UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 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): May 04, 2021

Ultragenyx Pharmaceutical Inc.

(Exact name of Registrant as Specified in Its 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

(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(s)	Name of each exchange on which registered
Common Stock, $0.001 par value	RARE	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 May 4, 2021, Ultragenyx Pharmaceutical Inc. issued a press release announcing its financial results for the three months ended March 31, 2021 (the "***Press Release***"). 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 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	**Description**
99.1	Press Release, dated May 4, 2021.
104	The cover page from the Company's Current Report on Form 8-K dated May 4, 2021 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.

Ultragenyx Pharmaceutical Inc.

Date: May 4, 2021

By: /s/ Mardi C. Dier

Mardi C. Dier
Executive Vice President & Chief Financial Officer

Exhibit 99.1

Contact Ultragenyx Pharmaceutical Inc.
Investors & Media
Joshua Higa
(415) 475-6370

Ultragenyx Reports First Quarter 2021 Financial Results and Corporate Update

First quarter 2021 total revenue of $99.4 million and Crysvita[1] revenue to Ultragenyx of $42.1 million

2021 Crysvita revenue in Ultragenyx territories[1] guidance of $180 million to $190 million reaffirmed

Strong Dojolvi launch continues with approximately 180 patients on reimbursed commercial therapy in the United States

NOVATO, Calif. – May 4, 2021 – Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development and commercialization of novel therapies for serious rare and ultra-rare genetic diseases, today reported its financial results for the first quarter 2021 and reaffirmed its financial guidance for 2021.

“We continue to have strong Crysvita and Dojolvi launches and execute on our development priorities across our pipeline,” said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. “In 2021, we will have ongoing pivotal trials for three gene therapies, one pivotal monoclonal antibody study for osteogenesis imperfecta, our first mRNA program for GSDIII, and an antisense oligonucleotide for Angelman with our partner GeneTx. These six clinical programs along with our commercial and early-stage programs make ours one of the most diversified pipelines in rare disease.”

First Quarter 2021 Financials

In the first quarter 2021, Crysvita revenue in Ultragenyx territories[1] increased 46% versus the first quarter 2020. This increase is driven by demand from pediatric and adult patients with X-linked hypophosphatemia (XLH) and patients with tumor induced osteomalacia (TIO), which became Crysvita’s second approved indication in the United States in June 2020.

Dojolvi revenue in the first quarter 2021 continued to build on the launch momentum after the U.S. Food and Drug Administration (FDA) approval in June 2020. As of the end of the first quarter 2021 the company has received approximately 230 completed start forms from approximately 115 unique prescribers. This has led to approximately 180 patients on reimbursed therapy as of the end of March 2021.

First quarter 2021 revenue included $42.8 million related to the technology transfer as part of the Daiichi Sankyo strategic manufacturing partnership around the HeLa PCL and HEK293 technologies. This revenue is expected to taper significantly through the end of this year as these activities come to a close.

Total operating expenses of $206.0 million in the first quarter 2021 increased 31% or $49.0 million versus the first quarter 2020, primarily driven by a $50.0 million expense for the upfront payment on the closing of the

license and collaboration agreement with Mereo Biopharma. Excluding this upfront payment, total operating expenses for the year are expected to increase modestly as the company prepares for and enrolls six clinical programs, including four registrational studies.

Net cash used in operations for the quarter ended March 31, 2021 was $159.3 million, compared to net cash used of $95.2 million for the same period in 2020. Cash, cash equivalents, and marketable debt securities were $1.0 billion as of March 31, 2021.

2021 Financial Guidance

Crysvita Guidance in Ultragenyx Territories

The company reaffirms the 2021 guidance range for Crysvita that was provided at the beginning of the year. This range is $180 million to $190 million and includes the North American profit share region and the other regions where product sales are recognized.

First Quarter 2021 Revenue and Selected Financial Data Tables

Revenues (dollars in thousands)

	Three Months Ended March 31,	
	2021	**2020**
Crysvita Collaboration and Product Revenues:		
North America Collaboration	$ 36,260	$ 27,215
ROW Product Sales	5,872	1,610
Crysvita in Ultragenyx Territories	42,132	28,825
EU Non-Cash Royalty Revenue	3,872	2,615
Total Crysvita Revenue	46,004	31,440
Dojolvi	7,034	1,444
Mepsevii	3,607	3,425
Daiichi Sankyo	42,750	—
Total Revenue	$ 99,395	$ 36,309

Selected Financial Data (dollars in thousands, except per share amounts)

	Three Months Ended March 31,	
	2021	**2020**
Total revenue	$ 99,395	$ 36,309
Operating expense:		
Cost of sales	5,188	(3,503)
Research and development	147,518	112,961
Selling, general and administrative	53,258	47,516
Total operating expense	205,964	156,974
Net loss	$ (136,141)	$ (119,025)
Net loss per share, basic and diluted	$ (2.03)	$ (2.05)

Program Updates and Upcoming Milestones

Dojolvi for the treatment of Long-chain Fatty Acid Oxidation Disorders, or LC-FAOD

- Health Canada approved Dojolvi for the treatment of adult and pediatric patients with LC-FAOD in February 2021 and the therapy was made commercially available to patients in Canada beginning in April 2021.
- Recently published data[2] under France's nominative Authorization for Temporary Use program , or Authorisations Temporaires d'Utilisation (ATU), of 18 pediatric and adult patients with LC-FAOD showed that Dojolvi (triheptanoin) led to reductions in LC-FAOD manifestations and was well-tolerated, with median follow-up duration of 22 months (range 9-228 months). When comparing the year prior to treatment to the first year receiving Dojolvi, annual emergency hospital care visits decreased from a mean of 1.12 to 0.17, or an 85% reduction, and the mean number of emergency home care events decreased from 16.82 to 2.83, an 83% reduction. Similarly, the cumulative annual number of days of emergency home care were reduced from 286 in the year prior to receiving Dojolvi to 51 in the first year receiving Dojolvi, an 82% reduction. Further improvements in the cumulative annual number of days of emergency home care were seen during the second year receiving Dojolvi.
- The efficacy and safety profile reported under the ATU is consistent with the results from prior reported data from the company-sponsored studies.

GTX-102 for the treatment of Angelman Syndrome, partnered with GeneTx

- A pre-application meeting with a national regulatory agency in Europe was held in which the company reviewed the available efficacy and safety data as well as its proposal for dosing and administration of GTX-102. Based on our discussion, the authority agreed in principle on the expansion of the trial to Europe using the proposed modified study design, dosing and administration strategy, pending review of the application. The application to enroll the clinical study in this region was recently submitted.
- In Canada, an amendment to the open clinical trial agreement was submitted and included the additional data and updated protocol and is pending review.
- Discussions with the FDA are continuing and a request for a meeting has been granted and will take place in the second quarter 2021. The meeting will review additional clinical data requested by the FDA regarding the previously treated patients that confirm that the serious adverse event in the previously treated patients has fully reversed as well as an amended dosing plan for the previously treated patients.
- The Phase 1/2 study is expected to resume in 2021, with clinical data on some patients still available before the end of 2021.

DTX401 for the treatment of Glycogen Storage Disease Type Ia, or GSDIa

- Ultragenyx completed the Scientific Advice process with the European Medicines Agency (EMA) and an End-of-Phase 2 meeting with the FDA.
- The Phase 3 design the study protocol has been submitted and endpoints are being finalized with regulators.

- The Phase 3 study is expected to initiate early in the second half of 2021.

DTX301 for the treatment of Ornithine Transcarbamylase, or OTC, Deficiency

- Late in the first quarter 2021, Ultragenyx met with the FDA in an End-of-Phase 2 meeting where the Phase 3 design and endpoints were finalized. The Phase 3 study will include a 64-week primary efficacy analysis period and enroll approximately 50 patients 12 years of age and older, randomized 1:1 to DTX301 (1.7 x 10^13 GC/kg dose) or placebo. The co-primary endpoints are the change in 24-hour plasma ammonia levels and the percentage of patients who achieve a response as measured by discontinuation or reduction in baseline disease management.
- The final Phase 3 study design incorporates Scientific Advice feedback from the EMA.
- The Phase 3 study is expected to initiate in the second half of 2021.

UX701 for the treatment of Wilson Disease

- During the first quarter 2021, the Investigational New Drug (IND) application cleared FDA review. A seamless, single-protocol Phase 1/2/3 study is expected to begin early in the second half of 2021.

UX053 for the treatment of Glycogen Storage Disease Type III, or GSDIII

- The IND application for UX053 for the treatment of GSDIII, the company's most advanced mRNA program, was cleared by the FDA in March 2021. Enrollment in a Phase 1/2 study is expected to begin in the second half of 2021.

1: Ultragenyx territories include the collaboration revenue from the North American profit share territory and other regions where revenue from product sales are recognized by Ultragenyx. This excludes the European territory revenue, which is recognized as non-cash royalty revenue since the rights were sold to Royalty Pharma in December 2019.

2: https://doi.org/10.1016/j.ymgme.2021.02.003.

Conference Call and Webcast Information

Ultragenyx will host a conference call today, Tuesday, May 4, 2021, at 2 p.m. PT/ 5 p.m. ET to discuss the first quarter 2021 financial results and provide a corporate update. The live and replayed webcast of the call will be available through the company's website at https://ir.ultragenyx.com/events.cfm. To participate in the live call by phone, dial (855) 797-6910 (USA) or (262) 912-6260 (international) and enter the passcode 6883837. The replay of the call will be available for one year.

About Ultragenyx

Ultragenyx is a biopharmaceutical company committed to bringing to patients novel therapies 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 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, the effects from the COVID-19 pandemic on the company's clinical activities, business and operating results, risks related to reliance on third party partners to conduct certain activities on the company's behalf, uncertainty and potential delays related to clinical drug development, 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 Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 12, 2021, and its subsequent periodic reports filed with the Securities and Exchange Commission

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Ultragenyx Pharmaceutical Inc.
Selected Statement of Operations Financial Data
(in thousands, except share and per share amounts)
(unaudited)

	Three Months Ended March 31,	
	2021	**2020**
Statement of Operations Data:		
Revenues:		
Collaboration and license	$ 79,010	$ 27,215
Product sales	16,513	6,479
Non-cash collaboration royalty revenue	3,872	2,615
Total revenues	99,395	36,309
Operating expenses:		
Cost of sales	5,188	(3,503)
Research and development	147,518	112,961
Selling, general and administrative	53,258	47,516
Total operating expenses	205,964	156,974
Loss from operations	(106,569)	(120,665)
Change in fair value of equity investments	(20,619)	7,668
Non-cash interest expense on liability related to the sale of future royalties	(8,418)	(8,082)
Other income (expense), net	(156)	2,463
Loss before income taxes	(135,762)	(118,616)
Provision for income taxes	(379)	(409)
Net loss	$ (136,141)	$ (119,025)
Net loss per share, basic and diluted	$ (2.03)	$ (2.05)
Weighted-average shares used in computing net loss per share, basic and diluted	67,102,342	57,995,999

Ultragenyx Pharmaceutical Inc.
Stock-Based Compensation and Selected License Fees included in Operating Expenses
(in thousands)
(unaudited)

	Three Months Ended March 31,	
	2021	**2020**
Stock-based compensation (non-cash)	$ 24,298	$ 20,172
GeneTx purchase option and extension	—	25,000
REGENXBIO license agreement	—	7,000
Mereo license and collaboration agreement	50,000	—

Ultragenyx Pharmaceutical Inc.
Selected Balance Sheet Financial Data
(in thousands)
(unaudited)

	March 31, 2021	December 31, 2020
Balance Sheet Data:		
Cash, cash equivalents, and marketable debt securities	$ 1,047,032	$ 1,212,039
Working capital	943,892	1,105,695
Total assets	1,597,763	1,759,555
Total stockholders' equity	1,054,582	1,154,375