(Address of principal executive offices) (Zip Code)

94949

60 Leveroni Court,

Novato, California

(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 August 6, 2014, the registrant had 31,703,990 shares of common stock issued and outstanding.

ULTRAGENYX PHARMACEUTICAL INC.

FORM 10-Q FOR THE QUARTER ENDED JUNE 30, 2014

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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 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

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.

ULTRAGENYX PHARMACEUTICAL INC.

CONDENSED BALANCE SHEETS

(In thousands, except share and per share amounts)

	June 30, 2014 (Unaudited)	December 31, 2013 (Note 2)		
Assets				
Current assets:				
Cash and cash equivalents	\$ 17,452	\$ 7,427		
Short-term investments	135,819	45,950		
Prepaid expenses and other current assets	4,945	1,848		
Total current assets	158,216	55,225		
Property and equipment, net	2,497	1,325		
Restricted cash	744	451		
Other assets	1,129	2,648		
Total assets	\$ 162,586	\$ 59,649		
Liabilities, Convertible Preferred Stock and Stockholders' Equity (Deficit) Current liabilities: Accounts payable	\$3,590	\$ 1,437		
Accrued liabilities	5,278	4,406		
Deferred rent—current portion	75	78		
Total current liabilities	8,943	5,921		
Convertible preferred stock warrant liability		3,419		
Other liabilities	560	200		
Total liabilities	9,503	9,540		
Commitments and contingencies (Note 10)				
Series A redeemable convertible preferred stock, par value of \$0.001 per share—nil and	1			
35,377,566 shares authorized; nil and 34,349,894 shares issued and outstanding as of				
June 30, 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 June 30, 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 June 30, 2014 and December 31, 2013	_			
Common stock, par value of \$0.001 per share—250,000,000 shares authorized;	30	4		
220,000,000 bilates addition,		·		
30,059,288 and 3,766,289 shares issued and outstanding as of June 30, 2014 and				

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December 31, 2013			
Additional paid-in capital	259,555		
Accumulated other comprehensive income (loss)	(48)	11	
Accumulated deficit	(106,454)	(74,836)
Total stockholders' equity (deficit)	153,083	(74,821)
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	\$ 162,586	\$ 59,649	
See accompanying notes.			

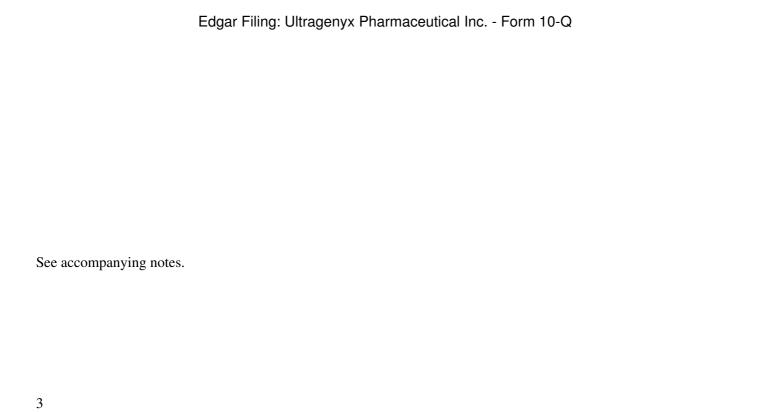
ULTRAGENYX PHARMACEUTICAL INC.

CONDENSED STATEMENTS OF OPERATIONS

(Unaudited)

(In thousands, except share and per share amounts)

	Three Months Ended June 30,		Six Months 30,	Ended June
	2014	2013	2014	2013
Operating expenses:				
Research and development	\$11,239	\$7,199	\$19,592	\$12,863
General and administrative	2,422	1,048	4,408	2,131
Total operating expenses	13,661	8,247	24,000	14,994
Loss from operations	(13,661) (8,247) (24,000) (14,994)
Other income (expense), net:				
Interest income	149	63	242	89
Other expense, net	(73) (406) (3,457) (420)
Total other income (expense), net	76	(343	(3,215) (331)
Net loss	\$(13,585) \$(8,590	\$(27,215)) \$(15,325)
Net loss attributable to common stockholders	\$(13,585) \$(10,829	\$(32,023)) \$(19,034)
Net loss per share attributable to common stockholders, basic				
and diluted	\$(0.45) \$(3.32	\$(1.25)) \$(6.19)
Shares used in computing net loss per share attributable to				
common				
stockholders, basic and diluted	30,055,943	3,257,806	25,697,40	7 3,076,907



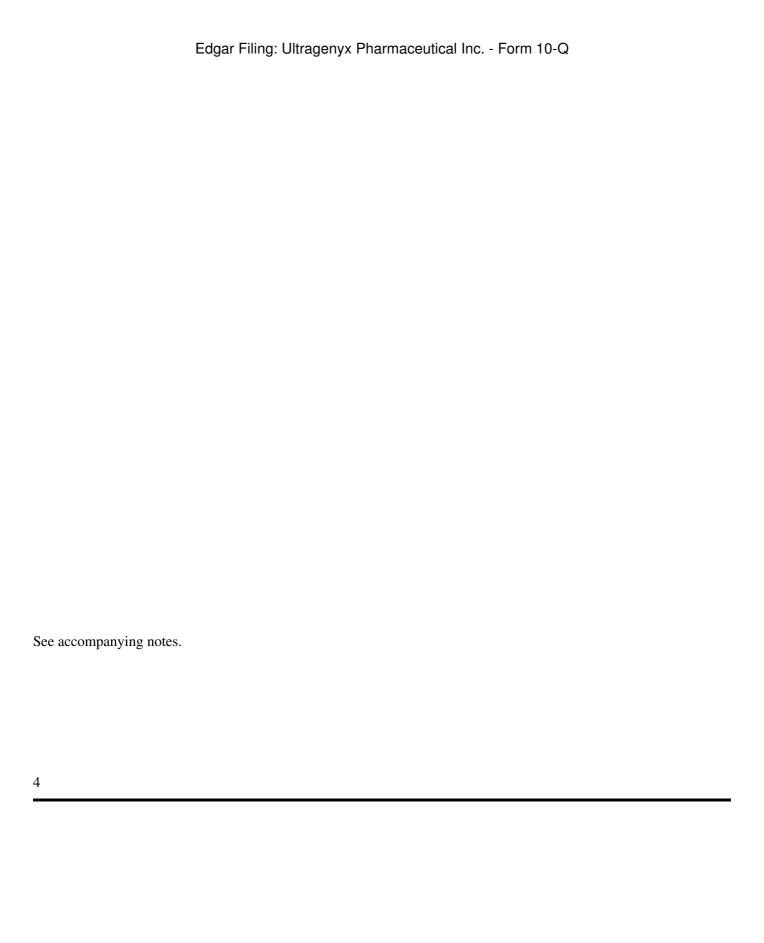
ULTRAGENYX PHARMACEUTICAL INC.

CONDENSED STATEMENTS OF COMPREHENSIVE LOSS

(Unaudited)

(In thousands)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2014	2013	2014	2013
Net loss	\$(13,585)	\$(8,590)	\$(27,215)	\$(15,325)
Other comprehensive income:				
Unrealized loss on available-for-sale securities	(2)	(66)	(59)	(77)
Total comprehensive loss	\$(13,587)	\$(8.656)	\$(27.274)	\$(15,402)



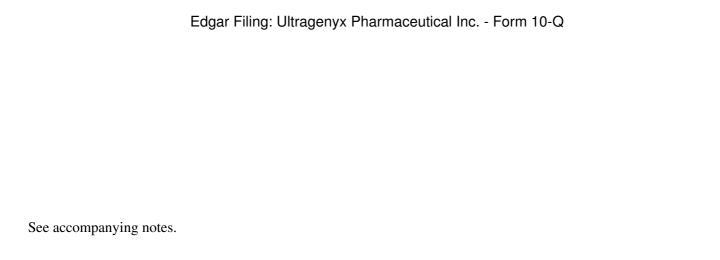
ULTRAGENYX PHARMACEUTICAL INC.

CONDENSED STATEMENTS OF CASH FLOWS

(Unaudited)

(In thousands)

	Six Months June 30,	Ended
	2014	2013
Operating activities:		
Net loss	\$(27,215)	\$(15,325)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	278	227
Amortization of premium (discount) on investment securities	1,396	365
Stock-based compensation	1,741	376
Revaluation of convertible preferred stock warrant liability	3,324	372
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(3,097)	(1,288)
Other assets	1,519	(129)
Accounts payable	1,960	886
Accrued liabilities and other liabilities	1,229	(164)
Net cash used in operating activities	(18,865)	(14,680)
Investing activities:		
Purchase of property and equipment	(1,257)	(75)
Purchase of investments	(129,298)	, ,
Proceeds from maturities of investments	37,974	—
Increase in restricted cash	(293)	(50)
Net cash used in investing activities	(92,874)	
Financing activities:		
Net proceeds from issuance of common stock	126,110	57
Payment of preferred stock dividend	(4,346)	
Net cash provided by financing activities	121,764	57
Net increase (decrease) in cash and cash equivalents	10,025	(72,683)
Cash and cash equivalents at beginning of period	7,427	86,190
Cash and cash equivalents at beginning of period	\$17,452	\$13,507
Cash and Cash equivalents at end of period	\$17,432	φ13,307
Supplemental disclosures of non-cash investing and financing information:		
Reclassification of warrant liability to equity upon conversion to		
common stock warrants	\$6,743	\$
Conversion of Series A and Series B preferred stock to common stock	\$129,360	\$ —
•		



ULTRAGENYX PHARMACEUTICAL INC.

Notes to Condensed Financial Statements

1. Organization

Ultragenyx Pharmaceutical Inc. (the Company) is a clinical-stage biotechnology company 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 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; 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; and a Phase 2 study of KRN23, an antibody targeting fibroblast growth factor 23, or FGF23, in pediatric patients with X-linked hypophosphatemia (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 June 30, 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 and six months ended June 30, 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 study 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 and to the Company's underwritten public offering in July 2014, were initially capitalized. IPO deferred offering costs of \$3.3 million were subsequently offset against IPO proceeds upon the completion of the IPO in February 2014. Underwritten public offering costs of \$444,000 were subsequently offset against offering proceeds on completion of our underwritten public offering in July 2014. As of June 30, 2014 and December 31, 2013, deferred offering costs of \$316,000 and \$2.3 million, respectively, 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.

Recently Issued Accounting Pronouncements

In June 2014, the Financial Accounting Standards Board issued Accounting Standards Update 2014-10, Development Stage Enterprises: Elimination of Certain Financial Reporting Requirements, Including an Amendment to Variable Interest Entities Guidance in Topic 810, Consolidation (ASU 2014-10). ASU 2014-10 removes the definition of a development stage entity from the Master Glossary of the Accounting Standards Codification, thereby removing the financial reporting distinction between development stage entities and other reporting entities from U.S. GAAP. ASU 2014-10 also eliminates the requirements for development stage entities to (1) present inception-to-date information in the statements of income, cash flows, and shareholder equity, (2) label the financial statements as those of a development stage entity, (3) disclose a description of the development stage activities in which the entity is engaged, and (4) disclose in the first year in which the entity is no longer a development stage entity that in prior years it had been in the development stage. The amendments also clarify that the guidance in Topic 275, Risks and Uncertainties, is applicable to entities that have not commenced planned principal operations. The amendments in ASU 2014-10 will be effective retrospectively except for the clarification to Topic 275, which shall be applied prospectively for annual reporting periods beginning after December 15, 2014, and interim periods therein. Early application of each of the amendments is permitted for any annual reporting period or interim period for which the entity's financial statements have not yet been issued. The Company elected to early adopt the provisions of ASU 2014-10 in the second quarter of 2014, as reflected in this Quarterly Report.

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):

	June 30. Level	el		
	1	Level 2	3	Total
Financial Assets:				
Money market funds	\$6,753	\$ —	\$ -	- \$6,753
Commercial paper		7,988	-	- 7,988
Corporate bonds		125,840	-	— 125,840
U.S. Government securities	-	3,002	-	_ 3,002
Total financial assets	\$6,753	\$136,830	\$ -	- \$143,583

	December 31, 2013			
	Level		Level	
	1	Level 2	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):

Six Months Ended June 30, 2014