Ultragenyx Pharmaceutical Inc.
Form 10-Q
May 12, 2014

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

x QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934.
For the quarterly period ended March 31, 2014

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 and 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 x NO ¨

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, 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 x NO ¨

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

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

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

As of May 7, 2014, the registrant had 30,035,894 shares of common stock issued and outstanding.

ULTRAGENYX PHARMACEUTICAL INC.

FORM 10-Q FOR THE QUARTER ENDED MARCH 31, 2014

INDEX

			Page
CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS			
Part I – Financial Information			
	Item 1.	Condensed Financial Statements – Unaudited	
		Condensed Balance Sheets	2
		Condensed Statements of Operations	3
		Condensed Statements of Comprehensive Loss	4
		Condensed Statements of Cash Flows	5
		Notes to Condensed Financial Statements	6
	Item 2.	Management's Discussion and Analysis of Financial Condition and Results of Operations	15
	Item 3.	Quantitative and Qualitative Disclosures About Market Risk	24
	Item 4.	Controls and Procedures	24
Part II – Other Information			
	Item 1.	Legal Proceedings	25
	Item 1A.	Risk Factors	25
	Item 2.	Unregistered Sales of Equity Securities and Use of Proceeds	52
	Item 3.	Defaults Upon Senior Securities	52
	Item 4.	Mine Safety Disclosures	52
	Item 5.	Other Information	52
	Item 6.	Exhibits	53
	Signatures		54

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q 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 on Form 10-Q 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 commencing our clinical studies and reporting results from same;
- the timing and likelihood of regulatory approvals for our product candidates;
- 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;
- the initiation, timing, progress, and results of future preclinical studies 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 expectations regarding the time during which we will be an emerging growth company under the JOBS Act;
- our financial performance and 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 on Form 10-Q 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 listed under Part II, Item 1A. Risk Factors and discussed elsewhere in this Quarterly Report on Form 10-Q. 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 on Form 10-Q 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 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.

1

ULTRAGENYX PHARMACEUTICAL INC.

(A Development Stage Company)

CONDENSED BALANCE SHEETS

(In thousands, except share and per share amounts)

	March 31, 2014 (Unaudited)	December 31, 2013
Assets		
Current assets:		
Cash and cash equivalents	$ 55,447	$ 7,427
Short-term investments	109,950	45,950
Prepaid expenses and other current assets	4,239	1,848
Total current assets	169,636	55,225
Property and equipment, net	1,777	1,325
Restricted cash	744	451
Other assets	550	2,648
Total assets	$ 172,707	$ 59,649
Liabilities, Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Current liabilities:		
Accounts payable	$ 3,495	$ 1,437
Accrued liabilities	3,238	4,406
Deferred rent—current portion	78	78
Total current liabilities	6,811	5,921
Convertible preferred stock warrant liability	—	3,419
Other liabilities	182	200
Total liabilities	6,993	9,540
Commitments and contingencies (Note 10)	—	—
Series A redeemable convertible preferred stock, par value of $0.001 per share—nil and 35,377,566 shares authorized; nil and 34,349,894 shares issued and outstanding as of March 31, 2014 and December 31, 2013	—	51,001
Series B convertible preferred stock, par value of $0.001 per share—nil and 27,081,680 shares authorized, issued and outstanding as of March 31, 2014 and December 31, 2013	—	73,929
Stockholders' equity (deficit):		
Preferred stock, par value of $0.001 per share—25,000,000 shares authorized; nil outstanding as of March 31, 2014 and December 31, 2013	—	—
Common stock, par value of $0.001 per share—250,000,000 shares authorized;	30	4

30,049,650 and 3,766,289 shares issued and outstanding as of March 31, 2014 and December 31, 2013		
Additional paid-in capital	258,599	—
Accumulated other comprehensive income (loss)	(46)	11
Deficit accumulated during the development stage	(92,869)	(74,836)
Total stockholders' equity (deficit)	165,714	(74,821)
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	$ 172,707	$ 59,649

See accompanying notes.

ULTRAGENYX PHARMACEUTICAL INC.

(A Development Stage Company)

CONDENSED STATEMENTS OF OPERATIONS

(Unaudited)

(In thousands, except share and per share amounts)

	Three Months Ended March 31,		Period from April 22, 2010 (Inception) Through March 31,
	2014	2013	2014
Operating expenses:			
Research and development	$8,353	$5,664	$ 54,499
General and administrative	1,986	1,083	11,916
Total operating expenses	10,339	6,747	66,415
Loss from operations	(10,339)	(6,747)	(66,415)
Other income (expense), net:			
Interest income	93	26	314
Interest expense	—	—	(318)
Other expense, net	(3,384)	(14)	(6,770)
Total other income (expense), net	(3,291)	12	(6,774)
Net loss	$(13,630)	$(6,735)	$ (73,189)
Net loss attributable to common stockholders	$(18,438)	$(8,205)	
Net loss per share attributable to common stockholders, basic and diluted	$(0.85)	$(2.84)	
Shares used in computing net loss per share attributable to common stockholders,			
basic and diluted	21,582,435	2,893,997	

See accompanying notes.

ULTRAGENYX PHARMACEUTICAL INC.

(A Development Stage Company)

CONDENSED STATEMENTS OF COMPREHENSIVE LOSS

(Unaudited)

(In thousands)

	Three Months Ended March 31,		Period from April 22, 2010 (Inception) Through March 31,
	2014	2013	2014
Net loss	$(13,630)	$(6,735)	$(73,189)
Other comprehensive income:			
Unrealized loss on available-for-sale securities	(57)	—	(46)
Total comprehensive loss	$(13,687)	$(6,735)	$(73,235)

See accompanying notes.

4

(A Development Stage Company)

CONDENSED STATEMENTS OF CASH FLOWS

(Unaudited)(In thousands)

	Three Months Ended March 31,		Period from April 22, 2010 (Inception) Through March 31,
	2014	2013	2014
Operating activities:			
Net loss	$(13,630)	$(6,735)	$(73,189)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	117	124	914
Noncash interest expense	—	—	318
Amortization of premium (discount) on investment securities	479	(6)	1,892
Stock-based compensation	795	191	2,597
Revaluation of convertible preferred stock warrant liability	3,324	(2)	6,540
Changes in operating assets and liabilities:			
Prepaid expenses and other current assets	(2,391)	(776)	(4,239)
Other assets	2,098	(96)	(550)
Accounts payable	2,058	1,339	3,495
Accrued liabilities and other liabilities	(1,186)	(332)	3,498
Net cash used in operating activities	(8,336)	(6,293)	(58,724)
Investing activities:			
Purchase of property and equipment	(569)	(177)	(2,691)
Purchase of investments	(87,998)	(14,923)	(151,951)
Proceeds from maturities of investments	23,462	—	40,063
Increase in restricted cash	(293)	—	(744)
Net cash used in investing activities	(65,398)	(15,100)	(115,323)
Financing activities:			
Net proceeds from issuance of convertible preferred stock	—	—	103,888
Net proceeds from issuance of common stock	126,100	1	126,402
Proceeds from issuance of promissory notes	—	—	3,550
Payment of preferred stock dividend	(4,346)	—	(4,346)
Net cash provided by financing activities	121,754	1	229,494
Net increase (decrease) in cash and cash equivalents	48,020	(21,392)	55,447
Cash and cash equivalents at beginning of period	7,427	86,190	—
Cash and cash equivalents at end of period	$55,447	$64,798	$55,447
Supplemental disclosures of non-cash investing and financing information:			
Issuance of convertible preferred stock warrants	$—	$—	$202
Issuance of Series A redeemable convertible preferred stock in lieu			

of cash dividend	$—	$—	$2,070
Reclassification of warrant liability to equity upon conversion to common stock warrants	$6,743	$—	$6,743
Conversion of interest accrued on promissory notes into Series A redeemable convertible preferred stock	$—	$—	$114
Conversion of promissory notes into Series A redeemable convertible preferred stock	$—	$—	$3,550
Conversion of Series A and Series B preferred stock to common stock	$129,360	$—	$129,360

See accompanying notes.

ULTRAGENYX PHARMACEUTICAL INC.

(A Development Stage Company)

Notes to Condensed Financial Statements

1. Organization

Ultragenyx Pharmaceutical Inc. (the Company) is a development stage biotechnology 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 an initial focus on serious and debilitating metabolic genetic diseases. The Company is currently conducting a Phase 2 extension study of sialic acid, extended release (SA-ER) in patients with hereditary inclusion body myopathy (HIBM), a progressive muscle-wasting disorder; a Phase 1/2 study of recombinant human beta-glucuronidase (rhGUS) in patients with mucopolysaccharidosis 7, or MPS 7, a rare lysosomal storage disease; a Phase 2 clinical study for triheptanoin for the treatment of patients with glucose transporter type-1 deficiency syndrome (Glut1 DS), a brain energy deficiency; and a Phase 2 clinical study of triheptanoin, 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 has also entered into a collaboration and license agreement with Kyowa Hakko Kirin Co., Ltd. (KHK) for KRN23, an antibody targeting fibroblast growth factor 23, or FGF23, intended for the treatment of X-linked hypophosphatemia, or XLH, a rare genetic disease that impairs bone growth.

On January 30, 2014, the Company’s registration statements on Form S-1 (File Nos. 333-192244 and 333-193675) relating to its initial public offering (IPO) of its common stock were declared effective by the Securities and Exchange Commission (SEC). The shares began trading on The NASDAQ Global Select Market on January 31, 2014. The public offering price of the shares sold in the offering was $21.00 per share. The IPO closed on February 5, 2014 and included 6,624,423 shares of common stock, which included 864,054 shares of common stock issued pursuant to the over-allotment option granted to the underwriters. The Company received total proceeds from the offering of $129.4 million, net of underwriting discounts and commissions of $9.7 million. After deducting offering expenses of approximately $3.3 million and a cash dividend of $4.3 million, which was paid to the preferred stockholders on the closing date, net proceeds were approximately $121.7 million. Upon the closing of the IPO, all shares of convertible preferred stock then outstanding converted into 19,598,486 shares of common stock and the Series A convertible preferred stock warrants were converted into warrants to purchase common stock.

Upon the effectiveness of the Amended and Restated Certificate of Incorporation of the Company on February 5, 2014, the number of shares of capital stock the Company is authorized to issue was increased to 275,000,000 shares, of which 250,000,000 shares are common stock and 25,000,000 shares are preferred stock. Both the common stock and preferred stock have a par value of $0.001 per share. There are no shares of preferred stock outstanding at March 31, 2014.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying unaudited condensed consolidated financial statements have been prepared in accordance with U.S. general accepted accounting principles ("U.S. 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 generally accepted accounting principles 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 March 24, 2014 with the SEC.

The results of operations for the three months ended March 31, 2014 are not necessarily indicative of the results to be expected for the year ending December 31, 2014. The condensed balance sheet as of December 31, 2013 has been derived from audited financial statements at that date but does not include all of the information required by U.S. GAAP for complete financial statements.

Reverse Stock Split

In January 2014, the Company's board of directors and its stockholders approved an amendment to the Company's amended and restated certificate of incorporation to effect a reverse split of shares of the Company's common stock on a 1-for-3.1345 basis (the "Reverse Stock Split"). The par values and the authorized shares of the common and convertible preferred stock were not adjusted as a result of the Reverse Stock Split, nor were the outstanding shares of preferred stock. All issued and outstanding common stock and related per share amounts contained in the financial statements have been retroactively adjusted to reflect this Reverse Stock Split for all periods presented. A proportional adjustment to the conversion ratio for each series of convertible preferred stock was also effected in connection with the Reverse Stock Split. The Reverse Stock Split was effected on January 17, 2014.

Use of Estimates

The preparation of condensed financial statements in conformity with U.S. 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 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, convertible preferred stock and related warrants, common stock, 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.

Cash and Cash Equivalents

The Company considers all highly liquid investments with original maturities of three months or less from the date of purchase to be cash equivalents. Cash equivalents consist primarily of amounts invested in money market accounts.

Short-Term Investments

All investments have been classified as “available-for-sale” and are carried at estimated fair value as determined based upon quoted market prices or pricing models for similar securities. Management determines the appropriate classification of its investments in debt securities at the time of purchase and reevaluates such designation as of each balance sheet date. Unrealized gains and losses are excluded from earnings and were reported as a component of comprehensive loss. Realized gains and losses and declines in fair value judged to be other than temporary, if any, on available-for-sale securities are included in interest income and other expense, respectively. The cost of securities sold is based on the specific-identification method. Interest on marketable securities is included in interest income.

Deferred Offering Costs

Deferred offering costs, which primarily consist of direct incremental accounting, legal and printing fees relating to the IPO, were initially capitalized. The deferred offering costs of $3.3 million were subsequently offset against IPO proceeds upon the completion of the IPO in February of 2014. As of December 31, 2013, $2.3 million of deferred offering costs were capitalized and included in prepaid and other current assets on the balance sheet.

Concentration of Credit Risk and Other Risks and Uncertainties

Financial instruments that potentially subject the Company to a concentration of credit risk consist of cash, cash equivalents, and marketable securities. The Company’s cash, cash equivalents, and short-term investments are held by financial institutions that management believes are of high credit quality. The Company’s investment policy limits investments to fixed income securities denominated and payable in U.S. dollars such as U.S. government obligations, money market instruments and funds, corporate bonds, and asset-backed securities and places restrictions on maturities and concentrations by type and issuer. Such deposits may, at times, exceed federally insured limits. The Company has not experienced any losses on its deposits of cash and cash equivalents and its accounts are monitored by management to mitigate risk. The Company is exposed to credit risk in the event of default by the financial institutions holding its cash and cash equivalents and corporate bond issuers to the extent recorded in the balance sheets.

Income Taxes

The Company uses the liability method of accounting for income taxes. Under this method, deferred tax assets and liabilities are determined based on the differences between the financial reporting and the tax bases of assets and liabilities and are measured using the enacted tax rates and laws that will be in effect when the differences are expected to reverse. The Company must then assess the likelihood that the resulting deferred tax assets will be realized. A valuation allowance is provided when it is more likely than not that some portion or all of a deferred tax asset will not be realized. Due to the Company's lack of earnings history, the net deferred tax assets have been fully offset by a valuation allowance.

The Company recognizes benefits of uncertain tax positions if it is more likely than not that such positions will be sustained upon examination based solely on their technical merits, as the largest amount of benefit that is more likely than not to be realized upon the ultimate settlement. The Company's policy is to recognize interest and penalties related to the underpayment of income taxes as a component of income tax expense or benefit. To date, there have been no interest or penalties charged in relation to the unrecognized tax benefits.

Net Loss per Share Attributable to Common Stockholders

Basic net loss per share attributable to common stockholders is calculated by dividing the net loss attributable to common stockholders by the weighted-average number of shares of common stock outstanding during the period, without consideration for common stock equivalents. The net loss attributable to common stockholders is calculated by adjusting the net loss of the Company for the accretion on the Series A convertible preferred stock and cumulative dividends paid on Series A and B convertible preferred stock. Diluted net loss per share attributable to common stockholders is the same as basic net loss per share attributable to common stockholders, since the effects of potentially dilutive securities are antidilutive.

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 and liabilities remeasured on a recurring basis based on the three-tier fair value hierarchy (in thousands):

	March 31, 2014			
	Level 1	Level 2	Level 3	Total
Financial Assets:				
Money market funds	$33,842	$—	$ —	$33,842
Commercial paper	—	5,985	—	5,985
Corporate bonds	—	118,231	—	118,231
U.S. Government securities	—	2,998	—	2,998
Total financial assets	$33,842	$127,214	$ —	$161,056

	December 31, 2013			
	Level 1	Level 2	Level 3	Total
Financial Assets:				
Money market funds	$6,847	$—	$—	$6,847
Commercial paper	—	1,000	—	1,000
Corporate bonds	—	44,950	—	44,950
Total financial assets	$6,847	$45,950	$—	$52,797
Financial Liabilities:				
Convertible preferred stock warrant liability	$—	$—	$3,419	$3,419
Total financial liabilities	$—	$—	$3,419	$3,419

The convertible preferred stock warrant liability was classified as a Level 3 liability. As of December 31, 2013, the Company determined the estimated fair value of the warrants using an option-pricing method to allocate the equity value of the Company to the warrants based on the Company's capital structure. The equity value was estimated using the back-solve method, whereby the equity value was derived from a recent transaction involving the Company's own securities. The key inputs used to determine value of the warrants was an estimated fair value of the Company's common stock of $12.14 per share, expected volatility of 70%, the expected time to liquidity event of 0.43 years and risk-free interest rate of 0.11%. The significant unobservable input used in the fair value measurement of the convertible preferred stock warrant liability was the equity value of the Company. Generally, increases (decreases) in the equity value of the Company would result in a directionally similar impact to the fair value measurement of the preferred stock warrant liability.

As of January 30, 2014, the Company determined the estimated fair value of the warrants using the Black-Scholes option-pricing model. Inputs used to determine the fair value included the value of the Company's common stock upon closing of the IPO of $21.00, the remaining contractual term of the warrants of 7.0 years, risk-free interest rate of 2.19% and expected volatility of 70%. The preferred stock warrants were converted to common stock warrants upon the completion of the IPO and are no longer subject to remeasurement.

The following table sets forth a summary of the changes in the estimated fair value of the Company's convertible preferred stock warrants, which were measured at fair value on a recurring basis until their conversion to common stock warrants and related reclassification to additional paid-in capital (in thousands):

	Three Months Ended March 31,	
	2014	2013
Fair value, beginning of period	$3,419	$518
Change in fair value recorded as a loss in other expense, net	3,324	(2)
Reclassification of warrant liability to additional paid-in capital	(6,743)	—
Fair value, end of period	$—	$516

4. Balance Sheet Components

Cash Equivalents and Short-term Investments

The fair values of cash equivalents and short-term investments classified as available-for-sale securities, consisted of the following:

	March 31, 2014			
	Amortized Cost	Gross Unrealized Gains	Losses	Estimated Fair Value
Money market funds classified as cash equivalents	$33,842	$—	$ —	$33,842
Corporate bonds classified as cash equivalents	17,263	2	(1)	17,264
Commercial Paper classified as short-term investments	5,985	—	—	5,985
Corporate bonds classified as short-term investments	101,017	18	(68)	100,967
U.S Government securities classified as short-term investments	2,995	3	—	2,998
Total	$161,102	$23	$ (69)	$161,056

	December 31, 2013			
	Amortized	Gross Unrealized Gains	Losses	Estimated

	Cost			Fair Value
Money market funds classified as cash equivalents	$6,847	$ —	$ —	$ 6,847
Commercial Paper classified as short-term investments	1,000	—	—	1,000
Corporate bonds classified as short-term investments	44,939	17	(6)	44,950
Total	$52,786	$ 17	$ (6)	$ 52,797

At March 31, 2014, the remaining contractual maturities of available-for-sale securities were less than two years. There have been no significant realized gains or losses on available-for-sale securities for the periods presented.

Accrued Liabilities

Accrued liabilities consist of the following (in thousands):

	March 31, 2014	December 31, 2013
Research and clinical trial expenses	$ 1,372	$ 1,886
Payroll and related expenses	1,557	2,360
Other	309	160
Total accrued liabilities	$ 3,238	$ 4,406

5. License and Research Agreements

Nobelpharma License Agreement

In September 2010, the Company entered into a collaboration and license agreement with Nobelpharma Co., Ltd. (Nobelpharma). Under the terms of this collaboration and license agreement, each party granted the other party a worldwide exclusive license under certain of that party's intellectual property related to the compound identified as N-acetylneuraminic acid, also known as sialic acid, to develop, manufacture, and commercialize products. Nobelpharma's licensed territory includes Japan and certain other Asian countries, and the Company's licensed territory includes the rest of the world.

Under the collaboration and license agreement, the Company paid Nobelpharma $110,500 (10 million Yen) for the license, which was recorded as research and development expense in 2010, and also issued 76,567 shares of common stock to Nobelpharma with a minimal value. The Company is required to pay Nobelpharma royalties based on net sales upon product sales commencement. In addition, the Company is required to make certain payments to Nobelpharma based upon achievement of certain development and approval milestones. The Company paid $495,000 in development milestone payments from inception through March 31, 2014. The remaining total aggregate payments, if all milestones are achieved by Nobelpharma, would be 200 million Yen (approximately $1.9 million based on the exchange rate at March 31, 2014). The Company will pay a high single digit royalty on net sales in the Company's territory and will receive a mid-single digit royalty on net sales in the Nobelpharma territory, excluding Japan, if such product sales are ever achieved. Net sales, as defined in the collaboration and license agreement, represent the net sales of products whereby the licensed compound is the active ingredient. If the products include other active ingredients, the portion of the net sales allocated to the licensed compound would be used in determining the royalty payments.

Saint Louis University License Agreement

In November 2010, the Company entered into a license agreement with Saint Louis University (SLU). Under the terms of this license agreement, SLU granted the Company an exclusive worldwide license to make, have made, use, import, offer for sale, and sell therapeutics related to SLU's beta-glucuronidase product for use in the treatment of human diseases.

Under the license agreement, the Company paid SLU an up-front fee of $10,000, which was recorded as research and development expense in 2010. The Company will be required to make a milestone payment of $100,000 upon approval of a glucuronidase-based enzyme therapy for treatment of MPS 7. Additionally, upon reaching a certain level of cumulative worldwide sales of the product, the Company will be required to pay to SLU a low single-digit royalty on net sales of the licensed products in any country or region, if such product sales are ever achieved.

AAI Pharma License Agreement

In March 2011, the Company entered into a license agreement with AAI Pharma Services Corp. (AAI Pharma). Under the terms of this license agreement, AAI Pharma granted the Company a fully paid-up, royalty-free, exclusive, perpetual, and irrevocable license to research, develop, make, have made, use, import, offer for sale, and sell products incorporating AAI Pharma's controlled release matrix solid dose oral tablet. Under the license agreement, the Company will pay a mid-single digit percentage of any sublicense revenue received by Ultragenyx related to the sublicense of AAI Pharma technology that had been initially licensed by Ultragenyx.

HIBM Research Group License Agreement

In April 2012, the Company entered into an exclusive license agreement with HIBM Research Group (HRG). Under the terms of this license agreement, HRG granted the Company an exclusive worldwide license to certain intellectual property related to the treatment of HIBM. Under the license agreement, the Company paid HRG an up-front fee of $25,000 which was recorded as research and development expense during the year ended December 31, 2012. The Company may make future payments that aggregate up to $300,000 and that are contingent upon attainment of various development and approval milestones. Additionally, the Company will pay to HRG a royalty of less than 1% of net sales of the licensed products in the licensed territories, if such product sales are ever achieved.

St. Jude Children’s Research Hospital License Agreement

In September 2012, the Company entered into a license agreement with St. Jude Children’s Research Hospital (St. Jude). Under the terms of this license agreement, St. Jude granted the Company an exclusive license under certain know-how to research, develop, make, use, offer to sell, import, and otherwise commercialize and exploit St. Jude’s protective protein, cathepsin, a protein product to treat, prevent, and/or diagnose galactosialidosis and other monogenetic diseases.

Under the license agreement, the Company paid St. Jude an up-front fee of $10,000 which was recorded as research and development expense during the year ended December 31, 2012. Additionally, the Company will pay to St. Jude a royalty of less than 1% on net sales of the licensed products in the licensed territories, if such product sales are ever achieved.

Baylor Research Institute License Agreement

In September 2012, the Company entered into a license agreement with Baylor Research Institute (BRI). Under the terms of this license agreement, BRI exclusively licensed to the Company certain intellectual property related to triheptanoin for North America. Under the license agreement, the Company paid BRI an up-front fee of $250,000 which was recorded as research and development expense during the year ended December 31, 2012. In June 2013, the Company notified BRI that it was exercising its option pursuant to the agreement to license the rights to triheptanoin in all territories outside of the United States, Canada and Mexico and paid the option exercise fee of $750,000.

The Company may make future payments of up to $10.5 million contingent upon attainment of various development milestones and $7.5 million contingent upon attainment of various sales milestones. Additionally, the Company will pay to BRI a mid-single digit royalty on net sales of the licensed product in the licensed territories, if such product sales are ever achieved.

Kyowa Hakko Kirin Collaboration and License Agreement

In August 2013, the Company entered into a collaboration and license agreement with Kyowa Hakko Kirin Co., Ltd. (KHK). Under the terms of this collaboration and license agreement, the Company and KHK will collaborate on the development and commercialization of certain products containing KRN23, an antibody directed towards FGF23, in the field of orphan diseases in the United States and Canada, or the profit share territory, and in the European Union, Switzerland, and Turkey, or the European territory, and the Company will have the right to develop and commercialize such products in the field of orphan diseases in Mexico and Central and South America, or Latin America. In the field of orphan diseases, and except for ongoing studies being conducted by KHK, the Company will be the lead party for development activities in the profit share territory and in the European territory until the applicable transition date. The Company will share the costs for development activities in the profit share territory and European territory conducted pursuant to the development plan before the applicable transition date equally with KHK. On the applicable transition date in the relevant territory, KHK will become the lead party and be responsible for these costs. However, the Company will continue to share the costs of the studies commenced prior to the applicable transition date equally with KHK. The Company has the primary responsibility for conducting certain research and development services. The Company is obligated to provide assistance in accordance with the agreed upon development plan as well as participate on various committees. If KRN23 is approved, the Company and KHK will share commercial responsibilities and profits in the profit share territory until the applicable transition date, KHK will commercialize KRN23 in the European territory and the Company will develop and commercialize KRN23 in Latin America. KHK will manufacture and supply KRN23 for clinical use globally and will manufacture and supply KRN23 for commercial use in the profit share territory and Latin America.

The Company is accounting for the agreement as a collaboration arrangement as defined in ASC 808, Collaborative Agreements; accordingly, the Company recognized $710,000 for its share of the costs as research and development expenses for the three months ended March 31, 2014. For the period from April 22, 2010 (Inception) through March 31, 2014, the Company recognized $1,514,000 for its share of the costs as research and development expenses.

6. Convertible Preferred Stock Warrants and Common Stock Warrants

Upon the closing of the Company’s IPO, the Convertible Preferred Stock warrants were converted into warrants to purchase common stock.

As of March 31, 2014, outstanding common stock warrants consisted of the following:

Common Stock Warrants:	Number of Warrants	Date Issued	Term	Exercise Price
Common stock	83,167	June 2010	10 years	$ 3.006
Common stock	203,759	February 2011	10 years	3.006
Common stock	66,533	June 2011	10 years	3.006
Total common stock warrants	353,459			

As of December 31, 2013, outstanding preferred stock warrants consisted of the following:

Convertible Preferred Stock Warrants:	Number of Warrants	Date Issued	Term	Exercise Price
Series A	241,803	June 2010	10 years	$ 1.034
Series A	592,417	February 2011	10 years	1.034
Series A	193,442	June 2011	10 years	1.034
Total convertible preferred stock warrants	1,027,662			

The fair value of the warrants was estimated to be $6.7 million and $3.4 million as of January 30, 2014 (pricing date of IPO) and December 31, 2013, respectively.

The Company recorded ($3.3 million), $2,000, and ($6.5 million) to other income (expense) for three months ended March 31, 2014 and 2013 and for the period from April 22, 2010 (Inception) through March 31, 2014, representing the change in fair value of the warrants for the respective period.

7. Convertible Preferred Stock

The holders of the Series A and Series B convertible preferred stock were entitled to receive cumulative dividends at the rate of $0.062 per share per annum, payable in the form of cash. Dividends accrued from day to day, whether or not declared, but were paid only when, as, and if declared by the Board of Directors. During 2012, $2.1 million of dividends were declared and paid to holders of Series A convertible preferred stock in the form of additional Series A convertible preferred stock. Dividends in arrears as of December 31, 2013 were $4.0 million for both series of preferred stock. Upon the closing of the IPO in February 2014, all shares of convertible preferred stock then outstanding automatically converted in 19,598,486 shares of common stock. In connection with the conversion of the convertible preferred stock, all accrued and outstanding dividends in the amount of $4.3 million were paid.

The Company initially recorded the Series A and Series B convertible preferred stock at their issuance price, which represents the carrying value. The Series A convertible preferred stock was redeemable at any time after June 16, 2017 once a written request to redeem such stock was received by the Company from holders of not less than seventy-five percent of the then outstanding Series A convertible preferred stock. As only the passage of time was required for the Series A convertible preferred stock to become redeemable, the difference in the initial carrying value of the Series A convertible preferred stock and their total redemption value was being accreted from the issuance date through the first redemption date of June 16, 2017. The Company recorded accretion of $4.4 million and $1.1 million for the three months ended March 31, 2014 and 2013, respectively. As a result of the conversion of the preferred stock to common stock in connection with the Company’s IPO, the Company is no longer accreting the Series A convertible preferred stock to its previously calculated redemption value.

8. Stock-Based Compensation

2011 Equity Incentive Plan

In 2011, the Company adopted the 2011 Equity Incentive Plan (the 2011 Plan). The 2011 Plan provides for the granting of stock-based awards to employees, directors, and consultants under terms and provisions established by the Board of Directors. Under the terms of the 2011 Plan, options may be granted at an exercise price not less than fair market value. For employees holding more than 10% of the voting rights of all classes of stock, the exercise prices for incentive stock options must be at least 110% of fair market of the common stock on the grant date, as determined by the Board of Directors. The terms of options granted under the 2011 Plan may not exceed ten years. Options granted generally vest over a period of four years. Typically, the vesting schedule for option grants to newly hired employees provides that 1/4 of the grant vests upon the first anniversary of the employee’s date of hire, with the remainder of the shares vesting monthly thereafter at a rate of 1/48 of the total shares subject to the option. All other employee options typically vest in equal monthly installments over the four-year vesting schedule. In connection with the Company’s IPO, no further grants will be made under this plan and all remaining shares available for grant were transferred to the 2014 Incentive Plan.

2014 Incentive Plan

In 2014, the Company adopted the 2014 Incentive Plan (the 2014 Plan), which became effective upon the closing of the Company's IPO in February 2014. The 2014 Plan had 2,250,000 shares of common stock available for future issuance at the time of its inception, which included 655,038 shares available under the 2011 Plan, which were transferred to the 2014 Plan upon adoption. The 2014 Plan provides for automatic annual increases in shares available for grant, beginning on January 1, 2015 through January 1, 2024. The 2014 Plan provides for the granting of stock-based awards to employees, directors, and consultants under similar terms, conditions and provisions as the 2011 Plan.

Founder's Stock

In connection with the Series A preferred stock financing, the Company entered into a stock repurchase agreement with the founder on June 16, 2011, whereby 2,552,241 shares of common stock previously owned by the founder were subject to repurchase by the Company at the original issuance price in the event that the founder's employment is terminated either voluntarily or involuntarily. The repurchase rights lapsed over a period of two years from June 16, 2011. The Company calculated the estimated fair value of these restricted shares at the time the restriction was added to the shares as $1,199,000 and recorded this amount as stock-based compensation ratably over the period that the repurchase rights lapsed. Stock-based compensation expense pertaining to the founder's stock was $0, $149,000, and $1,199,000 for the three months ended March 31, 2014 and 2013 and for the period from April 22, 2010 (Inception) through March 31, 2014, respectively.

The table below sets forth the functional classification of stock-based compensation expense, net of estimated forfeitures, for the periods presented (in thousands):

	Three Months Ended March 31,		Period from April 22, 2010 (Inception) Through March 31,
	2014	2013	2014
Research and development	$705	$30	$ 1,156
General and administrative	90	161	1,441
Total stock-based compensation	$795	$191	$ 2,597

9. Defined Contribution Plan

In March 2013, the Company began to sponsor a 401(k) retirement plan, in which substantially all of its full-time employees are eligible to participate. Eligible participants may contribute a percentage of their annual compensation to this plan, subject to statutory limitations. The Company has not provided any contributions to the plan since its inception through March 31, 2014.

10. Commitments and Contingencies

Commitments

The Company has various manufacturing, clinical, research, and other contracts with vendors in the conduct of the normal course of its business. As of March 31, 2014, the Company had a binding obligation for approximately $850,000 with a manufacturing vendor for the production of a drug substance for one of its product candidates. All other significant contracts as of March 31, 2014 were terminable, with varying provisions regarding termination. If a contract with a specific vendor were to be terminated, the Company would only be obligated for the products or services that the Company had received at the time the termination became effective.

Contingencies

While there are no legal proceedings the Company is aware of, the Company may become party to various claims and complaints arising in the ordinary course of business. Management does not believe that any ultimate liability resulting from any such claims will have a material adverse effect on its results of operations, financial position, or liquidity. However, management cannot give any assurance regarding the ultimate outcome of such claims, and their resolution could be material to the Company for any particular period, depending upon the level of income or loss for the period, as well as the Company’s balance sheet.

11. Net Loss per Share Attributable to Common Stockholders

The following table sets forth the computation of the basic and diluted net loss per share attributable to common stockholders (in thousands, except share and per share data):

	Three Months Ended March 31,	
	2014	2013
Numerator:		
Net loss	$(13,630)	$(6,735)
Accretion and dividends on convertible preferred stock	(4,808)	(1,470)
Net loss attributable to common stockholders	$(18,438)	$(8,205)
Denominator:		
Weighted-average common shares outstanding	21,582,435	3,461,161
Less: weighted-average unvested common shares subject to repurchase	—	(567,164)
Weighted-average shares used to compute net loss per share attributable to common stockholders, basic and diluted	21,582,435	2,893,997
Net loss per share attributable to common stockholders, basic and diluted	$(0.85)	$(2.84)

The following weighted-average outstanding common stock equivalents were excluded from the computation of diluted net loss per share attributable to common stockholders for the periods presented because including them would have been antidilutive:

	Three Months Ended March 31,	
	2014	2013
Convertible preferred stock	—	19,598,486
Stock options to purchase common stock	2,315,345	1,440,154
Common stock subject to repurchase	—	567,164
Warrants to purchase convertible preferred stock (as if converted)	—	353,459
Warrants to purchase common stock	353,459	—
	2,668,804	21,959,263

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

MANAGEMENT'S DISCUSSION AND ANALYSIS OF

FINANCIAL CONDITION AND RESULTS OF OPERATIONS

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with the accompanying unaudited consolidated financial statements and related notes in Item 1 and with the audited consolidated financial statements and the related notes included in our Annual Report on Form 10-K for the year ended December 31, 2013.

Overview

We are a development-stage biopharmaceutical company focused on the identification, acquisition, development, and commercialization of novel products for the treatment of rare and ultra-rare diseases, with an initial focus on serious, debilitating metabolic genetic diseases. We focus on diseases for which the unmet medical need is high, the biology for treatment is clear, and for which there are no approved therapies. Since our inception in 2010, we have in-licensed potential treatments for five different diseases that are currently in or have completed Phase 1/2 or Phase 2 clinical studies. Our strategy, which is predicated upon time- and cost-efficient drug development, allows us to pursue multiple programs in parallel with the goal of delivering safe and effective therapies to patients with the utmost urgency.

Our current pipeline consists of two product categories: biologics, including a monoclonal antibody and enzyme replacement therapies; and small-molecule substrate replacement therapies. Enzymes are proteins that the body uses to process materials needed for normal cellular function, and substrates are the materials upon which enzymes act. When enzymes or substrates are missing, the body is unable to perform its normal cellular functions, often leading to significant clinical disease. Several of our therapies are intended to replace deficient enzymes or substrates.

Our biologics pipeline includes the following three product candidates:

KRN23, or UX023, is an antibody targeting fibroblast growth factor 23, or FGF23, intended for the treatment of X-linked hypophosphatemia, or XLH, a rare genetic disease that impairs bone growth. We are developing KRN23 pursuant to our collaboration with Kyowa Hakko Kirin Co., Ltd., or KHK. KHK has completed one Phase 1 study, one Phase 1/2 study, and one longer-term Phase 1/2 study of KRN23 in adults with XLH. We plan to initiate a Phase 2 pediatric study in 2014. We also expect to continue the clinical development of KRN23 in adults with XLH.
rhGUS, or UX003, is an enzyme replacement therapy we are developing for the treatment of mucopolysaccharidosis 7, or MPS 7, a rare lysosomal storage disease that often leads to multi-system disease, pervasive skeletal disease, and early death. We initiated a Phase 1/2 clinical study in MPS 7 in December 2013.
rhPPCA, or UX004, is an enzyme replacement therapy in preclinical development for galactosialidosis, a rare lysosomal storage disease that can cause multi-system clinical disease similar to MPS 7 including enlarged liver, joint disease, abnormal bone development, short stature, and early death. We plan to continue preclinical development of rhPPCA during 2014.
Our substrate replacement therapy pipeline includes the following product candidates in development for three diseases:

Triheptanoin, or UX007, is a synthetic triglyceride with a specifically designed chemical composition being studied as an energy substrate replacement therapy in an international open-label Phase 2 study for the treatment of long-chain fatty acid oxidation disorders, or LC-FAOD. This is a set of rare metabolic diseases caused by the inability to convert fat into energy leading to low blood sugar, muscle rupture, and heart and liver disease.

Triheptanoin is also in a Phase 2 study for the treatment of glucose transporter type-1 deficiency syndrome, or Glut1 DS, a rare metabolic disease of brain energy deficiency that is characterized by seizures, developmental delay, and movement disorder.

SA-ER, or UX001, is an extended-release form of sialic acid in a Phase 2 extension study for the treatment of hereditary inclusion body myopathy, or HIBM, a neuromuscular disorder that causes muscle weakness and wasting. Data from the Phase 2 study were presented at the American Academy of Neurology (AAN) Annual Meeting in April 2014. We continue to treat the patients from the Phase 2 study in an extension study and anticipate that data from the extension study will be available in late 2014.

Product Candidates

The following table summarizes our current product candidate pipeline:

KRN23 (UX023) for the treatment of XLH

KRN23 is a fully human monoclonal antibody administered via subcutaneous injection that is designed to bind and reduce the biological activity of FGF23 to increase abnormally low phosphate levels in patients with XLH. Patients with XLH have low serum phosphate levels due to excessive phosphate loss into the urine, which is directly caused by the effect on kidney function of excess FGF23 production in bone cells. Low phosphate levels lead to poor bone mineralization and a variety of clinical manifestations, including rickets leading to bowing and other skeletal deformities, short stature, bone pain and fractures, poor quality bone, and muscle weakness. There is no approved drug therapy or treatment for the underlying cause of XLH. Most patients are managed using frequently dosed oral phosphate and vitamin D therapy, which is only partially effective at improving bone disease and growth and has significant side effects. Oral phosphate/vitamin D replacement therapy requires extremely close monitoring due to the potential for excessive phosphate levels and secondary increases in calcium, which can result in severe damage to the kidneys from excess calcium phosphate deposits and other complications. Additionally, some patients are unable to tolerate the regimen due to the chalky stool that results from taking large amounts of oral phosphate or the high frequency of dosing required.

In August 2013, we formed a collaboration with KHK to jointly develop and commercialize KRN23 for the treatment of XLH. KHK has conducted one Phase 1 study, one Phase 1/2 study and one longer-term Phase 1/2 study of KRN23 in adults with XLH. We reviewed safety and efficacy data from the Phase 1/2 studies prior to entering into our collaboration with KHK, and we entered into the collaboration based in part upon our conclusion that these data were supportive of further development (serum phosphate, renal tubular reabsorption of phosphate, and vitamin D levels were increased, and the product appeared well tolerated).

Results from the Phase 1 single dose study in 38 adult XLH patients were presented at the American Society for Bone and Mineral Research Annual Meeting in October 2013 and published in the Journal of Clinical Investigation in February 2014. The data demonstrated that KRN23 was well tolerated and increased serum phosphate, or phosphorus, as well as vitamin D levels. Of the 38 adult XLH patients, 12 received a single subcutaneous injection of KRN23 (at doses of 0.1, 0.3, 0.6, or 1.0 mg/kg), 17 received a single intravenous injection of KRN23 (at doses of 0.003, 0.01, 0.03, 0.1, or 0.3 mg/kg) and 9 received placebo. The effect of KRN23 on the increase in serum phosphate levels was comparable between intravenous and subcutaneous administration; however, time to reach peak effect was slower and duration of effect was greater with subcutaneous administration compared with intravenous administration. The demonstrated improvement in serum phosphate levels suggests that significant benefit could be expected. Corresponding changes were observed in renal tubular reabsorption of phosphate. Increases in vitamin D were also observed, suggesting improved intestinal absorption of both phosphate and calcium. Changes were not observed in serum calcium.

No serious adverse events were reported in the Phase 1 study. Approximately 83% of the subjects experienced at least one non-serious treatment-emergent adverse event, the most common of which were nausea and headache; no patients in the placebo or subcutaneous treatment arms reported these events. In the subcutaneous arm, two patients (approximately 17%) experienced elevated levels of the enzyme amylase in the blood, and two other patients (approximately 17%) experienced back pain. There did not appear to be a relationship between the incidence and

types of adverse events and the dose administered following a single dose of study drug.

We expect KHK to release data from the completed Phase 1/2 adult repeat-dose studies during 2014.

We plan to initiate a Phase 2 pediatric study in 2014, in patients with radiographic evidence of bone disease, following discussions with multiple regulatory agencies on our pediatric study design. Depending on the results of our Phase 2 pediatric study, we intend to conduct a Phase 3 pediatric trial. Given the high turnover and growth of bone during childhood and the critical role phosphate plays in bone growth, pediatric XLH patients have the highest morbidity and potential for benefit in a shorter timeframe. As a result, pediatric XLH patients may also have the greatest potential for improvement based on third-party data regarding enzyme replacement therapy in hypophosphatasia, which is another genetic bone disease with poor bone mineralization related to phosphate metabolism caused by a different, unrelated mechanism. We also expect to continue to develop KRN23 in adults with XLH and plan to conduct an adult Phase 2b study in parallel with our Phase 3 pediatric trial.

rhGUS (UX003) for the treatment of MPS 7

Recombinant human beta-glucuronidase, or rhGUS, is an intravenous, or IV, enzyme replacement therapy for the treatment of MPS 7, also known as Sly Syndrome. Patients with MPS 7 suffer from severe cellular and organ dysfunction that typically leads to death in the teens or early adulthood. MPS 7 is caused by a deficiency of the lysosomal enzyme beta-glucuronidase, which is required for the breakdown of certain complex carbohydrates known as glycosaminoglycans, or GAGs. The inability to properly break down GAGs leads to their accumulation in many tissues, resulting in a serious multi-system disease. There are currently no approved drug therapies for MPS 7.

We licensed exclusive worldwide rights to rhGUS-related know-how and cell lines from Saint Louis University in November 2010. We have conducted preclinical studies to support the chronic IV administration of rhGUS. Administration of rhGUS resulted in substantial distribution of enzyme, as well as reduction in tissue pathology in a wide variety of tissues, including the liver, spleen, lung, heart, kidney, muscle, bone, and brain. No adverse toxicology related to rhGUS was noted in these studies.

In December 2013 we initiated an open-label, Phase 1/2 study in the United Kingdom to evaluate the safety, tolerability, efficacy, and dose of IV administration every other week of rhGUS in up to five patients with MPS 7 who are between five and 30 years of age. The initial 12-week treatment period will be followed by a dose-titration period and a long-term extension study. We expect to release interim data from this study during 2014.

Preliminary data from the Phase 1/2 study were presented at the American College of Medical Genetics and Genomics (ACMG) Annual Clinical Genetics Meeting in March 2014. Results from three patients who had been administered 2 mg/kg of rhGUS every other week for two, six, and 12 weeks showed evidence of clearance of lysosomal storage as indicated by the decrease in urinary GAG excretion beginning at two weeks of treatment of approximately 30-50%. At the 12 week assessment of the first patient, absolute liver size was reduced by approximately 11%. This represents a 46% decrease in the excess liver size above normal for age and gender. The remaining patients have not yet reached the 12 week time point for liver size assessment. No serious adverse events were observed during up to 12 weeks of treatment, and no infusion-associated reactions were observed after a total of 13 infusions to date in these three subjects. The Phase 1/2 study will continue, and additional 12-week interim data are expected in the second half of 2014. If these results are supportive, we plan to initiate a pivotal Phase 3 study.

We are also supplying rhGUS to an investigator who is treating a single U.S. patient under an emergency investigational new drug, or eIND, application. Results from the treatment of this patient were presented at the Lysosomal Disease Network's 10th Annual World Symposium in February 2014. Preliminary data showed a reduction in lysosomal storage based on reduced excretion of urinary GAG and a reduction in the size of the enlarged liver and spleen. The patient showed an improvement of pulmonary function and no infusion-associated reactions during the first 14 weeks of treatment. The patient's caregivers also reported improved stamina and increased time spent in

school.

The European Medicines Agency, or EMA, has agreed that approval under exceptional circumstances could be possible for a proposed 12-patient placebo-controlled pivotal study in this disease with urinary GAG levels as a surrogate primary endpoint provided the data was strongly supportive of a favorable benefit/risk ratio. The EMA requested that some evidence or trend in improvement in clinical endpoints be observed to support the primary endpoint, but recognized that a statistically significant result on clinical endpoints was unlikely given the small number of patients expected to be enrolled in the study. The United States Food and Drug Administration, or FDA, has not yet agreed to the pivotal study plan and would like to see additional data correlating urinary GAG levels with other clinical endpoints, which we are collecting.

In addition to the above development plan, we intend to study MPS 7 patients under the age of five years, including potentially younger infants born with hydrops fetalis. Currently, these infants often die within a few months to one year, but enzyme replacement therapy might be able to reduce GAG storage and improve health and survival in these patients. This program would not start until we had obtained sufficient information from the Phase1/2 study to support the initiation of a trial in younger patients.

rhPPCA (UX004) for the treatment of galactosialidosis

Recombinant human protective protein cathepsin-A, or rhPPCA, which we in-licensed from St. Jude Children's Research Hospital in September 2012, is in preclinical development as an enzyme replacement therapy for galactosialidosis, a rare lysosomal storage disease for which there are no currently approved drug therapies. Similar to MPS patients, patients with galactosialidosis present with both soft tissue storage in the liver, spleen, and other tissues, as well as connective tissue (bone and cartilage) related disease. As with MPS 7, an enzyme deficiency results in accumulation of substrates in the lysosomes, causing skeletal and organ dysfunction, and death. We plan to continue preclinical development of rhPPCA during 2014.

Triheptanoin (UX007) for the treatment of LC-FAOD

We are developing triheptanoin for oral administration intended as a substrate replacement therapy for patients LC-FAOD. Triheptanoin is a medium odd-chain triglyceride of three seven-carbon fatty acids designed to provide substrate replacement for fatty acid metabolism and restore production of energy. Patients with LC-FAOD have a deficiency that impairs the ability to produce energy from fat, which can lead to depletion of glucose in the body, and severe liver, muscle, and heart disease, as well as death. There are currently no approved drugs or treatments specifically for LC-FAOD. The current standard of care for LC-FAOD includes diligent prevention of fasting combined with the use of low-fat/high-carbohydrate diets, carnitine supplementation in some cases, and medium even-chain triglyceride, or MCT, oil supplementation. Despite treatment with the current standard of care, many patients continue to suffer significant morbidity and mortality.

We licensed certain intellectual property rights relating to triheptanoin from Baylor Research Institute in August 2012. Triheptanoin has been studied clinically for 13 years in approximately 130 human subjects affected by a variety of diseases, including greater than 60 patients with LC-FAOD. Multiple investigator-sponsored open-label studies suggest clinical improvements with triheptanoin treatment, even for patients who were on standard of care. We recently presented a retrospective medical record review study assessing the clinical outcome of triheptanoin treatment on LC-FAOD subjects who have been participating in a compassionate use program at the University of Pittsburgh Medical Center. The data showed that treatment with triheptanoin appeared to reduce the frequency and severity of hospitalizations previously experienced by these patients for disease-related causes, including muscle rupture, hypoglycemia, and cardiomyopathy. A reduction in mean total hospital days per year from 17.55 to 5.40 (69%; $p = 0.0242$) was observed after transitioning from standard of care to triheptanoin therapy.

Triheptanoin is currently being evaluated in a prospective international open-label Phase 2 study in approximately 30 severely affected LC-FAOD patients. A principal goal of the study is to determine the appropriate clinical endpoints and patient population for testing in potential later-stage pivotal studies. The study will evaluate patients, ages 6 months to 35 years, exhibiting significant clinical manifestations of LC-FAOD despite current therapy. Prior to initiating treatment with triheptanoin, subjects will continue current therapy for four weeks to establish their baseline condition. Triheptanoin will then be titrated to an expected target dose of 25-35% of total daily caloric intake via oral administration, while ensuring tolerability. The study will assess the impact of triheptanoin on several endpoints, including cycle ergometer performance, 12-minute walk test, muscle strength, creatine kinase levels, hypoglycemia, liver size, cardiac disease, and major medical events. The patients will be followed to evaluate the effects of triheptanoin treatment on acute clinical pathophysiology associated with LC-FAOD over 24 weeks, then may continue treatment for an additional 54 weeks for observation of major medical events. Data from this study should be available in 2015.

Triheptanoin (UX007) for the treatment of Glut1 DS

We are also developing triheptanoin for patients with Glut1 DS. Glut1 DS is caused by a mutation affecting the gene that codes for Glut1, which is a protein that transports glucose from the blood into the brain. Because glucose is the primary source of energy for the brain, Glut1 DS results in a chronic state of brain energy deficiency and is characterized by seizures, developmental delay, and movement disorder. There are currently no approved drugs specific to Glut1 DS. The current standard of care for Glut1 DS is the ketogenic diet, an extreme high-fat (70-80% of daily calories as fat)/low-carbohydrate diet, which generates ketone bodies as an alternative energy source to glucose, and one or more antiepileptic drugs. The ketogenic diet can be effective in reducing seizures but compliance can be difficult, and the diet has demonstrated limited effectiveness in the treatment of developmental delay and movement disorders. In addition, ketogenic diet can lead to side effects including renal stones. In general, Glut 1 DS patients are considered relatively refractory to antiepileptic drugs with only approximately 10% achieving seizure control on antiepileptic drugs alone. There are currently no antiepileptic drugs approved specifically for patients with Glut 1 DS.

Triheptanoin is intended as a substrate replacement therapy to provide an alternative source of energy to the brain in Glut1 DS patients. Although an open-label investigator-sponsored clinical study is ongoing and the results have not yet been reported, there are anecdotal reports of benefit in terms of reduced seizures and improved development rate in some Glut1 DS subjects taking triheptanoin. In March 2014, we initiated a Phase 2 global, randomized, double-blind, placebo-controlled, parallel-group study of up to 50 patients who are currently not fully compliant with ketogenic diet and continue to have seizures. The primary efficacy objective is the reduction in frequency of seizures compared to placebo following a 6-week baseline period and subsequent 8-week placebo-controlled treatment period. The blinded treatment period will be followed by an open-label extension period in which patients will be treated with triheptanoin through week 52. Patient enrollment may be modified based on an interim analysis. We expect to release data from this trial in 2015.

We also continue to support investigator-sponsored trials studying triheptanoin across multiple indications.

SA-ER (UX001) for the treatment of HIBM

We are developing an extended-release, oral formulation of sialic acid, or SA-ER, for the treatment of hereditary inclusion body myopathy, or HIBM, which is also known as GNE myopathy. HIBM is characterized by severe progressive muscular myopathy, or disease in which muscle fibers do not function properly, with onset typically in the late teens or twenties. Patients with HIBM have a genetic defect in the gene coding for a particular enzyme that is involved in the first step in the biosynthesis of sialic acid. Therefore, HIBM patients have a sialic acid deficiency, which interferes with muscle function, leading to myopathy and atrophy. Patients typically lose major muscle function within ten to 20 years of diagnosis. There is no approved drug therapy for HIBM.

SA-ER is intended as a substrate replacement therapy designed to address sialic acid deficiency and restore muscle function in HIBM patients. We have conducted a Phase 2 randomized, double-blind, placebo-controlled study of SA-ER in 47 HIBM patients. Data from this study were presented at the American Academy of Neurology (AAN) Annual Meeting in April 2014.Patients in the study were initially randomized to receive placebo, 3 grams, or 6 grams of SA-ER per day. After 24 weeks, placebo patients crossed over to either 3 grams or 6 grams total daily dose, on a blinded basis, for an additional 24 weeks. The final analysis compared change at week 48 from baseline for the combined groups at 6 grams versus 3 grams of SA-ER. Assessments included pharmacokinetics, composites of upper extremity and lower extremity muscle strength as measured by dynamometry, other clinical endpoints, patient reported outcomes, and safety.

At 24 weeks, assessments of upper extremity composite of muscle strength showed a statistically significant difference in the 6 gram group compared to placebo (+2.33 kg; 5.5% relative difference from baseline; p=0.040). At 48 weeks, a statistically significant difference between the combined 6 gram group and the combined 3 gram group was observed (+3.44 kg; 8.5% relative difference from baseline; p=0.0033). Patients with less advanced disease (able to walk more than 200 meters at baseline), a predefined subset, showed a more pronounced difference (+4.69 kg; 9.7% relative difference from baseline; p=0.00055). The lower extremity composite showed a similar pattern of response but did not show a statistically significant difference between the dose groups. None of the groups showed a significant decline in the lower extremity composite during the treatment period. A positive trend was seen in patient-reported outcomes of functional activity. SA-ER appeared to be well tolerated with no serious adverse events observed to date in either dose group, and no dose-dependent treatment-emergent adverse events were identified. Most adverse events were mild to moderate and most commonly gastrointestinal and pain related to bone biopsy procedures.

We continue to treat these patients in an extension study evaluating an increased daily dosage of sialic acid based on the dose dependence observed at weeks 24 and 48. We anticipate that data from the extension study should be available in late 2014. We also plan to discuss data from this program with regulatory authorities during 2014.

Financial Operations Overview

We are considered a development-stage company under U.S. generally accepted accounting principles, or U.S. GAAP, and have only a limited operating history. To date, we have invested substantially all of our efforts and financial resources to identifying, acquiring, and developing our product candidates, including conducting clinical studies and providing general and administrative support for these operations. To date, we have funded our operations primarily from the sale of convertible preferred stock and equity securities.

We have never been profitable and have incurred net losses in each year since inception. Our net losses were $13.6 million and $6.7 million for the three months ended March 31, 2014 and 2013. As of March 31, 2014 we had incurred

cumulative net losses of $73.2 million. Substantially all of our net losses have resulted from costs incurred in connection with our research and development programs and from general and administrative costs associated with our operations.

Revenue

To date, we have not generated any revenue. We do not expect to receive revenue from any product candidates that we develop unless regulatory approvals are obtained for our products or we enter into collaborative agreements with third parties.

Research and Development Expenses

Research and development expenses consist primarily of costs incurred for the development of our product candidates, which include:

- expenses incurred under agreements with clinical study sites that conduct research and development activities on our behalf;
- expenses incurred under license agreements with third parties;
- employee and consultant-related expenses, which include salaries, benefits, travel, and stock-based compensation;
- laboratory and vendor expenses related to the execution of preclinical, non-clinical, and clinical studies;

- the cost of acquiring, developing, and manufacturing clinical study materials; and
- facilities, depreciation, and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance, and other supply costs.

We expense all research and development costs in the periods in which they are incurred. Costs for certain development activities are recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and clinical sites. Nonrefundable advance payments for goods or services to be received in future periods for use in research and development activities are deferred and capitalized. The capitalized amounts are then expensed as the related goods are delivered and the services are performed.

The largest component of our total operating expenses has historically been our investment in research and development activities, including the clinical development of our product candidates. We allocate research and development salaries, benefits, stock-based compensation, and indirect costs to our product candidates on a program-specific basis, and we include these costs in the program-specific expenses. We expect our research and development expenses will increase in absolute dollars in future periods as we continue to invest in research and development activities related to developing our product candidates, and as programs advance into later stages of development and we enter into larger clinical studies. The process of conducting the necessary clinical research to obtain FDA approval is costly and time consuming and the successful development of our product candidates is highly uncertain. As a result, we are unable to determine the duration and completion costs of our research and development projects or when and to what extent, if any, we will generate revenue from the commercialization and sale of any of our product candidates.

General and Administrative Expenses

General and administrative expenses consist primarily of personnel costs, allocated facilities costs, and other expenses for outside professional services, including legal, human resources, audit, and accounting services. Personnel costs consist of salaries, benefits, and stock-based compensation. We expect that our general and administrative expenses will increase in the future to support continued research and development activities, preparation for potential commercialization of our product candidates, and as a result of operating as a public company, including expenses related to compliance with the rules and regulations of the Securities and Exchange Commission, or SEC, and those of any national securities exchange on which our securities are traded, additional insurance expenses, investor relations activities, and other administration and professional services.

Interest income

Interest income consists of interest earned on our cash, cash equivalents, and short-term investments.

Other expense, net

Other expense, net primarily consists of gains and losses resulting from the remeasurement of our convertible preferred stock warrant liability. We recorded adjustments to the estimated fair value of the convertible preferred stock warrants until their conversion into warrants to purchase shares of our common stock at the completion of our initial public offering. At that time, we reclassified the convertible preferred stock warrant liability to additional paid-in capital which will no longer be subject to fair value adjustments.

Critical Accounting Policies and Significant Judgments and Estimates

Our management’s discussion and analysis of our financial condition and results of operations is based on our financial statements, which have been prepared in accordance with U.S. GAAP. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the

disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported expenses incurred during the reporting periods. Our estimates are based on our historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions. There have been no significant and material changes in our critical accounting policies during the three months ended March 31, 2014, as compared to those disclosed in “Management’s Discussion and Analysis of Financial Condition and Results of Operations-Critical Accounting Policies and Significant Judgments and Estimates” in our in our most recent Annual Report on Form 10-K filed with the SEC.

Results of Operations

Comparison of the three months ended March 31, 2014 and 2013:

Research and Development Expenses (dollars in thousands)

	Three Months Ended March 31, 2014	2013	Dollar Change	% Change
Development candidate:				
KRN23	$877	$—	$877	*
rhGUS	1,329	2,142	(813)	-38%
rhPPCA	110	71	39	55%
Triheptanoin (LC-FAOD)	1,382	866	516	60%
Triheptanoin (Glut 1 DS)	1,026	42	984	2343%
SA-ER	2,246	1,832	414	23%
Other research and development costs	1,383	711	672	95%
Total research and development expenses	$8,353	$5,664	$2,689	47%

*not meaningful

Research and development expenses increased $2.7 million for the three months ended March 31, 2014 compared to the same period in 2013. The increase in research and development expenses above is primarily due to:

· for KRN23, an increase of $0.9 million related to development of our pediatric trial design, other development planning, and regulatory activities, since the product candidate was in-licensed in August 2013;