

PTC THERAPEUTICS, INC.  
Form 10-Q  
August 07, 2014  
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**UNITED STATES**  
**SECURITIES AND EXCHANGE COMMISSION**

Washington, D.C. 20549

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**FORM 10-Q**

(Mark One)

**QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the quarterly period ended June 30, 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 number: 001-35969

## PTC Therapeutics, Inc.

(Exact name of registrant as specified in its charter)

**Delaware**

(State or other jurisdiction of incorporation or organization)

**04-3416587**

(I.R.S. Employer Identification Number)

**100 Corporate Court  
South Plainfield, NJ**

(Address of principal executive offices)

**07080**

(Zip Code)

**(908) 222-7000**

(Registrant's telephone number, including area code)

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

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate 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  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.

Large accelerated filer

Accelerated filer

Non-accelerated filer

Smaller reporting company

(Do not check if a 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

As of August 4, 2014 there were 30,069,897 shares of Common Stock, \$0.001 par value per share, outstanding.



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**FORWARD-LOOKING STATEMENTS**

This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Quarterly Report on Form 10-Q, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words anticipate, believe, estimate, expect, intend, may, plan, predict, project, target, potential, will, will continue, and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

The forward-looking statements in this Quarterly Report on Form 10-Q include, among other things, statements about:

- the timing and conduct of our clinical trials of Translarna (ataluren) for the treatment of Duchenne muscular dystrophy, cystic fibrosis and mucopolysaccharidosis type I, or MPS I, caused by nonsense mutations, as well as our trials in spinal muscular atrophy and BMI1, including statements regarding the timing of initiation, enrollment and completion of the trials and the period during which the results of the trials will become available;
- our plans to pursue development of Translarna for additional indications other than Duchenne muscular dystrophy, cystic fibrosis and MPS I, caused by nonsense mutations;
- our ability to advance our earlier stage programs, including our antibacterial program;
- our plans to pursue research and development of other product candidates;
- the potential advantages of Translarna;
- the rate and degree of market acceptance and clinical utility of Translarna;
- our ability to maintain the conditional marketing authorization of Translarna for the treatment of Duchenne muscular dystrophy caused by nonsense mutations, or nmDMD, in the European Economic Area;

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- the timing of and our ability to obtain additional marketing approvals of Translarna and our other product candidates, and the ability of Translarna and our other product candidates to meet existing or future regulatory standards;
- our estimates regarding the potential market opportunity for Translarna, including the size of eligible patient populations and our ability to identify such patients;
- our ability to expand the approved product label of Translarna for the treatment of nmDMD;
- our ability to commercialize Translarna in general, and specifically as a treatment for nmDMD, including our ability to successfully negotiate favorable pricing and reimbursement processes in the countries in which we may obtain regulatory approval;
- the timing and scope of our commercial infrastructure expansion, including the growth of our international presence in Europe and in other territories;
- the potential receipt of revenues from future sales of our product candidates, including our ability to earn a profit from sales or licenses of Translarna for the treatment of nmDMD;
- our sales, marketing and distribution capabilities and strategy;
- our ability to establish and maintain arrangements for the manufacture of Translarna and our other product candidates that are sufficient to meet clinical trial and commercial launch requirements;
- our estimates regarding expenses, future revenues, capital requirements and needs for additional financing, including our ability to maintain the level of our expenses consistent with our internal budgets and forecasts and to secure additional funds on favorable terms or at all;
- our intellectual property position;
- the impact of government laws and regulations;
- our competitive position; and



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- our expectations with respect to the development and regulatory status of our program directed against spinal muscular atrophy in collaboration with F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc., which we refer to collectively as Roche, and the Spinal Muscular Atrophy Foundation, or the SMA Foundation, and our estimates regarding future revenues from achievement of milestones in that program.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this Quarterly Report on Form 10-Q, particularly in Part II, Item 1A. Risk Factors, that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make.

You should read this Quarterly Report on Form 10-Q and the documents that we have filed as exhibits to this Quarterly Report on Form 10-Q and our Annual Report on Form 10-K for the year ended December 31, 2013 completely and with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward-looking statements whether as a result of new information, future events or otherwise, except as required by applicable law.

In this Quarterly Report on Form 10-Q, unless otherwise stated or the context otherwise requires, references to PTC, PTC Therapeutics, we, us, our and similar references refer to PTC Therapeutics, Inc. and, where appropriate, its subsidiaries. The trademarks, trade names and service marks appearing in this Quarterly Report on Form 10-Q are the property of their respective owners.



Table of Contents**PART I FINANCIAL INFORMATION****PTC Therapeutics, Inc.****Balance sheets (unaudited)****In thousands (except per share data)**

	<b>June 30, 2014</b>	<b>December 31, 2013</b>
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 21,172	\$ 15,414
Marketable securities	205,687	127,053
Prepaid expenses and other current assets	2,597	1,599
Grant and collaboration receivables, net	858	958
Total current assets	230,314	145,024
Fixed assets, net	6,417	6,730
Deposits and other assets	827	149
Total assets	\$ 237,558	\$ 151,903
<b>Liabilities and stockholders equity</b>		
Current liabilities:		
Accounts payable and accrued expenses	\$ 11,341	\$ 12,207
Current portion of long-term debt		49
Deferred revenue	242	878
Total current liabilities	11,583	13,134
Other long-term liabilities	2,219	2,227
Total liabilities	13,802	15,361
Stockholders equity:		
Preferred stock, \$0.001 par value. Undesignated 5,000,000 shares; issued and outstanding 0 shares at June 30, 2014 and December 31, 2013		
Common stock, \$0.001 par value. Authorized 125,000,000 shares; issued and outstanding 29,340,577 shares at June 30, 2014. Authorized 125,000,000 shares; issued and outstanding 23,803,282 shares at December 31, 2013	30	24
Additional paid-in capital	591,636	465,246
Accumulated other comprehensive income	90	70
Accumulated deficit	(368,000)	(328,798)
Total stockholders equity	223,756	136,542
Total liabilities and stockholders equity	\$ 237,558	\$ 151,903

See accompanying unaudited notes.

Table of Contents**PTC Therapeutics, Inc.****Statements of operations (unaudited)****In thousands (except per share data)**

	Three Months Ended June 30,		Six Months Ended June 30,	
	2014	2013	2014	2013
<b>Revenues:</b>				
Collaboration revenue	\$ 1,418	\$ 5,868	\$ 10,565	\$ 11,940
Grant revenue	259	986	329	2,056
<b>Total revenues</b>	<b>1,677</b>	<b>6,854</b>	<b>10,894</b>	<b>13,996</b>
<b>Operating expenses:</b>				
Research and development	18,313	14,712	34,202	25,969
General and administrative	8,733	6,595	16,273	11,056
<b>Total operating expenses</b>	<b>27,046</b>	<b>21,307</b>	<b>50,475</b>	<b>37,025</b>
Loss from operations	(25,369)	(14,453)	(39,581)	(23,029)
Interest income (expense), net	248	(114)	419	(6,276)
Other income (expense), net	17	(19)	(40)	34
Net loss	(25,104)	(14,586)	(39,202)	(29,271)
Deemed dividend				(18,249)
Gain on exchange of convertible preferred stock in connection with recapitalization				3,391
<b>Net loss attributable to common stockholders</b>	<b>\$ (25,104)</b>	<b>\$ (14,586)</b>	<b>\$ (39,202)</b>	<b>\$ (44,129)</b>
<b>Weighted-average shares outstanding:</b>				
Basic and diluted (in shares)	29,332,227	2,648,832	27,976,847	1,326,679
<b>Net loss per share applicable to common stockholders basic and diluted (in dollars per share)</b>	<b>\$ (0.86)</b>	<b>\$ (5.51)</b>	<b>\$ (1.40)</b>	<b>\$ (33.26)</b>

See accompanying unaudited notes.

Table of Contents**PTC Therapeutics, Inc.****Statements of comprehensive loss (unaudited)****In thousands**

	<b>Three Months Ended June 30,</b>		<b>Six Months Ended June 30,</b>	
	<b>2014</b>	<b>2013</b>	<b>2014</b>	<b>2013</b>
Net loss	\$ (25,104)	\$ (14,586)	\$ (39,202)	\$ (29,271)
Other comprehensive loss:				
Unrealized gain (loss) on marketable securities	10	(1)	20	(1)
Comprehensive loss	\$ (25,094)	\$ (14,587)	\$ (39,182)	\$ (29,272)

See accompanying unaudited notes.

Table of Contents**PTC Therapeutics, Inc.****Statements of cash flows (unaudited)****In thousands**

	<b>Six months ended June 30,</b>	
	<b>2014</b>	<b>2013</b>
<b>Cash flows from operating activities</b>		
Net loss	\$ (39,202)	\$ (29,271)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	1,150	1,222
Change in valuation of warrant liability	38	(34)
Non-cash interest expense		6,044
Amortization of premiums on investments	910	
Share-based compensation expense	7,983	2,502
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(998)	(760)
Grant and collaboration receivables	100	143
Deposits and other assets	(678)	41
Accounts payable and accrued expenses	(866)	3,035
Other long-term liabilities	(46)	3
Deferred revenue	(636)	(10,207)
Net cash used in operating activities	(32,245)	(27,282)
<b>Cash flows from investing activities</b>		
Purchases of fixed assets	(837)	(96)
Purchases of marketable securities	(132,603)	(11,604)
Maturities of marketable securities	53,079	20
Net cash used in investing activities	(80,361)	(11,680)
<b>Cash flows from financing activities</b>		
Payments on long-term debt	(49)	(2,174)
Net proceeds from sale of Series Four convertible preferred stock		60,785
Proceeds from exercise of options	30	
Net proceeds from public offerings	118,383	131,720
Net cash provided by financing activities	118,364	190,331
Net increase in cash and cash equivalents	5,758	151,369
Cash and cash equivalents, beginning of period	15,414	2,726
Cash and cash equivalents, end of period	\$ 21,172	\$ 154,095
<b>Supplemental disclosure of cash information</b>		
Cash paid for interest	\$ 1	\$ 282
<b>Supplemental disclosures of non-cash information related to investing and financing activities</b>		
Change in unrealized gain (loss) on marketable securities	\$ 20	\$ (1)
Change in carry value of preferred securities resulting from recapitalization	\$	\$ 3,391
IPO closing costs included in accounts payable and accrued expenses	\$	\$ 1,730

See accompanying unaudited notes.

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**PTC Therapeutics, Inc.**

**Notes to unaudited financial statements**

**June 30, 2014**

**In thousands (except per share data unless otherwise noted)**

**1. The Company**

PTC Therapeutics, Inc. (the Company or PTC) was incorporated as a Delaware corporation on March 31, 1998. During the second quarter of 2014, a wholly-owned subsidiary was established in Bermuda to hold certain intellectual property rights of the Company. In addition wholly-owned subsidiaries in Ireland and in Denmark were established during the second and third quarter of 2014, respectively. PTC is a biopharmaceutical company focused on the discovery and development of orally administered, proprietary small molecule drugs that target post-transcriptional control processes. The Company's internally discovered pipeline addresses multiple therapeutic areas, including rare disorders, oncology and infectious diseases. PTC has developed proprietary technologies that PTC applies in our drug discovery activities and in collaborations with leading biopharmaceutical companies.

The Company's lead candidate is ataluren, an investigational new drug in the US, for the treatment of patients with genetic disorders that arise from a type of genetic mutation known as a nonsense mutation. The brand name of ataluren is Translarna. On August 4, 2014, the Company was notified that the European Commission, or EC, granted conditional marketing authorization for Translarna for the treatment of Duchenne muscular dystrophy caused by nonsense mutations, or nmDMD, in ambulatory patients aged five years and older. The conditional marketing authorization allows the Company to market Translarna in the European Economic Area, or EEA, which is comprised of the 28 member states of the European Union plus Norway, Iceland and Liechtenstein. The conditional marketing authorization is subject to an annual review by the EMA and the Company will seek to renew the approval on an annual basis until its obligations have been fulfilled and the approval is converted from a conditional approval into a full approval.

The Company has devoted substantially all of its efforts to research and development, including clinical trials. The Company has not generated product revenue to date and is subject to a number of risks similar to those of other early stage companies, including dependence on key individuals, the difficulties inherent in the development of commercially usable products, the potential need to obtain additional capital necessary to fund the development of its products, and competition from other companies. As of June 30, 2014, the Company had an accumulated deficit of approximately \$368.0 million. The Company has financed its operations to date primarily through a public offering of common stock in February 2014, its initial public offering of common stock in June 2013 (see note 6 below), private placements of its convertible preferred stock, collaborations, bank debt, convertible debt financings, grant funding and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease area addressed by the Company's product candidates.

**2. Summary of significant accounting policies**

The Company's complete listing of significant accounting policies are described in note 2 of the notes to the Company's audited financial statements as of December 31, 2013 included in the Company's Annual Report on Form 10-K filed with the Securities and Exchange

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Commission (SEC) on March 6, 2014 (2013 Form 10-K). There have been no changes to our accounting policies during the quarter.

### **Basis of Presentation**

The accompanying unaudited financial information as of June 30, 2014 and for the three and six months ended June 30, 2014 and 2013 has been prepared by the Company pursuant to the rules and regulations of the SEC. Certain information and footnote disclosures normally included in financial statements prepared in accordance with generally accepted accounting principles in the United States (GAAP) have been condensed or omitted pursuant to such rules and regulations. These interim financial statements should be read in conjunction with the Company's audited financial statements as of December 31, 2013 and notes thereto included in the 2013 Form 10-K.

In the opinion of management, the unaudited financial information as of June 30, 2014 and for the three and six months ended June 30, 2014 and 2013 reflects all adjustments, which are normal recurring adjustments, necessary to present a fair statement of financial position, results of operations and cash flows. The results of operations for the three and six month periods ended June 30, 2014 are not necessarily indicative of the results to be expected for the year ended December 31, 2014 or for any other interim period or for any other future year.

### **Use of estimates**

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements, and the reported amounts of revenues and expenses during the reporting period. Actual results could differ from those estimates.

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**Recently issued accounting standard**

In May 2014, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) No. 2014-09, Revenue from Contracts with Customers. ASU 2014-09 will eliminate transaction- and industry-specific revenue recognition guidance under current GAAP and replace it with a principle-based approach for determining revenue recognition. ASU 2014-09 will require that companies recognize revenue based on the value of transferred goods or services as they occur in the contract. The ASU also will require additional disclosure about the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts, including significant judgments and changes in judgments and assets recognized from costs incurred to obtain or fulfill a contract. ASU 2014-09 is effective for annual reporting periods beginning after December 15, 2016. Early application is not permitted. Entities can transition to the standard either retrospectively or as a cumulative-effect adjustment as of the date of adoption. Presently, the Company is assessing what effect the adoption of ASU 2014-09 will have on its financial statements and accompanying notes.

**3. Fair value of financial instruments and marketable securities**

The Company follows the fair value measurement rules, which provides guidance on the use of fair value in accounting and disclosure for assets and liabilities when such accounting and disclosure is called for by other accounting literature. These rules establish a fair value hierarchy for inputs to be used to measure fair value of financial assets and liabilities. This hierarchy prioritizes the inputs to valuation techniques used to measure fair value into three levels: Level 1 (highest priority), Level 2, and Level 3 (lowest priority).

- Level 1 Unadjusted quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the balance sheet date.
  
- Level 2 Inputs other than quoted prices included within Level 1 that are observable for the asset or liability, either directly or indirectly. Level 2 inputs include quoted prices for similar assets and liabilities in active markets, quoted prices for identical or similar assets or liabilities in markets that are not active, inputs other than quoted prices that are observable for the asset or liability (i.e., interest rates, yield curves, etc.), and inputs that are derived principally from or corroborated by observable market data by correlation or other means (market corroborated inputs).
  
- Level 3 Inputs are unobservable and reflect the Company's assumptions as to what market participants would use in pricing the asset or liability. The Company develops these inputs based on the best information available.

Cash equivalents are reflected in the accompanying financial statements at fair value. The carrying amount of grant and collaboration receivables, accounts payable and accrued expenses, and debt approximates fair value due to the short-term nature of those instruments.

Fair value of certain marketable securities is based upon market prices using quoted prices in active markets for identical assets quoted on the last day of the period. In establishing the estimated fair value of the remaining investments, the Company used the fair value as determined by its

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investment advisors using observable inputs other than quoted prices.

The Company reviews its investments on a periodic basis for other-than-temporary impairments. This review is subjective, as it requires management to evaluate whether an event or change in circumstances has occurred in that period that may have a significant adverse effect on the fair value of the investment.

The following represents the fair value using the hierarchy described above for the Company's financial assets and liabilities that are required to be measured at fair value on a recurring basis as of June 30, 2014 and December 31, 2013:

	<b>June 30, 2014</b>			
	<b>Total</b>	<b>Quoted prices in active markets for identical assets (level 1)</b>	<b>Significant other observable inputs (level 2)</b>	<b>Significant unobservable inputs (level 3)</b>
Marketable securities	\$ 205,687	\$	\$ 205,687	\$
Warrant liability	96			96

	<b>December 31, 2013</b>			
	<b>Total</b>	<b>Quoted prices in active markets for identical assets (level 1)</b>	<b>Significant other observable inputs (level 2)</b>	<b>Significant unobservable inputs (level 3)</b>
Marketable securities	\$ 127,053	\$	\$ 127,053	\$
Warrant Liability	58			58



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The following is a summary of marketable securities accounted for as available-for-sale securities at June 30, 2014 and December 31, 2013:

	Amortized Cost		June 30, 2014 Gross Unrealized		Fair Value
			Gains	Losses	
Corporate debt securities	\$ 166,564	\$	185	\$ (77)	\$ 166,672
Government obligations	39,033		5	(23)	39,015
	\$ 205,597	\$	190	\$ (100)	\$ 205,687

	Amortized Cost		December 31, 2013 Gross Unrealized		Fair Value
			Gains	Losses	
Commercial paper	\$ 14,993	\$	5	\$	\$ 14,998
Corporate debt securities	111,989		97	(31)	112,055
	\$ 126,982	\$	102	\$ (31)	\$ 127,053

At June 30, 2014 and December 31, 2013, the Company held securities with an unrealized loss position that were not considered to be other-than-temporarily impaired as the Company has the ability to hold such investments until recovery of their fair value.

Marketable securities on the balance sheet at June 30, 2014 and December 31, 2013 mature as follows:

	June 30, 2014	
	Less Than 12 Months	More Than 12 Months
Corporate debt securities	\$ 71,387	\$ 95,285
Government obligations		39,015
Total Marketable securities	\$ 71,387	\$ 134,300

	December 31, 2013	
	Less Than 12 Months	More Than 12 Months
Commercial paper	\$ 14,998	\$
Corporate debt securities	54,159	57,896
Total Marketable securities	\$ 69,157	\$ 57,896

**Level 3 valuation**

The warrant liability is classified in Other long-term liabilities on the Company's balance sheet. The warrant liability is marked-to-market each reporting period with the change in fair value recorded as a gain or loss within Other income (expense), net on the Company's statement of operations until the warrants are exercised, expire or other facts and circumstances lead the warrant liability to be reclassified as an equity instrument. The fair value of the warrant liability is determined at each reporting period by utilizing the Black-Scholes option pricing model.

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The table presented below is a summary of changes in the fair value of the Company's Level 3 valuation for warrant liability for the period ended June 30, 2014:

	<b>Level 3 assets</b>	
Beginning balance as of December 31, 2013	\$	58
Change in fair value of warrant liability		38
Ending balance as of June 30, 2014	\$	96

Fair value of the warrant liability is estimated using an option-pricing model, which includes variables such as the expected volatility based on guideline public companies, the stock fair value, and the estimated time to a liquidity event. The significant assumptions used in preparing the option pricing model for valuing the Company's warrants as of June 30, 2014 include (i) volatility (81% - 83%), (ii) risk free interest rate (0.88% - 1.62%), (iii) strike price (\$128.00), (iv) fair value of common stock (\$26.14), and (v) expected life

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(2.96 - 5.23 years). The significant assumptions used in preparing the option pricing model for valuing the Company's warrants as of December 31, 2013 include (i) volatility (61-89%), (ii) risk free interest rate (0.07% - 2.10%), (iii) strike price (\$128.00 - \$2,520.00), (iv) fair value of common stock (\$16.97), and (v) expected life (0.30 - 5.70 years). See Note 6 for a description of the warrants issued in connection with the convertible notes.

#### 4. Other comprehensive income (loss) and accumulated other comprehensive items

Other comprehensive income (loss) includes changes in equity that are excluded from net income (loss), such as unrealized gains and losses on marketable securities.

The following table summarizes other comprehensive income and the changes in accumulated other comprehensive items for the three months ended June 30, 2014:

	Unrealized Gains On Marketable Securities	Total Accumulated Other Comprehensive Items
Balance at March 31, 2014	\$ 80	\$ 80
Other comprehensive income before reclassifications	10	10
Amounts reclassified from other comprehensive items		
Other comprehensive income	10	10
Balance at June 30, 2014	\$ 90	\$ 90

The following table summarizes other comprehensive income and the changes in accumulated other comprehensive items for the six months ended June 30, 2014:

	Unrealized Gains On Marketable Securities	Total Accumulated Other Comprehensive Items
Balance at December 31, 2013	\$ 70	\$ 70
Other comprehensive income before reclassifications	20	20
Amounts reclassified from other comprehensive items		
Other comprehensive income	20	20
Balance at June 30, 2014	\$ 90	\$ 90

**5. Accounts payable and accrued expenses**

Accounts payable and accrued expenses at June 30, 2014 and December 31, 2013 consist of the following:

	<b>June 30, 2014</b>	<b>December 31, 2013</b>
Employee compensation, benefits, and related accruals	\$ 4,149	\$ 5,103
Consulting and contracted research	3,851	4,006
Professional fees	1,814	1,294
Accounts payable	613	1,124
Other	914	680
	\$ 11,341	\$ 12,207

**6. Capital structure****2013 Recapitalization**

During January and February of 2013, the Company entered into a bridge financing arrangement with certain existing investors providing for the issuance by the Company of an aggregate of \$6 million of convertible promissory notes and warrants to purchase 2,527,675 shares of Series One convertible preferred stock (Series One) and Series Two convertible preferred stock (Series Two). The warrants have a per share exercise price of \$0.01, and as such, they are referred to as penny warrants. This bridge financing was closed in anticipation of the March 2013 Series Four financing event, which the Company refers to as the 2013 recapitalization.

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The Company allocated the proceeds of the convertible promissory notes between debt and warrant liability. Since the value of the warrants exceeded the proceeds from the convertible notes issued to existing investors, the value of the warrant in excess of the proceeds is considered a deemed dividend and reflected as an equity transaction in the financial statements. The Company recorded \$6.0 million to interest expense related to the debt discount associated with the convertible debt during the quarter ended March 31, 2013.

On March 7, 2013, the Company closed a private placement of a new series of convertible preferred stock that resulted in the 2013 recapitalization. In this private placement, the Company issued and sold an aggregate of 4,497,035 shares of its Series Four senior preferred stock (Series Four) for an aggregate purchase price of approximately \$54.0 million. Including the \$6.0 million raised with the bridge financing, total gross proceeds raised during the quarter ended March 31, 2013 was approximately \$60.0 million. In addition, the Company issued an aggregate of 502,919 shares of Series Four upon the share settlement of the convertible promissory notes described above that were issued in January and February 2013.

In connection with this private placement, the Company effected a one-for-120 reverse stock split of its common stock and an exchange of outstanding shares of Series One, Series Two and Series Three convertible preferred stock (Series Three) into an aggregate of 6,700,487 shares of a new series of Series Five junior preferred stock (Series Five). In addition, the Company issued an aggregate of 2,527,675 shares of Series One and Series Two upon the exercise of the warrants issued in connection with the bridge loan that were immediately exchanged for 2,095,515 shares of Series Five during the 2013 recapitalization.

The Company accounted for the 2013 recapitalization as an extinguishment of its Series One, Series Two and Series Three convertible preferred stock and recorded the Series Five shares at their fair value as of the recapitalization date. In accordance with authoritative accounting guidance, the Company recorded a gain attributable to the common stockholders on the extinguishment of the Series One, Series Two and Series Three. The gain of approximately \$3.4 million represents the excess of the Series One, Series Two and Series Three over the fair value of the shares Series Five issued in connection with the recapitalization.

**Initial Public Offering**

In June 2013, the Company closed the initial public offering of its common stock pursuant to a registration statement on Form S-1, as amended. The Company issued and sold an aggregate of 9,627,800 shares of common stock under the registration statement at a public offering price of \$15.00 per share, including 1,255,800 shares pursuant to the exercise by the underwriters of an over-allotment option. The Company received net proceeds from the initial public offering of approximately \$131.6 million, after deducting underwriting discounts and commissions and other offering expenses payable by the Company.

Upon closing the initial public offering, all outstanding shares of the Series Four and Series Five were converted into 14,170,956 shares of common stock.

**Follow-On Offering**

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In February 2014, the Company closed a follow-on public offering of its common stock pursuant to a registration statement on Form S-1, as amended. The Company issued and sold an aggregate of 5,163,265 shares of common stock under the registration statement at a public offering price of \$24.50 per share, including 673,469 shares pursuant to the exercise by the underwriters of an over-allotment option. The Company received net proceeds from the follow-on public offering of approximately \$118.4 million, after deducting underwriting discounts and commissions and other offering expenses payable by the Company.

### Warrants

All of the Company's outstanding warrants were classified as liabilities as of June 30, 2014 and December 31, 2013 because they contained non-standard antidilution provisions.

The following is a summary of the Company's outstanding warrants as of June 30, 2014:

	Warrant shares	Exercise price	Expiration
Common stock	6,250	\$ 128.00	2017
Common stock	7,030	\$ 128.00	2019 and 2020

The following is a summary of the Company's outstanding warrants as of December 31, 2013:

	Warrant shares	Exercise price	Expiration
Common stock	1,428	\$ 128.00	2014
Common stock	6,250	\$ 128.00	2017
Common stock	7,030	\$ 128.00	2019 and 2020
Common stock	452	\$ 2,520.00	2014

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In connection with the 2013 recapitalization, all of the Series Two outstanding warrants became warrants to purchase Series Five. In connection with the Company's initial public offering all of the Series Five outstanding warrants became warrants to purchase common stock.

## 7. Net loss per share

Basic earnings per share is computed by dividing net loss available to common stockholders by the weighted-average number of common shares outstanding. Diluted earnings per share is computed by dividing net income (loss) available to common stockholders by the weighted-average number of common shares plus the effect of dilutive potential common shares outstanding during the period.

The following tables set forth the computation of basic and diluted net income (loss) per share for common stockholders:

	<b>Three months ended June 30,</b>	
	<b>2014</b>	<b>2013</b>
<b>Numerator</b>		
Net loss attributable to common stockholders	\$ (25,104)	\$ (14,586)
<b>Denominator</b>		
Denominator for basic and diluted net loss per share	29,332,227	2,648,832
<b>Net loss per share:</b>		
Basic and diluted	\$ (0.86)*	\$ (5.51)*

\* In the three months ended June 30, 2014 and 2013, the Company experienced a net loss and therefore did not report any dilutive share impact.

	<b>Six months ended June 30,</b>	
	<b>2014</b>	<b>2013</b>
<b>Numerator</b>		
Net loss	\$ (39,202)	\$ (29,271)
Deemed dividend		(18,249)
Gain on exchange of convertible preferred stock in connection with recapitalization		3,391
Net loss attributable to common stockholders	\$ (39,202)	\$ (44,129)
<b>Denominator</b>		
Denominator for basic and diluted net loss per share	27,976,847	1,326,679
<b>Net loss per share:</b>		
Basic and diluted	\$ (1.40)*	\$ (33.26)*

\* In the six months ended June 30, 2014 and 2013, the Company experienced a net loss and therefore did not report any dilutive share impact.

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The following table shows historical dilutive common share equivalents outstanding, which are not included in the above historical calculation, as the effect of their inclusion is anti-dilutive during each period.

	2014	As of June 30, 2013
Stock Options	3,182,963	2,048,737
Unvested restricted stock	729,320	1,128,672
Total	3,912,283	3,177,409

### 8. Stock award plan

On March 5, 2013, the Company's Board of Directors approved the 2013 Stock Incentive Plan, which provides for the granting of stock option awards, stock appreciation rights, restricted stock, restricted stock units and other stock-based awards in the aggregate of



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739,937 shares of common stock. On March 5, 2013, the Board approved a grant of 735,324 shares of restricted stock and 4,613 stock options. There are no additional shares available for issuance under this plan.

In May 2013, the Company's Board of Directors and stockholders increased by 2,500,000 the number of shares authorized under the 2009 Stock Incentive Plan, which provides for the granting of stock option awards, restricted stock awards, and other stock-based and cash-based awards.

In May 2013, the Company's Board of Directors and stockholders approved the 2013 Long Term Incentive Plan, which became effective upon the closing of the Company's IPO. The 2013 Long Term Incentive Plan provides for the grant of incentive stock options, nonstatutory stock options, restricted stock awards and other stock-based awards. The number of shares of common stock reserved for issuance under the 2013 Long Term Incentive Plan is the sum of (1) 122,296 shares of common stock available for issuance under the Company's 2009 Equity and Long Term Incentive Plan and 2013 Stock Incentive Plan, (2) the number of shares (up to 3,040,444 shares) equal to the sum of the number of shares of common stock subject to outstanding awards under the Company's 1998 Employee, Director and Consultant Stock Option Plan and 2013 Stock Incentive Plan that expire, terminate or are otherwise surrendered, cancelled, forfeited or repurchased by the Company at their original issuance price pursuant to a contractual repurchase right plus (3) an annual increase, to be added on the first day of each fiscal year until the expiration of the 2013 Long Term Incentive Plan, equal to the lowest of 2,500,000 shares of common stock, 4% of the number of shares of common stock outstanding on the first day of the fiscal year and an amount determined by the Company's Board of Directors.

A summary of stock option activity is as follows:

	Number of options	Weighted- average exercise price	Weighted- average remaining contractual term	Aggregate intrinsic value (in thousands)
Outstanding at December 31, 2013	2,095,592	\$ 20.24		
Granted	1,174,946	\$ 27.78		
Exercised	(2,750)	\$ 10.85		
Forfeited	(84,825)	\$ 11.61		
Outstanding at June 30, 2014	3,182,963	\$ 23.26	9.09 years	\$ 29,263
Vested or Expected to vest at June 30, 2014	2,956,469	\$ 21.69	9.14 years	\$ 27,526
Exercisable at June 30, 2014	629,159	\$ 39.44	8.53 years	\$ 9,018

From January 1, 2014 through June 30, 2014, the Company issued a total of 1,174,946 stock options to various employees. Of those, 183,750 were inducement grants for non-statutory stock options. The awards were made pursuant to the NASDAQ inducement grant exception as a component of our new hires' employment compensation.

The fair value of grants made in the period ended June 30, 2014 was contemporaneously estimated on the date of grant using the following assumptions:

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	Six months ended June 30, 2014	
Risk-free interest rate	0.11%	2.03%
Expected volatility	89%	91%
Expected term	5.5 years	6.25 years

The Company assumed no expected dividends for all grants. The weighted average grant date fair value of options granted during the six month period ended June 30, 2014 was \$20.75 per share.

The Company uses the simplified method to determine the expected term of options. Under this method, the expected term represents the average of the vesting period and the contractual term. The expected volatility of share options was estimated based on a historical volatility analysis of peers that were similar to the Company with respect to industry, stage of life cycle, size, and financial leverage. The risk-free rate of the option is based on U.S. Government Securities Treasury Constant Maturities yields at the date of grant for a term similar to the expected term of the option.

*Restricted Stock Awards* Restricted stock awards are granted subject to certain restrictions, including in some cases service conditions (restricted stock). The grant-date fair value of restricted stock awards, which has been determined based upon the market value of the Company's shares on the grant date, is expensed over the vesting period.

The following table summarizes information on the Company's restricted stock:

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	<b>Restricted Stock</b>	
	<b>Number of Shares</b>	<b>Weighted Average Grant Date Fair Value</b>
January 1, 2014	1,110,226	\$ 10.68
Granted		
Vested	(371,280)	\$ 10.60
Forfeited	(9,626)	\$ 10.66
Unvested at June 30, 2014	729,320	\$ 10.72

The Company recorded share-based compensation expense in the statement of operations as follows:

	<b>Three Months Ended June 30,</b>		<b>Six Months Ended June 30,</b>	
	<b>2014</b>	<b>2013</b>	<b>2014</b>	<b>2013</b>
Research and development	\$ 2,209	\$ 1,107	\$ 4,153	\$ 1,364
General and administrative	2,069	774	3,830	1,138
Total	\$ 4,278	\$ 1,881		