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Ultragenyx Announces Burosumab Data Presentations at ASBMR 2017 Annual Meeting

NOVATO, Calif., Aug. 24, 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 upcoming presentations of data highlighting burosumab for the treatment of X-linked hypophosphatemia (XLH) and tumor-induced osteomalacia (TIO) at the American Society for Bone and Mineral Research (ASBMR) 2017 Annual Meeting taking place September 8-11 in Denver, Colorado.

Kyowa Hakko Kirin, Kyowa Kirin International and Ultragenyx Pharmaceutical Inc. have been collaborating in development and commercialization of burosumab globally based on the collaboration and license agreement between Kyowa Hakko Kirin and Ultragenyx.

Two oral presentations will highlight a late breaking abstract on 24 week data from the adult Phase 3 study (n=134) and 64 week data from the pediatric Phase 2 study (n=52) in XLH patients

Oral Presentation #LB-1159: A Phase 3 Randomized, 24 Week, Double-Blind, Placebo-Controlled Study Evaluating the Efficacy of Burosumab, an Anti-FGF23 Antibody, in Adults with X-Linked Hypophosphatemia (XLH)

- Monday, September 11, 11:15 AM — 11:25 AM MDT
- Mile High Ballroom, Colorado Convention Center

Oral Presentation #1154: Burosumab (KRN23), a Fully Human Anti-FGF23 Monoclonal Antibody for X-linked Hypophosphatemia (XLH): Final 64-Week Results of a Randomized, Open-label, Phase 2 Study of 52 Children

- Monday, September 11, 09:45 AM - 10:00 AM MDT
- Mile High Ballroom, Colorado Convention Center

Three poster presentations will highlight additional burosumab data including functional patient reported outcomes from the pediatric Phase 2 study, Phase 2 study in Tumor Induced Osteomalacia (TIO), and the Phase 2 study in pediatric patients under 5 years old

Poster #FR0331 (shown twice): Effects of Burosumab (KRN23), a Fully Human Anti-FGF23 Monoclonal Antibody, on Functional Outcomes in Children with X-linked Hypophosphatemia (XLH): Final Results from a Randomized, 64-week, Open-label Phase 2 Study

- Friday, September 8, 05:00 PM — 07:00 PM MDT and Saturday, September 9, 12:30 PM — 02:30 PM MDT
- ASBMR Discovery Hall - Exhibit Hall A & B1, Colorado Convention Center

Poster #SU0325: Effects of Burosumab (KRN23), a Human Monoclonal Antibody to FGF23, in Patients with Tumor-Induced Osteomalacia (TIO) or Epidermal Nevus Syndrome (ENS)

- Sunday, September 10, 12:30 PM — 02:30 PM MDT
- ASBMR Discovery Hall - Exhibit Hall A, Colorado Convention Center

Poster #MO0695: The Effects of Burosumab (KRN23), a Fully Human Anti-FGF23 Monoclonal Antibody, on Phosphate Metabolism and Rickets in 1 to 4-Year-Old Children with X-linked Hypophosphatemia (XLH)

- Monday, September 11, 12:00 PM — 02:00 PM MDT
- ASBMR Discovery Hall - Exhibit Hall A & B1, Colorado Convention Center

About Burosumab

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 and planned presentations regarding ongoing or additional studies for its product candidates, 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, the timing of our regulatory filings and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations and 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 July 28, 2017, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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