PTC THERAPEUTICS, INC.
Form 10-Q
May 06, 2014
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UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 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

o 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	**04-3416587**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification Number)
100 Corporate Court **South Plainfield, NJ**	**07080**
(Address of principal executive offices)	(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 x No o

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 o

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

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

As of May 5, 2014 there were 30,074,453 shares of Common Stock, $0.001 par value per share, outstanding.

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FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form10-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 , w 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 ataluren for the treatment of Duchenne muscular dystrophy and cystic fibrosis caused by nonsense mutations, including statements regarding the timing of initiation and completion of the trials and the period during which the results of the trials will become available;

- the timing of and our ability to obtain marketing approval, including conditional approval in the European Union, of ataluren and our other product candidates, and the ability of ataluren and our other product candidates to meet existing or future regulatory standards;

- our expectations with respect to 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;

- the potential receipt of revenues from future sales of ataluren;

- our plans to pursue development of ataluren for additional indications other than Duchenne muscular dystrophy and cystic fibrosis caused by nonsense mutations;

- our plans to pursue research and development of other product candidates;

- the potential advantages of ataluren;

- the rate and degree of market acceptance and clinical utility of ataluren;
- our estimates regarding the potential market opportunity for ataluren;
- our sales, marketing and distribution capabilities and strategy;
- our ability to establish and maintain arrangements for manufacture of ataluren and our other product candidates;
- our intellectual property position;
- the impact of government laws and regulations;
- our competitive position; and
- our estimates regarding expenses, future revenues, capital requirements and needs for additional financing.

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 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 subsidiary. The trademarks, trade names and service marks appearing in this this Quarterly Report on Form 10-Q are the property of their respective owners.

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PART I FINANCIAL INFORMATION

Item 1. Financial Statements.

PTC Therapeutics, Inc.

Balance sheets (unaudited)

In thousands (except per share data)

	March 31, 2014	December 31, 2013
Assets		
Current assets:		
Cash and cash equivalents	$ 115,855	$ 15,414
Marketable securities	130,730	127,053
Prepaid expenses and other current assets	1,957	1,599
Grant and collaboration receivables, net	838	958
Total current assets	249,380	145,024
Fixed assets, net	6,328	6,730
Deposits and other assets	132	149
Total assets	$ 255,840	$ 151,903
Liabilities and stockholders equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 8,735	$ 12,207
Current portion of long-term debt	12	49
Deferred revenue	492	878
Total current liabilities	9,239	13,134
Other long-term liabilities	2,259	2,227
Total liabilities	11,498	15,361
Stockholders equity:		
Preferred stock, $0.001 par value. Undesignated 5,000,000 shares; issued and outstanding 0 shares at March 31, 2014 and December 31, 2013		
Common stock, $0.001 par value. Authorized 125,000,000 shares; issued and outstanding 29,325,997 shares at March 31, 2014. Authorized 125,000,000 shares; issued and outstanding 23,803,282 shares at December 31, 2013	30	24
Additional paid-in capital	587,128	465,246
Accumulated other comprehensive loss	80	70
Accumulated deficit	(342,896)	(328,798)
Total stockholders equity	244,342	136,542
Total liabilities and stockholders equity	$ 255,840	$ 151,903

See accompanying unaudited notes.

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PTC Therapeutics, Inc.

Statements of operations (unaudited)

In thousands (except per share data)

	Three Months Ended March 31,	
	2014	**2013**
Revenues:		
Collaboration revenue	$ 9,147	$ 6,072
Grant revenue	70	1,070
Total revenues	9,217	7,142
Operating expenses:		
Research and development	15,889	11,257
General and administrative	7,540	4,461
Total operating expenses	23,429	15,718
Loss from operations	(14,212)	(8,576)
Interest income (expense), net	171	(6,162)
Other (expense) income, net	(57)	54
Net loss	(14,098)	(14,684)
Deemed dividend		(18,249)
Gain on exchange of convertible preferred stock in connection with recapitalization		3,391
Net loss attributable to common stockholders	$ (14,098)	$ (29,542)
Weighted-average shares outstanding:		
Basic and diluted (in shares)	24,492,487	4,526
Net loss per share applicable to common stockholders basic and diluted (in dollars per share)	$ (0.58)	$ (6,527.30)

See accompanying unaudited notes.

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PTC Therapeutics, Inc.

Statements of comprehensive loss (unaudited)

In thousands

	Three Months Ended March 31,	
	2014	**2013**
Net loss	$ (14,098)	$ (14,684)
Other comprehensive loss:		
Unrealized gain on marketable securities	10	
Comprehensive loss	$ (14,088)	$ (14,684)

See accompanying unaudited notes.

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PTC Therapeutics, Inc.

Statements of cash flows (unaudited)

In thousands

	Three months ended March 31,	
	2014	**2013**
Cash flows from operating activities		
Net loss	$ (14,098)	$ (14,684)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	588	625
Change in valuation of warrant liability	55	(54)
Non-cash interest expense		6,023
Amortization of premiums on investments	414	
Share-based compensation expense	3,705	621
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(358)	(25)
Grant and collaboration receivables	120	68
Deposits and other assets	17	39
Accounts payable and accrued expenses	(3,472)	4,446
Other long-term liabilities	(23)	1
Deferred revenue	(386)	(4,907)
Net cash used in operating activities	(13,438)	(7,847)
Cash flows from investing activities		
Purchases of fixed assets	(186)	(21)
Purchases of marketable securities	(25,354)	
Maturities of marketable securities	21,273	
Net cash used in investing activities	(4,267)	(21)
Cash flows from financing activities		
Payments on long-term debt	(37)	(1,069)
Net proceeds from sale of Series Four convertible preferred stock		56,458
Net proceeds from secondary offering	118,183	
Net cash provided by financing activities	118,146	55,389
Net increase in cash and cash equivalents	100,441	47,521
Cash and cash equivalents, beginning of period	15,414	2,726
Cash and cash equivalents, end of period	$ 115,855	$ 50,247
Supplemental disclosure of cash information		
Cash paid for interest	$ 1	$ 162
Supplemental disclosures of non-cash information related to investing and financing activities		
Change in unrealized gain on marketable securities	$ 10	$
Change in carry value of preferred securities resulting from recapitalization	$	$ 3,391

See accompanying unaudited notes.

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PTC Therapeutics, Inc.

Notes to unaudited financial statements

March 31, 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. The Company 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 has devoted substantially all of its efforts to research and development, including clinical trials. The Company has not completed development of any drugs. 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 March 31, 2014, the Company had an accumulated deficit of approximately $342.9 million. The Company has financed its operations to date primarily through a public offering in February 2014, its initial public offering 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. The Company believes that its existing cash, cash equivalents, and marketable securities provide for sufficient resources to fund its currently planned operations through 2016.

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 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 March 31, 2014 and for the three months ended March 31, 2014 and 2013 has been prepared by the Company, without audit, 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 March 31, 2014 and for three months ended March 31, 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 month period ended March 31, 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.

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.

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- 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 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 March 31, 2014 and December 31, 2013:

	March 31, 2014			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities	$ 130,730	$	$ 130,730	$
Warrant liability	113			113

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

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

	March 31, 2014			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 3,998	$ 2	$	$ 4,000
U.S. corporate debt securities	126,651	115	(36)	126,730
	$ 130,649	$ 117	$ (36)	$ 130,730

	December 31, 2013			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 14,993	$ 5	$	$ 14,998
U.S. corporate debt securities				