Ultragenyx Announces Upcoming Setrusumab (UX143) Presentations at the ASBMR 2024 Annual Meeting

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NOVATO, Calif., Sept. 26, 2024 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE) today announced that it will present seven abstracts related to its ongoing late-stage program evaluating setrusumab (UX143) and osteogenesis imperfecta (OI), including a late-breaker oral presentation of the 14-month data from the Phase 2/3 *Orbit* study, at the American Society for Bone and Mineral Research (ASBMR) 2024 Annual Meeting. The meeting is being held September 27-30, 2024, in Toronto, Canada.

“Presentations at this year’s ASBMR meeting add to our growing knowledge of the real-world impact and burden of OI and underscore the urgent need for an innovative therapy for these patients,” stated Eric Crombez, M.D., chief medical officer at Ultragenyx. “We will also present the phase 2 *Orbit* study results that we previously announced in June, which demonstrate a rapid and clinically meaningful increase in bone mineral density and a corresponding decrease in annualized fracture rate through month 14.”

Details of the setrusumab presentations:

Title: Integrated Bone Biomarker Analyses to Define Setrusumab Mechanism of Action in Pediatric and Young Adult Subjects with Osteogenesis Imperfecta and to Inform Dose Selection in the Orbit Study
Session: Welcome Reception and Plenary Poster Session
Presentation Number: Plenary Poster (#Fri-423)
Session Date / Time: Friday, September 27, 5:30 p.m. - 7:30 p.m. ET
Will also be presented during the Clinical Career Spotlight Rapid Fire session (#Sun-423) and during Poster Session I (#Sun-423)

Title: Burden of Illness of Osteogenesis Imperfecta in Ontario, Canada
Session: Late Breaking Poster Session I
Presentation Number: #Sat-LB 592
Session Date / Time: Saturday, September 28, 2:15 p.m.- 3:45 p.m. ET

Title: Manifestations and Comorbid Conditions Among Patients with Osteogenesis Imperfecta (OI): A US Retrospective Claims Database Analysis
Session: Poster Session I
Presentation Number: #Sat-447
Session Date / Time: Saturday, September 28, 2:15 p.m.- 3:45 p.m. ET

Title: Fracture Rates for Patients Living with Osteogenesis Imperfecta (OI): Real-world Results from US Retrospective Claims
Session: Poster Session I
Presentation Number: #Sat-446
Session Date / Time: Saturday, September 28, 2:15 p.m. - 3:45 p.m. ET

Title: Population Pharmacokinetics (PK) and PK/Pharmacodynamics Analyses to Select the Phase 3 Dose of Setrusumab in Pediatric Patients with Osteogenesis Imperfecta: Results from Phase 2 of the Orbit Study
Session: Oral Presentations: Pregnancy Associated Bone Loss and Other Rare Bone Diseases
Presentation Number: #1063
Presentation Date / Time: Sunday, September 29, 11:45 a.m. - 12:00 p.m. ET

Title: Healthcare Resource Use (HRU) and Costs for Patients Living with Osteogenesis Imperfecta (OI): Results from US Retrospective Claims
Session: Poster Session II
Presentation Number: #Sun-446
Session Date / Time: Sunday, September 29, 2:15 p.m. - 3:45 p.m. ET

Title: Sustained Reduction in Fracture Rate in Patients with OI Treated with Setrusumab: Fourteen Month Data from Phase 2 of the Phase 2/3 Orbit Study
Session: Late-Breaking Oral Presentations: Clinical Science
Presentation Number: #1125
Presentation Date / Time: Monday, September 30, 12:00 p.m. - 12:15 p.m. ET

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 genetic variants 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. Patients with OI also exhibit inadequate production of new bone and excess bone resorption, resulting in decreased bone mineral density, bone fragility and weakness. OI can also lead to bone deformities, abnormal spine curvature, pain, decreased mobility, and short stature. No treatments are globally approved for OI, which affects approximately 60,000 people in commercially accessible geographies.

About Setrusumab (UX143)
Setrusumab is a fully human monoclonal antibody that inhibits sclerostin, a negative regulator of bone formation. Blocking sclerostin is expected to increase new bone formation, bone mineral density and bone strength in OI. In mouse models of OI, the use of anti-sclerostin antibodies was shown to increase bone formation, improve bone mass to normal levels, and increase bone strength against fracture force testing to normal levels.

In 2019, Mereo BioPharma completed the Phase 2b dose-finding study (ASTEROID) for setrusumab in 112 adults with OI. 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 based on the collaboration and license agreement between the parties. 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 therapies to patients for the treatment of serious rare and ultrarare 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 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 2, 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-/mycompany/).

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