PTC THERAPEUTICS, INC.
Form 10-Q
November 09, 2015
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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 September 30, 2015

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 x
Non-accelerated filer o (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 November 4, 2015 there were 34,271,694 shares of Common Stock, $0.001 par value per share, outstanding.

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PART I FINANCIAL INFORMATION

PART II OTHER INFORMATION

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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 Form10-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, might, plan, predict, project, target, potential, should, 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 of PTC s planned regulatory filings with respect to the results of our Phase 3 confirmatory trial in nonsense mutation Duchenne muscular dystrophy, or nmDMD, including the completion of our new drug application, or NDA, with the U.S. Food and Drug Administration, or FDA, our submission of the clinical trials results with the European Medicines Agency, or EMA, and other filings with regulatory bodies outside of the United States and European Economic Area, or EEA;

- the timing and conduct of our clinical trials and studies of Translarna (ataluren) for the treatment of cystic fibrosis, mucopolysaccharidosis type I, or MPS I, and aniridia, caused by nonsense mutations, as well as our studies in spinal muscular atrophy and our cancer stem cell program, 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;

- the rate and degree of market acceptance and clinical utility of Translarna;

- our ability to commercialize Translarna in general, and specifically as a treatment for nmDMD, including the timing of such commercialization and our ability to successfully negotiate adequate pricing and reimbursement processes on a timely basis, or at all, in the countries in which we may obtain regulatory approval, including the countries in the European Economic Area;

- the timing of and our ability to obtain additional marketing authorizations for Translarna and our other product candidates, and the ability of Translarna and our other product candidates to meet existing or future regulatory standards;

- our ability to obtain additional and maintain existing reimbursed named patient and cohort early access programs for Translarna for the treatment of nmDMD on adequate terms;

- 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;

- 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 Translarna and other 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, including the ability of our third party manufacturers to manufacture and deliver Translarna in commercially sufficient quantities and the ability of distributors to process orders in a timely manner and satisfy its other obligations to us;

- 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 plans to pursue development of Translarna for additional indications other than nmDMD and cystic fibrosis, MPS I, and aniridia, caused by nonsense mutations;

- our ability to maintain the marketing authorization of Translarna for the treatment of nmDMD in the EEA, which is

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conditioned upon, among other things, submission of the final report, including additional efficacy and safety data from our Phase 3 confirmatory trial in nmDMD during 2015 and which is subject to annual review and renewal by the EMA following its reassessment of the risk benefit balance of the authorization;

- 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;

- 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

- our expectations with respect to the development and regulatory status of our product candidate and 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, 2014 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.

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PART I FINANCIAL INFORMATION

Item 1. Financial Statements.

PTC Therapeutics, Inc.

Consolidated Balance Sheets (unaudited)

In thousands (except per share data)

	September 30, 2015	December 31, 2014
Assets		
Current assets:		
Cash and cash equivalents	$ 140,712	$ 49,748
Marketable securities	230,809	265,493
Prepaid expenses and other current assets	4,627	3,885
Receivables, net	8,016	4,445
Total current assets	384,164	323,571
Fixed assets, net	8,799	9,159
Deposits and other assets	3,137	489
Total assets	$ 396,100	$ 333,219
Liabilities and stockholders equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 31,695	$ 29,121
Deferred revenue		3,354
Total current liabilities	31,695	32,475
Long-term debt	93,198	
Other long-term liabilities	2,056	2,277
Total liabilities	126,949	34,752
Stockholders equity:		
Common stock, $0.001 par value. Authorized 125,000,000 shares; issued and outstanding 33,915,059 shares at September 30, 2015. Authorized 125,000,000 shares; issued and outstanding 32,898,392 shares at December 31, 2014	34	33
Additional paid-in capital	812,294	721,722
Accumulated other comprehensive loss	(1,127)	(737)
Accumulated deficit	(542,050)	(422,551)
Total stockholders equity	269,151	298,467
Total liabilities and stockholders equity	$ 396,100	$ 333,219

See accompanying unaudited notes.

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PTC Therapeutics, Inc.

Consolidated Statements of Operations (unaudited)

In thousands (except per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
Revenues:				
Net product revenue	$ 9,772	$ 81	$ 21,002	$ 81
Collaboration revenue	2	716	547	11,280
Grant revenue	2	897	2,483	1,226
Total revenues	9,776	1,694	24,032	12,587
Operating expenses:				
Research and development	30,640	18,765	86,768	52,967
Selling, general and administrative	21,368	10,530	56,193	26,803
Total operating expenses	52,008	29,295	142,961	79,770
Loss from operations	(42,232)	(27,601)	(118,929)	(67,183)
Interest (expense)/income, net	(852)	354	170	774
Other expense, net	(51)	(35)	(507)	(75