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Ultragenyx Announces Completion of Enrollment in Phase 3 Study of KRN23 in Adults with X-Linked Hypophosphatemia (XLH)

NOVATO, Calif., July 28, 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 that it has completed patient enrollment in the Phase 3 study of KRN23 for the treatment of adults with X-linked hypophosphatemia (XLH). Data from the study are expected in 2017.

"Many adult patients with XLH have substantial disability, pain and stiffness due to complications from their disease, and are in need of a new treatment option. This study will help evaluate the potential effect of blocking FGF23 on this debilitating disease, with the hope that we may bring this potential therapy to all XLH patients who could benefit from it," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx.

The Phase 3 study is a randomized, double-blind, placebo-controlled clinical study designed to assess the efficacy and safety of monthly KRN23 in 134 adult XLH patients in the US, EU, Canada, Japan, and Korea who are experiencing pain at screening. The primary endpoint of the study is a comparison between active and placebo groups for the change in serum phosphorus levels from baseline through 24 weeks. The key secondary endpoint is the change from baseline of the Brief Pain Inventory Question 3 (BPI Q3; pain at its worst in the last 24 hours) over 24 weeks. Other secondary endpoints include patient reported outcomes assessing skeletal pain, stiffness, fatigue, motor function, and quality of life in these patients.

Ultragenyx is also conducting an open-label bone quality Phase 3 study in approximately 14 adult XLH patients evaluating the potential impact of KRN23 on osteomalacia via bone biopsy.

In addition, the company continues to study KRN23 in pediatric patients with XLH, through an ongoing Phase 2 study and a Phase 3 study that is expected to begin in mid-2016.

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 multiple divided doses each day 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 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.

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 the potential sufficiency of clinical data for regulatory approval, timing for study commencement and completion, and 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 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 March 10, 2016, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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