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Ultragenyx Announces Positive Results From a Long-Term Phase 1/2 Study of KRN23 in Adult Patients With X-Linked Hypophosphatemia

All Patients Continued to Demonstrate Increases in Serum Phosphorus and the Majority Maintained Levels in the Normal Range

NOVATO, Calif., Sept. 15, 2014 (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 presentation of results from a long-term Phase 1/2 extension study, conducted by Kyowa Hakko Kirin Pharma, Inc., of the investigational fully human anti-FGF23 monoclonal antibody KRN23 (UX023) in adult patients with X-linked hypophosphatemia (XLH). The cumulative 16-month data, combining the four-month dose escalation period data from INT-001 and the 12-month extension data from INT-002, were presented at the American Society of Bone and Mineral Research (ASBMR) Annual Meeting in Houston.

"By binding and inhibiting FGF23, patients treated with KRN23 demonstrated increases in phosphate levels over the cumulative 16-month treatment period," said Sunil Agarwal, M.D., Chief Medical Officer of Ultragenyx. "Based on these encouraging results, we plan to continue development in adult XLH patients and are enrolling pediatric XLH patients in our ongoing Phase 2 study."

The Phase 1/2 extension study (INT-002) was designed to evaluate long-term safety and efficacy following an initial four-month dose escalation study (INT-001) that was conducted in the US and Canada. During the extension study, 22 adult patients with XLH were evaluated over an additional 12 months. Patients received monthly subcutaneous injections of KRN23 administered at a dose range of 0.1 to 1.0 mg/kg. Data from the INT-001 study were previously presented at the ICE/ENDO joint meeting of The Endocrine Society and The International Congress of Endocrinology in June 2014.

Data from the INT-002 study demonstrated that the increases in serum phosphorus levels, urinary phosphorus reabsorption, and 1,25 dihydroxy vitamin D levels observed in the initial INT-001 study were generally sustained during the 12-month extension. All patients continued to demonstrate increases in serum phosphorus levels. Approximately 52.6%-85.7% of subjects in the extension study had serum phosphorus levels that reached the normal range (2.5 to 4.5 mg/dL) at peak time on Day 7 or Day 14 after each dose over this 12-month period.

The mean increases in markers of bone remodeling (procollagen type I N propeptide (P1NP) and osteocalcin) observed in INT-001 were also generally sustained.

KRN23 was generally safe and well tolerated over the cumulative treatment period. The most common treatment-related adverse events were injection site reaction, arthralgia, diarrhea, restless legs syndrome, injection site erythema, injection site pain, upper abdominal pain, headache, and decreased neutrophil count (the neutrophil changes were not associated with any significant infections). Serious adverse events were reported in three subjects but were all considered unrelated to KRN23. One patient discontinued treatment due to nephrolithiasis and one patient discontinued due to restless legs syndrome. There were no significant changes in parathyroid hormone or renal ultrasound. Serum calcium levels did not change significantly, and mild hypercalcemia was observed intermittently in two subjects. Urinary calcium was not increased, and three subjects had only transient hypercalciuria. No anti-KRN23 antibodies were observed.

Ultragenyx and Kyowa Hakko Kirin Co., Ltd. (Tokyo:4151) initiated a Phase 2 study of KRN23 in pediatric patients in the US and EU in June 2014 and expect to continue the clinical development of KRN23 in adults with XLH.

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 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. Studies suggest there are approximately 12,000 XLH patients in the United States. XLH is a distinctive bone 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, osteopenia, frequent/poorly healing microfractures, spinal

stenosis, enthesopathy, and osteoarthritis.

Most patients are managed using oral phosphate replacement and vitamin D (calcitriol) therapy, which requires frequent divided doses and careful medical monitoring. It is partially effective at reducing rickets, but it does not improve growth and can be challenging to optimize the dose without increasing the risk of depositing phosphate-calcium precipitates in the kidneys (nephrocalcinosis).

About KRN23 and FGF23

KRN23 is an investigational recombinant fully human monoclonal IgG_1 antibody, discovered by KHK, against the phosphaturic hormone fibroblast growth factor 23 (FGF23). It is being developed 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 restore normal phosphate reabsorption from the kidney and increase the production of vitamin D, which enhances intestinal absorption of phosphate and calcium. Ultragenyx and KHK 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 Co., Ltd. (KHK) is a leading biopharmaceutical company in Japan focusing on its core business area of oncology, nephrology, and immunology/allergy. KHK 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. Kyowa Hakko Kirin Pharma, Inc. is a subsidiary of KHK.

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 the plans for continued development of KRN23 in adult and pediatric patients, 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, that these results (which were in adult patients) may not translate into similar safety or efficacy in pediatric patients, 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 May 12, 2014, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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