Ultragenyx Pharmaceutical Inc.
Form 10-Q
November 03, 2017

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934.
For the quarterly period ended September 30, 2017

OR

TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934.
For the transition period from to .

Commission File No. 001-36276

ULTRAGENYX PHARMACEUTICAL INC.

(Exact name of registrant as specified in its charter)

| | |
|---|---|
| Delaware | 27-2546083 |
| (State or other jurisdiction of incorporation or organization) | (I.R.S. Employer Identification No.) |

| | |
|---|---|
| 60 Leveroni Court | |
| Novato, California | 94949 |
| (Address of principal executive offices) | (Zip Code) |

(415) 483-8800

(Registrant's telephone number, including area code)

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days.
YES NO

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Website, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). YES NO

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act. (Check one):

| | | |
|---|---|---|
| Large accelerated filer | | Accelerated filer |
| Non-accelerated filer | (Do not check if a smaller reporting company) | Smaller reporting company |
| 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.

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act).
YES NO

As of October 30, 2017, the registrant had 42,589,056 shares of common stock issued and outstanding.

ULTRAGENYX PHARMACEUTICAL INC.

FORM 10-Q FOR THE QUARTER ENDED SEPTEMBER 30, 2017

INDEX


| | | | Page |
|---|---|---|---|
| CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS | | | 1 |
| Part I – Financial Information | | | |
| | Item 1. | Condensed Consolidated Financial Statements – Unaudited | |
| | | Condensed Consolidated Balance Sheets | 2 |
| | | Condensed Consolidated Statements of Operations | 3 |
| | | Condensed Consolidated Statements of Comprehensive Loss | 4 |
| | | Condensed Consolidated Statements of Cash Flows | 5 |
| | | Notes to Condensed Consolidated Financial Statements | 6 |
| | Item 2. | Management's Discussion and Analysis of Financial Condition and Results of Operations | 13 |
| | Item 3. | Quantitative and Qualitative Disclosures About Market Risk | 20 |
| | Item 4. | Controls and Procedures | 20 |
| Part II – Other Information | | | |
| | Item 1. | Legal Proceedings | 22 |
| | Item 1A. | Risk Factors | 22 |
| | Item 2. | Unregistered Sales of Equity Securities and Use of Proceeds | 50 |
| | Item 3. | Defaults Upon Senior Securities | 50 |
| | Item 4. | Mine Safety Disclosures | 51 |
| | Item 5. | Other Information | 51 |
| | Item 6. | Exhibits | 51 |
| | Signatures | | 52 |

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q (the "Quarterly Report") contains forward-looking statements that involve risks and uncertainties. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this Quarterly Report are forward-looking statements. In some cases, you can identify forward-looking statements by words such as "anticipate," "believe," "contemplate," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "seek," "should," "target," "will," "would," or the negative of these w comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- our expectations regarding the timing of clinical study commencements and reporting results from same;
- the timing and likelihood of regulatory approvals for our product candidates;
- the anticipated indications for our product candidates, if approved;
- the potential market opportunities for commercializing our product candidates;
- our expectations regarding the potential market size and the size of the patient populations for our product candidates, if approved for commercial use;
- estimates of our expenses, future revenue, capital requirements, and our needs for additional financing;
- our ability to develop, acquire, and advance product candidates into, and successfully complete, clinical studies;
- the implementation of our business model and strategic plans for our business and product candidates and the integration and performance of any businesses we acquire;
- the initiation, timing, progress, and results of ongoing and future preclinical and clinical studies, and our research and development programs;
- the scope of protection we are able to establish and maintain for intellectual property rights covering our product candidates;
- our ability to maintain and establish collaborations or obtain additional funding;
- our ability to maintain and establish relationships with third parties, such as contract research organizations, suppliers, and distributors;
- our financial performance and the expansion of our organization;
- our ability to obtain supply of our product candidates;
- developments and projections relating to our competitors and our industry; and
- other risks and uncertainties, including those listed under Part II, Item 1A. Risk Factors.

Any forward-looking statements in this Quarterly Report reflect our current views with respect to future events or to our future financial performance and involve known and unknown risks, uncertainties, and other factors that may cause our actual results, performance, or achievements to be materially different from any future results, performance, or achievements expressed or implied by these forward-looking statements. Factors that may cause actual results to differ materially from current expectations include, among other things, those discussed under Part II, Item 1A. Risk Factors and discussed elsewhere in this Quarterly Report. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

This Quarterly Report also contains estimates, projections, and other information concerning our industry, our business, and the markets for certain diseases, including data regarding the estimated size of those markets, and the incidence and prevalence of certain medical conditions. Information that is based on estimates, forecasts, projections, market research, or similar methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances reflected in this information. Unless otherwise expressly stated, we obtained this industry, business, market, and other data from, or derived such data based on information in, reports, research surveys, studies, and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data, and similar sources.

PART I. FINANCIAL INFORMATION

Item 1. Financial Statements

ULTRAGENYX PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

(Unaudited)

(In thousands, except share amounts)

| | September 30, 2017 | December 31, 2016 |
|---|---|---|
| Assets | | |
| Current assets: | | |
| Cash and cash equivalents | $60,407 | $ 161,120 |
| Short-term investments | 310,659 | 219,028 |
| Restricted cash | 461 | 1,411 |
| Prepaid expenses and other current assets | 19,376 | 20,136 |
| Total current assets | 390,903 | 401,695 |
| Property and equipment, net | 16,030 | 17,055 |
| Restricted cash | 1,805 | 2,076 |
| Long-term investments | 24,964 | 117,963 |
| Other assets | 1,345 | 1,837 |
| Total assets | $435,047 | $ 540,626 |
| | | |
| Liabilities and Stockholders' Equity | | |
| Current liabilities: | | |
| Accounts payable | $9,288 | $ 5,364 |
| Accrued liabilities | 54,278 | 54,554 |
| Deferred rent—current portion | 695 | 341 |
| Total current liabilities | 64,261 | 60,259 |
| Other liabilities | 5,228 | 6,393 |
| Total liabilities | 69,489 | 66,652 |
| Stockholders' equity: | | |
| Preferred stock — 25,000,000 shares authorized; nil outstanding as of September 30, 2017 and December 31, 2016 | — | — |
| Common stock — 250,000,000 shares authorized; 42,488,312 and 41,240,230 shares issued and outstanding as of September 30, 2017 and December 31, 2016, respectively | 42 | 41 |
| Additional paid-in capital | 1,123,861 | 1,003,561 |
| Accumulated other comprehensive income (loss) | (7,404) | 905 |
| Accumulated deficit | (750,941) | (530,533) |
| Total stockholders' equity | 365,558 | 473,974 |

| | | |
|---|---|---|
| Total liabilities and stockholders' equity | $435,047 | $ 540,626 |

See accompanying notes.

ULTRAGENYX PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

(Unaudited)

(In thousands, except share and per share amounts)

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|---|---|---|---|---|
| | 2017 | 2016 | 2017 | 2016 |
| Revenue | $198 | $111 | $198 | $128 |
| Operating expenses: | | | | |
| Research and development | 60,412 | 48,711 | 170,117 | 132,458 |
| General and administrative | 23,499 | 17,183 | 62,189 | 45,128 |
| Total operating expenses | 83,911 | 65,894 | 232,306 | 177,586 |
| Loss from operations | (83,713) | (65,783) | (232,108) | (177,458) |
| Other income (expense), net: | | | | |
| Interest income | 1,117 | 922 | 3,350 | 2,877 |
| Other income (expense), net | 3,373 | (46) | 8,368 | (6) |
| Total other income (expense), net | 4,490 | 876 | 11,718 | 2,871 |
| Loss before income taxes | (79,223) | (64,907) | (220,390) | (174,587) |
| Income tax provision | (4) | — | (18) | — |
| Net loss | $(79,227) | $(64,907) | $(220,408) | $(174,587) |
| Net loss per share, basic and diluted | $(1.87) | $(1.64) | $(5.22) | $(4.46) |
| Shares used in computing net loss per share, basic and diluted | 42,471,606 | 39,551,923 | 42,222,413 | 39,184,994 |

See accompanying notes.

ULTRAGENYX PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS

(Unaudited)

(In thousands)

| | Three Months Ended September 30, | | Nine Months Ended September 30, | |
|---|---|---|---|---|
| | 2017 | 2016 | 2017 | 2016 |
| Net loss | $(79,227) | $(64,907) | $(220,408) | $(174,587) |
| Other comprehensive income (loss): | | | | |
| Foreign currency translation adjustments | (3,326) | (28) | (8,377) | (17) |
| Unrealized gain (loss) on available-for-sale securities | 145 | (212) | 68 | 897 |
| Other comprehensive income (loss): | (3,181) | (240) | (8,309) | 880 |
| Total comprehensive loss | $(82,408) | $(65,147) | $(228,717) | $(173,707) |

See accompanying notes.

ULTRAGENYX PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

(Unaudited)

(In thousands)

| | Nine Months Ended September 30, | |
|---|---|---|
| | 2017 | 2016 |
| Operating activities: | | |
| Net loss | $(220,408) | $(174,587) |
| Adjustments to reconcile net loss to net cash used in operating activities: | | |
| Stock-based compensation | 48,473 | 34,771 |
| Amortization of premium on investment securities, net | 1,451 | 4,230 |
| Depreciation and amortization | 3,311 | 2,267 |
| Non-cash license fee from collaboration arrangement | — | 700 |
| Foreign currency remeasurement gain | (8,431) | — |
| Changes in operating assets and liabilities: | | |
| Prepaid expenses and other current assets | 760 | (5,833) |
| Other assets | 492 | (1,273) |
| Accounts payable | 3,879 | 3,842 |
| Accrued liabilities and other liabilities | (1,547) | 22,588 |
| Net cash used in operating activities | (172,020) | (113,295) |
| Investing activities: | | |
| Purchase of property and equipment | (1,894) | (9,522) |
| Purchase of investments | (230,490) | (311,523) |
| Proceeds from the sale of investments | 27,642 | 94,289 |
| Proceeds from maturities of investments | 202,833 | 326,228 |
| (Increase) decrease in restricted cash | 1,221 | (1,202) |
| Net cash provided by (used in) investing activities | (688) | 98,270 |
| Financing activities: | | |
| Proceeds from issuance of common stock in connection with at-the-market offering, net | 67,616 | 33,700 |
| Proceeds from issuance of common stock in connection with collaboration agreement, net | — | 26,362 |
| Proceeds from issuance of common stock from equity awards, net | 4,212 | 3,674 |
| Net cash provided by financing activities | 71,828 | 63,736 |
| Effect of exchange rate changes on cash | 167 | — |
| Net increase (decrease) in cash and cash equivalents | (100,713) | 48,711 |
| Cash and cash equivalents at beginning of period | 161,120 | 93,569 |
| Cash and cash equivalents at end of period | $60,407 | $142,280 |

See accompanying notes.

5

ULTRAGENYX PHARMACEUTICAL INC.

Notes to Condensed Consolidated Financial Statements

1. Organization

Ultragenyx Pharmaceutical Inc. (the Company) is a biopharmaceutical company and was incorporated in California on April 22, 2010. The Company subsequently reincorporated in the state of Delaware in June 2011.

The Company is focused on the identification, acquisition, development, and commercialization of novel products for the treatment of rare and ultra-rare diseases, with a focus on serious, debilitating genetic diseases. The Company has completed a Phase 3 study of vestronidase alfa (recombinant human beta-glucuronidase or rhGUS) in patients with mucopolysaccharidosis 7 (MPS 7), a rare lysosomal storage disease, and is conducting Phase 2 and Phase 3 studies of burosumab (KRN23 or UX023), an antibody targeting fibroblast growth factor 23 (FGF23), in pediatric and adult patients with X-linked hypophosphatemia (XLH) and a Phase 2 study in tumor induced osteomalacia (TIO), both rare diseases that impair bone mineralization; a Phase 3 study for UX007 in patients with glucose transporter type-1 deficiency syndrome (Glut1 DS), a brain energy deficiency, who are experiencing movement disorders; and a Phase 2 clinical study of UX007 in patients severely affected by long-chain fatty acid oxidation disorders (LC-FAOD), a genetic disorder in which the body is unable to convert long chain fatty acids into energy. The Company operates as one reportable segment.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying unaudited condensed consolidated financial statements include the amounts of the Company and our wholly-owned subsidiaries and have been prepared in accordance with U.S. generally accepted accounting principles (GAAP) for interim financial information and in accordance with the instructions to Form 10-Q and Rule 10-01 of Regulation S-X. Accordingly, they do not include all of the information and footnotes required by GAAP for complete financial statements. The unaudited interim consolidated financial statements have been prepared on the same basis as the annual financial statements. In the opinion of management, the accompanying unaudited condensed consolidated financial statements reflect all adjustments (consisting only of normal recurring adjustments) considered necessary for a fair presentation. These financial statements should be read in conjunction with the audited financial statements and notes thereto for the preceding fiscal year contained in the Company’s Annual Report on Form 10-K filed on February 17, 2017 with the United States Securities and Exchange Commission (SEC).

The results of operations for the three and nine months ended September 30, 2017 are not necessarily indicative of the results to be expected for the year ending December 31, 2017. The condensed consolidated balance sheet as of December 31, 2016 has been derived from audited financial statements at that date, but does not include all of the information required by GAAP for complete financial statements.

Use of Estimates

The accompanying consolidated financial statements have been prepared in accordance with GAAP. The preparation of the consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent liabilities and the reported amounts of expenses in the consolidated financial statements and the accompanying notes. On an ongoing basis, management evaluates its estimates, including those related to clinical trial accruals, fair value of assets and liabilities, income taxes, and stock-based compensation. Management bases its estimates on historical experience and

on various other market-specific and relevant assumptions that management believes to be reasonable under the circumstances. Actual results could differ from those estimates.

Revenue Recognition

In May 2014, the Financial Accounting Standards Board (FASB), issued Accounting Standards Update (ASU) 2014-09, Revenue from Contracts with Customers (ASC 606), to supersede nearly all existing revenue recognition guidance under GAAP. The core principle of ASU 2014-09 is to recognize revenues when promised goods or services are transferred to customers in an amount that reflects the consideration that is expected to be received for those goods or services. ASU 2014-09 defines a five-step process to achieve this core principle and, in doing so, it is possible more judgment and estimates may be required within the revenue recognition process than are required under existing GAAP, including identifying performance obligations in a contract, estimating the amount of variable consideration to include in the transaction price and allocating the transaction price to each separate performance obligation. In March, April, May and December 2016, the FASB issued ASU 2016-08, Revenue from Contracts with Customers: Principal versus Agent Considerations, ASU 2016-10, Revenue from Contracts with Customers: Identifying Performance Obligations and Licensing, ASU 2016-12, Revenue from Contracts with Customers: Narrow-Scope Improvements and Practical Expedients to provide supplemental adoption guidance and clarification to ASU 2014-09, and ASU 2016-20, Technical Corrections and Improvements to Topic 606, Revenue from Contracts with Customers, respectively. The effective date for these new standards is the same as the effective date and transition requirements for ASU 2014-09. The Company has early adopted the new revenue standard as of January 1, 2017 using a full retrospective application to each prior reporting period presented. Through January 1, 2017, the

Company had recorded a cumulative inception to date total of $0.1 million of revenues. The adoption did not have an effect on the Consolidated Financial Statements on the adoption date and no adjustment to prior year consolidated financial statements was required.

Product sales revenue

The Company recognizes revenue from sales of vestronidase alfa (UX003) on a “named patient” basis, which is allowed in certain countries prior to the commercial approval of the product in the territory. Under ASC 606, revenue from product sales is recognized at the point in time when the product is shipped to the customer. Prior to recognizing revenue, the Company makes estimates of the transaction price, including variable consideration that is subject to a constraint. Amounts of variable consideration are included in the transaction price to the extent that it is probable that a significant reversal in the amount of cumulative revenue recognized will not occur when the uncertainty associated with the variable consideration is subsequently resolved.

License and collaboration agreements

The Company has license and collaboration agreements with Kyowa Hakko Kirin Co., Ltd. (KHK) and Takeda Pharmaceutical Company Limited (Takeda). These license and collaboration agreements are within the scope of ASC 808, Collaborative Agreements, which provides guidance on the presentation and disclosure of collaborative arrangements. Funding received related to research and development services and pre-commercialization costs are classified as a reduction of research and development expenses and general and administrative expenses, respectively in the consolidated statement of operations because the provision of such services for collaborative partners are not considered to be part of the Company’s ongoing major or central operations.

Stock-Based Compensation

In March 2016, the FASB issued ASU 2016-09, Compensation — Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting, which simplifies several aspects of the accounting for employee share-based payments, including income tax consequences, application of award forfeitures to expense, classification on the statement of cash flows, and classification of awards as either equity or liabilities. The Company adopted ASU 2016-09 as of January 1, 2017. On January 1, 2017, there was $19.7 million of cumulative unrecognized excess tax benefits which was fully offset by a corresponding increase in the valuation allowance. The adoption did not have any other impact on the Consolidated Financial Statements on the adoption date.

Recently Issued Accounting Pronouncements

In February 2016, the FASB issued ASU No. 2016-02, Leases, which requires an entity that is a lessee to record a right of use asset and a corresponding lease liability on the balance sheet for all leases with terms longer than 12 months. This guidance also requires disclosures about the amount, timing, and uncertainty of cash flows arising from leases. This guidance is effective for annual reporting periods beginning after December 15, 2018, and interim periods within those annual periods, using a modified retrospective approach, and early adoption is permitted. The Company is evaluating the effect that this guidance will have on its Consolidated Financial Statements and related disclosures.

In October 2016, the FASB issued ASU 2016-16, Income Taxes - Intra-Entity Transfers of Assets Other Than Inventory, which requires entities to recognize the income tax consequences of an intra-entity transfer of an asset other than inventory when the transfer occurs. The standard is effective for fiscal years beginning after December 15, 2017, including interim periods within those fiscal years. Early adoption is permitted as of the beginning of a fiscal year.

The new standard must be adopted using a modified retrospective transition method which is a cumulative-effective adjustment to retained earnings as of the beginning of the first effective reporting period. The Company is evaluating the effect that this guidance will have on its Consolidated Financial Statements and related disclosures.

3. Fair Value Measurements

Financial assets and liabilities are recorded at fair value. The carrying amount of certain financial instruments, including cash and cash equivalents, accounts payable and accrued liabilities approximate fair value due to their relatively short maturities. Assets and liabilities recorded at fair value on a recurring basis in the balance sheets are categorized based upon the level of judgment associated with the inputs used to measure their fair values. Fair value is defined as the exchange price that would be received for an asset or an exit price that would be paid to transfer a liability in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. The authoritative guidance on fair value measurements establishes a three-tier fair value hierarchy for disclosure of fair value measurements as follows:

Level 1—Inputs are unadjusted, quoted prices in active markets for identical assets or liabilities at the measurement date;

Level 2—Inputs are observable, unadjusted quoted prices in active markets for similar assets or liabilities, unadjusted quoted prices for identical or similar assets or liabilities in markets that are not active, or other inputs that are observable or can be corroborated by observable market data for substantially the full term of the related assets or liabilities; and

Level 3—Unobservable inputs that are significant to the measurement of the fair value of the assets or liabilities that are supported by little or no market data.

The following tables set forth the fair value of the Company's financial assets remeasured on a recurring basis based on the three-tier fair value hierarchy (in thousands):

| | September 30, 2017 | | | |
|---|---|---|---|---|
| | Level 1 | Level 2 | Level 3 | Total |
| Financial Assets: | | | | |
| Money market funds | $42,229 | $— | $ — | $42,229 |
| Corporate bonds | — | 127,486 | | |