Ultragenyx and Mereo BioPharma to Present Setrusumab Data Update at ASBMR

September 10, 2022

NOVATO, Calif. and MOUNTAIN VIEW, Calif. and LONDON, Sept. 09, 2022 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), ("Ultragenyx"), a biopharmaceutical company focused on the development and commercialization of novel products for serious rare and ultra-rare genetic diseases and Mereo BioPharma Group plc (NASDAQ: MREO), ("Mereo"), a clinical-stage biopharmaceutical company focused on rare diseases and oncology, today announced that additional data from Mereo's Phase 2b ASTEROID study related to setrusumab (UX143) and information on the Phase 2/3 *Orbit* study will be presented at the American Society for Bone and Mineral Research 2022 Annual Meeting (ASBMR) being held September 9-12, 2022, in Austin, Texas. Setrusumab is a monoclonal antibody in development for the treatment of Osteogenesis Imperfecta (OI), a rare disease affecting approximately 60,000 individuals in the US and Europe.

Ultragenyx will present the latest results from the previously presented ASTEROID study and an overview of the design of the ongoing global, seamless Phase 2/3 *Orbit* study at the meeting. Data will also be presented from the IMPACT Survey, the largest collection of data in OI and a joint research project between the Osteogenesis Imperfecta Foundation (OIF), and the umbrella organization Osteogenesis Imperfecta Federation Europe (OIFE) and their members, supported by Mereo BioPharma.

Details of the ASBMR data presentations are as follows:

Abstract Title: *Orbit*: A Randomized, Double-Blind, Placebo-controlled, Phase 2/3 Study to Assess the Efficacy and Safety of Setrusumab in Pediatric and Young Adult Participants with Osteogenesis Imperfecta (Ominsky M et al.)
Presentation: Poster
Session Date and Time: Saturday, September 10, 2022, 13:00-15:00 Central Time
Presentation Number: LB SAT-897

Abstract Title: The IMPACT survey provides unique insights into the experiences of adults with osteogenesis imperfecta (OI) through self-reported data (Rauch F et al.)
Presentation: Poster
Session Date and Time: Sunday, September 11, 2022, from 13:00-15:00 Central Time
Presentation Number: SUN-667

Abstract Title Changes in BMD and Bone Turnover Markers Following Discontinuation of Setrusumab After 1 Year of Treatment in Adults with Osteogenesis Imperfecta (Javaid K et al.)
Presentation: Oral Presentation
Session Date and Time: Monday, September 12, 2022, from 11:45-12:00 Central Time
Presentation Number: 1112

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 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 various mouse models of OI, the use of anti-sclerostin antibodies was shown to stimulate bone formation, improve bone mass and density, and reduce bone fragility as reflected in increased long bone strength and reduced the number of fractures.

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. Off-treatment results were followed.

Ultragenyx and Mereo are collaborating on the development of setrusumab globally based on the collaboration and license agreement between the parties. The companies are planning 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 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 the company's website at: www.ultragenyx.com.

About Mereo BioPharma
Mereo BioPharma is a biopharmaceutical company focused on the development of innovative therapeutics for rare diseases and in oncology and plans to commercialize selected rare disease programs. The Company has developed a portfolio of six clinical stage product candidates. The Company has two rare disease product candidates, setrusumab for the treatment of osteogenesis imperfecta (OI) and alvelestat for the treatment of severe Alpha-1 antitrypsin deficiency (AATD) and Bronchiolitis Obliterans Syndrome (BOS). The Company's partner, Ultragenyx Pharmaceutical, Inc.,

has initiated a pivotal Phase 2/3 pediatric study in young adults (5-25 years old) for setrusumab in OI and expects to initiate a study in pediatric patients (2-5 years old) in the second half of 2022. The partnership with Ultragenyx includes potential milestone payments of up to $254 million and royalties to Mereo on Ultragenyx territories. Mereo has retained EU and UK commercial rights and will pay Ultragenyx royalties on those territories. Alvelestat has received U.S. Orphan Drug Designation for the treatment of AATD and positive top-line data were recently reported from a Phase 2 proof-of-concept study in North America, Europe and the UK. Mereo's lead oncology product candidate, etigilimab (anti-TIGIT), is currently in an open label Phase 1b/2 basket study evaluating anti-TIGIT in combination with an anti-PD-1 in a range of tumor types including three rare tumors and three gynecological carcinomas, cervical, ovarian, and endometrial carcinomas. The Company's second oncology product, navicixizumab, for the treatment of late line ovarian cancer, has completed a Phase 1 study and has been partnered with OncXerna Therapeutics, Inc., formerly Oncologie, Inc. The global licensing agreement with OncXerna includes payments of up to $300 million in milestones and royalties.

Mereo Forward-Looking Statements
This press release contains "forward-looking statements." All statements other than statements of historical fact contained in this press release are forward-looking statements within the meaning of Section 27A of the United States Securities Act of 1933, as amended (the "Securities Act"), and Section 21E of the United States Securities Exchange Act of 1934, as amended (the "Exchange Act"). Forward-looking statements usually relate to future events and anticipated revenues, earnings, cash flows or other aspects of our operations or operating results. Forward-looking statements are often identified by the words "believe," "expect," "anticipate," "plan," "intend," "foresee," "should," "would," "could," "may," "estimate," "outlook" and similar expressions, including the negative thereof. The absence of these words, however, does not mean that the statements are not forward-looking. These forward-looking statements are based on Mereo's current expectations, beliefs and assumptions concerning future developments and business conditions and their potential effect on Mereo. While management believes that these forward-looking statements are reasonable as and when made, there can be no assurance that future developments affecting Mereo will be those that it anticipates.

All of Mereo's forward-looking statements involve known and unknown risks and uncertainties, some of which are significant or beyond its control and assumptions that could cause actual results to differ materially from Mereo's historical experience and its present expectations or projections. You should carefully consider the foregoing factors and the other risks and uncertainties that affect Mereo's business, including those described in the "Risk Factors" section of its latest Annual Report on Form 20-F, reports on Form 6-K and other documents furnished or filed from time to time by Mereo with the Securities and Exchange Commission. Mereo wishes to caution you not to place undue reliance on any forward-looking statements, which speak only as of the date hereof. Mereo undertakes no obligation to publicly update or revise any of our forward-looking statements after the date they are made, whether as a result of new information, future events or otherwise, except to the extent required by law.

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 business plans and objectives, the therapeutic potential and clinical benefits of its product candidates, expectations regarding the safety and tolerability of its product candidates, and future clinical developments for its product candidates 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 the company's clinical development programs, 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 ability of the company to successfully develop UX143, the effects of the COVID-19 pandemic on the company's clinical activities, business and operating results, risks related to reliance on third party partners to conduct certain activities on Ultragenyx's behalf; the potential for any license or collaboration agreement, including Ultragenyx's collaboration agreement with Mereo, to be terminated, uncertainty and potential delays related to clinical drug development, the risk that clinical outcomes demonstrated in interim data from Ultragenyx's clinical trials may materially change or increased incidents of adverse events as patient enrollment continues and/or more patient data becomes available, Ultragenyx's ability to achieve its projected development goals in its expected timeframes, risks and uncertainties related to the regulatory approval process, 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 on July 29, 2022, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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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