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Ultragenyx Announces First Patient Enrolled in Global Phase 3 Study of KRN23 in Adults With X-Linked Hypophosphatemia (XLH)

NOVATO, Calif., Dec. 03, 2015 (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 the initiation of the Phase 3 study of KRN23 for the treatment of adults with XLH (X-linked hypophosphatemia). XLH is an inherited metabolic bone disease characterized by short stature, skeletal deformities, bone pain, fractures, and muscle weakness. Ultragenyx and Kyowa Hakko Kirin entered into a collaboration and license agreement in August 2013 to develop and commercialize KRN23.

"Many adult patients with XLH continue to have significant complications from their disease and are in need of treatment options," said Sunil Agarwal, M.D, Chief Medical Officer of Ultragenyx. "The initiation of this Phase 3 pivotal study in adults with XLH is a significant step forward in our overall development program for patients with this debilitating disease."

The Phase 3 study is a, randomized, double-blind, placebo-controlled clinical study that will assess the efficacy and safety of monthly KRN23 at 24 weeks in approximately 120 adult XLH patients in the US, EU, Canada, Japan, and Korea. The primary endpoint of the study will be serum phosphorus levels through 24 weeks and the key secondary endpoint is the Brief Pain Inventory Question 3 (pain at its worst in the last 24 hours) at Week 24. Other secondary endpoints include patient reported outcomes assessing skeletal pain, stiffness, fatigue, motor function, and quality of life in these patients. Ultragenyx also plans to initiate a 48-Week open-label bone quality study in approximately ten adult XLH patients evaluating the potential impact of KRN23 on the underlying osteomalacia via bone biopsy.

About X-Linked Hypophosphatemia (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 frequent divided doses and careful medical monitoring.

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, a disease 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 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, 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.

Multiple clinical studies of KRN23 in adult patients with XLH have been completed and Ultragenyx and Kyowa Hakko Kirin intend to continue development of KRN23 in adults with XLH. In addition, a Phase 2 study in pediatric patients with XLH is ongoing.

KRN23 is also being developed for tumor-induced osteomalacia (TIO), a disease characterized by typically benign tumors that produce excess levels of FGF23, which can lead to severe osteomalacia, fractures, bone and muscle pain, and muscle weakness.

About Ultragenyx

Ultragenyx is a clinical-stage biotechnology 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.

About Kyowa Hakko Kirin

Kyowa Hakko Kirin is a leading biopharmaceutical company in Japan focusing on its core business area of oncology, nephrology, and immunology/allergy. Kyowa Hakko Kirin leverages antibody-related leading-edge technologies to discover and develop innovative new drugs aiming to become a global specialty pharmaceutical company which contributes to the health and well-being of people around the world.

For more information, please visit www.kyowa-kirin.com.

Forward-Looking Statements

Except for the historical information contained herein, the matters set forth in this press release, including statements regarding plans for a bone quality study, 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 availability or commercial potential of our 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 the Company in general, see Ultragenyx's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on November 10, 2015, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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