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): July 30, 2020

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))

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. ☐

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

Item 2.02 Results of Operations and Financial Condition.

On July 30, 2020, Ultragenyx Pharmaceutical Inc. issued a press release announcing its financial results for the three months and six months ended June 30, 2020 (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 July 30, 2020.
104	The cover page from the Company's Current Report on Form 8-K dated July 30, 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 thereunto duly authorized.

Ultragenyx Pharmaceutical Inc.

Date: July 30, 2020

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

Exhibit 99.1

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

Ultragenyx Reports Second Quarter 2020 Financial Results and Corporate Update

Two U.S. FDA approvals in second quarter 2020

Second quarter 2020 total revenue of $61.7 million and Crysvita revenue in Ultragenyx territories of $32.4 million

Maintains full year 2020 guidance based on global COVID-19 impact to date

NOVATO, Calif. – July 30, 2020 – Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development and commercialization of novel products for serious rare and ultra-rare genetic diseases, today reported its financial results for the quarter ended June 30, 2020 and maintained its full year 2020 financial guidance for Crysvita in Ultragenyx territories.

“In the second quarter we made significant progress expanding the commercial reach of Crysvita and Mepsevii despite the COVID environment. We also expanded our commercial portfolio with two FDA approvals in a two-week period as Dojolvi for LC-FAOD and Crysvita for TIO became the third and fourth approvals for Ultragenyx,” said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. “We also presented positive updates at ASGCT on both of our clinical gene therapy programs, DTX401 for GSDIa and DTX301 for OTC deficiency, and we are heading towards Phase 3 studies for each of them.”

Second Quarter 2020 Financial Results

Net Revenues
For the second quarter of 2020, Ultragenyx reported $61.7 million in total revenue. Ultragenyx recognized $32.4 million in total Crysvita revenue in the Ultragenyx territories, which includes $29.8 million in collaboration revenue in the North American profit share territory and net product sales in other regions of$2.5 million. Total royalty revenue related to European Crysvita sales were $5.0 million, which includes $1.5 million recognized on sales that occurred prior to January 1, 2020. Mepsevii® (vestronidase alfa) product revenue for the second quarter of 2020 was $4.2 million, and UX007 named patient revenue was $1.3 million. Revenue for the second quarter also includes $18.9 million of revenue related to the collaboration and license agreement with Daiichi Sankyo that was executed in March 2020.

Revenue for the six months ended June 30, 2020 was $98.0 million, including $61.2 million in total Crysvita revenue in the Ultragenyx territories. Crysvita collaboration revenue in the North American profit share

territory was $57.0 million and net Crysvita product sales in other regions were $4.2 million. Total royalty revenue related to European Crysvita royalties was $7.6 million in the first half of 2020, which includes $1.5 million recognized on sales that occurred prior to January 1, 2020. Mepsevii product revenue for the six months ended June 30, 2020 was $7.6 million and UX007 named patient revenue was $2.8 million. The technology transfer services with Daiichi Sankyo were initiated during the second quarter of 2020 and Ultragenyx began recognizing revenue during the period related to the collaboration and license agreement. For the three and six months ended June 30, 2020, revenue related to this agreement was $18.9 million.

Operating Expenses
Total operating expenses for the second quarter of 2020 were $124.8 million, including non-cash stock-based compensation of $22.4 million. Total operating expenses for the second quarter of 2019 were $136.6 million, including a $15.6 million research and development expense from the Arcturus collaboration amendment, and non-cash stock-based compensation of $22.2 million.

Total operating expenses for the six months ended June 30, 2020 were $281.7 million, which includes $25.0 million to maintain the option to acquire GeneTx, $7.0 million to license certain vectors from REGENXBIO, and non-cash stock-based compensation of $42.6 million. This is compared with $254.0 million for the same period in 2019, which includes a $15.6 million research and development expense from the Arcturus collaboration amendment and non-cash stock-based compensation $42.4 million. The increase in total operating expenses is due to the increase in commercial, development, and general and administrative costs as the company commercializes, grows, and advances its portfolio.

For the second quarter of 2020, Ultragenyx reported net income of $25.3 million, or $0.42 per basic share and $0.41 per diluted share, compared with a net loss for the second quarter of 2019 of $99.2 million, or $1.72 per share, basic and diluted. For the six months ended June 30, 2020, net loss was $93.7 million, or $1.59 per share, basic and diluted, compared with a net loss for the same period in 2019 of $195.9 million, or $3.54 per share, basic and diluted. The net income for the second quarter of 2020 and the net loss for the six months ended June 30, 2020 includes a $95.2 million unrealized gain and a $102.9 million unrealized gain for the three and six months ended June 30, 2020, respectively, from the fair value adjustment on the investment in Arcturus equity. Net cash used in operations for the first six months of 2020 was $7.8 million, which includes $134.9 million of operating cash received from Daiichi Sankyo related to the collaboration and license agreement, compared to net cash used of $184.8 million for the same period in 2019.

Cash, Cash Equivalents and Investments
Cash, cash equivalents, and investments were $817.5 million as of June 30, 2020.

2020 Guidance

Crysvita Guidance in Ultragenyx Territories
The company continues to maintain its guidance range for 2020 Crysvita revenue in the Ultragenyx territories between $125.0 million and $140.0 million, but will continue to monitor the COVID-19 pandemic situation. Ultragenyx territories include the collaboration revenue from the North American profit share territory (U.S. and Canada) and other regions where revenue from product sales are recognized by Ultragenyx (Latin America,

Turkey). 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.

Corporate Update

Arcturus Collaboration

· In May 2020, Ultragenyx exercised its option to purchase 600,000 shares of Arcturus common stock at $16.00 per share. Upon completion of the new equity purchase, Ultragenyx owned 3,000,000 shares of Arcturus common stock and continued to be its largest shareholder.

COVID-19 Update

· Despite the ongoing pandemic, the company has been able to maintain an uninterrupted supply of medicine to patients around the world as no significant disruptions to manufacturing or distribution activities have been experienced. There has been some impact to preclinical manufacturing, clinical study site conduct, and regulatory interactions. The company is adapting clinical and commercial strategies to ensure continuity of clinical and commercial programs during this unprecedented time.

Program Updates and Upcoming Milestones

Dojolvi for Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD): Approved by FDA on June 30, 2020

· The U.S. FDA approved Dojolvi for the treatment of pediatric and adult patients for all forms of LC-FAOD with a molecularly-confirmed diagnosis. Dojolvi is the first FDA-approved therapy for these lifelong and life-threatening genetic disorders and is now available to patients in the U.S.

· Dojolvi has been submitted for approval with ANVISA in Brazil and has been submitted to Health Canada after being granted priority review.

Crysvita for Tumor-Induced Osteomalacia (TIO): Approved by FDA on June 18, 2020

· The U.S. FDA approved Crysvita for the treatment of fibroblast growth factor 23 (FGF23)-related hypophosphatemia in tumor-induced osteomalacia (TIO) associated with phosphaturic mesenchymal tumors that cannot be curatively resected or localized in adults and pediatric patients 2 years of age and older. This is the second FDA approval for Crysvita, which was first approved in April 2018 for the treatment of X-linked hypophosphatemia (XLH).

DTX401 for Glycogen Storage Disease Type Ia (GSDIa): Positive data from confirmatory cohort in Phase 1/2 study presented at American Society of Gene & Cell Therapy (ASGCT) Annual Meeting

· As presented at ASGCT, all three patients in the third, confirmatory dose cohort (6.0 x 10^12 GC/kg) demonstrated increased time to hypoglycemia and substantial reductions in cornstarch usage. Prolonged periods

of hyperglycemia were observed in Cohort 3 with the implementation of continuous glucose monitoring (CGM), which is indicative of early transgene expression and glucose release from the liver. The early transgene expression resulted in faster cornstarch reductions in Cohort 3, with a mean reduction of 57% at week 12 compared with 38% and 14% in the first and second cohorts, respectively. Across all three cohorts in the study, 100 percent of patients have demonstrated meaningful and sustained cornstarch regimens over time and significant increases in time to hypoglycemia. After longer-term follow-up, four of six patients in the first two cohorts have discontinued daytime cornstarch.

· Barring delays related to COVID-19, the company expects to release additional data on Cohort 3 in the second half of 2020, intends to hold an end-of-Phase 2 meeting with the U.S. FDA, and potentially initiate a Phase 3 study in early 2021.

DTX301 for Ornithine Transcarbamylase (OTC) Deficiency: Updated positive data from Phase 1/2 study presented at ASGCT

· Updated data presented at ASGCT confirmed that all three patients in the third dose cohort (1.0 x 10^13 GC/kg) are responders to DTX301 as shown by sustained meaningful increases in the rate of ureagenesis or reductions in ammonia levels. Six of nine patients across all three cohorts have now responded to the gene therapy, including three females and three males. All three complete responders, those who have discontinued all ammonia scavengers and liberalized their diets, remain clinically and metabolically stable after longer-term follow-up.

· A fourth cohort of three patients at the same Cohort 3 dose is planned using prophylactic steroids. Dosing is still on hold due to COVID-19 but data are expected by the end of 2020. Ultragenyx intends to hold an end-of-phase 2 meeting with the FDA based on the first three cohorts, with Phase 3 study initiation currently expected in the first half of 2021.

DTX201 / BAY 2599023 Partnered with Bayer for Hemophilia A: Cohort 3 data from Phase 1/2 study presented at International Society on Thrombosis and Haemostasis (ISTH) 2020 Congress

· Three cohorts with two patients each have been dosed with AAVhu37 (DTX201 / BAY 2599023), using material from Ultragenyx's proprietary HeLa manufacturing platform. Initial data from the third dose cohort (2.0 x 10^13 GC/kg) and longer-term data from the lower dose cohorts were presented at ISTH. The data demonstrated a dose response with FVIII expression of 72.1% and 12.9% at weeks 29 and 26 in the two Cohort 3 patients. No spontaneous bleeds were reported after Cohort 3 patients reached peak expression despite prophylaxis discontinuation. One traumatic bleed occurred with no need for Factor VIII treatment. No adverse events related to DTX201 were observed, and ALT elevations in both patients were successfully treated with corticosteroids. Longer-term data from the two earlier cohorts showed sustained FVIII expression up to 16 months with no loss of expression.

GTX-102 for Angelman Syndrome Conducted by Partner, GeneTx: First two cohorts have been fully enrolled

· The first two cohorts of two patients each have been fully enrolled with all patients receiving multiples doses in the Phase 1/2 study of GTX-102 in Angelman Syndrome. Safety and efficacy data from the first two

dose escalating cohorts are currently being evaluated and enrollment and dosing at the next dose levels are expected to resume shortly. Preliminary data from the study are expected in the first half of 2021.

Crysvita for X-Linked Hypophosphatemia (XLH): Positive opinion from Committee for Medicinal Products for Human Use (CHMP)

· In Europe, Crysvita received a positive opinion from the CHMP, for the use of Crysvita for the treatment of XLH in adolescents and adults. This could lead to an expansion of the current market authorization beyond the current approval for children 1 year of age and older and adolescents with growing skeletons. A formal decision from the European Commission (EC) is expected in the second half of 2020.

Mepsevii for Mucopolysaccharidosis Type VII (MPS VII): Positive opinion from CHMP to expand the Summary of Product Characteristics in Europe

· The CHMP provided a positive opinion on a type II variation that would expand the EMA approval to include long-term effects of Mepsevii on the reduction of urinary glycosaminoglycans (uGAGs) and improvements in the multi-domain clinical responder index (MDRI) and 6-minute walk test (6MWT). A formal decision from the EC is expected in the second half of 2020.

Other Gene Therapy Platform Updates

· An IND application for UX701 in Wilson disease, a larger rare metabolic disease, is expected by the end of 2020 barring any delays in nonclinical or manufacturing activities.

· Ultragenyx and Daiichi Sankyo initiated technology transfer activities related to the non-exclusive license agreement executed in the first quarter of 2020 with respect to Ultragenyx's HeLa PCL and HEK293 transient transfection manufacturing technology platforms for AAV-based gene therapy products. Daiichi Sankyo will pay $25.0 million upon completion of transfer of both platforms, as well as single-digit royalties on net sales of products manufactured in either system.

Conference Call and Webcast Information

Ultragenyx will host a conference call today, Thursday, July 30, 2020, at 2 p.m. PT/ 5 p.m. ET to discuss the second quarter 2020 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 1808389. The replay of the call will be available for one year.

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, the effects from the COVID-19 pandemic on the company's commercialization activities, business and operating results, smaller than anticipated market opportunities for the company's products and product candidates, manufacturing risks, competition from other therapies or products, uncertainties related to insurance coverage and reimbursement status of the company's newly approved products, the company's evolving integrated commercial organization, 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 May 7, 2020, and its subsequent periodic reports filed with the Securities and Exchange Commission.

###

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	**2019**	**2020**	**2019**
Statement of Operations Data:				
Revenues:				
Collaboration and license	$ 50,161	$ 19,247	$ 77,376	$ 33,485
Product sales	8,066	4,902	14,545	8,836
Non-cash collaboration royalty revenue	3,482	—	6,097	—
Total revenues	61,709	24,149	98,018	42,321
Operating expenses:				
Cost of sales	1,803	766	(1,700)	1,218
Research and development	80,709	96,045	193,670	174,150
Selling, general and administrative	42,252	39,812	89,768	78,641
Total operating expenses	124,764	136,623	281,738	254,009
Loss from operations	(63,055)	(112,474)	(183,720)	(211,688)
Change in fair value of investment in Arcturus equity securities	95,200	9,828	102,868	9,828
Non-cash interest expense on liability related to the sale of future royalties	(8,429)	—	(16,511)	—
Other income (expense), net	2,014	3,687	4,477	6,361
Income (loss) before income taxes	25,730	(98,959)	(92,886)	(195,499)
Provision for income taxes	(415)	(213)	(824)	(429)
Net income (loss)	$ 25,315	$ (99,172)	$ (93,710)	$ (195,928)
Net income (loss) per share:				
Basic	$ 0.42	$ (1.72)	$ (1.59)	$ (3.54)
Diluted	$ 0.41	$ (1.72)	$ (1.59)	$ (3.54)
Weighted average shares used in computing net income (loss) per share:				
Basic	59,995,617	57,519,308	58,996,278	55,376,336
Diluted	61,146,231	57,519,308	58,996,278	55,376,336

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

	June 30, 2020	**December 31, 2019**
Balance Sheet Data:		
Cash, cash equivalents, and available-for-sale investments	$ 817,484	$ 760,404
Working capital	686,310	747,717
Total assets	1,314,041	1,135,496
Total stockholders' equity	695,587	653,764