May 19, 2014

Ultragenyx Announces KRN23 Phase 1/2 Study Data to be Presented at ICE/ENDO Meeting on June 23rd and June 24th

NOVATO, Calif., May 19, 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 upcoming presentation of three abstracts from a Phase 1/2 clinical study, conducted by Kyowa Hakko Kirin Pharma, Inc. (KKP), of four months of treatment with the human monoclonal anti-FGF23 antibody KRN23 (UX023) in adult patients with X-linked hypophosphatemia (XLH). XLH is an inherited metabolic bone disease characterized by short stature, skeletal deformities, bone pain, fractures, and muscle weakness.

Results from the Phase 1/2 dose-escalation study (INT-001) in 28 adult XLH patients will be presented at the 2014 ICE/ENDO joint meeting of The Endocrine Society and The International Congress of Endocrinology in Chicago. The abstracts being presented are available on the ICE/ENDO website.

The following abstract will be presented orally on June 24th at 9:30am CT.

E. Imel, X. Zhang, M. Ruppe, T. Weber, M. Klausner, T. Ito, M. Vergeire, J. Humphrey, F. Glorieux, A. Portale, K. Insogna, M. Peacock, T. Carpenter

The First Multi-Dose Trial of a Human Anti-FGF23 (Fibroblast Growth Factor 23) Antibody (KRN23) in Adults with X-Linked Hypophosphatemia (XLH)

The following two abstracts will be presented as posters on June 23rd between 1:00pm and 3:00pm CT.

M. Ruppe, X. Zhang, E. Imel, T. Weber, M. Klausner, T. Ito, M. Vergeire, J. Humphrey, F. Glorieux, A. Portale, K. Insogna, M. Peacock, T. Carpenter

Effect of Four Monthly Doses of a Human Monoclonal Anti-FGF23 (Fibroblast Growth Factor 23) Antibody (KRN23) on Quality of Life in X-Linked Hypophosphatemia (XLH)

X. Zhang, E. Imel, M. Ruppe, T. Weber, M. Klausner, T. Ito, M. Vergeire, J. Humphrey, F. Glorieux, A. Portale, K. Insogna, M. Peacock, T. Carpenter

Pharmacokinetics (PK) and Pharmacodynamics (PD) Following Four Monthly Doses of a Human Monoclonal Anti-FGF23 (Fibroblast Growth Factor 23) Antibody (KRN23) in Adults with X-Linked Hypophosphatemia (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 and osteoarthritis.

Most patients are managed using oral phosphate replacement and vitamin D (calcitriol) therapy, which is poorly tolerated and only partially effective at restoring bone physiology and growth. Current treatment with oral phosphate requires close monitoring and can result in complications such as secondary hyperparathyroidism, hypercalciuria, hypercalcemia and nephrocalcinosis. XLH was originally called vitamin D-resistant rickets because doses of vitamin D effective for the treatment of vitamin D-deficient nutritional rickets did not have an impact on phosphate levels in these patients.

About KRN23 and FGF23

KRN23 is a recombinant fully human monoclonal IgG_1 antibody against the phosphaturic hormone fibroblast growth factor 23 (FGF23) 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 Co., Ltd. (KHK) entered into a collaboration and license agreement in August 2013 to develop and commercialize KRN23.

About Ultragenyx

Ultragenyx is a development-stage biotechnology company committed to bringing to market novel products for the treatment of rare and ultra-rare diseases, with an initial 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 the timing of release of additional data, the severity of XLH, the number of patients in the United States who have XLH and the intended result of administration of KRN23, 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 May 12, 2014, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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