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): November 02, 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 November 2, 2021, Ultragenyx Pharmaceutical Inc. issued a press release announcing its financial results for the three months ended September 30, 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 November 2, 2021.
104	The cover page from the Company's Current Report on Form 8-K dated November 2, 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 thereunto duly authorized.

Ultragenyx Pharmaceutical Inc.

Date: November 2, 2021

By: /s/ Mardi C. Dier

Mardi C. Dier
Executive Vice President & Chief Financial Officer

Exhibit 99.1

Contact Ultragenyx Pharmaceutical Inc.
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Ultragenyx Reports Third Quarter 2021 Financial Results and Corporate Update

Third quarter 2021 total revenue of $81.6 million including Crysvita[1] revenue to Ultragenyx of $50.3 million

2021 Crysvita revenue in Ultragenyx territories[1] now expected towards the upper end of the$180 million to $190 million range previously provided

NOVATO, Calif. – November 02, 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 third quarter 2021.

“In the third quarter we executed on key commercial and clinical milestones, including the resumption of the GTX-102 study for the treatment of Angelman syndrome and the initiation of our seamless Phase 1/2/3 study of UX701 for the treatment of Wilson disease. Looking ahead, we are preparing to initiate four additional studies across multiple modalities, including two Phase 3 gene therapies, a Phase 2/3 anti-sclerostin monoclonal antibody, and a Phase 1/2 leveraging our first mRNA program,” said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. “The breadth of our clinical programs with the strength of our balance sheet put us in position to deliver disease-modifying therapies across a spectrum of rare diseases.”

Third Quarter 2021 Financials and Full Year Crysvita Guidance Update

In the third quarter 2021, Crysvita revenue in Ultragenyx territories[1] increased 35% versus the third quarter 2020. For the full year 2021, the company now expects the 2021 Crysvita revenue to be towards the upper end of the guidance range of $180 million to $190 million that was previously provided.

Dojolvi revenue in the third quarter 2021 grew 176% versus the third quarter of 2020, which was the first quarter following FDA approval. As of the end of the third quarter 2021, the company had received approximately 310 completed start forms from approximately 145 unique prescribers. This has led to approximately 250 patients on reimbursed therapy as of the end of September 2021.

Third quarter 2021 revenue included $12.1 million related to the technology transfer as part of the Daiichi Sankyo strategic manufacturing partnership around the company’s producer cell line and HEK293 technologies. The technology transfer activities and resulting revenue is expected to be substantially complete in the fourth quarter 2021.

Total operating expenses of $171.5 million in the third quarter 2021 increased 30% or $39.7 million versus the third quarter 2020, primarily driven by pipeline advancements. For the year, total operating expenses are expected to increase modestly as the company continues the commercial launch of Dojolvi and supports six clinical programs, including four pivotal studies.

Net cash used in operations for the nine months ended September 30, 2021 was $284.4 million, compared to net cash used of $69.8 million for the same period in 2020 which included approximately $154 million of operating cash received in 2020 from Daiichi Sankyo related to the collaboration and license agreement. Cash, cash equivalents, and marketable debt securities were $941.4 million as of September 30, 2021.

Third Quarter 2021 Revenue and Selected Financial Data Tables

Revenues (dollars in thousands)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Crysvita Collaboration and Product Revenues:				
North America Collaboration	$ 42,971	$ 34,058	$ 120,987	$ 91,079
ROW Product Sales	7,378	3,277	16,150	7,436
Crysvita in Ultragenyx Territories	50,349	37,335	137,137	98,515
EU Royalty Revenue	4,665	3,331	13,454	10,926
Total Crysvita Revenue	55,014	40,666	150,591	109,441
Dojolvi	10,654	3,862	27,735	6,638
Mepsevii	3,918	4,076	12,924	11,686
Daiichi Sankyo	12,061	32,866	76,767	51,723
Total Revenue	$ 81,647	$ 81,470	$ 268,017	$ 179,488

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Total revenue	$ 81,647	$ 81,470	$ 268,017	$ 179,488
Operating expense:				
Cost of sales	4,175	2,348	12,499	648
Research and development	113,417	87,314	374,140	280,984
Selling, general and administrative	53,883	42,123	160,551	131,891
Total operating expense	171,475	131,785	547,190	413,523
Net loss	$ (72,998)	$ (68,845)	$ (331,567)	$ (162,555)
Net loss per share, basic and diluted	$ (1.08)	$ (1.13)	$ (4.91)	$ (2.73)

Program Updates and Upcoming Milestones

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

- The U.S. FDA removed the clinical hold, allowing GeneTx to begin dosing naïve pediatric patients in the Phase 1/2 study of GTX-102 in patients with Angelman syndrome.
- The first patients in Canada have been dosed, with dosing in the U.K. and U.S. currently expected to begin in the fourth quarter of 2021.
- A preliminary update on the first four patients in the study is expected to be available around the end of the year, with complete data from the full cohort of 12 patients anticipated in mid-2022.

Gene Therapy Clinical Program Updates

- UX701 for the treatment of Wilson disease: Multiple patients with Wilson disease have been successfully screened and are enrolled in the baseline monitoring period prior to dosing in the seamless Phase 1/2/3 study. Following the initial screening that includes testing for pre-existing antibodies to the AAV9 capsid, patients will be evaluated to ensure stable measures of disease during a 6-to 12-week baseline monitoring period after which they will then be dosed with either UX701 or placebo.

- DTX401 for the treatment of Glycogen Storage Disease Type Ia: The first patients in the U.S. and Canada are expected to enter a 4- to 8-week baseline screening period around the end of 2021 after which they would receive a single dose of DTX401 or placebo.
- DTX301 for the treatment of Ornithine Transcarbamylase Deficiency: The first patients in the U.S. are expected to enter a 4- to 8- week baseline screening period around the end of 2021 after which they would receive a single dose of DTX301 or placebo.
- 14th International Congress of Inborn Errors of Metabolism (ICIEM): Ten abstracts have been accepted for this hybrid conference that will be held November 21-24, 2021. Of the five oral presentations accepted, two will include longer-term follow-up data from the Phase 1/2 clinical studies of DTX301 for OTC deficiency and DTX401 for GSDIa.

UX143 for the treatment of Osteogenesis Imperfecta, or OI

- At the 2021 American Society for Bone and Mineral Research (ASBMR) annual meeting, additional secondary endpoint data from the Phase 2b ASTEROID study demonstrated that treatment with UX143 resulted in dose-dependent increase in P1NP serum levels, a marker of bone formation, and decrease in CTx serum levels, a marker of bone resorption confirming the mechanism of action of sclerostin inhibition over the 12-month treatment period. Observed improvements in bone mineral density were continuous over the 12 months of the study, with comparable gains achieved in the first and second 6 months of treatment in the high dose group despite temporal changes in biomarkers.
- A Phase 2/3 study in children and young adults is expected to initiate around the end of 2021, with additional studies in other age groups to follow.

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.

Conference Call and Webcast Information

Ultragenyx will host a conference call today, Tuesday, November 2, 2021, at 2 p.m. PT/ 5 p.m. ET to discuss the third 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 1098326. 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 Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on August 3, 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 September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Statement of Operations Data:				
Revenues:				
Collaboration and license	$ 55,048	$ 66,924	$ 197,998	$ 144,300
Product sales	21,950	11,215	56,809	25,760
Non-cash collaboration royalty revenue	4,649	3,331	13,210	9,428
Total revenues	81,647	81,470	268,017	179,488
Operating expenses:				
Cost of sales	4,175	2,348	12,499	648
Research and development	113,417	87,314	374,140	280,984
Selling, general and administrative	53,883	42,123	160,551	131,891
Total operating expenses	171,475	131,785	547,190	413,523
Loss from operations	(89,828)	(50,315)	(279,173)	(234,035)
Change in fair value of equity investments	25,702	(11,520)	(25,963)	91,348
Non-cash interest expense on liability related to the sale of future royalties	(8,683)	(8,582)	(25,618)	(25,093)
Other income (expense), net	(7)	1,885	211	6,362
Loss before income taxes	(72,816)	(68,532)	(330,543)	(161,418)
Provision for income taxes	(182)	(313)	(1,024)	(1,137)
Net loss	$ (72,998)	$ (68,845)	$ (331,567)	$ (162,555)
Net loss per share, basic and diluted	$ (1.08)	$ (1.13)	$ (4.91)	$ (2.73)
Weighted-average shares used in computing net loss per share, basic and diluted	67,875,363	60,687,177	67,533,671	59,564,163

Ultragenyx Pharmaceutical Inc.
Selected Noncash Activity and License Fees included in Operating Expenses
(in thousands)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Non-cash stock based compensation	$ 26,990	$ 20,341	$ 78,430	$ 62,922
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)

	September 30, 2021	December 31, 2020
Balance Sheet Data:		
Cash, cash equivalents, and marketable debt securities	$ 941,360	$ 1,212,039
Working capital	710,951	1,105,695
Total assets	1,484,801	1,759,555
Total stockholders' equity	933,674	1,154,375