PTC THERAPEUTICS, INC.
Form 10-Q
August 04, 2016
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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 June 30, 2016

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 x	Accelerated filer o
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 August 1, 2016 there were 34,247,719 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:

- our ability to resolve the matters set forth in the Refuse to File letter we received from the United States Food and Drug Administration, or FDA, in connection with our New Drug Application, or NDA, for Translarna (ataluren) for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, including whether the appeal we filed with the FDA results in successful reversal of the Refuse to File decision in a timely manner, or ever, and in the event that the Refuse to File decision is reversed, whether such reversal results in a timely or successful review of our NDA, and whether we will be required to perform additional clinical and non-clinical trials or analyses at significant cost and whether such trials, if successful, may enable FDA review of an NDA submission by us and, ultimately, may support approval of Translarna for nmDMD in the U.S.;

- the timing and outcome of the opinion of the European Medicines Agency s, or EMA s, Committee for Medicinal Products for Human Use, or CHMP, with respect to our request for renewal of our marketing authorization of Translarna for the treatment of nmDMD in the European Economic Area, or EEA, which is subject to annual review and renewal by the European Commission following reassessment of the risk-benefit balance of the authorization by the European Medicines Agency, among other things;

- our ability to design an acceptable new clinical trial in nmDMD with input from the EMA, including with respect to matters of scope, length, and conduct and, if successfully designed, our ability to enroll, fund, and complete such trial;

- the nature of any conditions or restrictions that may be placed on any renewal of the marketing authorization by the European Commission in the event that the CHMP issues a positive opinion with respect to renewal;

- 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;

- when Translarna will be available to nmDMD patients in England;

- 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, or at all;

- our estimates regarding the potential market opportunity for Translarna, including, in general, the size of eligible patient populations and our ability to identify such patients;

- our regulatory submissions, including with respect to timing and outcome of regulatory review and determinations in connection with our submission with the EMA related to a variation to our marketing authorization to include Translarna as a treatment for nonsense mutation cystic fibrosis, or nmCF, as well as our other submissions with regulatory bodies outside of the EEA;

- our estimates regarding expenses, future revenues, third party discounts and rebates, 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;

- the timing and conduct of our clinical trials and studies of Translarna for the treatment of nmCF, nmDMD, mucopolysaccharidosis type I, or MPS I, aniridia, and Dravet syndrome/CDKL5, each 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;

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

- the ability and willingness of patients and healthcare professionals to access Translarna through alternative means if pricing and reimbursement negotiations in the applicable territory do not have a positive outcome, including whether patients in Germany will continue to be able to access Translarna via a reimbursed importation pathway provided under German law and whether such pathway, if utilized, will minimize any access issues for German patients while maintaining a sustainable price;

- 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 maintain the current label under the marketing authorization in the EEA or expand the approved product label of Translarna for the treatment of nmDMD, whether pursuant to our recently initiated Phase 2 study of Translarna for nmDMD in pediatric patients, or otherwise;

- 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 clinically and commercially sufficient quantities and the ability of distributors to process orders in a timely manner and satisfy their 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, nmCF, MPS I, aniridia, and Dravet/CDKL5, caused by nonsense mutations;

- our ability to advance our earlier stage programs, including our cancer stem cell program;
- our plans to pursue research and development of other product candidates;
- the potential advantages of Translarna;
- 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 candidates 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, 2015 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

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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, the Company, 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.

All website addresses given in this Quarterly Report on Form 10-Q are for information only and are not intended to be an active link or to incorporate any website information into this document.

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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)

	June 30, 2016	December 31, 2015
Assets		
Current assets:		
Cash and cash equivalents	$ 35,658	$ 58,022
Marketable securities	237,235	280,903
Prepaid expenses and other current assets	4,776	5,930
Trade receivables, net	19,765	11,094
Total current assets	297,434	355,949
Fixed assets, net	7,601	8,974
Deposits and other assets	528	358
Total assets	$ 305,563	$ 365,281
Liabilities and stockholders equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 42,183	$ 45,247
Deferred revenue	726	139
Total current liabilities	42,909	45,386
Long-term debt	94,936	91,848
Other long-term liabilities	2,094	2,046
Total liabilities	139,939	139,280
Stockholders equity:		
Common stock, $0.001 par value. Authorized 125,000,000 shares; issued and outstanding 34,083,319 shares at June 30, 2016. Authorized 125,000,000 shares; issued and outstanding 33,916,559 shares at December 31, 2015	34	34
Additional paid-in capital	837,850	820,165
Accumulated other comprehensive income (loss)	885	(1,200)
Accumulated deficit	(673,145)	(592,998)
Total stockholders equity	165,624	226,001
Total liabilities and stockholders equity	$ 305,563	$ 365,281

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 June 30,		Six Months Ended June 30,	
	2016	**2015**	**2016**	**2015**
Revenues:				
Net product revenue	$ 15,437	$ 6,161	$ 34,314	$ 11,230
Collaboration and grant revenue	196	613	214	3,026
Total revenues	15,633	6,774	34,528	14,256
Operating expenses:				
Research and development	28,827	28,190	60,226	56,128
Selling, general and administrative	23,366	17,210	49,304	34,825
Total operating expenses	52,193	45,400	109,530	90,953
Loss from operations	(36,560)	(38,626)	(75,002)	(76,697)
Interest (expense) income, net	(2,060)	498	(4,016)	1,022
Other expense, net	(387)	(88)	(1,107)	(456)
Loss before income tax expense	(39,007)	(38,216)	(80,125)	(76,131)
Income tax benefit (expense)	93	(145)	(22)	(145)
Net loss attributable to common stockholders	$ (38,914)	$ (38,361)	$ (80,147)	$ (76,276)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	34,000,333	33,600,653	33,959,751	33,335,674
Net loss per share basic and diluted (in dollars per share)	$ (1.14)	$ (1.14)	$ (2.36)	$ (2.29)

See accompanying unaudited notes.

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PTC Therapeutics, Inc.

Consolidated Statements of Comprehensive Loss (unaudited)

In thousands

	Three Months Ended June 30,		Six Months Ended June 30,	
	2016	**2015**	**2016**	**2015**
Net loss	$ (38,914)	$ (38,361)	$ (80,147)	$ (76,276)
Other comprehensive loss:				
Unrealized (loss) gain on marketable securities, net of tax	(40)	(224)	618	(99)
Foreign currency translation (loss) gain	(159)	465	1,467	341
Comprehensive loss	$ (39,113)	$ (38,120)	$ (78,062)	$ (76,034)

See accompanying unaudited notes.

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PTC Therapeutics, Inc.

Consolidated Statements of Cash Flows (unaudited)

In thousands

	Six months ended June 30,	
	2016	**2015**
Cash flows from operating activities		
Net loss	$ (80,147)	$ (76,276)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	1,664	1,319
Change in valuation of warrant liability	47	(72)
Non-cash interest expense	2,941	
Amortization of premiums on investments	1,140	915
Amortization of debt issuance costs	147	
Share-based compensation expense	17,651	18,076
Benefit for deferred income taxes	(244)	
Unrealized foreign currency transaction losses, net	963	
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	1,163	(356)
Trade receivables, net	(8,480)	(669)
Deposits and other assets	(170)	258
Accounts payable and accrued expenses	(3,435)	(6,039)
Other long-term liabilities	1	(46)
Deferred revenue	587	(3,354)
Net cash used in operating activities	(66,172)	(66,244)
Cash flows from investing activities		
Purchases of fixed assets	(275)	(1,177)
Purchases of marketable securities	(46,256)	(44,988)
Sale and redemption of marketable securities	89,645	83,468
Net cash provided by investing activities	43,114	37,303
Cash flows from financing activities		
Proceeds from exercise of options	34	8,072
Net cash provided by financing activities	34	8,072
Effect of exchange rate changes on cash	660	341
Net decrease in cash and cash equivalents	(22,364)	(20,528)
Cash and cash equivalents, beginning of period	58,022	49,748
Cash and cash equivalents, end of period	$ 35,658	$ 29,220
Supplemental disclosure of cash information		
Cash paid for interest	$ 2,263	$
Cash paid for income taxes	$ 264	$
Supplemental disclosures of non-cash information related to investing and financing activities		
Change in unrealized gain (loss) on marketable securities, net of tax	$ 618	$ (99)

See accompanying unaudited notes.

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PTC Therapeutics, Inc.

Notes to Consolidated Financial Statements (unaudited)

June 30, 2016

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. PTC is a global biopharmaceutical company focused on the discovery, development and commercialization of orally administered, small molecule therapeutics targeting an area of RNA biology referred to as post transcriptional control. Post-transcriptional control processes are the regulatory events that occur in cells during and after a messenger RNA molecule is copied from DNA through the transcription process. PTC has discovered all of its compounds currently under development using its proprietary technologies. PTC plans to continue to develop these compounds both on its own and through selective collaboration arrangements with leading pharmaceutical and biotechnology companies. PTC s internally discovered pipeline addresses multiple therapeutic areas, including rare disorders and oncology.

PTC s lead product candidate is ataluren, an investigational new drug in the United States, for the treatment of patients with genetic disorders that arise from a type of genetic mutation known as a nonsense mutation. The Company holds worldwide commercialization rights to ataluren for all indications in all territories. The brand name of ataluren is Translarna .

The Company received conditional marketing authorization from the European Commission in August 2014 for Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, in ambulatory patients aged five years and older in the 31 member states of the European Economic Area, or EEA. The marketing authorization is subject to annual review and renewal by the European Commission following reassessment by the European Medicines Agency, or EMA, of the risk-benefit balance of the authorization, or the annual EMA reassessment, as well as the Company s satisfaction of any conditions and obligations that have been or may be placed upon the marketing authorization. The Company has been informed that the annual EMA assessment procedure cannot be completed by mid-year 2016. During 2016, the Company s revenues have been and are expected to be primarily generated from sales of Translarna for the treatment of nmDMD in countries in the EEA where pricing and reimbursement approval is obtained at acceptable levels and in other territories where the Company is permitted to distribute Translarna under reimbursed early access programs, or EAPs. The Company 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, 2016, the Company had an accumulated deficit of approximately $673.1 million. The Company has financed its operations to date primarily through the private offering in August 2015 of 3.00% convertible senior notes due 2022 (see Note 9), public offerings of common stock in February 2014 and October 2014, its initial public offering of common stock in June 2013, 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, 2015 included in the Company s Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on February 29, 2016 (2015 Form 10-K).

Basis of Presentation

The accompanying financial information as of June 30, 2016 and for the three and six months ended June 30, 2016 and 2015 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, 2015 and notes thereto included in the 2015 Form 10-K.

In the opinion of management, the unaudited financial information as of June 30, 2016 and for the three and six months ended June 30, 2016 and 2015 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, 2016 are not necessarily indicative of the results to be expected for the year ended December 31, 2016 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 amounts reported in the financial statements and accompanying notes. Significant estimates in these consolidated financial statements have been made in connection with the calculation of net product sales, certain accruals related to the Company s research

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and development expenses, stock-based compensation, valuation procedures for the convertible notes and the provision for or benefit from income taxes. Actual results could differ from those estimates. Changes in estimates are reflected in reported results in the period in which they become known.

Inventories and cost of product revenue

In 2014, the Company was notified that the European Commission, or EC, granted marketing authorization for Translarna for the treatment of nmDMD in ambulatory patients aged five years and older. The conditional marketing authorization allows the Company to market Translarna for the treatment of nmDMD in the 31 member states of European Economic Area. The launch in these countries is on a country by country basis. This marketing authorization is subject to annual review and renewal by the EC following reassessment by the European Medicines Agency, or EMA, of the risk benefit balance of the authorization, which the Company refers to as the annual EMA reassessment. In the third quarter of 2015, the EMA approved the annual renewal of the marketing authorization for Translarna for the treatment of nmDMD. The authorization was further conditioned on the Company s submission of the final report, including additional efficacy and safety data, from ACT DMD and the Company s ability to implement measures, including pharmacovigilance plans that are detailed in the risk management plan for Translarna that was submitted to EMA. In January 2016, the Company submitted the final ACT DMD report to the EMA. The Company made this submission as a type II variation request that sought to have this initial condition to its marketing authorization removed and a full marketing authorization granted. In February 2016, the Company also submitted a marketing authorization renewal request with the EMA.

While the Company has been informed that the renewal assessment procedure cannot be completed by mid-year 2016, it expects that, pursuant to applicable regulations, its current marketing authorization status will remain valid while the annual EMA reassessment is ongoing and until it is concluded with an opinion from the European Commission with respect to renewal of its marketing authorization. Based on its interpretation of applicable regulatory timeframes, the Company believes the annual EMA reassessment could be completed, at the earliest, by the end of 2016.

The Company plans to seek to renew the marketing authorization on an annual basis until the Company s obligations have been fulfilled and the approval is converted from a conditional approval into a full approval. If the Company fails to satisfy such requirements, or if it is determined that the balance of risks and benefits of using Translarna changes materially, the EC could, at the EMA s recommendation, vary, suspend, withdraw or refuse to renew the marketing authorization for Translarna or require additional clinical trials.

There continues to be substantial risk that regulators could suspend or not renew the Company s marketing authorization in the future. As such, as of the date of this filing, the Company has not capitalized inventory given the near term uncertainty with respect to the long term utilization of Translarna finished product for commercial use. Had the Company capitalized as inventory all of the its Translarna product that is available for commercial sale on hand as of June 30, 2016, the value of that inventory would have been approximately $1.2 million. In addition, had the Company expensed the cost of Translarna product sold as a cost of sales, the gross profit margin would have been greater than 90%, which the Company believes is consistent with the cost of producing small molecule therapeutics for orphan drug diseases in the pharmaceutical industry. The Company will continue to assess the appropriateness of inventory capitalization based on the outcome of applicable regulatory approvals which are expected later this year.

Revenue Recognition

The Company recognizes revenue when amounts are realized or realizable and earned. Revenue is considered realizable and earned when the following criteria are met: (1) persuasive evidence of an arrangement exists; (2) delivery has occurred or services have been rendered; (3) the price is fixed or determinable; and (4) collection of the amounts due are reasonably assured.

Net Product Sales

The Company s net product sales have consisted solely of sales of Translarna for the treatment of nmDMD in territories outside of the U.S. The Company began recognizing revenue for payments received under the reimbursed EAPs for Translarna in nmDMD patients in select countries in the third quarter of 2014. The Company has now established a pattern of collectability and, since January 2015, the Company recognizes revenue from product sales when there is persuasive evidence that an arrangement exists, title to product and associated risk of loss has passed to the customer, the price is fixed or determinable, collectability is reasonably assured and the Company has no further performance obligations in accordance with Financial Accounting Standards Board (FASB) Accounting Standards Codification (ASC) Subtopic 605-15, Revenue Recognition Products.

The Company has recorded revenue on sales where Translarna is available either on a commercial basis or through a reimbursed EAP program. Orders for Translarna are generally received from hospital and retail pharmacies and, in some cases, one of the Company s third-party partner distributors. The Company s third-party distributors act as intermediaries between the Company and end users and do not typically stock significant quantities of Translarna. The ultimate payor for Translarna is typically a government authority or institution or a third-party health insurer.

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The Company records revenue net of estimated third party discounts and rebates. Allowances are recorded as a reduction of revenue at the time revenues from product sales are recognized. These allowances are adjusted to reflect known changes in factors and may impact such allowances in the quarter those changes are known.

Collaboration and Grant Revenue

The terms of these agreements typically include payments to the Company of one or more of the following: nonrefundable, upfront license fees; milestone payments; research funding and royalties on future product sales. In addition, the Company generates service revenue through agreements that generally provide for fees for research and development services and may include additional payments upon achievement of specified events.

The Company evaluates all contingent consideration earned, such as a milestone payment, using the criteria as provided by the Financial Accounting Standards Board (FASB), guidance on the milestone method of revenue recognition. At the inception of a collaboration arrangement, the Company evaluates if a milestone payment is substantive. The criteria requires that (1) the Company determines if the milestone is commensurate with either its performance to achieve the milestone or the enhancement of value resulting from our activities to achieve the milestone; (2) the milestone be related to past performance; and (3) the milestone be reasonable relative to all deliverable and payment terms of the collaboration arrangement. If these criteria are met then the contingent milestones can be considered a substantive milestone and will be recognized as revenue in the period that the milestone is achieved. The Company recognizes royalties as earned in accordance with the terms of various research and collaboration agreements. If not substantive, the contingent consideration is allocated to the existing units of accounting based on relative selling price and recognized following the same basis previously established for the associated unit of accounting.

The Company recognizes revenue for reimbursements of research and development costs under collaboration agreements as the services are performed. The Company records these reimbursements as revenue and not as a reduction of research and development expenses as the Company has the risks and rewards as the principal in the research and development activities.

Recently issued accounting standards

In May 2014, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) No. 2014-09, Revenue from Contracts with Customers (Topic 606) . ASU No. 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 No. 2014-09 includes the required steps to achieve the core principle that an entity should recognize revenue to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. The ASU will also 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. With the issuance of ASU No. 2015-14 in August 2015, the FASB deferred the effective date of the revenue recognition guidance to reporting periods beginning after December 15, 2017. Early adoption of the standard is permitted but not before the original effective date, which was for reporting periods beginning after December 15, 2016. With the issuance of ASU No. 2016-08 in March 2016 and ASU No. 2016-10 in April 2016, the FASB further amended guidance on recording revenue on a gross versus a net basis and on identifying performance obligations and licensing, respectively. The Company expects to adopt this guidance when effective and continues to evaluate the effect that the updated standard, as well as additional amendments, may have on its consolidated financial statements and accompanying notes.

In August 2014, the FASB issued ASU 2014-15, Presentation of Financial Statements Going Concern (Subtopic 205-40): Disclosure of Uncertainties about an Entity s Ability to Continue as a Going Concern , which defines management s responsibility to assess an entity s ability to continue as a going concern, and to provide related footnote disclosures if there is substantial doubt about its ability to continue as a going concern. The pronouncement is effective for annual reporting periods ending after December 15, 2016 with early adoption permitted. The adoption of this guidance is not expected to have a significant impact on the Company s financial statements.

In April 2015, the FASB issued ASU No. 2015-03, Interest Imputation of Interest (Subtopic 835-30): Simplifying the Presentation of Debt Issuance Costs topic of the Codification . This standard provides a simplified presentation of debt issuance costs and requires that debt issuance costs related to a recognized debt liability to be presented on the balance sheet as a direct deduction from the carrying amount of that debt liability, consistent with debt discounts. The standard is effective for public companies for annual periods beginning after December 15, 2015. The Company adopted the guidance on January 1, 2016 on a retrospective basis and reclassed $2.8 million from Deposits and other assets to Long-term debt on the balance sheet as of December 31, 2015. The Company s unamortized debt issuance cost at June 30, 2016 was $2.6 million which is included within Long-term debt on the consolidated balance sheet.

In November 2015, the FASB issued ASU No. 2015-17, Income Taxes (Topic 740): Balance Sheet Classification of Deferred Taxes . This standard requires all deferred tax assets and liabilities to be classified as non-current on the balance sheet instead of separating deferred taxes into current and non-current amounts. In addition, valuation allowance allocations between current and non-current deferred tax assets are no longer required because those allowances also will be classified as non-current. This standard is effective for public companies for annual periods beginning after December 15, 2016. Earlier application is permitted as of the beginning of an interim or annual reporting period. The Company s deferred tax assets is provided with full valuation allowance as of June 30, 2016. As such, the Company does not expect that this standard will have a significant impact upon adoption.

In January 2016, the FASB issued ASU No. 2016-01, Financial Instruments - Overall (Subtopic 825-10): Recognition and Measurement of Financial Assets and Financial Liabilities . This standard enhances the reporting model for financial instruments, which includes amendments to address aspects of recognition, measurement, presentation and disclosure. The new guidance affects all

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reporting organizations (whether public or private) that hold financial assets or owe financial liabilities. ASU 2016-01 is effective for years beginning after December 15, 2017, including interim periods within those fiscal years. The Company expects to adopt this guidance when effective and is currently assessing what effect the adoption of ASU No. 2016-01 will have on its consolidated financial statements and accompanying notes.

In February 2016, the FASB issued ASU No. 2016-02, Leases (Topic 842) . This standard will require organizations that lease assets with lease terms of more than 12 months to recognize assets and liabilities for the rights and obligations created by those leases on their balance sheets. The ASU will also require new qualitative and quantitative disclosures to help investors and other financial statement users better understand the amount, timing, and uncertainty of cash flows arising from leases. The standard is effective for public companies for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2018, with early adoption permitted. The Company expects to adopt this guidance when effective and is currently assessing what effect the adoption of ASU No. 2016-02 will have on its consolidated financial statements and accompanying notes.

In March 2016, the FASB issued ASU No. 2016-09, Compensation Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting . This standard requires the recognition of all income tax effects of awards in the income statement when the awards vest or are settled, with Additional Paid in Capital (APIC) pools to be eliminated. In addition, the standard will increase the amount an employer can withhold to cover income taxes on awards and still qualify for the exception to liability classification for shares used to satisfy the employer s statutory income tax withholding obligation as well as allowing companies to elect whether to account for forfeitures of share-based payments by recognizing forfeitures of awards as they occur or estimating the number of awards expected to be forfeited and adjusting the estimate when it is likely to change, as is currently required. This standard is effective for public companies for fiscal years beginning after December 15, 2016 and interim periods within those years, with early adoption permitted but only if all of the guidance is adopted in the same period. The Company expects to adopt this guidance when effective and is currently assessing what effect the adoption of ASU No. 2016-09 will have on its consolidated financial statements and accompanying notes.

In June 2016, the FASB issued ASU No. 2016-13, Financial Instruments Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments . This standard requires financial assets measured at amortized cost basis to be presented at the net amount expected to be collected. This standard is effective for public companies who are SEC filers for fiscal years beginning after December 15, 2019, including interim periods within those years. The Company expects to adopt this guidance when effective and is assessing what effect the adoption of ASU 2016-13 will have on its consolidated 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 and investments 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.

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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 June 30, 2016 and December 31, 2015:

	June 30, 2016			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities	$ 237,235	$	$ 237,235	$
Warrant liability	$ 1	$	$	$ 1
Stock appreciation rights liability	$ 140	$	$	$ 140

	December 31, 2015			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities	$ 280,903	$	$ 280,903	$
Warrant Liability	$ 48	$	$	$ 48
Stock appreciation rights liability	$	$	$	$

No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the periods ended June 30, 2016 and December 31, 2015.

The following is a summary of marketable securities accounted for as available-for-sale securities at June 30, 2016 and December 31, 2015:

	June 30, 2016			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 11,969	$ 21	$	$ 11,990
Corporate debt securities	210,640	274	(26)	210,888
Government obligations	14,353	4		14,357
	$ 236,962	$ 299	$ (26)	$ 237,235

	December 31, 2015			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 26,877	$ 80	$	$ 26,957
Corporate debt securities	226,959		(640)	226,319
Government obligations	27,656	3	(32)	27,627
	$ 281,492	$ 83	$ (672)	$ 280,903

At June 30, 2016 and December 31, 2015, 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. Unrealized gains and losses are reported as a component of accumulated other comprehensive (loss) income in stockholders equity. As of June 30, 2016 and December 31, 2015, the Company did not have any realized gains/losses from the sale of marketable securities.

Marketable securities on the balance sheet at June 30, 2016 and December 31, 2015 mature as follows:

	June 30, 2016	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 11,990	$
Corporate debt securities	159,714	51,174
Government obligations	14,357	
Total Marketable securities	$ 186,061	$ 51,174

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	December 31, 2015	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 26,957	$
Corporate debt securities	140,831	85,488
Government obligations	18,994	8,633
Total Marketable securities	$ 186,782	$ 94,121

The Company classifies all of its securities as current as they are all available for sale and are available for current operations.

Level 3 valuation

The warrant liability is classified in Other long-term liabilities on the Company s consolidated balance sheets. The warrant liability is marked-to-market each reporting period with the change in fair value recorded as a gain or loss within Other expense, net, on the Company s consolidated statements 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.

The stock appreciation rights (SARs) liability is classified in Other long-term liabilities on the Company s consolidated balance sheets. The SARs liability is marked-to-market each reporting period with the change in fair value recorded as compensation expense on the Company s consolidated statements of operations until the SARS vest. The fair value of the SARs liability is determined at each reporting period by utilizing the Black-Scholes option pricing model.

The table presented below is a summary of changes in the fair value of the Company s Level 3 valuations for the warrant liability and SARs liability for the period ended June 30, 2016:

	Level 3 liabilities	
	Warrants	SARs
Beginning balance as of December 31, 2015	$ 48	$
Change in fair value	(47)	140
Ending balance as of June 30, 2016	$ 1	$ 140

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, 2016 include (i) volatility (75% 77%), (ii) risk free interest rate (0.45% 0.71%), (iii) strike price ($128.00-$2,520.00), (iv) fair value of common stock ($7.02), and (v) expected life (0.96 3.23 years). The significant assumptions used in preparing the option pricing model for valuing the Company s warrants as of December 31, 2015 include (i) volatility (62%-70%), (ii) risk free interest rate (0.86% 1.54%), (iii) strike price ($128.00 $2,520.00), (iv) fair value of common stock ($32.40), and (v) expected life (1.50 3.70 years).

Fair value of the SARs 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 SARs as of June 30, 2016 include (i) volatility (70%), (ii) risk free interest rate (0.36% 0.86%), (iii) strike price ($6.76-$30.86), (iv) fair value of common stock ($7.02), and (v) expected life (0.52 3.52 years).

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 tables summarize other comprehensive income (loss) and the changes in accumulated other comprehensive items for the three and six months ended June 30, 2016:

	Unrealized Gains/(Losses) On Marketable Securities, net of tax	Foreign Currency Translation	Total Accumulated Other Comprehensive Items
Balance at March 31, 2016	$ 69	$ 1,015	$ 1,084
Other comprehensive loss before reclassifications	(40)	(159)	(199)
Amounts reclassified from other comprehensive items			
Other comprehensive loss	(40)	(159)	(199)
Balance at June 30, 2016	$ 29	$ 856	$ 885

	Unrealized Gains/(Losses) On Marketable Securities, net of tax	Foreign Currency Translation	Total Accumulated Other Comprehensive Items
Balance at December 31, 2015	$ (589)	$ (611)	$ (1,200)
Other comprehensive income before reclassifications	618	1,467	2,085
Amounts reclassified from other comprehensive items			
Other comprehensive income	618	1,467	2,085
Balance at June 30, 2016	$ 29	$ 856	$ 885

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5. Accounts payable and accrued expenses

Accounts payable and accrued expenses at June 30, 2016 and December 31, 2015 consist of the following:

	June 30, 2016	December 31, 2015
Employee compensation, benefits, and related accruals	$ 4,688	$ 11,187
Consulting and contracted research	12,846	13,753
Professional fees	1,638	2,523
Accounts payable	16,800	11,940
Accrued severance	995	
Other	5,216	5,844
	$ 42,183	$ 45,247

6. Warrants

All of the Company s outstanding warrants were classified as liabilities as of June 30, 2016 and December 31, 2015 because they contained non-standard antidilution provisions.

The following is a summary of the Company s outstanding warrants as of June 30, 2016 and December 31, 2015:

	Warrant shares	Exercise price	Expiration
Common stock	6,250	$ 128.00	2017
Common stock	7,030	$ 128.00	2019
Common stock	130	$ 2,520.00	2019

7. Net loss per share

Basic earnings per share is computed by dividing net income (loss) by the weighted-average number of common shares outstanding. Diluted earnings per share is computed by dividing net income (loss) 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 loss per share:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2016	2015	2016	2015
Numerator				
Net loss	$ (38,914)	$ (38,361)	$ (80,147)	$ (76,276)
Denominator				
Denominator for basic and diluted net loss per share	34,000,333	33,600,653	33,959,751	33,335,674
Net loss per share:				
Basic and diluted	$ (1.14)*	$ (1.14)*	$ (2.36)*	$ (2.29)*

*In the three and six months ended June 30, 2016 and 2015, the Company experienced a net loss and therefore did not report any dilutive share impact.

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.

	As of June 30,	
	2016	2015
Stock Options	5,969,382	4,663,852
Unvested restricted stock awards and units	274,490	353,135
Total	6,243,872	5,016,987

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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 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 Equity and Long Term 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, 2009 Equity and Long Term Incentive 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. As of June 30, 2016, awards for 326,101 shares of common stock are available for issuance.

From January 1, 2016 through June 30, 2016, the Company issued a total of 1,403,045 stock options to various employees. Of those, 93,100 were inducement grants for non-statutory stock options. The inducement grant awards were made pursuant to the NASDAQ inducement grant exception as a material component of our new hires employment compensation and not under the 2013 Long Term Incentive Plan.

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, 2015	4,826,477	$ 37.20		
Granted	1,403,045	$ 29.06		

Exercised	(3,125)	$	10.85			
Forfeited/Cancelled	(257,015)	$	47.36			
Outstanding at June 30, 2016	5,969,382	$	35.02	8.21 years	$	6
Vested or Expected to vest at June 30, 2016	3,361,980	$	35.89	8.66 years	$	5
Exercisable at June 30, 2016	2,357,502	$	33.60	7.50 years	$	

The fair value of grants made in the six months ended June 30, 2016 was contemporaneously estimated on the date of grant using the following assumptions:

	Six months ended June 30, 2016
Risk-free interest rate	1.31% 2.24%
Expected volatility	67% 71%
Expected term	5.05 10.00 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, 2016 was $17.98 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.

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Restricted Stock Awards Restricted stock awards are granted subject to certain restrictions, including in some cases service or time 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.

Restricted Stock Units Restricted stock units are granted subject to certain restrictions, including in some cases service or time conditions (restricted stock). The grant-date fair value of restricted stock units, 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 awards and units:

	Restricted Stock Awards and Units	
	Number of Shares	Weighted Average Grant Date Fair Value
January 1, 2016	344,335	$ 10.85
Granted	141,185	$ 30.86
Vested	(163,635)	$ 10.85
Forfeited	(47,395)	$ 18.19
Unvested at June 30, 2016	274,490	$ 19.86

The Company recorded share-based compensation expense in the statement of operations related to incentive stock options, nonstatutory stock options, restricted stock awards and restricted stock units as follows:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2016	2015	2016	2015