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Ultragenyx Receives Breakthrough Therapy Designation for KRN23 in Pediatric Patients with X-Linked Hypophosphatemia

NOVATO, Calif., June 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 received Breakthrough Therapy Designation from the U.S. Food and Drug Administration (FDA) for KRN23 for the treatment of X-linked hypophosphatemia (XLH) in pediatric patients one year of age and older. Ultragenyx and Kyowa Hakko Kirin entered into a collaboration and license agreement in August 2013 to develop and commercialize KRN23.

"KRN23 is the first potential disease-specific treatment option in development for patients with XLH, a chronically debilitating and deformative bone disease that can impact a patient's daily functioning and quality of life starting from a very young age," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "We are encouraged by this Breakthrough Therapy Designation, and we look forward to working closely with the FDA to bring this potential new therapy to XLH patients as soon as possible."

This Breakthrough Therapy Designation is based on interim 40-week data from the first 36 patients enrolled in the ongoing pediatric Phase 2 study of KRN23 for the treatment of XLH. According to the FDA, Breakthrough Therapy Designation is intended to expedite the development and review of therapies for serious or life-threatening conditions and whose preliminary clinical evidence indicates that the drug may demonstrate substantial improvement on one or more clinically significant endpoints over existing therapies. Under the designation, the FDA provides intensive guidance, organizational commitment involving senior managers, and eligibility for rolling and priority review of the application; this process is intended to ensure that therapies for serious conditions are approved and available to patients as soon as it can be concluded that the therapies' benefits justify their risks.

In addition to the ongoing Phase 2 study, Ultragenyx plans to initiate a Phase 3 study of KRN23 in pediatric patients with XLH in mid-2016. A Phase 3 program of KRN23 in adult patients with XLH is ongoing.

About X-Linked Hypophosphatemia (XLH)

XLH is a life-long devastating 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 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, frequent/poorly healing pseudofractures, spinal stenosis, enthesopathy, and osteoarthritis.

There are no existing therapies that act directly on the phosphate wasting condition of XLH. 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 and fibroblast growth factor 23 (FGF23)

KRN23 is an investigational recombinant fully human monoclonal IgG1 antibody, discovered by Kyowa Hakko Kirin, against the phosphaturic hormone 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. KRN23 is intended to directly act on the phosphate wasting condition of XLH.

In addition to XLH, KRN23 is 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.

Ultragenyx Forward-Looking Statements
Except for the historical information contained herein, the matters set forth in this press release, including statements regarding the management and oversight of the Company's programs and the regulatory status of the Company's KRN23 program, 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 development programs, future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. Forward-looking statements include (but are not limited to) expectations around the potential benefits of receiving Breakthrough Therapy Designation. Further, the receipt of Breakthrough Therapy Designation is no indication of greater chances of success in later clinical studies or greater probabilities of receiving ultimate regulatory approval of 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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