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Ultragenyx Initiates New Development Program Studying KRN23 for the Treatment of Tumor-Induced Osteomalacia

NOVATO, Calif., Jan. 6, 2015 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (Nasdaq:RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, announced the initiation of a new development program for the human monoclonal anti-FGF23 antibody KRN23 (UX023) in tumor-induced osteomalacia (TIO). TIO results from typically benign tumors that produce excess levels of fibroblast growth factor 23 (FGF23), which can lead to severe osteomalacia, fractures, bone and muscle pain, and muscle weakness. Ultragenyx intends to initiate a Phase 2 study of KRN23 in six adult TIO patients in the first half of 2015.

KRN23 is being developed under a license and collaboration agreement between Ultragenyx and Kyowa Hakko Kirin Co., Ltd. KRN23 is being evaluated in a separate Phase 2 clinical study for pediatric patients with X-linked hypophosphatemia (XLH) and has completed multiple Phase 1/2 studies in adults with XLH.

"We are pleased to announce the start of our Phase 2 program in TIO. This decision was based on the clear biological rationale and the particularly high unmet medical need in these patients when tumor resection is not feasible," commented Sunil Agarwal, M.D., Ultragenyx's Chief Medical Officer. "We anticipate interim safety and efficacy data from the Phase 2 study by the end of 2015. With the start of the TIO program, the KRN23 development plan now includes two diseases, TIO and XLH, both sharing the same underlying pathology of excess FGF23 production."

The open-label, dose-finding Phase 2 clinical study will evaluate safety and efficacy in approximately six adult patients. The primary objectives of the study are to establish the dose, dosing regimen, and safety profile of treatment with KRN23 in TIO patients. Preliminary clinical effects of KRN23 treatment will be evaluated by radiographic assessments, muscle strength, walking ability, and by patient-reported measures of pain, disability, and quality of life. Markers of bone health and changes in serum phosphorus and other biochemical measures will also be followed.

The study will consist of a 16-week individual dose-titration period followed by a 28-week treatment period. The goal of the dose-titration period is to identify the individualized dose of KRN23 required to achieve stable serum phosphorus levels in the target range. Patients will receive subcutaneous injections of KRN23 once every four weeks.

About TIO

TIO, and its skin lesion variant, epidermal nevus syndrome (ENS), are caused by typically benign tumors 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 can be resected; however, there are cases in which resection of the tumor is not feasible or recurrence of the tumor occurs after resection. In patients for whom the tumor is inoperable, the current standard of care 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. There are an estimated 500-1,000 patients with TIO in the United States, and approximately half of all cases are inoperable.

About KRN23 and FGF23

KRN23 is an investigational recombinant fully human monoclonal IgG1 antibody against the phosphaturic hormone FGF23 being developed to treat TIO and XLH, both of which are 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. KRN23 is designed to bind to and thereby inhibit the excessive biological activity of FGF23. By blocking excess FGF23, KRN23 is intended to restore normal phosphate reabsorption from the kidney and increase the production of vitamin D, which enhances intestinal absorption of phosphate and calcium. Ultragenyx and Kyowa Hakko Kirin Co., Ltd. entered into a collaboration and license agreement in August 2013 to develop and commercialize KRN23.

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 http://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 timing of the initiation of a Phase 2 clinical study, timing of availability of interim data from the Phase 2 clinical study, the design of the study, and the number of patients who will participate in the 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 candidate. 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, 2014, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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