Mereo BioPharma and Ultragenyx Present Data from the Phase 2b ASTEROID Study of UX143 (setrusumab) in Osteogenesis Imperfecta (OI) at the American Society for Bone and Mineral Research (ASBMR) 2021 Annual Meeting

Previously reported data demonstrated dose-dependent, statistically significant bone-building effect at multiple anatomical sites in adult patients with OI that was consistent across subtypes of OI

Dose-dependent improvements in serum biomarkers of bone turnover with UX143 therapy confirm sclerostin inhibition mechanism of action

London and Novato, Calif., October 1, 2021 - Ultragenyx Pharmaceutical Inc. (Nasdaq: RARE), a biopharmaceutical company focused on the development and commercialization of novel products for serious rare and ultra-rare diseases, and Mereo BioPharma Group plc (Nasdaq: MREO), a clinical stage biopharmaceutical company focused on oncology and rare diseases, today presented secondary endpoint data on UX143 (setrusumab) from the Phase 2b ASTEROID study for the Treatment of Osteogenesis Imperfecta (OI). Data were presented at the American Society for Bone and Mineral Research (ASBMR) 2021 Annual Meeting in an oral presentation by Suzanne Jan de Beur, M.D., an associate professor of medicine at The Johns Hopkins University School of Medicine and president of ASBMR.

Previously reported analyses of pre-specified endpoints in the Phase 2b ASTEROID study demonstrated a clear, dose-dependent, statistically significant bone-building effect at multiple anatomical sites in adult patients with OI that was consistent across all subtypes of OI studied, including Types I, III and IV. This effect was determined by evaluating areal bone mineral density (BMD) over baseline at the lumbar spine, as measured by DXA and reaching 8.97% at 12 months in the highest dose cohort ($p<0.001$). The data also show a statistically significant and consistent improvement in bone strength (stiffness) over baseline at the wrist and tibia measured by Finite Element Analysis (FEA) in both medium and high dose cohorts ($p<0.05$). There was also a statistically significant improvement in bone strength (failure load) at the wrist at the highest dose ($p<0.01$). New data presented today at ASBMR demonstrate that treatment with UX143 resulted in dose-dependent increase in P1NP serum levels, a marker of bone formation, and decrease in CTx serum levels, a marker of bone resorption, confirming the mechanism of action of sclerostin inhibition. These changes peaked at month one and declined thereafter, an expected effect based on data from prior clinical studies of sclerostin antibodies tested in osteoporosis. Observed improvements in BMD were continuous, with comparable gains achieved in the first and second 6 months of treatment in the high dose group despite temporal changes in biomarkers.

*"*The continuous gains in BMD over the twelve months of the ASTEROID study clearly warrant further investigation of the longer-term treatment effect of UX143 in patients with osteogenesis imperfecta,*"* stated Francis Glorieux, M.D., Ph.D., Founding Director of the Genetics Unit at the Shriners Hospital for Children in Montreal and one of the study's co-ordinating PIs.

"These data analyses provide a clear picture of the sclerostin-inhibition effect of UX143 that resulted in both a statistically significant bone building effect and a significant increase in bone strength based on pre-specified measures in adults across all subtypes of OI studied. With Ultragenyx, we plan to advance UX143 into a comprehensive late-stage program in pediatric and adult patients across OI sub-types I, III and IV," said

Denise Scots-Knight, Ph.D., Chief Executive Officer of Mereo. “We would like to thank Dr. Jan de Beur for today’s presentation, and the co-ordinating Primary Investigators Dr. Glorieux, Dr. Kassim Javaid and Dr. Jay Shapiro for their work on the study.”

Top-line 12-month data from the ASTEROID study were reported in November 2019, with full data, including secondary endpoint analyses, reported in January 2020.

About UX143 (setrusumab)
UX143 is a fully human monoclonal antibody that inhibits sclerostin, a protein that acts on a key bone-signalling pathway and inhibits the activity of bone-forming cells. The goal of blocking inhibitory effects of sclerostin is to create new bone formation, increase production of collagen, and increase bone mineral density and strength. Sclerostin inhibition also reduces excessive bone resorption, further enhancing the 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, reduce bone fragility, increase long bone stiffness and strength, and reduce the number of fractures. UX143 is being evaluated to treat osteogenesis imperfecta (OI), and a Phase 2b study (ASTEROID) dose-finding study in 112 adults was concluded in 2019.

Mereo BioPharma (MREO) and Ultragenyx are collaborating on the development of UX143 globally based on the collaboration and license agreement between the parties. The companies are planning a comprehensive late-stage program to continue development of UX143 in pediatric and young adult patients across OI sub-types I, III and IV.

About the Phase 2b ASTEROID Study
ASTEROID was a 12-month, randomized, double-blind, Phase 2b dose-finding study in 112 adults diagnosed with type I, III or IV Osteogenesis Imperfecta and a confirmed COL1A1/COL1A2 mutation who had fractured over the previous 5 years. The study evaluated three dose levels of UX143, 2 mg/kg (low), 8 mg/kg (medium), and 20 mg/kg (high). ASTEROID was the largest prospectively-designed interventional clinical study to be performed in this patient group. The primary endpoint of the study was the change over baseline in Tr.vBMD of the wrist at 12 months, followed by changes in bone strength measured by Finite Element Analysis (FEA) (hierarchical), assessed using HR-pQCT. While the primary endpoint was not met, UX143 did demonstrate a clear benefit in multiple pre-specified endpoint analyses, including improvements from baseline in bone strength (stiffness) at the wrist and tibia, and areal BMD at the lumbar spine, as measured by DXA. Additional secondary endpoints included HR-pQCT parameters (such as total vBMD), bone biomarkers, Patient Reported Outcomes (PRO) and Quality of Life measures. Fracture data were also collected throughout the duration of the study, although the trial was not statistically powered for fractures.

About Osteogenesis Imperfecta
Osteogenesis Imperfecta (OI) is a rare genetic disorder that is characterized by fragile bones and reduced bone mass resulting in bones that break easily. In severe cases patients may experience hundreds of fractures in a lifetime. In addition, people with OI often suffer muscle weakness, early hearing loss, fatigue, curved bones, scoliosis, respiratory problems and short stature, leading to significant impacts on overall health and quality of life. In the majority of people with OI (estimated at approximately 90%) the condition is caused by a dominant mutation in a gene coding for type I collagen, a key component of healthy bone. Current treatment for OI is supportive, focusing on minimizing fractures and maximizing mobility, but to date, there are no EU or FDA approved treatments.

About Ultragenyx Pharmaceutical Inc.
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 and commercialization of innovative therapeutics that aim to improve outcomes for oncology and rare diseases. The Company has developed a portfolio of six clinical-stage product candidates. Mereo's lead oncology product candidate, etigilimab (Anti-TIGIT), has recently advanced into 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 a number of gynecological carcinomas including 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 Company has two rare disease product candidates: alvelestat for the treatment of severe Alpha-1 antitrypsin deficiency (AATD), which is being investigated in an ongoing Phase 2 proof-of-concept study in the U.S. and Europe, for which the Company expects to report on in late 2021, and setrusumab for the treatment of osteogenesis imperfecta (OI). In September 2020, the FDA granted Rare Pediatric Disease designation to setrusumab for the treatment of OI. In December 2020, the Company signed a license and collaboration agreement for setrusumab in OI with Ultragenyx Pharmaceutical Inc.

Ultragenyx Forward-Looking Statements

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 for UX143, the therapeutic potential and clinical benefits of UX143, expectations regarding the safety and tolerability of UX143, and future clinical 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 ability of the company and Mereo BioPharma to successfully develop UX143 , the effects from 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 the company's behalf, uncertainty and potential delays related to clinical drug development, 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 August 3, 2021, and its subsequent periodic reports filed with the Securities and Exchange Commission.

Mereo BioPharma 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 the Company's current expectations, beliefs and assumptions concerning future developments and business conditions and their potential effect on the Company. While management believes that these forward-looking statements are reasonable as and when made, there can be no assurance that future developments affecting the Company will be those that it anticipates.

All of the Company'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 the Company's historical experience and its present expectations or projections. You should carefully consider the foregoing factors and the other risks and uncertainties that affect the Company'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 the Company with the Securities and Exchange Commission. The Company wishes to caution you not to place undue reliance on any forward-looking statements, which speak only as of the date hereof. The Company 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.

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