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Ultragenyx Granted EU Orphan Drug Designation for KRN23 for the Treatment of X-Linked Hypophosphatemia

Novato, CA, Oct. 30, 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 that the European Commission has granted orphan medicinal product designation for recombinant human monoclonal IgG1 antibody for fibroblast growth factor 23 (KRN23 or UX023) for the treatment of X-linked hypophosphatemia (XLH). XLH is an inherited metabolic bone disease characterized by short stature, skeletal deformities, bone pain, fractures, and muscle weakness.

KRN23 is being developed under a license and collaboration agreement between Ultragenyx and Kyowa Hakko Kirin Co., Ltd. Multiple clinical studies in adult patients with XLH have been completed and a Phase 2 study in pediatric patients with XLH is ongoing. The U.S. Food and Drug Administration granted orphan drug designation for KRN23 in XLH in 2009.

"Pediatric patients with XLH can develop significant skeletal deformities and short stature and adult patients can experience many long-term problems including severe joint pain and stiffness, muscle weakness, fractures, and an overall decreased quality of life," commented Sunil Agarwal, M.D., Chief Medical Officer of Ultragenyx. "We believe that, by targeting the underlying mechanism of XLH, KRN23 can help both pediatric and adult patients with this disease."

The European Commission grants orphan drug status for medicinal products intended to treat diseases or conditions that affect fewer than five in 10,000 people in the European Union. The designation provides certain benefits and incentives in the EU, including protocol assistance, fee reductions, and ten years of market exclusivity once the medicine is on the market.

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. 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 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. It is partially effective at reducing rickets in pediatric patients, 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 IgG1 antibody, discovered by Kyowa Hakko Kirin, 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 Kyowa Hakko Kirin 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. Kyowa Hakko Kirin Pharma, Inc. is a subsidiary of Kyowa Hakko Kirin.

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 potential for KRN23 to help both pediatric and adult patients with XLH, 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 August 11, 2014, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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