



Ultragenyx Announces Sale of a Portion of Future North American Royalties on Crysvida® (burosumab) for \$500 Million to OMERS Capital Markets

July 14, 2022

Strengthens balance sheet with non-dilutive capital at an attractive cost

Competitive process results in the sale of 30% of the Ultragenyx royalty interest, subject to a 1.45x cap

Proceeds to fund planned clinical and commercial activities

NOVATO, Calif., July 14, 2022 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development and commercialization of novel products for serious rare and ultra-rare genetic diseases, today announced the sale of 30% of the company's royalty interest from Kyowa Kirin Co., Ltd on the future sales of Crysvida® (burosumab) in the United States (U.S.) and Canada to OMERS, one of Canada's largest defined benefit pension plans, for \$500 million. OMERS' right to receive royalty payments is based on net sales of the product beginning in April 2023 and total payments are capped at 1.45 times the purchase price.

"In North America, Crysvida has generated more than \$1.3 billion net sales in the first four years on the market making it one of the most successful launches in the rare disease field. This non-dilutive financing bolsters Ultragenyx's balance sheet, funds the ongoing commercialization of multiple approved medicines and the advancement of our diverse clinical pipeline," said Mardi Dier, Chief Financial Officer of Ultragenyx. "We are pleased to partner with OMERS on this transaction who, like us, recognizes the ongoing and future value of Crysvida, the tremendous success of the launch and the significance for patients who will continue to benefit from this important medicine."

"Ultragenyx has successfully launched Crysvida, meaningfully improving the lives of thousands of pediatric and adult patients with two rare diseases. We are proud to invest in a product that has made a difference for so many and that aligns with the investment strategy at OMERS," said Rob Missere, Managing Director and Head of Life Sciences, OMERS Capital Markets. "This deal furthers our mandate of delivering steady, long-term returns to our more than 541,000 members."

Cowen acted as exclusive financial advisor to Ultragenyx on the transaction. Gibson Dunn LLP acted as legal advisor to Ultragenyx. Davies Ward Phillips & Vineberg LLP and Latham & Watkins LLP acted as legal advisors to OMERS.

About Crysvida

Crysvida (burosumab) is a recombinant fully human monoclonal IgG1 antibody, discovered by Kyowa Kirin, against the phosphaturic hormone 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 TIO and other hypophosphatemic conditions, including XLH, is caused by excessive levels and activity of FGF23. Crysvida is designed to bind to and thereby inhibit the biological activity of FGF23. By blocking excess FGF23 in patients with TIO and XLH, Crysvida is intended to increase phosphate reabsorption from the kidney and increase the production of vitamin D, which enhances intestinal absorption of phosphate and calcium.

In North America, Crysvida is approved by the U.S. Food and Drug Administration (FDA) and by Health Canada for the treatment of X-linked hypophosphatemia (XLH) and FGF23-related hypophosphatemia in tumor induced osteomalacia (TIO).

Kyowa Kirin and Ultragenyx have been collaborating in the development and commercialization of Crysvida globally based on the collaboration and license agreement between the parties.

U.S. INDICATION

Crysvida® (burosumab-twza) is a fibroblast growth factor 23 (FGF23)-blocking antibody indicated for the treatment of:

- X-linked hypophosphatemia (XLH) in adult and pediatric patients 6 months of age and older.
- FGF23-related hypophosphatemia in tumor-induced osteomalacia (TIO) associated with phosphaturic mesenchymal tumors that cannot be curatively resected or localized in adult and pediatric patients 2 years of age and older.

IMPORTANT SAFETY INFORMATION

CONTRAINDICATIONS

- With oral phosphate and/or active vitamin D analogs (e.g., calcitriol, paricalcitol, doxercalciferol, calcifediol).
- When serum phosphorus is within or above the normal range for age.
- In patients with severe renal impairment or end stage renal disease.

WARNINGS AND PRECAUTIONS

Hypersensitivity

- Discontinue Crysvida if serious hypersensitivity reactions occur and initiate appropriate medical treatment.

Hyperphosphatemia and Risk of Nephrocalcinosis

- For patients already taking Crysvida, dose interruption and/or dose reduction may be required based on a patient's serum phosphorus levels.
- Patients with TIO who undergo treatment of the underlying tumor should have dosing interrupted and adjusted to prevent hyperphosphatemia.

Injection Site Reactions

- Discontinue Crysvida if severe injection site reactions occur and administer appropriate medical treatment.

ADVERSE REACTIONS

Pediatric XLH Patients

- Adverse reactions reported in 10% or more of Crysvida-treated pediatric XLH patients across all studies are: pyrexia, injection site reaction, cough, vomiting, pain in extremity, headache, tooth abscess, dental caries, diarrhea, vitamin D decreased, toothache, constipation, myalgia, rash, dizziness, and nausea.
- Post-marketing experience reported in pediatric XLH patients receiving Crysvida – blood phosphorus increased.

Adult XLH Patients

- Adverse reactions reported in more than 5% of Crysvida-treated adult XLH patients and in at least 2 patients more than placebo in one study are: back pain, headache, tooth infection, restless legs syndrome, vitamin D decreased, dizziness, constipation, muscle spasms, and blood phosphorus increased.
- Spinal stenosis is prevalent in adults with XLH, and spinal cord compression has been reported. It is unknown if Crysvida therapy exacerbates spinal stenosis or spinal cord compression.

Adult TIO Patients

- Adverse reactions reported in more than 10% of Crysvida-treated adult TIO patients in two studies are: tooth abscess, muscle spasms, dizziness, constipation, injection site reaction, rash, and headache.

USE IN SPECIFIC POPULATIONS

- There are no available data on Crysvida use in pregnant women to inform a drug-associated risk of adverse developmental outcomes. Serum phosphorus levels should be monitored throughout pregnancy. Report pregnancies to the Kyowa Kirin, Inc. Adverse Event reporting line at 1-888-756-8657.
- There is no information regarding the presence of Crysvida in human milk or the effects of Crysvida on milk production or the breastfed infant.

Report side effects to the FDA at www.fda.gov/medwatch. You may also report side effects to Kyowa Kirin, Inc. at 1-888-756-8657.

Please see full [Prescribing Information](#) for a complete discussion of the risks associated with Crysvida.

About Ultragenyx

Ultragenyx is a biopharmaceutical company committed to bringing novel therapies to patients for the treatment of serious rare and ultra-rare genetic diseases. The company has built a diverse portfolio of approved medicines and treatment candidates aimed at addressing diseases with high unmet medical need and clear biology, for which there are typically no approved therapies treating the underlying disease.

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 OMERS Capital Markets and OMERS

OMERS Capital Markets is the capital markets investment division of OMERS, one of Canada's largest defined benefit pension plans. Capital Markets' diverse programs, flexible investment strategies and specialized teams, including across public equities, private credit, and structured investments, enable it to pursue opportunities that don't fit into traditional categories. OMERS Life Sciences provides royalty financings and other non-dilutive solutions to biopharma companies and academic institutions, supporting their efforts to address unmet medical needs and improve the quality of life of patients around the world.

Founded in 1962, OMERS manages \$121 billion in net assets as of December 31, 2021. OMERS is a jointly-sponsored pension plan, with 1,000 participating employers ranging from large cities to local agencies, and over half a million active, deferred, and retired members. OMERS members include union and non-union employees of municipalities, school boards, local boards, transit systems, electrical utilities, emergency services and children's aid societies across Ontario. Contributions to the Plan are funded equally by members and employers. OMERS teams work in Toronto, London, New York, Amsterdam, Luxembourg, Singapore, Sydney and other major cities across North America and Europe – serving members and employers and originating and managing a diversified portfolio of high-quality investments in public markets, private equity, infrastructure, and real estate.

Forward-Looking Statements and Use of Digital Media

Except for the historical information contained herein, the matters set forth in this press release, including statements related to Ultragenyx's expectations and projections regarding its future operating results and financial performance, anticipated cost or expense reductions, the timing, progress and plans for its clinical programs and clinical studies, future regulatory interactions, and the components 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, collaboration with third parties, 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 effects from the COVID-19 pandemic on the company's clinical and commercial activities and business and operating results, risks related to reliance on third party partners to conduct certain activities on the company's behalf, including under our collaboration agreement with Kyowa Kirin, our limited experience in generating revenue from product sales, risks related to product liability

lawsuits, our dependence on Kyowa Kirin for the commercial supply of Crysvita, fluctuations in buying or distribution patterns from distributors and specialty pharmacies, the transition back to Kyowa Kirin of our exclusive rights to promote Crysvita in the United States and Canada and unexpected costs, delays, difficulties or adverse impact to revenue related to such transition, smaller than anticipated market opportunities for the company's products and product candidates, manufacturing risks, competition from other therapies or products, and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations, the company's future operating results and financial performance, the timing of clinical trial activities and reporting results from same, and the availability or commercial potential of Ultragenyx's products and 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 Ultragenyx in general, see Ultragenyx's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on May 6, 2022, and its subsequent periodic reports filed with the SEC.

In addition to its SEC filings, press releases and public conference calls, Ultragenyx uses its investor relations website and social media outlets to publish important information about the company, including information that may be deemed material to investors, and to comply with its disclosure obligations under Regulation FD. Financial and other information about Ultragenyx is routinely posted and is accessible on Ultragenyx's Investor Relations website (<https://ir.ultragenyx.com/>) and LinkedIn website (<https://www.linkedin.com/company/ultragenyx-pharmaceutical-inc-/mycompany/>).

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