (ASBMR) October 13-16, 2023, in Vancouver, British Columbia.

Ultragenyx and its collaborators will present late-breaking presentations on new, updated data from the Phase 2 portion of the Phase 2/3 *Orbit* study evaluating setrusumab on annualized clinical fracture rate in patients aged 5 to <26 years, and data on the burden of fractures for individuals living with OI. The company will also present information on the Phase 3 *Cosmic* study design comparing setrusumab to intravenous bisphosphonate (IV-BP) in patients aged 2 to <5 years.

Details of the setrusumab presentations are as follows:

Abstract Title: Evaluating Setrusumab for the Treatment of Osteogenesis Imperfecta: Phase 2 Data from the Phase 2/3 *ORBIT* Study (Lewiecki, E.M. et al.)
Session: Late-breaking poster session I
Session Date and Time: Saturday, Oct 14, 2023, 13:30–15:00 PDT
Presentation Number: LB SAT-650

Abstract Title: Burden of Fractures for Individuals Living with Osteogenesis Imperfecta: Integrating Real-world Claims Data in the United States and Caregiver Panel Interviews (Byers, H. et al.)
Session: Late-breaking poster session I
Session Date and Time: Saturday, Oct 14, 2023, 13:30–15:00 PDT
Presentation Number: LB SAT-648

Abstract Title: *Cosmic*: An open-label, randomized, active-controlled, phase 3 study of setrusumab compared with bisphosphonates in pediatric subjects with osteogenesis imperfecta (Krolczyk, S. et al.)
Session: Poster session I
Session Date and Time: Saturday, Oct 14, 2023, 13:30–15:00 PDT
Presentation Number: SAT-500

Ultragenyx is leading the clinical development of setrusumab as part of a collaboration and license agreement with Mereo BioPharma Group plc (NASDAQ: MREO), a clinical-stage biopharmaceutical company focused on rare diseases. The companies previously announced positive data from the dose-selection Phase 2 portion of the *Orbit* study showing that setrusumab rapidly induced bone production in OI-affected patients.

U.S. residents can learn more by visiting ultraclinicaltrials.com.

About Osteogenesis Imperfecta (OI)
Osteogenesis Imperfecta (OI) includes a group of genetic disorders impacting bone metabolism. Approximately 85% to 90% of OI cases are caused by mutations in the *COL1A1 or COL1A2* genes, leading to either reduced or abnormal collagen and changes in bone metabolism. The collagen mutations in OI can result in increased bone brittleness, which contributes to a high rate of fractures, including at atypical sites. Patients with OI also exhibit increased bone resorption (breakdown of old bone) and inadequate production of new bone, which leads to decreased bone mass, bone fragility and weakness. OI can also lead to bone deformities, abnormal spine curvature, pain, decreased mobility, and short stature. No treatments are approved for OI, which affects approximately 60,000 people in the developed world.

About Setrusumab (UX143)
Setrusumab is a fully human monoclonal antibody that inhibits sclerostin, a protein that acts on a key bone-signaling pathway that inhibits the maturation and activity of bone-forming cells. The goal of blocking inhibitory effects of sclerostin is to increase new bone formation, bone mineral density and bone strength. Sclerostin inhibition also reduces excessive bone resorption, further enhancing its impact on bone density. In mouse models of OI, the use of anti-sclerostin antibodies was shown to stimulate bone formation, improve bone mass and density, and increase bone strength against fracture force testing.

Mereo BioPharma's Phase 2b study (*ASTEROID*) treatment phase of the dose-finding study of setrusumab for the treatment of OI in 112 adults was concluded in 2019. The *ASTEROID* study demonstrated treatment with setrusumab resulted in a clear, dose-dependent and statistically significant effect on bone formation and bone density at multiple anatomical sites among adult participants with OI.

Ultragenyx and Mereo BioPharma are collaborating on the development of setrusumab globally. The companies have developed a comprehensive late-stage program to continue development of setrusumab in pediatric and young adult patients across OI sub-types I, III and IV.

About Ultragenyx
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 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 tolerability and safety of UX143, and future clinical and regulatory developments for UX143 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 3, 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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