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Ultragenyx and Kyowa Hakko Kirin Announce FDA Acceptance and Priority Review Designation of Burosumab's Biologics License Application

Novato, CA, Tokyo, Japan and London, UK — October 10, 2017 — Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, Kyowa Hakko Kirin Co., Ltd. (Kyowa Hakko Kirin) and Kyowa Kirin International PLC (Kyowa Kirin International) today announce that the U.S. Food and Drug Administration (FDA) has accepted the Biologics License Application (BLA) for burosumab to treat pediatric and adult patients with X-Linked Hypophosphatemia (XLH) and has granted Priority Review status. Burosumab previously received Breakthrough Therapy Designation from the FDA for the treatment of XLH in pediatric patients one year of age and older.

The FDA has granted burosumab Priority Review status, which is available to drugs that treat a serious condition and, if approved, would provide a significant improvement in safety or effectiveness. The Prescription Drug User Fee Act (PDUFA) action date for the BLA is April 17, 2018. The Agency has not informed the companies whether an advisory committee meeting to discuss the application will be needed. The FDA previously designated burosumab as a drug for a "rare pediatric disease", enabling issuance of a priority review voucher if burosumab is approved.

"XLH is a debilitating disease and there are no current treatment options that address the underlying cause," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "We are pleased that the FDA has granted priority review and are looking forward to working with the agency in the coming months with the goal of bringing this potential new treatment to patients as quickly as possible."

"Burosumab is the culmination of Kyowa Hakko Kirin's research activity in diseases in which high levels of FGF 23 are a causative factor," said Mitsuo Satoh, Executive Officer, Vice President, Head of Research and Development Division of Kyowa Hakko Kirin. "I believe burosumab has the potential to be an effective treatment option for patients with conditions with excess FGF23 such as XLH and tumor induced osteomalacia and we will keep working to provide this advance in therapy for patients as soon as possible."

Dr. Tom Stratford, President and Chief Executive of Kyowa Kirin International, said "Burosumab has the potential to treat both pediatric and adult patients with XLH and we welcome the fact that the FDA has granted burosumab priority review status, meaning that sufferers may be able to have access to this treatment earlier."

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

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 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.

About Kyowa Kirin

Kyowa Hakko Kirin Co., Ltd. is a research-based life sciences company, with special strengths in biotechnologies. In the core therapeutic areas of oncology, nephrology and immunology/allergy, Kyowa Hakko Kirin leverages leading-edge biotechnologies centered on antibody technologies, to continually discover innovative new drugs and to develop and market those drugs world-wide. In this way, the company is working to realize its vision of becoming a Japan-based global specialty pharmaceutical company that contributes to the health and wellbeing of people around the world.

Kyowa Kirin International PLC is a wholly owned subsidiary of Kyowa Hakko Kirin and is a rapidly growing specialty pharmaceutical company engaged in the development and commercialization of prescription medicines for the treatment of unmet therapeutic needs in Europe and the United States. Kyowa Kirin International is headquartered in Scotland.

You can learn more about the business at: 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 Ultragenyx's plans or expectations regarding future regulatory interactions and the potential timing and success of filings for regulatory approvals, 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.