Ultragenyx Announces Sale of Future European Royalties on Crysvita® (burosumab) for $320 Million to Royalty Pharma

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NOVATO, Calif. and NEW YORK, Dec. 18, 2019 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, and Royalty Pharma today announced that Ultragenyx has sold to Royalty Pharma for $320 million a royalty right due to Ultragenyx from Kyowa Kirin Co., Ltd for the net sales of Crysvita® (burosumab) in the European Union (EU), the United Kingdom (UK), and Switzerland. Crysvita has received conditional marketing authorization in Europe for the treatment of X-linked hypophosphatemia (XLH) with radiographic evidence of bone disease in children 1 year of age and older and adolescents with growing skeletons, and an application for the expanded use in adults with XLH is currently under review by the European Medicines Agency.

The agreement will automatically expire in the event aggregate royalty payments received by Royalty Pharma are equal to or greater than 1.9 times the purchase price prior to December 31, 2030, or in the event aggregate royalty payments are equal to or greater than 2.5 times the purchase price if the prior threshold is not met by the end of 2030. Once the threshold amount has been met, the EU, UK, and Switzerland royalty payments will revert back to Ultragenyx.

“This non-dilutive royalty financing provides Ultragenyx with capital to support our strong launches of Crysvita in North America and Latin America and Mepsevii worldwide, as well as the advancement of our clinical and translational research programs,” said Shalini Sharp, Chief Financial Officer of Ultragenyx. “Crysvita is the first treatment option for XLH that targets the underlying cause of the disease and we are pleased that Royalty Pharma, a recognized leader in providing innovative capital in life sciences, has invested in the future of this therapy.”

Royalty Pharma’s CEO, Pablo Legorreta, stated, “We are pleased to have the opportunity to work with Ultragenyx on this win-win transaction, in which Ultragenyx was able to pull forward capital from a passive, EU royalty stream to power its current strategic plan. Royalty Pharma adds a high quality asset that is an excellent addition to our diversified portfolio of leading biopharmaceutical royalties.”

Perella Weinberg Partners LP and J. Wood Capital Advisors LLC acted as financial advisors and Gibson, Dunn & Crutcher LLP acted as legal advisor to Ultragenyx on the transaction. Goodwin Procter LLP and Maiwald Patentanwalts GmbH acted as legal advisors to Royalty Pharma.

About Crysvita
Crysvita (burosumab-twza) 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. Crysvita is designed to bind to and thereby inhibit the biological activity of FGF23. By blocking excess FGF23 in patients with TIO and XLH, Crysvita is intended to increase phosphate reabsorption from the kidney and increase the production of vitamin D, which enhances intestinal absorption of phosphate and calcium.

Crysvita is approved by the U.S. Food and Drug Administration (FDA) for the treatment of X-linked hypophosphatemia (XLH) in adult and pediatric patients six months of age and older, and by Health Canada and Brazil’s National Health Surveillance Agency (ANVISA) for the treatment of XLH in adult and pediatric patients one year of age and older. It is approved by Japan’s Ministry of Health, Labor and Welfare (MHLW) for the treatment of FGF23-related hypophosphatemic rickets and osteomalacia. The medicine has received European conditional marketing authorization for the treatment of XLH with radiographic evidence of bone disease in children 1 year of age and older and adolescents with growing skeletons, and an application for the expanded use in adults with XLH is currently under review by the European Medicines Agency.

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

INDICATION *(IN THE U.S.)*
Crysvita is indicated for the treatment of X-linked hypophosphatemia (XLH) in adult and pediatric patients 6 months of age and older.

IMPORTANT SAFETY INFORMATION
Crysvita should not be taken if:

- An oral phosphate supplement and/or a specific form of vitamin D supplement are taken (such as calcitriol, paricalcitol, doxercalciferol, calcifediol).
- Phosphorus levels from a blood sample are within or above the normal range for age.
- Kidney problems are present.

What is the most important information to know about Crysvita?

- Some patients developed allergic reactions (rash and hives) while taking Crysvita. Doctors will monitor for symptoms of an allergic reaction while Crysvita is taken.
- High levels of phosphorus in the blood have been reported in some patients taking Crysvita. This may be related to a risk of high calcium levels in the kidneys. Doctors will collect samples to monitor levels.
- Administration of Crysvita may result in reactions at the injection site, such as hives, reddening of the skin, rash, swelling, bruising, pain,

severe itching of the skin, and collection of blood outside of a blood vessel (hematoma).

What are the possible side effects of Crysvita?

- The most common adverse reactions that were seen in children with XLH are:
  • Fever
  • Injection site reaction
  • Cough
  • Vomiting
  • Pain in arms and legs
  • Headache
  • Tooth infection
  • Dental cavities
  • Diarrhea
  • Decreased vitamin D levels
  • Toothache
  • Constipation
  • Muscle pain
  • Rash
  • Dizziness
  • Nausea

- The most common adverse reactions that were seen in adults with XLH are:
  • Back pain
  • Headache
  • Tooth infection
  • Restless leg syndrome
  • Decreased vitamin D levels
  • Dizziness
  • Muscle spasms
  • Constipation
  • Phosphorus levels increased in the blood

- Narrowing of the spaces within the spine is common in adults with XLH and pressure on the spinal cord has been reported in adults taking Crysvita. It is not known if taking Crysvita worsens the narrowing of the spaces within the spine or the pressure on the spinal cord.

Before taking Crysvita, doctors should be informed about all medications (including supplements) and medical conditions, including if:

- One is taking oral phosphate and/or active vitamin D (such as calcitriol, paricalcitol, doxercalciferol, calcifediol).

- One is pregnant, thinks she may be pregnant, or plans to become pregnant. There is not enough experience to know if Crysvita may harm an unborn baby. Report pregnancies to the Kyowa Kirin, Inc. Adverse Event reporting line at 1-888-756-8657.

- One is breastfeeding or plans to breastfeed. There is not enough experience to know if Crysvita passes into breast milk. Women should talk with their doctors about the best way to feed their babies while taking Crysvita.

While taking Crysvita, doctors should be informed if one experiences:

- An allergic reaction such as rash or hives

- A rash, swelling, bruising or other reaction at the injection site

- New or worsening restless leg syndrome

These are not all the possible side effects of Crysvita. Doctors should be contacted for medical advice about side effects.

Side effects may be reported to the FDA at (800) FDA-1088 or www.fda.gov/medwatch. Side effects may also be reported to Kyowa Kirin, Inc. at 1-888-756-8657.

Please see full Prescribing Information for additional Important Safety Information.

About Ultragenyx Pharmaceutical Inc.
Ultragenyx is a biopharmaceutical company committed to bringing to patients novel products for the treatment of serious rare and ultra-rare genetic diseases. The company has built a diverse portfolio of approved therapies and product candidates aimed at addressing diseases with high unmet medical need and clear biology for treatment, 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 Royalty Pharma
Founded in 1996, Royalty Pharma is the industry leader in acquiring pharmaceutical royalties, with over $16 billion in royalty assets. Royalty Pharma funds innovation in life sciences both directly and indirectly: directly when it partners with life sciences companies to co-develop and co-fund products in late-stage clinical trials, and indirectly when it acquires existing royalty interests from the original innovators (academic institutions, research

hospitals, foundations and inventors). The company's portfolio includes royalty interests in over 50 approved products including AbbVie and J&J's Imbruvica, Astellas and Pfizer's Xtandi, Biogen's Tysabri, Gilead's HIV franchise, Merck's Januvia, Novartis' Promacta, and Vertex's Kalydeco, Symdeko and Trikafta. Royalty Pharma is also a leading investor in pre-approval royalties, having since 2011 invested over $5.6 billion in royalties on pre-approval products and committed over $1.2 billion to direct R&D funding in exchange for royalties. For more information, visit www.royaltypharma.com

Forward-Looking Statements
Except for the historical information contained herein, the matters set forth in this press release, including statements related to Ultragenyx's expectations regarding plans for its clinical programs and future regulatory interactions, 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, including our collaboration with Kyowa Kirin, 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 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 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 on November 6, 2019, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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