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): August 2, 2018

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))

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 7.01 Regulation FD Disclosure.

On August 2, 2018, Ultragenyx Pharmaceutical Inc. (the "***Company***") held its earnings call for its financial results for the three and six months ended June 30, 2018 (the "***Earnings Call***"). Due to audio quality issues during the Earnings Call, the Company is furnishing herewith as Exhibit 99.1 a copy of the prepared remarks used for the Earnings Call.

The information set forth under Item 7.01 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 any such filing.

Item 9.01 Financial Statements and Exhibits

(d) **Exhibits**

Exhibit No.	**Description**
99.1	Call Script

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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: August 2, 2018

Ultragenyx Pharmaceutical Inc.

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

Ultragenyx Second Quarter 2018 Financial Results Conference Call
August 2, 2018

Attendees: Emil Kakkis, Shalini Sharp, Danielle Keatley

Danielle Keatley – Opening Comments

Good afternoon and welcome to the Ultragenyx Pharmaceutical financial results and corporate update conference call for the second quarter 2018. We have issued a press release detailing our financial results, which you can find on our website at Ultragenyx.com. I am Danielle Keatley, Senior Director of Investor Relations and Corporate Communications. With me today are Emil Kakkis, Chief Executive Officer and President, and Shalini Sharp, Chief Financial Officer.

I would like to remind investors that this call will include forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995, including, but not limited to, the types of statements identified as forward looking in our Quarterly Report on Form 10-Q that was filed on May 8, our Quarterly Report on Form 10-Q for the quarter ended June 30, 2018 that will be filed soon, and our subsequent periodic reports filed with the SEC, which will all be available on our website in the Investors' section. These forward-looking statements represent our views only as of the date of this call and involve substantial risks and uncertainties, including many that are beyond our control. Please note that actual results could differ materially from those projected in any forward-looking statement.

For a further description of the risks and uncertainties that could cause actual results to differ materially from those expressed in the forward-looking statements, as well as risks relating to our business, see our periodic reports filed with the SEC.

I’ll now turn the call over to Emil.

Emil Kakkis – Corporate Highlights

Good afternoon everyone and thank you for joining us. The first half of 2018 has been a transformational period for us with strong initial launches for both Crysvita and Mepsevii, as well as progress on the development front across our programs.

I'll start first with the Crysvita program. We launched in the U.S. on April 27 which means that by June 30, we only had two months of commercial activity this quarter. The early launch is going very well. At the end of the quarter, we had received approximately 300 completed patient start forms from more than 150 unique prescribers across 38 states. This shows broad adoption well beyond the ten U.S. clinical trial sites. At this point the majority of major metabolic bone centers have prescribed Crysvita, and approximately two thirds of patients with completed start forms have been naïve to treatment with Crysvita. Among the completed start forms, we continue to see a 60/40 split among pediatric and adult patients, which indicates substantial interest among both adults and children with XLH. We are also encouraged by the number of patients who have been converted to commercial therapy to date. We continue to work through the reimbursement process for the majority of patients with completed start forms. As anticipated, this takes about 30 to 90 days during this medical exception period with a newly approved therapy.

The payer mix as of the end of the quarter was approximately 70% private plans, with the majority of remaining plans from government payers. We have seen medical policies from a mix of national and regional payers, covering about 100

million lives. The policies have been consistent with the broad Crysvita label for the treatment of XLH in adult and pediatric patients one year of age and older, and the policies have not differentiated between children and adults.

In these early days of launch, we are continuing to see new start forms come in and new patients be identified by our patient diagnosis liaison team. This reinforces our belief in our estimate of 3,000 pediatric patients and 9,000 adult patients in the United States. We regularly hear positive feedback and support from XLH patients, and we look forward to sharing our launch progress in the coming quarters.

As our launch continues, we have also succeeded in developing additional clinical data which support Crysvita's superiority to conventional therapy of oral phosphate and active vitamin D. In May, we reported data from our pediatric Phase 3 randomized active controlled study, the first study to directly compare Crysvita to conventional phosphate therapy. In this study we showed that Crysvita had a rapid and profound effect on the underlying XLH disease with normalization or near normalization of serum phosphorus achieved on average with Crysvita that was not achievable with conventional therapy. This strong improvement in phosphorus levels led to significant improvement in bone disease, as patients treated with Crysvita were 39 times more likely to achieve substantial healing of rickets compared to patients treated with conventional therapy.
In addition to these new Phase 3 results, the *New England Journal of Medicine* recently published the results from the Phase 2 study of Crysvita in children aged 5 to 12 years, the pivotal study for the pediatric indication.

In summary, the Crysvita launch is off to a strong start and we also continue to strengthen the clinical evidence base of Crysvita with encouraging data.

Turning to Mepsevii, the U.S. launch has been underway for eight months and is progressing well. We continue to identify new patients with this extremely rare disease and we continue to receive start forms, giving us confidence in our estimate of 200 patients worldwide. Following the U.S. approval, we are now responding to requests for named patient programs in other countries. We will also be expanding our efforts into Europe where the CHMP recently adopted a positive opinion recommending the marketing authorization of Mepsevii under exceptional circumstances. This should be followed by a decision from the European Commission in the third quarter of 2018. Reimbursement in Europe happens on a country by country basis, and can take up to 24 months.

I'll also provide an update on UX007 in long-chain fatty acid oxidation disorders, or LC-FAOD. Over the past few months, we have been working with FDA to determine the acceptability of an NDA based on data from the Phase 2 study as well as additional supportive data. In their review, the FDA noted that the effect on reducing hospitalizations was substantial and important. However, they questioned whether the results were confounded by the changes in dietary care of patients during the study, due to differences in dose levels of UX007 and MCT oil. More specifically, the protocol-specified UX007 dose was 30% and is higher than the usual MCT dose that targets 20% but ranged from 0 to 30% in the study subjects at baseline. The change in target UX007 dose was predetermined in the

protocol based on years of prior academic work, and the range of changes in individual patients' MCT doses was very broad and not proportionally correlated with improvement. After reviewing additional information that we provided, the FDA has maintained their concern regarding whether the improvement observed with UX007 was confounded by diet. However we, as well as many investigators and patients, believe that the reduction in events of this magnitude had to be due to the UX007 drug at that dose. As a result we are continuing discussions with FDA, and when these discussions with FDA and further discussions with EMA conclude, we will determine if and what additional study may be needed. Most importantly, we believe that the submitted data from the Phase 2 study, the retrospective medical record review, the emergency IND cardiomyopathy cases, and a randomized controlled study showing an effect on cardiac function are sufficient to support early filings. These data are on par with many other rare disease products filed and approved. We are vigorously pursuing a path forward to get this potential therapy to patients as quickly as possible and recognize the importance of doing so. We expect to have an update on progress with FDA and EMA in the second half of 2018, and I look forward to sharing an update as we know more.

We also continue to make progress with our gene therapy programs, and in July we dosed the first patient in our Phase 1/2 DTX 401 study, an adeno-associated virus vector based gene therapy for the treatment of glycogen storage disease type Ia, or GSDIa. The FDA also granted Fast Track designation to the program, to help facilitate the development and expedite the review of this therapy for patients with this highly debilitating disease that can lead to severe hypoglycemia,

seizures and death. The first, lowest dose cohort of the study will enroll three patients, and we expect data in the second half of this year.

Our preclinical pipeline is also progressing nicely, and our plan continues to be to generate a new IND every one to two years from this portfolio.

With that I will turn the call over to Shalini to provide an overview of our financial results.

Shalini Sharp – Financial Results

Thank you, Emil, and good afternoon everyone. We issued a press release earlier today that included a financial update, which I will briefly summarize.

Net loss for the second quarter of 2018 was $52.7 million or $1.06 per share basic and diluted, compared with a net loss of $72.9 million or $1.72 per share basic and diluted for the second quarter of 2017. This includes a $40.3 million gain from our portion of the sale of the priority review voucher received upon Crysvita approval.

For the first half of 2018, cash used in operations was $165.6 million compared to $110 million in the first half of 2017. This includes adjustments for significant non-cash charges, including stock-based compensation expense of $38.4 million, $12.2 million in depreciation and amortization, and $5.8 million in non-cash foreign currency remeasurement losses resulting from restructuring of some of our foreign subsidiaries.

Non-cash charges such as stock-based compensation expense and depreciation and amortization expense are expected to continue to increase quarter over quarter and year over year.

For the second quarter of 2018, we reported $12.8 million in total revenue, which includes $8.9 million in revenue from our research agreement with Bayer. For Crysvita, we recognized $1.6 million in profit sharing and royalty revenue from our collaboration and license agreement with Kyowa Hakko Kirin. This includes $1.1 million in collaboration revenue in the U.S profit share territory, where Crysvita became commercially available on April 27, one month into this quarter. It also includes $0.5 million in royalty revenue in the European territory, where Crysvita received conditional marketing authorization on February 23 of this year. There were nominal net product sales for Crysvita in other regions. Mepsevii product revenue for the second quarter of 2018 was $2.0 million, and UX007 named patient revenue was $0.2 million.

At this early point in our product launches, we will not be providing any financial guidance. Emil provided you today with some metrics including patient start forms, unique prescribers and payer mix to provide insight into the Crysvita launch progress. These are metrics that we would likely only provide in the first quarters of launch, and we will continue to evaluate the appropriate time to provide these and other metrics and/or revenue guidance as we gain experience with both Crysvita and Mepsevii. As a reminder it can take 30 to 90 days to obtain reimbursement, and we are committed to putting patients on therapy without reimbursement as soon as possible.

Our total operating expenses were $107.7 million for the second quarter of 2018, including research and development costs of $76.8 million and SG&A costs of $30.7 million. Our research and development costs will continue to increase as we advance our clinical programs, including gene therapy, and preclinical translational research programs with SG&A increasing to support multiple product launched in multiple territories. We expect the proportion of our operating expenses attributable to R&D versus SG&A to remain generally consistent, and we expect total opex to continue to increase quarter over quarter for the remainder of the year.

We ended the second quarter with $547.1 million in cash, cash equivalents, and investments on the balance sheet, including the proceeds from the two PRV sales. We believe that our cash resources should be sufficient to support the initial years of launch for Mepsevii and Crysvita.

I will now turn the call back to Emil.

Emil Kakkis – Closing Comments

Thank you, Shalini. As you can see, we have made a tremendous amount of progress with the recent launches of Crysvita and Mepsevii.

In addition to our launch activities, we are advancing our clinical and translational programs. We have a number of important catalysts across our pipeline over the remaining half of 2018.

- *For Crysvita:*
- We are expecting data from the Phase 2 study of Crysvita in patients with tumor-induced osteomalacia in the second half of 2018. The co-primary endpoints in this 48 week study are change in serum phosphorus and key biopsy parameters of osteomalacia. We previously presented data showing significant increases in mean serum phosphorus levels and bone turnover markers in patients at 24 weeks, and improvements in histomorphometric indices of osteomalacia in 3 of the 4 patients with bone biopsies who had completed 48 weeks of treatment.

- *For UX007:*
- As we discussed earlier, we expect additional clarity from both FDA and EMA on potential regulatory filings for UX007 in patients with LC-FAOD in the second half of the year.
- Our Phase 3 study of UX007 in patients with Glut1DS with movement disorders is fully enrolled and on track, and we expect data in the second half of the year.

- *Turning to our gene therapy programs:*
- *For DTX301, our AAV gene therapy for the treatment of OTC deficiency:*

- All three patients are enrolled in our second dose cohort of the study, and we expect to announce data from this cohort in the second half of the year. After reviewing data from this second cohort, we have the option to move to a higher dose, or depending upon the data, we may elect to study DTX301 in an additional three patients at the same dose.

- *For DTX401, our AAV gene therapy for the treatment of GSD1a:*
- The first patient in our Phase 1/2 study was dosed last week, and we expect data from this first lowest dose cohort of patients in the second half of 2018.

I look forward to updating you throughout the year on our progress.

Let’s move to your questions. Operator, can you please provide the instructions for the Q&A portion of the call?

[Q&A]

Danielle

Thank you. This concludes our call today, and a replay will be available soon. If there are any additional questions, please contact us by phone or at ir@ultragenyx.com. Thank you for joining us.