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, 2023

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, 2023, Ultragenyx Pharmaceutical Inc. issued a press release announcing its financial results for the three months ended March 31, 2023 (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, 2023.
104	The cover page from the Company's Current Report on Form 8-K dated May 4, 2023 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, 2023

By: /s/ Emil D. Kakkis, M.D, Ph.D.
Emil D. Kakkis, M.D., Ph.D.
President and Chief Executive Officer

Exhibit 99.1

Contacts Ultragenyx Pharmaceutical Inc.
Investors
Joshua Higa
ir@ultragenyx.com

Media
Jeff Blake
media@ultragenyx.com

Ultragenyx Reports First Quarter 2023 Financial Results and Corporate Update

First quarter 2023 total revenue of $100.5 million, Crysvita® revenue of $76.0 million and Dojolvi® revenue of $14.3 million

Total revenue grew 26% and Crysvita revenue grew 28% versus the first quarter 2022

Reaffirmed 2023 expected total revenue guidance between $425 million to $450 million, Crysvita revenue of $325 million to $340 million, and Dojolvi revenue of $65 million to $75 million

NOVATO, Calif. – May 04, 2023 – Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development and commercialization of novel products for serious rare and ultrarare genetic diseases, today reported its financial results for the quarter ended March 31, 2023 and reaffirmed its financial guidance for 2023.

“In the first quarter we continued the strong growth in product revenue and advanced our clinical programs including completion of the Phase 2 portion of our pivotal study of UX143 in osteogenesis imperfecta. We are analyzing the data and look forward to sharing the results soon. Once completed, we will initiate enrollment of the Phase 3 portion in mid-2023,” said Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx. “We are also making rapid progress in our Phase 1/2 study of GTX-102 in Angelman syndrome, dosing in the expansion cohorts and activating sites in new countries.”

First Quarter 2023 Selected Financial Data Tables and Financial Results

Revenues (dollars in thousands), (unaudited)

	Three Months Ended March 31,	
	2023	2022
Crysvita		
Revenue in profit-share territory	$ 49,906	$ 45,164
Product revenue	21,234	9,394
Non-cash royalty revenue in European territory	4,882	4,838
Total Crysvita revenue	76,022	59,396
Dojolvi	14,303	12,429
Mepsevii	8,480	4,861
Evkeeza	212	—
Daiichi Sankyo	1,479	3,249
Total revenues	$ 100,496	$ 79,935

Net Revenues

Ultragenyx reported $100.5 million in total revenue for the first quarter of 2023, which represents 26% growth compared to the first quarter 2022. The growth is primarily driven by increased demand for Crysvita in Latin America and steady growth from the other regions and products. The first quarter 2023 also included $1.5 million of revenue from the technology transfer which was completed during the quarter as part of the collaboration and license agreement with Daiichi Sankyo.

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

	Three Months Ended March 31,	
	2023	2022
Total revenues	$ 100,496	$ 79,935
Operating expense:		
Cost of sales	12,257	6,100
Research and development	165,698	143,155
Selling, general and administrative	76,646	67,312
Total operating expense	254,601	216,567
Net loss	$ (163,972)	$ (152,320)
Net loss per share, basic and diluted	$ (2.33)	$ (2.19)

Operating Expenses

Total operating expenses for the first quarter of 2023 were $254.6 million, including non-cash stock-based compensation of $31.9 million. In 2023, annual operating expenses are expected to decrease as the company manages headcount and increases operational leverage while executing on high-value programs.

Net Loss
For the first quarter of 2023, Ultragenyx reported net loss of $164.0 million, or $2.33 per share basic and diluted, compared with a net loss for the first quarter of 2022 of $152.3 million, or $2.19 per share, basic and diluted.

Cash, Cash Equivalents and Marketable Debt Securities
Cash, cash equivalents, and marketable debt securities were approximately $714.6 million as of March 31, 2023.

2023 Financial Guidance
For the full year 2023, the company reaffirms:

- Total revenue in the range of $425 million to $450 million
- Crysvita revenue in the range of $325 million to $340 million. This includes all regions where Ultragenyx will recognize revenue, including the royalties in Europe, which have been ongoing, and the royalties in North America, which began in April 2023.
- Dojolvi revenue in the range of $65 million to $75 million
- Net Cash Used in Operations is expected to be less than $400 million

Recent Updates and Clinical Milestones

UX143 (setrusumab) monoclonal antibody for Osteogenesis Imperfecta (OI): Phase 2 enrollment complete; Phase 2 data and Phase 3 initiation expected in mid-2023
Ultragenyx is continuing to dose patients in the Phase 2/3 *Orbit* study of UX143 in pediatric and adult patients with OI aged five to <26 years. All patients have completed the Phase 2 portion of the study with data expected in mid-2023. These data are expected to include changes in bone biomarkers response and some data on bone mineral density that will be used to establish the dosing algorithm for the Phase 3 portion of the study. The Phase 3 is expected to initiate in mid-2023.

In addition, in the second quarter of 2023, Ultragenyx intends to initiate *Cosmic*, a randomized study in children under age five with serious bone disease, which compares bisphosphonates to UX143. Younger pediatric patients with OI often have a much higher fracture rate than other age groups driving clinical urgency for better treatment options. The primary endpoint is expected to be the annualized rate of fractures.

GTX-102 antisense oligonucleotide for Angelman syndrome: Phase 1/2 dosing in the expansion cohorts

Outside of the U.S., the dose escalation phase of this study has been completed and the company recently transitioned to dosing patients in the expansion cohorts, which are designed to verify the GTX-102 dose and treatment regimen that will be used in the Phase 3 program. The study has also expanded geographically with multiple new sites activated in Europe and Australia. Approximately 40 patients will be enrolled across Cohort A (ages four to <eight years) and Cohort B (ages eight to <18 years) that will evaluate the same safety, pharmacokinetic, and efficacy measures as the dose escalating cohorts.

Patients from the dose escalation cohorts continue to exhibit encouraging dose and time-dependent clinical activity following longer-term treatment and maintenance dosing. No additional treatment-related serious adverse events or lower extremity weakness adverse events have occurred since our prior update in January 2023. As of May 2023, 14 patients have had at least six months of exposure to GTX-102 including nine patients with more than one year of continuous therapy. The next data update is expected in the second half of 2023.

In the U.S., patients are continuing to receive GTX-102 at the lower loading and maintenance dose. Productive discussions with the FDA are ongoing to harmonize the dosing strategy with the ex-U.S. protocol.

DTX401 AAV gene therapy for Glycogen Storage Disease Type Ia (GSDIa): Dosing in Phase 3 study complete

Ultragenyx has randomized and dosed the last patient in the Phase 3 study. The 48-week study has fully enrolled patients eight years of age and older, randomized 1:1 to DTX401 or placebo. The primary endpoint is the reduction in oral glucose replacement with cornstarch while maintaining glucose control. Phase 3 data are expected in the first half of 2024.

DTX301 AAV gene therapy for Ornithine Transcarbamylase (OTC) Deficiency: Phase 3 study dosing patients

Ultragenyx is randomizing and dosing patients in the ongoing Phase 3 study. The pivotal, 64-week study will include approximately 50 patients, randomized 1:1 to DTX301 or placebo. The primary endpoints are response as measured by removal of ammonia-scavenger medications and protein-restricted diet and change in 24-hour ammonia levels.

UX701 AAV gene therapy for Wilson Disease: Stage 1 of pivotal clinical study dosing patients; expect interim Stage 1 enrollment completion in the second half of 2023

Dosing in the first stage of the pivotal study is ongoing under an amended protocol that removes placebo from the dose finding stage and includes five patients per cohort. During this stage of the study, safety and efficacy of up to three dose levels of UX701 will be evaluated and a dose will be selected for further evaluation in Stage 2. Completion of Stage 1 enrollment is

expected in the second half of 2023 with data expected in the first half of 2024 that would include safety and potentially initial signs of clinical activity.

UX053 mRNA for glycogen storage disease type III (GSDIII): Phase 1/2 single ascending dose cohort enrolled; data in 1H23
Dosing in the single ascending dose stage (SAD) of the Phase 1/2 study of UX053 for the treatment of GSDIII has been completed with no safety issues observed. The company has decided to not enroll patients in the multiple ascending dose cohorts at this time to allow greater focus on other late-stage and larger indication clinical programs. The data from the SAD cohort are being analyzed and are expected in the second quarter of 2023.

Conference Call and Webcast Information

Ultragenyx will host a conference call today, Thursday, May 4, 2023, at 2 p.m. PT/ 5 p.m. ET to discuss the first quarter 2023 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-presentations. To participate in the live call, please register by clicking on the following link (https://register.vevent.com/register/BI1183803cc36b46baa6367e521e12e605), and you will be provided with dial in details. The replay of the call will be available for one year.

About Ultragenyx

Ultragenyx is a biopharmaceutical company committed to bringing novel therapies to patients for the treatment of serious rare and ultrarare 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.

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 uncertainty of clinical drug development and unpredictability and lengthy process for obtaining regulatory approvals, risks related to serious or undesirable side effects of our product candidates, the company's ability to achieve its projected development goals in its expected timeframes, 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 Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on February 17, 2023, 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/).

###

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

	Three Months Ended March 31,	
	2023	2022
Statement of Operations Data:		
Revenues:		
Collaboration and license	$ 51,385	$ 48,413
Product sales	44,229	26,684
Royalty revenue	4,882	4,838
Total revenues	100,496	79,935
Operating expenses:		
Cost of sales	12,257	6,100
Research and development	165,698	143,155
Selling, general and administrative	76,646	67,312
Total operating expenses	254,601	216,567
Loss from operations	(154,105)	(136,632)
Change in fair value of equity investments	(334)	(9,329)
Non-cash interest expense on liability related to the sale of future royalties	(15,636)	(6,584)
Other income, net	6,598	783
Loss before income taxes	(163,477)	(151,762)
Provision for income taxes	(495)	(558)
Net loss	$ (163,972)	$ (152,320)
Net loss per share, basic and diluted	$ (2.33)	$ (2.19)
Weighted-average shares used in computing net loss per share, basic and diluted	70,368,478	69,516,668

Ultragenyx Pharmaceutical Inc.
Selected Activity included in Operating Expenses
(in thousands)
(unaudited)

	Three Months Ended March 31,	
	2023	2022
Non-cash stock based compensation	$ 31,939	$ 29,387

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

	March 31, 2023	December 31, 2022
Balance Sheet Data:		
Cash, cash equivalents, and marketable debt securities	$ 714,567	$ 896,732
Working capital	548,157	622,689
Total assets	1,383,404	1,545,444
Total stockholders' equity	220,481	352,494