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Ultragenyx Provides Regulatory Update on Burosumab (KRN23)

BLA filing for Burosumab planned with current clinical data package in 2H 2017

NOVATO, Calif. , June 23, 2017 (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 reached agreement with the FDA at a Pre-Biologics License Application (pre-BLA) meeting on the clinical package to support the burosumab (KRN23) BLA filing for X-linked hypophosphatemia (XLH). At the meeting, the FDA agreed that the BLA can be submitted based on available clinical data and confirmed that both pediatric and adult indications would be included in the review. Based on the agreement, the submission of the burosumab BLA is planned for the second half of 2017.

"We are pleased to come to an agreement with the FDA on the content of the burosumab clinical data package for the BLA filing and look forward to working with the agency during the review process," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "We appreciate the FDA's collaboration in awarding the therapy BTD status and working on a prompt filing plan for the burosumab BLA."

Specifically for the pediatric XLH population, the FDA agreed that the 64 week data from Study CL201 in 5-12 year olds and 24 week data from Study CL205 in 1-4 year olds would be sufficient for review. The FDA confirmed that data from the pediatric Phase 3 study (CL301) would not be required for the BLA filing. To support the adult XLH indication, the FDA agreed that the review would be based on the totality of the data from the adult Phase 3 study CL303 including fracture healing data. FDA would also accept some bone biopsy data from the 48 week open label bone quality Phase 3 study in adults if available during the review as supportive evidence.

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 (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 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. Most pediatric patients and some adult patients are managed using oral phosphate replacement and vitamin D (calcitriol) therapy, which requires multiple divided doses each day and careful medical monitoring.

About Burosumab (KRN23)

Burosumab is an investigational recombinant fully human monoclonal IgG_1 antibody, discovered by Kyowa Hakko Kirin, against the phosphaturic hormone fibroblast growth factor 23 (FGF23). FGF23 is a hormone that reduces serum levels of phosphorus and active vitamin D by regulating phosphate excretion and active vitamin D production by the kidney. Burosumab is being developed by Ultragenyx and Kyowa Hakko Kirin to treat XLH and tumor-induced osteomalacia (TIO), diseases characterized by excess levels of FGF23. Phosphate wasting in XLH and TIO is caused by excessive levels and activity of FGF23. Burosumab is designed to bind to and thereby inhibit the biological activity of FGF23. By blocking excess FGF23 in patients with XLH and TIO, burosumab is intended to increase phosphate reabsorption from the kidney and increase the production of vitamin D, which enhances intestinal absorption of phosphate and calcium.

A clinical program studying burosumab in adults and pediatric patients with XLH is ongoing. Burosumab is also being developed for 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.

Forward-Looking Statements

Except for the historical information contained herein, the matters set forth in this press release, including statements regarding Ultragenyx's expectations regarding potential indications for its product candidates, discussions with the FDA and sufficiency for, and timing of, regulatory submissions, 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, such as the regulatory approval process, and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations. 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 5, 2017, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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