Ultragenyx and Kyowa Kirin International Announce Marketing Authorisation Application for KRN23 Filed and Accepted for Review by European Medicines Agency

Novato, CA and London, UK — January 5, 2017 — Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE) and Kyowa Kirin International PLC (KKI), a wholly owned subsidiary of Kyowa Hakko Kirin Co., Ltd. (Kyowa Hakko Kirin), today announced that the European Medicines Agency (EMA) has accepted for review the Marketing Authorisation Application (MAA) for KRN23 for the treatment of X-linked hypophosphatemia (XLH). The MAA was filed and accepted in late 2016, and an opinion from the Committee for Medicinal Products for Human Use (CHMP) is expected in the second half of 2017. Ultragenyx and Kyowa Hakko Kirin entered into a collaboration and licence agreement in August 2013 to develop and commercialise KRN23.

"Based on the positive results from multiple studies in paediatric and adult patients with XLH, we are pursuing conditional marketing authorisation in order to accelerate access to this therapy for patients," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx.

"X-linked hypophosphatemia is a debilitating condition which causes long term pain and distress among sufferers and for which there are no currently approved treatments that target the underlying cause," said Dr. Tom Stratford, President and CEO of KKI. "As part of Kyowa Hakko Kirin, we strive to contribute to the health and wellbeing of people around the world through advances in life sciences and technologies. The acceptance of this filing brings us one step closer to addressing the unmet medical needs of patients who suffer from X-linked hypophosphatemia."

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. XLH is a distinctive disease characterised by inadequate mineralisation 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 paediatric patients and some adult patients are managed using oral phosphate replacement and active vitamin D (calcitriol) therapy, which requires multiple divided doses each day and monitoring for potential risks such as nephrocalcinosis, hypercalciuria, and hyperparathyroidism.

About KRN23

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 by Ultragenyx and Kyowa Hakko Kirin to treat XLH

and TIO, diseases characterised by excess activity of 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. Phosphate wasting in XLH and TIO 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 and TIO, KRN23 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 Phase 3 programme studying KRN23 in adults and Phase 2 and Phase 3 studies in paediatric patients with XLH are ongoing. KRN23 is also being developed for tumour-induced osteomalacia (TIO), a disease characterised by typically benign tumours that produce excess levels of FGF23, which can lead to severe osteomalacia, fractures, bone and muscle pain, and muscle weakness.

Ultragenyx and Kyowa Hakko Kirin plan to submit a biologics licence application (BLA) to the U.S. FDA for KRN23 in the second half of 2017. The companies continue to discuss the details of the planned submission with FDA.

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

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 centred

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 realise its vision of becoming a Japan-based global specialty pharmaceutical company that contributes to the health and wellbeing of people around the world.

You can learn more about the business at: www.kyowa-kirin.com.

Ultragenyx 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 the timing of release of additional data for its product candidates, plans to initiate additional studies for its product candidates and timing regarding these studies, plans regarding ongoing studies for existing programmes, plans to make regulatory submissions and the timing for those submissions and the expected timing for an opinion from the CHMP, 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 programmes, 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 success of our drug development programmes, including KRN23. 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 November 8, 2016, and its subsequent periodic reports filed with the Securities and Exchange Commission.