Ultragenyx Announces Crysvita® (burosumab) and UX143 (setrusumab) Data Presentations at Upcoming American Society for Bone and Mineral Research (ASBMR) 2021 Annual Meeting

September 27, 2021

NOVATO, Calif., Sept. 27, 2021 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development and commercialization of novel therapies for serious rare and ultra-rare genetic diseases, today announced that new data highlighting Crysvita® (burosumab-twza) for the treatment of X-linked hypophosphatemia (XLH) and UX143 (setrusumab) for the treatment of osteogenesis imperfecta (OI) will be presented at the American Society for Bone and Mineral Research (ASBMR) 2021 Annual Meeting. The meeting will take place October 1-4 in-person in San Diego with programming also virtually accessible in real-time.

Details for the presentations are as follows:

X-Linked Hypophosphatemia

Oral Presentations

- Neurological and Psychiatric Manifestations of X-Linked Hypophosphatemia in a Longitudinal Cohort Study: XLH Disease Monitoring Program (XLH-DMP)
  - Presentation #1019: Friday, October 1, 2:15-2:30 p.m. PT
  - Presenter: Suzanne Jan de Beur, M.D.

- Burosumab Improves Lower Limb Alignment in Children with X-Linked Hypophosphatemia
  - Presentation #1020: Friday, October 1, 2:30-2:45 p.m. PT
  - Presenter: David Frumberg, M.D.

Poster Presentations

- Patient Perspective: XLH Requires Whole-Body, Whole-Life, Whole-Family Care
  - SAT-268: Saturday, October 2, 1:00-3:00 p.m. PT

- Adolescents with X-linked hypophosphatemia (XLH): first year of real-world data from the XLH Disease Monitoring Program (DMP)
  - SAT-269: Saturday, October 2, 1:00-3:00 p.m. PT

- Rarediseasegenes.com/phex: A comprehensive locus specific database of PHEX gene variants associated with X-linked hypophosphatemia vastly increases the number of known variants.
  - SUN-274: Sunday, October 3, 1:00-3:00 p.m. PT

Osteogenesis Imperfecta

Oral Presentation

- Setrusumab for the Treatment of Osteogenesis Imperfecta (OI): Results from the Phase 2b ASTEROID Study
  - Presentation #1016: Friday, October 1, 1:30-1:45 p.m. PT
  - Presenter: Suzanne Jan de Beur, M.D.

Poster Presentations

- Safety Evaluation of Setrusumab in Juvenile CD-1 Mice
  - VPP-714: Saturday, October 2 (virtual poster)

- Baseline Patient Demographics and Disease Characteristics in Adults with Osteogenesis Imperfecta (OI) in the Phase 2b ASTEROID Study
  - VPP-688: Saturday, October 2 (virtual poster)

- The Patient Clinical Journey and Socioeconomic Impact of Osteogenesis Imperfecta: A Systematic Review
  - SUN-280: Sunday, October 3, 1:00-3:00 p.m. PT

About Crysvita

Crysvita (burosumab-twza) is a recombinant fully human monoclonal IgG1 antibody, discovered by Kyowa Kirin, against the phosphaturic hormone FGF23. FGF23 is a hormone that reduces serum levels of phosphorus and active vitamin D by regulating phosphate excretion and active vitamin D production by the kidney. Phosphate wasting in tumor-induced osteomalacia (TIO) and other hypophosphatemic conditions, including X-linked hypophosphatemia (XLH), is caused by excessive levels and activity of FGF23. Crysvita is designed to bind to and thereby inhibit the biological activity of FGF23. By blocking excess FGF23 in patients with TIO and XLH, Crysvita is intended to increase phosphate reabsorption from the kidney and increase the production of active vitamin D, which enhances intestinal absorption of phosphate and calcium.

Crysvita is approved by the U.S. FDA for the treatment of XLH in adult and pediatric patients six months of age and older and FGF23-related hypophosphatemia in TIO associated with phosphaturic mesenchymal tumors that cannot be curatively resected or localized in adults and pediatric patients 2 years of age and older, and by Health Canada and Brazil's National Health Surveillance Agency (ANVISA) for the treatment of XLH in adult and pediatric patients one year of age and older. In Japan, it is approved by the Ministry of Health, Labor and Welfare (MHLW) for the treatment of FGF23-related hypophosphatemic rickets and osteomalacia. In Europe, Crysvita has received European conditional marketing authorization for the treatment of XLH with radiographic evidence of bone disease in adult and pediatric patients one year of age and older.

Kyowa Kirin and Ultragenyx have been collaborating on the development and commercialization of Crysvita globally based on the collaboration and license agreement between the parties.

About UX143 (setrusumab)
UX143 is a fully human monoclonal antibody that inhibits sclerostin, a protein that acts on a key bone-signaling 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.

Crysvita U.S. INDICATION
Crysvita® (burosumab-twza) is a fibroblast growth factor 23 (FGF23)-blocking antibody indicated for the treatment of:

- X-linked hypophosphatemia (XLH) in adult and pediatric patients 6 months of age and older.
- FGF23-related hypophosphatemia in tumor-induced osteomalacia (TIO) associated with phosphaturic mesenchymal tumors that cannot be curatively resected or localized in adult and pediatric patients 2 years of age and older.

IMPORTANT SAFETY INFORMATION
CONTRAINDICATIONS

- With oral phosphate and/or active vitamin D analogs (e.g., calcitriol, paricalcitol, doxercalciferol, calcifediol).
- When serum phosphorus is within or above the normal range for age.
- In patients with severe renal impairment or end stage renal disease.

WARNINGS AND PRECAUTIONS
Hypersensitivity

- Discontinue Crysvita if serious hypersensitivity reactions occur and initiate appropriate medical treatment.

Hyperphosphatemia and Risk of Nephrocalcinosis

- For patients already taking Crysvita, dose interruption and/or dose reduction may be required based on a patient's serum phosphorus levels.
- Patients with TIO who undergo treatment of the underlying tumor should have dosing interrupted and adjusted to prevent hyperphosphatemia.

Injection Site Reactions

- Discontinue Crysvita if severe injection site reactions occur and administer appropriate medical treatment.

ADVERSE REACTIONS
Pediatric XLH Patients

- Adverse reactions reported in 10% or more of Crysvita-treated pediatric XLH patients across all studies are: pyrexia, injection site reaction, cough, vomiting, pain in extremity, headache, tooth abscess, dental caries, diarrhea, vitamin D decreased, toothache, constipation, myalgia, rash, dizziness, and nausea.
- Post-marketing experience reported in pediatric XLH patients receiving Crysvita – blood phosphorus increased.

Adult XLH Patients

- Adverse reactions reported in more than 5% of Crysvita-treated adult XLH patients and in at least 2 patients more than

placebo in one study are: back pain, headache, tooth infection, restless legs syndrome, vitamin D decreased, dizziness, constipation, muscle spasms, and blood phosphorus increased.
- Spinal stenosis is prevalent in adults with XLH, and spinal cord compression has been reported. It is unknown if Crysvita therapy exacerbates spinal stenosis or spinal cord compression.

Adult TIO Patients

- Adverse reactions reported in more than 10% of Crysvita-treated adult TIO patients in two studies are: tooth abscess, muscle spasms, dizziness, constipation, injection site reaction, rash, and headache.

USE IN SPECIFIC POPULATIONS

- There are no available data on Crysvita use in pregnant women to inform a drug-associated risk of adverse developmental outcomes. Serum phosphorus levels should be monitored throughout pregnancy. Report pregnancies to the Kyowa Kirin, Inc. Adverse Event reporting line at 1-888-756-8657.
- There is no information regarding the presence of Crysvita in human milk or the effects of Crysvita on milk production or the breastfed infant.

PATIENT COUNSELING INFORMATION

- Advise patients not to use any oral phosphate and/or active vitamin D analog products.
- Instruct patients to contact their physician if hypersensitivity reactions, injection site reactions, and restless leg syndrome induction or worsening of symptoms occur.

Side effects may be reported to the FDA at (800) FDA-1088 or www.fda.gov/medwatch. Side effects may also be reported to Kyowa Kirin, Inc. at 1-888-756-8657.

Please see full Prescribing Information for a complete discussion of the risks associated with CRYSVITA.

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.

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 future operating results and financial performance, anticipated cost or expense reductions, the timing, progress and plans for its clinical programs and clinical studies, future regulatory interactions, and the components and timing of regulatory submissions 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 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.

Contacts
Ultragenyx Pharmaceutical, Inc.
Investors
Joshua Higa
415-660-0951

Media
Carolyn Wang
415-225-5050

Mereo Biopharma
Simon Craig

+44 7977 037817

Kyowa Kirin Co., Ltd.
Media
Hiroki Nakamura
+81-3-5205-7205
Email: media@kyowakirin.com