
**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 11, 2015

Ultragenyx Pharmaceutical Inc.
(Exact name of registrant as specified in its charter)

Delaware
(State or other jurisdiction
of incorporation)

001-36276
(Commission
File Number)

27-2546083
(I.R.S. Employer
Identification No.)

60 Leveroni Court, Novato, California
(Address of principal executive offices)

94949
(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))
-
-

Item 2.02. Results of Operations and Financial Condition.

On May 11, 2015, Ultragenyx Pharmaceutical Inc. issued a press release announcing its financial results for the three months ended March 31, 2015 (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

<u>Exhibit No.</u>	<u>Description</u>
99.1	Press Release, dated May 11, 2015

* * *

- 2 -

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: May 11, 2015

Ultragenyx Pharmaceutical Inc.

By: /s/ Shalini Sharp

Shalini Sharp

Senior Vice President, Chief Financial Officer



Contact Ultragenyx Pharmaceutical Inc.
Investors & Media
Robert Anstey
844-758-7273

Ultragenyx Reports First Quarter 2015 Financial Results and Corporate Update

NOVATO, CA – May 11, 2015 – Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, today reported its financial results and corporate update for the first quarter ended March 31, 2015.

“In the first quarter we continued to execute the development plans for our broad pipeline of four clinical-stage products for six rare diseases,” commented Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. “We have also made progress in our strategy of adding incremental value within our current programs with the release of positive data from an investigator-sponsored trial in the movement disorder phenotype of Glut1 deficiency, which will lead to an expansion of that clinical program.”

First Quarter 2015 Financial Results

For the first quarter of 2015, Ultragenyx reported a net loss attributable to common stockholders of \$21.4 million, or \$0.63 per share, basic and diluted, compared with a net loss attributable to common stockholders for the first quarter of 2014 of \$18.4 million, or \$0.85 per share, basic and diluted. First quarter of 2014 net loss attributable to common stockholders differs from net loss due to dividends and other charges related to outstanding preferred stock, which was converted into common stock upon the company’s initial public offering.

Total operating expenses for the first quarter of 2015 were \$21.5 million compared with \$10.3 million for the first quarter of 2014. The increase in total operating expenses is due to the increase in clinical trial, manufacturing, other development activities, and supportive general and administrative costs as the company’s pipeline continues to advance to the late stages of development.

Cash, cash equivalents, and short-term investments were \$342.6 million as of March 31, 2015. Based on current operating levels, the company expects that existing cash, cash equivalents, and short-term investments will be sufficient to fund operations into 2018.

Recent Highlights & Upcoming Milestones

Corporate Update

- **New board member appointed.** In April 2015, Ultragenyx appointed Daniel G. Welch to its board of directors. Mr. Welch is an Executive Partner at Sofinnova Ventures and was Chairman, CEO, and President of InterMune until its acquisition by Roche in October 2014.

KRN23 anti-FGF23 Monoclonal Antibody in X-Linked Hypophosphatemia (XLH)

- **Development in pediatric and adult XLH advancing on schedule.** Preliminary phosphate and safety data at 16 weeks from the Phase 2 study in 36 pediatric patients are expected in the second quarter of 2015. Initiation of a randomized, placebo-controlled study in adult patients is expected in the second half of 2015.
- **Long-term extension study of KRN23 in adult XLH patients initiated.** Patients who had previously participated in the Phase 1 and Phase 1/2 studies of KRN23 completed by our collaborative partner Kyowa Hakko Kirin are being re-enrolled in a long-term Phase 2b extension study. Enrollment has begun, and multiple patients have resumed receiving KRN23 monthly injections.

KRN23 anti-FGF23 Monoclonal Antibody in Tumor-Induced Osteomalacia (TIO)

- **Interim Phase 2 data expected late 2015 or early 2016.** The Phase 2 study of KRN23 in TIO continues to enroll and treat patients. In an effort to address the needs of patients with significant bone disease, additional sites are being added in order to lessen the travel requirements.

rhGUS in Mucopolysaccharidosis 7 (MPS 7)

- **Pivotal Phase 3 study in MPS 7 continues to enroll patients.** Agreement with the U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA) was reached for the single pivotal Phase 3 study design. Data are expected in the first half of 2016.
- **Initiation of new clinical study in MPS 7 patients under five years of age expected in mid-2015.** The study is designed to include MPS 7 patients with non-immune hydrops fetalis (NIHF), a severe neonatal condition in which patients retain a significant amount of fluid and that leads to death in early infancy in more than half of patients. The NIHF phenotype of MPS 7 likely contributes to the rarity of the disease. Currently, one four-month old patient is receiving infusions under an emergency investigational new drug (IND) application and may later be transferred to the new study.

Triheptanoin in Glut1 Deficiency Syndrome (Glut1 DS) and Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD)

- **Positive data from investigator-sponsored trial in Glut1 DS presented at American Academy of Neurology (AAN) Annual Meeting.** Treatment with triheptanoin resulted in a 90% reduction in the number of paroxysmal motor events in six patients with movement disorders associated with Glut1 DS ($p=0.028$). After withdrawal of triheptanoin, the number of events increased substantially ($p=0.043$). In addition, improvements in the clinical global impression scale and normalization of induction of brain energy metabolism during visual stimulation were observed after treatment. Triheptanoin was well tolerated in all patients. Two patients were considered not compliant for reasons unrelated to safety and were excluded from the analysis.
- **Company-sponsored study in Glut1 DS movement disorders planned.** Based on the results presented at AAN, Ultragenyx intends to initiate a randomized, double-blind, placebo-controlled study of triheptanoin in this phenotype. The study is currently being designed, and discussions with regulators are anticipated to commence in the second half of 2015.
- **Additional orphan drug designations received.** The FDA granted orphan drug designation for triheptanoin in LC-FAOD, and the EMA Committee for Orphan Medicinal Products granted orphan medicinal product designation for Glut1 DS. These new designations are in addition to the orphan drug designation granted by the FDA for Glut1 DS in October 2014.

Sialic Acid Extended-Release (SA-ER) in Hereditary Inclusion Body Myopathy (HIBM)

- **Late-stage development and regulatory plan on track.** Progress continues to be made toward both key late-stage milestones for the SA-ER program. The initiation of a global Phase 3 study is expected in mid-2015. A conditional marketing authorization filing with the EMA for stabilization or slowing of decline in upper extremity muscle strength is planned for the second half of 2015.

Conference Call & Webcast Information

Ultragenyx will host a conference call today, Monday, May 11, 2015 at 5pm ET, to discuss the first quarter 2015 financial results and corporate update. The live and replayed webcast of the call will be available through the company's website at <http://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 3243-3559. The replay of the call will be available for one year.

About Ultragenyx

Ultragenyx is a clinical-stage biopharmaceutical company committed to bringing to market novel products for the treatment of rare and ultra-rare diseases, with a focus on serious, debilitating genetic diseases. Founded in 2010, the company has rapidly built a diverse portfolio of product candidates with the potential to address diseases for which the unmet medical need is high, the biology for treatment is clear, and for which there are no approved therapies.

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 regarding Ultragenyx's expectations regarding sufficiency of existing cash, cash equivalents and short-term investments to fund operations for projected periods of time, timing of release of additional data for its product candidates, plans to initiate additional studies for its product candidates and timing regarding these studies, plans regarding ongoing studies for existing programs and intent to file for conditional approval, 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, 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 uncertainties inherent in the clinical drug development process, including the regulatory approval process, the timing of our regulatory filings and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations and the availability or commercial potential of our 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 the company in general, see Ultragenyx's Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 27, 2015, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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

	Three Months Ended March 31,	
	2015	2014
Statements of Operations Data:		
Operating expenses:		
Research and development	\$ 17,364	\$ 8,353
General and administrative	4,138	1,986
Total operating expenses	21,502	10,339
Loss from operations	(21,502)	(10,339)
Other income (expense)	123	(3,291)
Net loss	\$ (21,379)	\$ (13,630)
Net loss attributable to common stockholders	\$ (21,379)	\$ (18,438)
Net loss per share attributable to common stockholders, basic and diluted	\$ (0.63)	\$ (0.85)
Shares used to compute net loss per share attributable to common stockholders, basic and diluted	34,008,830	21,582,435

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

	March 31,	December 31,
	2015	2014
Balance Sheet Data:		
Cash, cash equivalents and short-term investments	\$ 342,566	\$ 187,487
Working capital	336,271	180,899
Total assets	355,840	197,967
Total stockholders' equity	341,119	184,945