August 23, 2016

Ultragenyx Announces KRN23 Data Presentations at ASBMR 2016 Annual Meeting

NOVATO, Calif., Aug. 23, 2016 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ:RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, today announced upcoming presentations of data highlighting KRN23 for the treatment of X-linked hypophosphatemia (XLH) and tumor-induced osteomalacia (TIO) at the American Society for Bone and Mineral Research (ASBMR) 2016 Annual Meeting taking place September 16-19 in Atlanta, Georgia.

Two oral presentations will highlight 40-week (n=52) and 64-week (n=36) data from the Phase 2 pediatric XLH study, as well as results from the ongoing Phase 2 TIO study:

Oral Presentation #1154: A Randomized, Open-label Phase 2 Study of KRN23, a Fully Human Anti-FGF23 Monoclonal Antibody, in 52 Children with X-linked Hypophosphatemia (XLH)

- Monday, September 19, 9:45 - 10:00 AM ET
- Room 412, Georgia World Congress Center

Oral Presentation #1098: Effects of KRN23, an Anti-FGF23 Antibody, in Patients With Tumor Induced Osteomalacia and Epidermal Nevus Syndrome: Results from an Ongoing Phase 2 Study

- Sunday, September 18, 10:15 - 10:30 AM ET
- Sidney Marcus Auditorium, Georgia World Congress Center

Posters highlight additional KRN23 data including functional patient-reported outcomes from the Phase 2 pediatric XLH study, and data from the open-label extension study in adults with XLH:

Poster #FR0331 and #SA0331 (shown twice): Evaluating the Effects of KRN23, a Fully Human Anti-FGF23 Monoclonal Antibody, on Functional Outcomes in Children with X-linked Hypophosphatemia (XLH): 40-week Interim Results from a Randomized, Open-label Phase 2 Study

- Friday, September 16, 5:30 - 7:00 PM ET and Saturday, September 17, 12:30 — 2:30 PM ET
- ASBMR Discovery Hall - Expo Hall A1, Georgia World Congress Center

Poster #MO0319: Clinical and Radiographic Characteristics of Adult X-linked Hypophosphatemia (XLH) in a Cohort of Patients Treated with KRN23, an Antibody to FGF23

- Monday, September 19, 12:30 - 2:30 PM ET
- ASBMR Discovery Hall - Expo Hall A1, Georgia World Congress Center

About XLH

XLH is a disorder of phosphate metabolism caused by phosphate wasting in the urine leading to severe hypophosphatemia. XLH is the most common heritable form of rickets (the softening and weakening of bones) that is inherited as an X-linked dominant trait affecting both males and females, though some reports indicate that the disease may be more severe in males. XLH is a distinctive disease characterized by inadequate mineralization of bone that leads to a spectrum of abnormalities, including rickets, progressive bowing of the leg, osteomalacia, bone pain, waddling gait, short stature, gross motor impairment, muscle weakness, frequent/poorly healing pseudofractures, spinal stenosis, enthesopathy, and osteoarthritis. Most pediatric patients and some adult patients are managed using oral phosphate replacement and vitamin D (calcitriol) therapy, which requires multiple divided doses each day and monitoring for potential risks such as nephrocalcinosis, hypercalciuria, and hyperparathyroidism.

About TIO

TIO, and a skin lesion variant, epidermal nevus syndrome (ENS)-associated osteomalacia, are caused by typically benign tumors or lesions that produce excess levels of FGF23, causing phosphate wasting in the urine that leads to severe hypophosphatemia, osteomalacia, muscle weakness, fatigue, bone pain, and fractures. The symptoms rapidly resolve if the causal tumors or lesion can be resected; however, there are cases in which resection is not feasible or recurrence of the tumor occurs after resection. In patients for whom the tumor or lesion is inoperable, the current treatment consists of oral phosphate and/or vitamin D replacement. Efficacy of this treatment is often limited, as it does not treat the underlying disease and its benefits must be balanced with monitoring for potential risks such as nephrocalcinosis, hypercalciuria, and hyperparathyroidism.

About KRN23

KRN23 is an investigational recombinant fully human monoclonal IgG1 antibody, discovered by Kyowa Hakko Kirin, against the phosphaturic hormone fibroblast growth factor 23 (FGF23). It is being developed by Ultragenyx and Kyowa Hakko Kirin to treat XLH and TIO, diseases characterized by excess activity of FGF23. FGF23 is a hormone that reduces serum levels of phosphorus and vitamin D by regulating phosphate excretion and vitamin D production by the kidney. Phosphate wasting in XLH and TIO is caused by excessive levels and activity of FGF23. KRN23 is designed to bind to and thereby inhibit the excessive biological activity of FGF23. By blocking excess FGF23 in patients with XLH and TIO, KRN23 is intended to increase phosphate reabsorption from the kidney and increase the production of vitamin D, which enhances intestinal absorption of phosphate and calcium.

About Ultragenyx

Ultragenyx is a clinical-stage biopharmaceutical company committed to bringing to market novel products for the treatment of rare and ultra-rare diseases, with a focus on serious, debilitating genetic diseases. Founded in 2010, the company has rapidly built a diverse portfolio of product candidates with the potential to address diseases for which the unmet medical need is high, the biology for treatment is clear, and for which there are no approved therapies.

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 regarding Ultragenyx's expectations regarding the timing of release of additional data for its product candidates, plans to initiate additional studies for its product candidates and timing regarding these studies, plans regarding ongoing studies for existing programs and its intent to file for conditional approval, 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, 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 uncertainties inherent in the clinical drug development process, including the regulatory approval process, the timing of our regulatory filings, and other matters that could affect the success of our drug development programs, including KRN23. 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 the Company in general, see Ultragenyx's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on August 9, 2016, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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