UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d) of the
Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): January 13, 2020

ULTRAGENYX PHARMACEUTICAL INC.

(Exact name of registrant as specified in charter)

Delaware	**001-36276**	**27-2546083**
(State or other jurisdiction of incorporation)	(Commission File Number)	(IRS Employer Identification No.)

60 Leveroni Court, Novato, California	**94949**
(Address of principal executive offices)	(Zip Code)

Registrant's telephone number, including area code: (415) 483-8800

Not Applicable
(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol	Name of each exchange on which registered
Common Stock, $0.001 par value	**RARE**	**The Nasdaq Global Select Market**

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 2.02 Results of Operations and Financial Condition.

On January 13, 2020, Ultragenyx Pharmaceutical Inc. (the "***Company***") issued a press release (the "***Press Release***") announcing preliminary unaudited fiscal year 2019 revenue, cash and investments at 2019 fiscal year end and 2020 revenue guidance for Crysvita® in the Company's territories. The Company expects to report its full year results for the 2019 fiscal year in February 2020. A copy of the Press Release is furnished herewith as Exhibit 99.1.

The information set forth under Item 2.02 and in Exhibit 99.1 shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, except as shall be expressly set forth by specific reference in such filing.

Item 7.01 Regulation FD Disclosure

On January 13, 2020, the Company posted a presentation (the "***Presentation***") to its website at www.ultragenyx.com in the "*Events and Presentations*" subsection of the "Investors" tab. The Company is scheduled to present the Presentation at the 38th Annual J.P. Morgan Healthcare Conference on January 14, 2020.

The information set forth under Item 7.01 and in the Presentation shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, except as shall be expressly set forth by specific reference in any such filing.

Item 8.01 Other Events.

On January 13, 2020, the Company issued a joint press release (the "***Joint Press Release***") with Kyowa Kirin Co., Ltd. announcing that they have submitted a supplemental Biologics License Application (sBLA) to the U.S. Food and Drug Administration for Crysvita® (burosumab) for the treatment of FGF23-related hypophosphatemia associated with phosphaturic mesenchymal tumors (tumor-induced osteomalacia; TIO) that cannot be curatively resected or localized. A copy of the Joint Press Release is filed as Exhibit 99.2.

Item 9.01 Financial Statements and Exhibits

(d) **Exhibits**

Exhibit No.	**Description**
99.1	Press Release, dated January 13, 2020.
99.2	Joint Press Release, dated January 13, 2020.
104	The cover page from the Company's Current Report on Form 8-K dated January 13, 2020 formatted in Inline XBRL.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Date: January 13, 2020

Ultragenyx Pharmaceutical Inc.

By: /s/ Shalini Sharp
Shalini Sharp
Executive Vice President, Chief Financial Officer

Exhibit 99.1

Contact Ultragenyx Pharmaceutical Inc.
Investors & Media
Danielle Keatley
415-475-6876

Ultragenyx Reports Preliminary 2019 Revenue and Provides 2020 Crysvita Revenue Guidance

Preliminary 2019 total revenue is approximately $102 million to $104 million

Preliminary 2019 Crysvita revenue to Ultragenyx of approximately $86 million to $88 million

2020 Crysvita revenue in Ultragenyx territories is expected to be in the range of $125 million to $140 million excluding EU royalty revenue

Year-end 2019 cash balance of greater than $750 million

NOVATO, Calif. – January 13, 2020 – Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development of novel products for serious rare and ultra-rare genetic diseases, today reported preliminary unaudited 2019 revenue and cash and investments at year end 2019, and provided 2020 revenue guidance for Crysvita in Ultragenyx territories.

“We passed the $100 million revenue threshold in 2019 based on the strong launches of Crysvita and Mepsevii. We aim to build on this success in 2020 with the potential approvals of UX007 in LC-FAOD and Crysvita in TIO,” said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. “This growing commercial foundation will continue to enable significant pipeline advancement as we execute on our gene therapy clinical studies and initiate multiple new clinical programs.”

2019 Preliminary Revenue, 2020 Crysvita Revenue Guidance, and 2019 Ending Cash Position

2019 Preliminary Revenue (unaudited)

Total revenue for the year ended December 31, 2019 is approximately $102 million to $104 million. Total Crysvita revenue to Ultragenyx is approximately $86 million to $88 million in 2019, which includes collaboration revenue in the North American profit share territory, royalty revenue in the European territory from Kyowa Kirin Co. Ltd., and product revenue for Crysvita in other regions.

2020 Crysvita Guidance

For 2020, Crysvita revenue in the Ultragenyx territories is estimated to be between $125 million and $140 million. Ultragenyx territories include the North American profit share territory and other regions where collaboration revenue and product sales are recognized by

Ultragenyx. The 2020 Crysvita revenue guidance excludes the European territory royalty revenue, the rights to which were sold to Royalty Pharma. Beginning January 1, 2020, the company will no longer receive cash payments from the EU territory royalty until the respective threshold amount is met; however, the company will continue to record the royalty as “non-cash” revenue.

The company is not providing total 2020 revenue guidance at this time. The preliminary revenue results are based on management's initial analysis of operations for the quarter and year ended December 31, 2019. The 2019 revenue and cash position included in this release are preliminary and prior to the completion of review and audit procedures by Ultragenyx’s external auditors, and are therefore subject to adjustment. The Company expects to issue full financial results for the fourth quarter and fiscal year 2019 in February 2020.

2019 Ending Cash Position (unaudited) and 2020 Expected Net Cash Burn Rate

Cash, cash equivalents, and available-for-sale investments were greater than $750 million as of December 31, 2019, including proceeds of $320 million received from the sale of the company’s royalty interest in Crysvita in the European territory. The company also expects a more than 20 percent reduction in net cash burn (net cash used in operations plus capital expenditures) in 2020 compared to 2019.

Recent Updates and Upcoming Milestones

Crysvita for X-linked Hypophosphatemia: Non-dilutive $320 million royalty sale of future European royalties

- In December, Ultragenyx sold to Royalty Pharma for $320 million its royalty interest in Crysvita in the European territory, where it is being commercialized by Kyowa Kirin Co., Ltd.

Crysvita for Tumor-Induced Osteomalacia (TIO): Supplemental Biologics License Application (sBLA) submitted

- Ultragenyx submitted the sBLA to the U.S. FDA on December 18, 2019 and expects to hear back from FDA on submission acceptance and review designation in February 2020.

UX007 for Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD): NDA under review by U.S. FDA

- The U.S. FDA accepted for review the New Drug Application (NDA) and has set a Prescription Drug User Fee Act (PDUFA) date of July 31, 2020. The FDA has indicated that it is not currently planning to hold an advisory committee meeting to discuss the application.

DTX301 for Ornithine Transcarbamylase (OTC) Deficiency: Positive data from higher cohort of Phase 1/2 study; data from prophylactic steroid cohort in second half 2020

- Recent positive data from Cohort 3 indicated two confirmed responders and a potential third responder out of three total patients, as well as a new responder in Cohort 2. There are currently up to six responders of the nine dosed to date with a more consistent response at higher doses.

- Ultragenyx is initiating a fourth cohort (n=3) using prophylactic steroids at the same dose as Cohort 3. The first patient is expected to be enrolled in the first half of 2020, and data from the prophylactic steroid cohort are expected in the second half of 2020.

DTX401 for Glycogen Storage Disease Type Ia (GSDIa): Positive data from Phase 1/2 study; enrollment complete in confirmatory cohort and data expected in first half 2020

- Enrollment is complete in the confirmatory cohort of three patients at the second dose cohort, with data expected in the first half of 2020. Following the results of the confirmatory cohort, a Phase 3 study could begin in the second half of 2020.

GTX-102 for Angelman Syndrome: Partnered program with GeneTx; IND expected in first half 2020

- An investigational new drug (IND) application is expected in the first half of 2020. In August 2019, Ultragenyx and GeneTx announced a partnership to develop GTX-102 with Ultragenyx receiving an exclusive option to acquire GeneTx.

UX701 for Wilson Disease: IND expected in second half 2020

- An IND application is expected in the second half of 2020 for a new gene therapy for Wilson disease, a larger rare metabolic disease. UX701 will be the company's second clinical program to utilize its HeLa manufacturing system. The Hemophilia A program partnered with Bayer uses the HeLa system and has released its first clinical data.

Ultragenyx to Present at 38th Annual J.P. Morgan Healthcare Conference

- Dr. Kakkis will present on Tuesday, January 14, 2020 at 12:00 p.m. PT in San Francisco. The live and archived webcast of the presentation will be accessible from the company's website at http://ir.ultragenyx.com/events.cfm

About Ultragenyx

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.

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 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, Ultragenyx's reliance on its third party partner, Kyowa Kirin Co. ,Ltd., for the supply of Crysvita, smaller than anticipated market opportunities for the company's products and product candidates, the company's evolving commercial infrastructure, uncertainties related to insurance coverage and reimbursement approval for the company's products, manufacturing risks, the uncertainties inherent in the clinical drug development process, including the potential for substantial delays and the risk that earlier study results may not be predictive of future study results, the lack of predictability in the regulatory approval process, the timing of regulatory filings and approvals (including whether such approvals can be obtained), 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 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 on November 6, 2019, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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Exhibit 99.2

Ultragenyx and Kyowa Kirin Announce Submission of Supplemental Biologics License Application to U.S. FDA for Crysvita® (burosumab) for Tumor-Induced Osteomalacia (TIO)

Novato, Calif. and Tokyo, January 13, 2020 — Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, and Kyowa Kirin Co., Ltd., (Kyowa Kirin, TYO: 4151) today announced that they submitted a supplemental Biologics License Application (sBLA) to the U.S. Food and Drug Administration (FDA) on December 18, 2019, for Crysvita® (burosumab) for the treatment of FGF23-related hypophosphatemia associated with phosphaturic mesenchymal tumors (tumor-induced osteomalacia; TIO) that cannot be curatively resected or localized. The companies expect to hear back from FDA on submission acceptance and review designation in February 2020.

“Approximately half of patients with TIO have tumors that cannot be surgically removed, leaving them with no other current treatment options,” said Camille L. Bedrosian, M.D., Chief Medical Officer of Ultragenyx. “We look forward to continuing to work closely with the FDA during the review process, with the goal of bringing Crysvita to patients with TIO in the U.S.”

The sBLA package includes data from two single-arm Phase 2 studies, a 144-week study in 14 adult patients conducted by Ultragenyx in the U.S. and an 88-week study in 13 adult patients conducted by Kyowa Kirin in Japan and South Korea. In both studies, Crysvita was associated with increases in serum phosphorus and serum 1,25-dihydroxyvitamin D levels. Increased phosphate levels led to improvements in osteomalacia, mobility and vitality. Bone scans also demonstrated an increase in healed fractures and a decrease in new fractures during Crysvita treatment. During the studies, adverse events generally reflected the patients’ underlying disease, and there were no serious treatment-related adverse events.

Crysvita is approved by the 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.

See below for Important Safety Information for Crysvita in X-linked hypophosphatemia.

About Tumor-Induced Osteomalacia (TIO)
TIO is caused by typically benign tumors that produce excess levels of fibroblast growth factor 23 (FGF23), causing phosphate wasting in the urine that leads to severe hypophosphatemia, osteomalacia, muscle weakness, fatigue, bone pain and fractures. The symptoms rapidly resolve if the causal tumors or lesion can be resected; however, there are cases in which resection is not feasible or recurrence of the tumor occurs after resection. In patients for whom the tumor or lesion is inoperable, the current treatment consists of oral phosphate and/or vitamin D replacement. Efficacy of this management is often limited, as it does not treat the underlying disease and its benefits must be balanced with monitoring for potential risks such as nephrocalcinosis, hypercalciuria and hyperparathyroidism. An estimated 500-1,000 people in the United States have TIO, and approximately half of all cases are inoperable.

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.

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.

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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 commits to innovate drug discovery driven by state-of-the-art technologies. The company focuses on creating new values in the four therapeutic areas: nephrology, oncology, immunology/allergy and neurology. Under the Kyowa Kirin brand, the employees from 36 group companies across North America, EMEA and Asia/Oceania unite to champion the interests of patients and their caregivers in discovering solutions wherever there are unmet medical needs. You can learn more about the business of Kyowa Kirin at www.kyowakirin.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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