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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

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, 2018

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	**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 ☑ 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, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer", "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer ☐	Accelerated filer ☑
Non-accelerated filer ☐ (Do not check if a smaller reporting company)	Smaller reporting company ☐
	Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

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 3, 2018, there were 46,713,886 shares of Common Stock, $0.001 par value per share, outstanding.

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PTC Therapeutics, Inc.

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," "might," "plan," "predict," "project," "target," "potential," "will," "would," "could," "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 expectations with respect to the closing of our planned acquisition of Agilis Biotherapeutics, Inc., or Agilis, and the other transactions contemplated in conjunction with the acquisition, including with respect to matters of timing, including the satisfaction of closing conditions, the anticipated financial impact and potential benefits to us, integration of Agilis into our business and any product candidates we may acquire from Agilis into our business strategy assuming completion of the acquisition and other matters related to the acquisition;

- our expectations and the potential financial impact and benefits related to our Collaboration and Licensing Agreement with Akcea Therapeutics, Inc., or Akcea, including with respect to the timing of regulatory approval of Tegsedi™ (inotersen) and Waylivra™ (volanesorsen) in countries which we are licensed to commercialize them, the potential commercialization of Tegsedi and Waylivra, and the our expectations with respect to contingent payments to Akcea based on the potential achievement of certain regulatory milestones and royalty payments by us to Akcea based on our potential achievement of certain net sales thresholds;

- our ability to negotiate, secure and maintain adequate pricing, coverage and reimbursement terms and processes on a timely basis, or at all, with third-party payors for Emflaza™ (deflazacort) for the treatment of Duchenne muscular dystrophy, or DMD, in the United States and for Translarna™ (ataluren) for the treatment of nonsense mutation DMD, or nmDMD, in the European Economic Area, or EEA, and other countries in which we have or may obtain regulatory approval, or in which there exist significant reimbursed early access programs, or EAP programs;

- our ability to maintain our marketing authorization of Translarna for the treatment of nmDMD in the EEA (which is subject to the specific obligation to conduct and submit the results of Study 041 to the European Medicines Agency, or EMA, and annual review and renewal by the European Commission following reassessment of the benefit-risk balance of the authorization by the EMA);

- our ability to enroll, fund, and complete Study 041, a multicenter, randomized, double-blind, 18-month, placebo-controlled clinical trial of Translarna for the treatment of nmDMD followed by an 18-month open label extension, according to the protocol agreed with the EMA, and by the trial's deadline;

- the anticipated period of market exclusivity for Emflaza for the treatment of DMD in the United States under the Orphan Drug Act of 1983, or the Orphan Drug Act, the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act and through any grant of pediatric exclusivity;

- our ability to complete the United States Food and Drug Administration, or FDA, post-marketing requirements to the marketing authorization of Emflaza or any requirements necessary to obtain any grant of pediatric exclusivity;

- our expectations with respect to our acquisition of all rights to Emflaza from Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC), or Marathon, including with respect to our ability to realize the anticipated benefits of the acquisition (including with respect to future revenue generation and contingent payments to Marathon based on annual net sales);

- our ability to complete any dystrophin study necessary in order to resolve the matters set forth in the FDA's denial of our appeal to the Complete Response Letter we received from the FDA in connection with our New Drug Application, or NDA, for Translarna for the treatment of nmDMD, and our ability to perform additional clinical trials, non-clinical studies or CMC assessments or analyses at significant cost;

- the timing and scope of our continued commercialization of Translarna as a treatment for nmDMD in the EEA or other territories outside of the United States;

- our ability to obtain additional and maintain existing reimbursed named patient and cohort EAP programs for Translarna for the treatment of nmDMD on adequate terms, or at all;

- our estimates regarding the potential market opportunity for Translarna, Emflaza, Tegsedi, Waylivra or any other product candidate, including the size of eligible patient populations and our ability to identify such patients;
- 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 ongoing, planned and potential future clinical trials and studies of Translarna for the treatment of nmDMD, aniridia, and Dravet syndrome/CDKL5, each caused by nonsense mutations, as well as our studies in spinal muscular atrophy and our oncology program, including 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, Emflaza, Tegsedi and Waylivra;
- 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;
- the timing of, and our ability to obtain additional marketing authorizations for, Translarna and our other product candidates;
- the ability of Translarna, Emflaza, Tegsedi and Waylivra and our other product candidates to meet existing or future regulatory standards;
- our ability to maintain the current labeling under the marketing authorization in the EEA or expand the approved product label of Translarna for the treatment of nmDMD;
- the potential receipt of revenues from future sales of Translarna, Emflaza and other product candidates, including our ability to earn a profit from sales or licenses of Translarna for the treatment of nmDMD in the countries in which we have or may obtain regulatory approval and of Emflaza for the treatment of DMD in the United States;
- the potential impact that enrollment, funding and completion of Study 041 may have on our revenue growth;
- our sales, marketing and distribution capabilities and strategy, including the ability of our third-party manufacturers to manufacture and deliver Translarna and Emflaza and any other product candidate 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, Emflaza and our other product candidates that are sufficient to meet clinical trial and commercial launch requirements;
- our ability to satisfy our obligations under the terms of the credit and security agreement with MidCap Financial Trust, or MidCap Financial, as administrative agent and MidCap Financial and certain other financial institutions as lenders thereunder;
- our other regulatory submissions, including with respect to timing and outcome of regulatory review;
- our plans to pursue development of Translarna for additional indications;
- our ability to advance our earlier stage programs, including our oncology program;
- our plans to pursue research and development of other product candidates;
- whether we may pursue business development opportunities, including potential collaborations, alliances, and acquisition or licensing of assets and our ability to successfully develop or commercialize any assets to which we may gain rights pursuant to such business development opportunities;
- the potential advantages of Translarna, Emflaza, Tegsedi and Waylivra and any other product candidate;
- our intellectual property position;
- the impact of government laws and regulations;
- the impact of litigation that has been brought against us and certain of our current and former officers or of litigation that we are pursuing against others;
- 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 as well as in Part I, Item 1A. Risk Factors in our Annual Report on Form 10-K for the year ended December 31, 2017, and in Part II, Item 1A. Risk Factors in our Quarterly Report on Form 10-Q for the period ended March 31, 2018, 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 our planned acquisition of Agilis or 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, 2017 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,” “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.

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

PTC Therapeutics, Inc.
Consolidated Balance Sheets (unaudited)
In thousands (except per share data)

	June 30, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 223,788	$ 111,792
Marketable securities	72,318	79,454
Trade receivables, net	59,383	40,394
Inventory	13,852	10,754
Prepaid expenses and other current assets	6,305	6,669
Total current assets	375,646	249,063
Fixed assets, net	8,217	8,376
Intangible assets, net	126,290	132,993
Deposits and other assets	1,620	1,221
Total assets	$ 511,773	$ 391,653
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 82,534	$ 76,446
Current portion of long-term debt	1,666	—
Deferred revenue	—	3,937
Other current liabilities	2,274	1,665
Total current liabilities	86,474	82,048
Deferred revenue - long-term	10,540	7,954
Long-term debt	147,204	144,971
Other long-term liabilities	153	243
Total liabilities	244,371	235,216
Stockholders' equity:		
Common stock, $0.001 par value. Authorized 125,000,000 shares; issued and outstanding 46,680,482 shares at June 30, 2018. Authorized 125,000,000 shares; issued and outstanding 41,612,395 shares at December 31, 2017	47	42
Additional paid-in capital	1,105,124	966,534
Accumulated other comprehensive income	1,855	3,969
Accumulated deficit	(839,624)	(814,108)
Total stockholders' equity	267,402	156,437
Total liabilities and stockholders' equity	$ 511,773	$ 391,653

See accompanying unaudited notes.

PTC Therapeutics, Inc.
Consolidated Statements of Operations (unaudited)
In thousands (except per share data)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Revenues:				
Net product revenue	$ 68,170	$ 47,891	$ 124,151	$ 74,334
Collaboration and grant revenue	573	71	654	176
Total revenues	68,743	47,962	124,805	74,510
Operating expenses:				
Cost of product sales, excluding amortization of acquired intangible asset	2,572	758	5,616	797
Amortization of acquired intangible asset	5,593	—	11,022	—
Research and development	32,607	30,835	63,970	58,198
Selling, general and administrative	33,545	28,866	66,514	54,365
Total operating expenses	74,317	60,459	147,122	113,360
Loss from operations	(5,574)	(12,497)	(22,317)	(38,850)
Interest expense, net	(2,884)	(3,008)	(6,187)	(5,227)
Other (expense) income, net	(673)	(1,820)	332	(2,139)
Loss before income tax expense	(9,131)	(17,325)	(28,172)	(46,216)
Income tax expense	(389)	(150)	(610)	(316)
Net loss attributable to common stockholders	$ (9,520)	$ (17,475)	$ (28,782)	$ (46,532)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	46,137,833	39,621,738	46,257,397	36,978,528
Net loss per share—basic and diluted (in dollars per share)	$ (0.21)	$ (0.44)	$ (0.62)	$ (1.26)

See accompanying unaudited notes.

PTC Therapeutics, Inc.
Consolidated Statements of Comprehensive Loss (unaudited)
In thousands

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Net loss	$ (9,520)	$ (17,475)	$ (28,782)	$ (46,532)
Other comprehensive loss:				
Unrealized gain (loss) on marketable securities	40	(9)	(83)	(31)
Foreign currency translation (loss) gain	(3,138)	2,884	(2,031)	3,515
Comprehensive loss	$ (12,618)	$ (14,600)	$ (30,896)	$ (43,048)

See accompanying unaudited notes.

PTC Therapeutics, Inc.
Consolidated Statements of Cash Flows (unaudited)
In thousands

	Six Months Ended June 30,	
	2018	**2017**
Cash flows from operating activities		
Net loss	$ (28,782)	$ (46,532)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	12,243	1,448
Change in valuation of warrant liability	—	3
Non-cash interest expense	3,644	3,274
Loss on disposal of asset	—	47
Amortization of premiums and accretion of discounts on investments, net	(230)	365
Amortization of debt issuance costs	256	185
Share-based compensation expense	15,831	16,914
Unrealized foreign currency transaction (gains) losses, net	(764)	1,648
Changes in operating assets and liabilities:		
Inventory	(3,393)	(2,806)
Prepaid expenses and other current assets	254	(416)
Trade receivables, net	(20,429)	(6,762)
Deposits and other assets	(419)	(463)
Accounts payable and accrued expenses	2,225	12,452
Other liabilities	485	457
Deferred revenue	3,204	4,604
Net cash used in operating activities	(15,875)	(15,582)
Cash flows from investing activities		
Purchases of fixed assets	(1,187)	(579)
Purchases of marketable securities	(28,656)	(19,467)
Sale and redemption of marketable securities	35,939	144,357
Acquisition, including transaction costs	—	(76,424)
Net cash provided by investing activities	6,096	47,887
Cash flows from financing activities		
Proceeds from exercise of options	3,592	535
Net proceeds from public offerings	117,874	—
Proceeds from shares issued under employee stock purchase plan	1,299	557
Debt issuance costs related to secured term loan	—	(432)
Proceeds from issuance of secured term loan	—	40,000
Net cash provided by financing activities	122,765	40,660
Effect of exchange rate changes on cash	(990)	1,725
Net increase in cash and cash equivalents	111,996	74,690
Cash and cash equivalents, beginning of period	111,792	58,321
Cash and cash equivalents, end of period	$ 223,788	$ 133,011
Supplemental disclosure of cash information		
Cash paid for interest	$ 3,838	$ 2,474
Cash paid for income taxes	$ 758	$ 334

See accompanying unaudited notes.

PTC Therapeutics, Inc.
Notes to Consolidated Financial Statements (unaudited)
June 30, 2018
In thousands (except per share data unless otherwise noted)

1. The Company

PTC Therapeutics, Inc. (the "Company" or "PTC") is a science-led global biopharmaceutical company focused on the discovery, development and commercialization of clinically-differentiated medicines that provide benefits to patients with rare disorders. The Company has launched two rare disease products and has a global commercial footprint. The Company's recent ability to commercialize products is the foundation that drives its continued investment in a robust pipeline of transformative medicines and its mission to provide access to best-in-class treatments for patients who have an unmet medical need.

The Company has two products, Translarna™ (ataluren) and Emflaza™ (deflazacort), for the treatment of Duchenne muscular dystrophy, or DMD, a rare, life threatening disorder. Translarna received marketing authorization from the European Commission in August 2014 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. In July 2018, the European Commission approved a label-extension request to our marketing authorization for Translarna in the EEA to include patients from two to up to five years of age. Emflaza is approved in the United States for the treatment of DMD in patients five years and older.

The Company's marketing authorization for Translarna in the EEA is subject to annual review and renewal by the European Commission following reassessment by the European Medicines Agency, or EMA, of the benefit-risk balance of the authorization, which the Company refers to as the annual EMA reassessment. This marketing authorization is further subject to the specific obligation to conduct and submit the results of a multi-center, randomized, double-blind, 18-month, placebo-controlled trial, followed by an 18-month open-label extension, according to an agreed protocol, in order to confirm the efficacy and safety of Translarna in the approved patient population. The final report on the trial and open-label extension is to be submitted by the Company to the EMA by the end of the third quarter of 2021. The Company refers to the trial and open-label extension together as Study 041.

The marketing authorization in the EEA was last renewed in July 2018 and is effective, unless extended, through August 5, 2019. The renewal was based on the Company's commitment to conduct Study 041 and the totality of the clinical data available from its trials and studies of Translarna for the treatment of nmDMD, including the safety and efficacy results of the Phase 2b and Phase 3 clinical trials. The primary efficacy endpoint was not achieved in either trial within the pre-specified level of statistical significance.

In June 2014, the Company initiated reimbursed early access programs, or EAP programs, for Translarna for nmDMD patients in selected territories in the EEA and recorded its first sales of Translarna in the third quarter of 2014 pursuant to an EAP program. In December 2014, the Company recorded its first commercial sales in Germany. As of June 30, 2018, Translarna was available in over 25 countries on a commercial basis or pursuant to an EAP program. The Company expects to expand its commercial activities across the EEA pursuant to the marketing authorization granted by the EMA throughout 2018 and future years, subject to continued renewal of its marketing authorization following annual EMA reassessments and successful completion of pricing and reimbursement negotiations. Concurrently, the Company plans to continue to pursue EAP programs in select countries where those mechanisms exist, both within the EEA and in other countries that will reference the marketing authorization in the EEA.

Translarna is an investigational new drug in the United States. During the first quarter of 2017, the Company filed a New Drug Application, or NDA, over protest with the United States Food and Drug Administration, (the "FDA"), for which the FDA granted a standard review. In October 2017, the Office of Drug Evaluation I of the FDA issued a complete response letter for the NDA, stating that it was unable to approve the application in its current form. In response, the Company filed a formal dispute resolution request with the Office of New Drugs of the FDA. In February 2018, the Office of New Drugs of the FDA denied PTC's appeal of the Complete Response Letter. In its response, the Office of New Drugs recommended a possible path forward for the ataluren NDA submission based on the accelerated approval pathway. This would involve a re-submission of an NDA containing the current data on effectiveness of ataluren with new data to be generated on dystrophin production in nmDMD patients' muscles. The Company intends to follow the FDA's recommendation and will collect such dystrophin data using newer technologies via procedures and methods that it is currently designing and expects to initiate such a study by the end of 2018. Additionally, should a re-submission of an NDA receive accelerated approval, the Office of New Drugs stated that Study 041, which is currently enrolling, could serve as the confirmatory post-approval trial required in connection with the accelerated approval framework.

The NDA, which seeks approval of Translarna for the treatment of nmDMD in the United States, was initially submitted by the Company in December 2015. In February 2016, following the submission, the Company received a Refuse to File letter from the FDA regarding the NDA. The FDA stated in the Refuse to File letter that the NDA was not sufficiently complete to permit a substantive review. Specifically, the Company was notified in the letter that, in the view of the FDA, both the Phase 2b and Phase 3

ACT DMD trials were negative and do not provide substantial evidence of effectiveness and that the NDA did not contain adequate information regarding the abuse potential of Translarna. Additionally, the FDA stated that the Company had proposed a post-hoc adjustment of ACT DMD that eliminates data from a majority of enrolled patients. During July 2016, the Company appealed the Refuse to File decision via the formal dispute resolution process within FDA's Center for Drug Evaluation and Research; however, this appeal was denied by the FDA's Office of Drug Evaluation I in October 2016.

On April 20, 2017, the Company completed its acquisition of all rights to Emflaza, or the Transaction. Emflaza is approved in the United States for the treatment of DMD in patients five years and older. The Transaction was completed pursuant to an asset purchase agreement, dated March 15, 2017, as amended on April 20, 2017, (the "Asset Purchase Agreement"), by and between the Company and Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC), or Marathon. The Transaction was accounted for as an asset acquisition. The assets acquired by the Company in the Transaction include intellectual property rights related to Emflaza, inventories of Emflaza, and certain contractual rights related to Emflaza. The Company assumed certain liabilities and obligations in the Transaction arising out of, or relating to, the assets acquired in the Transaction.

Upon the closing of the Transaction, the Company paid to Marathon total upfront consideration comprised of $75.0 million in cash, funded through cash on hand, and 6,683,598 shares of the Company's common stock. The number of shares of common stock issued at closing was determined by dividing $65.0 million by the volume weighted average price per share of the Company's common stock on the Nasdaq Stock Market for the 15 trading-day period ending on the third trading day immediately preceding the closing. Marathon will be entitled to receive contingent payments from the Company based on annual net sales of Emflaza beginning in 2018, up to a specified aggregate maximum amount over the expected commercial life of the asset, and a single $50.0 million sales-based milestone, in each case subject to the terms and conditions of the Asset Purchase Agreement.

As of June 30, 2018, the Company had an accumulated deficit of approximately $839.6 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, October 2014 and April 2018, 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. Since 2014, the Company has also relied on revenue generated from net sales of Translarna for the treatment of nmDMD in territories outside of the United States, and in May 2017, the Company began to recognize revenue generated from net sales of Emflaza for the treatment of DMD in the United States. The Company expects that the cash flows from the sales of its products, together with the Company's cash, cash equivalents and marketable securities, will be sufficient to fund its operations for at least the next twelve months.

2. Summary of significant accounting policies

The Company's complete listing of significant accounting policies is set forth in Note 2 of the notes to the Company's audited financial statements as of December 31, 2017 included in the Company's Annual Report on Form 10-K filed with the Securities and Exchange Commission (the "SEC") on March 6, 2018 (the "2017 Form 10-K"). Additional significant accounting policies adopted during the six month period ended June 30, 2018 are discussed in further detail below.

Basis of presentation

The accompanying financial information as of June 30, 2018 and for the three and six months ended June 30, 2018 and 2017 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, 2017 and notes thereto included in the 2017 Form 10-K.

In the opinion of management, the unaudited financial information as of June 30, 2018 and for the three and six months ended June 30, 2018 and 2017 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, 2018 are not necessarily indicative of the results to be expected for the year ended December 31, 2018 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 and development expenses, stock-based compensation, valuation procedures for the convertible notes, allowance for

doubtful accounts, inventory, acquired intangible assets, 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.

Inventory and cost of product sales

Inventory

Inventories are stated at the lower of cost and net realizable value with cost determined on a first-in, first-out basis by product. The Company capitalizes inventory costs associated with products following regulatory approval when future commercialization is considered probable and the future economic benefit is expected to be realized. Translarna and Emflaza product which may be used in clinical development programs are included in inventory and charged to research and development expense when the product enters the research and development process and no longer can be used for commercial purposes. Inventory used for marketing efforts are charged to selling, general and administrative expense.

The following table summarizes the components of the Company's inventory for the periods indicated:

	June 30, 2018	December 31, 2017
Raw materials	$ 727	$ 452
Work in progress	4,712	3,912
Finished goods	8,413	6,390
Total inventory	$ 13,852	$ 10,754

The Company periodically reviews its inventories for excess amounts or obsolescence and writes down obsolete or otherwise unmarketable inventory to its estimated net realizable value. The Company has not recorded any inventory write downs as of the current period. Additionally, though the Company's product is subject to strict quality control and monitoring which it performs throughout the manufacturing processes, certain batches or units of product may not meet quality specifications resulting in a charge to cost of product sales.

Cost of product sales

Cost of product sales consists of the cost of inventory sold, manufacturing and supply chain costs, storage costs, amortization of the acquired intangible asset and royalty payments associated with net product sales.

Revenue recognition

In May 2014, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") No. 2014-9, "Revenue from Contracts with Customers (Topic 606)". ASU No. 2014-9 eliminated transaction- and industry-specific revenue recognition guidance under FASB Accounting Standards Codification ("ASC") Subtopic 605-15, Revenue Recognition-Products (Topic 605) and replaced it with a principle-based approach for determining revenue recognition. ASC Topic 606 requires entities to 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. On January 1, 2018, the Company adopted ASC Topic 606 using the modified retrospective approach, a practical expedient permitted under Topic 606, and applied this approach only to contracts that were not completed as of January 1, 2018. The Company calculated a one-time transition adjustment of $3.3 million, which was recorded on January 1, 2018 to the opening balance of accumulated deficit, related to the product sales of Emflaza. The ASC 606 transition adjustment recorded for Emflaza resulted in sales being recognized earlier than under Topic 605, as the deferred revenue recognition model (sell-through) is not applicable under Topic 606. The one-time adjustment consisted of $3.9 million in deferred revenue offset by $0.6 million of variable consideration. The information presented for the periods prior to January 1, 2018 has not been adjusted and is reported under Topic 605.

Periods prior to January 1, 2018

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

Prior to the second quarter of 2017, the Company's net product sales consisted of sales of Translarna for the treatment of nmDMD in territories outside of the U.S. 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 the Company's third-party partner distributors. Revenue is recognized when risk of ownership has transferred. The Company's third-party partner 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.

In May 2017, the Company began the commercialization of Emflaza in the U.S. The Company recorded product revenue related to the sales of Emflaza in the U.S. in accordance with ASC 605-15, when persuasive evidence of an arrangement exists, delivery has occurred and title of the product and associated risk of loss has passed to the customer, the price is fixed or determinable and collection from the customer has been reasonably assured. Due to the early stage of the product launch, the Company determined that it was not able to reliably make certain estimates, including returns, necessary to recognize product revenue upon shipment to distributors. As a result, the Company recorded net product revenue for Emflaza using a deferred revenue recognition model (sell-through). Under the deferred revenue model, the Company does not recognize revenue until Emflaza is shipped to the specialty pharmacy.

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 ASC 605-28, Revenue Recognition—Milestone Method. 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 its 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.

Periods commencing January 1, 2018

The Company's net product revenue consists of sales of Translarna in territories outside of the U.S. and sales of Emflaza in the U.S., both for the treatment of DMD.

Net product revenue

The Company recognizes revenue when its performance obligations with its customers have been satisfied. The Company's performance obligations are to provide Translarna or Emflaza based on customer orders from distributors, hospitals, specialty pharmacies or retail pharmacies. The performance obligations are satisfied at a point in time when the Company's customer obtains control of either Translarna or Emflaza, which is typically upon delivery. The Company invoices its customers after the products have been delivered and invoice payments are generally due within 30 to 90 days of invoice date. The Company determines the transaction price based on fixed consideration in its contractual agreements. Contract liabilities arise in certain circumstances when consideration is due for goods the Company has yet to provide. As the Company has identified only one distinct performance obligation, the transaction price is allocated entirely to either product sales of Translarna or Emflaza. In determining the transaction price, a significant financing component does not exist since the timing from when the Company delivers product to when the customers pay for the product is typically less than one year. Customers in certain countries pay in advance of product delivery. In those instances, payment and delivery typically occur in the same month.

The Company records product sales net of any variable consideration, which includes discounts, allowances, rebates and distribution fees. The Company uses the expected value or most likely amount method when estimating its variable consideration, unless discount or rebate terms are specified within contracts. Historically, returns of Translarna and Emflaza are immaterial to the financial statements. The identified variable consideration is recorded as a reduction of revenue at the time revenues from product sales are recognized. These estimates for variable consideration are adjusted to reflect known changes in factors and may impact such estimates in the quarter those changes are known. Revenue recognized does not include amounts of variable consideration that are constrained.

In relation to customer contracts, the Company incurs costs to fulfill a contract but does not incur costs to obtain a contract. These costs to fulfill a contract do not meet the criteria for capitalization and are expensed as incurred.

Upon adoption of ASC Topic 606 on January 1, 2018, the Company elected the following practical expedients:

- Portfolio Approach - the Company applied the Portfolio Approach to contract reviews within its identified revenue streams that have similar characteristics and the Company believes this approach would not differ materially than if applying ASC Topic 606 to each individual contract.

- Significant Financing Component - the Company expects the period between when it transfers a promised good to a customer and when the customer pays for the good or service to be one year or less.

- Immaterial Performance Obligations - the Company disregards promises deemed to be immaterial in the context of the contract.

- Shipping and Handling Activities - the Company considers any shipping and handling costs that are incurred after the customer has obtained control of the product as a cost to fulfill a promise.

Shipping and handling costs associated with finished goods delivered to customers are recorded as a selling expense.

Collaboration 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.

At the inception of a collaboration arrangement, the Company needs to first evaluate if the arrangement meets the criteria in ASC Topic 808 “Collaborative Arrangements” to then determine if ASC Topic 606 is applicable by considering whether the collaborator meets the definition of a customer. If the criteria are met, the Company assesses the promises in the arrangement to identify distinct performance obligations.

For licenses of intellectual property, the Company assesses, at contract inception, whether the intellectual property is distinct from other performance obligations identified in the arrangement. If the licensing of intellectual property is determined to be distinct, revenue is recognized for nonrefundable, upfront license fees when the license is transferred to the customer and the customer can use and benefit from the license. If the licensing of intellectual property is determined not to be distinct, then the license will be bundled with other promises in the arrangement into one distinct performance obligation. The Company needs to determine if the bundled performance obligation is satisfied over time or at a point in time. If the Company concludes that the nonrefundable, upfront license fees will be recognized over time, the Company will need to assess the appropriate method of measuring proportional performance.

For milestone payments, the Company assesses, at contract inception, whether the development or sales-based milestones are considered probable of being achieved. If it is probable that a significant revenue reversal will occur, the Company will not record revenue until the uncertainty has been resolved. Milestone payments that are contingent upon regulatory approval are not considered probable of being achieved until the applicable regulatory approvals or other external conditions are obtained as such conditions are not within the Company's control. If it is probable that a significant revenue reversal will not occur, the Company will estimate the milestone payments using the most likely amount method. The Company will re-assess the development and sales-based milestones each reporting period to determine the probability of achievement.

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.

Allowance for doubtful accounts

The Company maintains an allowance for estimated losses resulting from the inability of its customers to make required payments. The Company estimates uncollectible amounts based upon current customer receivable balances, the age of customer receivable balances, the customer's financial condition and current economic trends. The allowance for doubtful accounts was $0.5 million as of June 30, 2018 and $0.8 million as of December 31, 2017.

Income Taxes

On December 22, 2017, the U.S. government enacted the 2017 Tax Cuts and Jobs Act (the 2017 Tax Act), which significantly revises U.S. tax law by, among other provisions, lowering the U.S. federal statutory income tax rate to 21%, imposing a mandatory one-time transition tax on previously deferred foreign earnings, and eliminating or reducing certain income tax deductions. The Global Intangible Low-tax Income (GILTI) provisions of the 2017 Tax Act require the Company to include in its U.S. income tax return foreign subsidiary earnings in excess of an allowable return on the foreign subsidiary's tangible assets. The Company has elected to account for GILTI tax in the period in which it is incurred, and therefore has not provided any deferred tax impacts of GILTI in its consolidated financial statements for the period ended June 30, 2018.

ASC 740, Income Taxes requires the effects of changes in tax laws to be recognized in the period in which the legislation is enacted. However, due to the complexity and significance of the 2017 Tax Act's provisions, the SEC issued SAB 118, which allows companies to record the tax effects of the 2017 Tax Act on a provisional basis based on a reasonable estimate, and then, if necessary, subsequently adjust such amounts during a limited measurement period as more information becomes available. The measurement period ends when a company has obtained, prepared, and analyzed the information necessary to finalize its accounting, but cannot extend beyond one year from enactment. The 2017 Tax Act does not have a material impact on the Company's financial statements since its deferred temporary differences are fully offset by a valuation allowance and the Company does not have any significant off shore earnings from which to record the mandatory transition tax. However, given the significant complexity of the 2017 Tax Act, anticipated guidance from the U.S. Treasury about implementing the 2017 Tax Act, and the potential for additional guidance from the SEC or the FASB related to the 2017 Tax Act, these estimates may be adjusted during the measurement period. The Company continues to analyze the changes in certain income tax deductions, assess calculations of earnings and profits in certain foreign subsidiaries, including if those earnings which are held in cash or other assets and gather additional data to compute the full impacts on the Company's deferred and current tax assets and liabilities.

Recently issued accounting standards

In February 2016, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") No. 2016-2, "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. 2016-2 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.

In February 2018, the FASB issued ASU 2018-02, "Income Statement — Reporting Comprehensive Income (Topic 220): Reclassification of Certain Tax Effects from Accumulated Other Comprehensive Income". This standard permits the reclassification of tax effects stranded in other comprehensive income as a result of tax reform to retained earnings related to the change in federal tax rate in addition to other stranded effects that relate to the Tax Cuts and Job Act ("the Act") but do not directly relate to the change in the federal rate. ASU 2018-02 is effective for fiscal years beginning after December 15, 2018, including interim periods within those fiscal years with early adoption permitted for periods for which financial statements have not yet been issued or made available for issuance. The Company expects to adopt this guidance when effective and is currently assessing what effect the adoption of ASU No. 2018-02 will have on its consolidated financial statements and accompanying notes.

In June 2018, the FASB issued ASU 2018-07, "Compensation — Stock Compensation (Topic 718), Improvements to Nonemployee Share-Based Payment Accounting". This standard expands the scope of ASC 718 to include share-based payments granted to nonemployees in exchange for goods or services used or consumed in the entity's own operations and supersedes the guidance in ASC 505-50. The ASU retains the existing cost attribution guidance, which requires entities to recognize compensation cost for nonemployee awards in the same period and in the same manner they would if they paid cash for the goods or services, but it

moves the guidance to ASC 718. ASU 2018-07 is effective for fiscal years beginning after December 15, 2018, including interim periods within those fiscal years with early adoption permitted for periods for which financial statements have not yet been issued or made available for issuance. The Company expects to adopt this guidance when effective and is currently assessing what effect the adoption of ASU No. 2018-07 will have on its consolidated financial statements and accompanying notes.

Impact of recently adopted accounting pronouncements

In May 2014, the FASB issued ASU No. 2014-09, “Revenue from Contracts with Customers (Topic 606)”. ASU No. 2014-09 eliminated transaction- and industry-specific revenue recognition guidance under FASB Accounting Standards Codification (“ASC”) Subtopic 605-15, Revenue Recognition-Products and replaced it with a principle-based approach for determining revenue recognition. ASC Topic 606 requires entities to 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. On January 1, 2018, the Company adopted ASC Topic 606 using the modified retrospective approach and applied this approach only to contracts that were not completed as of January 1, 2018. The Company calculated a one-time transition adjustment of $3.3 million, which was recorded on January 1, 2018 to deferred revenue and accumulated deficit, related to the product sales of Emflaza. The information presented for the periods prior to January 1, 2018 has not been restated and is reported under ASC Topic 605.

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 reporting organizations (whether public or private) that hold financial assets or owe financial liabilities. The Company adopted ASU 2016-01 during the three months ended March 31, 2018. In March 2018, the FASB issued ASU 2018-04, "Investments - Debt Securities (Topic 320) and Regulated Operations (Topic 980): Amendments to SEC Paragraphs Pursuant to the SEC Staff Accounting Bulletin ("SAB") No. 117 and SEC Release No. 33-9273 (SEC Update)". This standard supersedes SEC paragraphs in ASC 320, Investments-Debt Securities, as a result of the issuance of SAB 117 and also updates the Codification for a 2011 SEC release and is effective when a registrant adopts ASU 2016-01, which in the case of the Company was during the three months ended March 31, 2018. The adoption of these standards did not have a material impact on the Company’s financial position or results of operations for the period ended and as of June 30, 2018.

In August 2016, the FASB issued ASU No. 2016-15, “Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments”. This standard clarifies the presentation of certain specific cash flow issues in the Statement of Cash Flows. The Company adopted ASU 2016-15 during the three months ended March 31, 2018. The adoption of this standard did not have a material impact on the Company’s financial position or results of operations for the period ended and as of June 30, 2018.

In November 2016, the FASB issued ASU No. 2016-18, “Statement of Cash Flows (Topic 230): Restricted Cash”. This standard requires entities to show the changes in the total of cash, cash equivalents, restricted cash and restricted cash equivalents in the statement of cash flows and no longer present transfers between cash and cash equivalents and restricted cash and restricted cash equivalents in the statement of cash flows. The Company adopted ASU 2016-18 during the three months ended March 31, 2018. The adoption of this standard did not have a material impact on the Company’s financial position or results of operations for the period ended and as of June 30, 2018.

In May 2017, the FASB issued ASU No. 2017-09, "Stock Compensation (Topic 718): Scope of Modification Accounting". This standard clarifies when changes to the terms or conditions of a share-based payment award must be accounted for as a modification, with entities applying the modification accounting guidance if the value, vesting conditions or classification of the award changes. In addition to all disclosures about modifications that are required under the current guidance, entities will be also required to disclose that compensation expense has not changed if applicable. The Company adopted ASU 2017-09 during the three months ended March 31, 2018. The adoption of this standard did not have a material impact on the Company’s financial position or results of operations for the period ended and as of June 30, 2018.

3. Fair value of financial instruments and marketable securities

The Company follows the fair value measurement rules, which provide 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 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 June 30, 2018 and December 31, 2017:

	June 30, 2018			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities	$ 72,318	$ —	$ 72,318	$ —
Warrant liability	$ 1	$ —	$ —	$ 1
Stock appreciation rights liability	$ 2,274	$ —	$ —	$ 2,274

	December 31, 2017			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities	$ 79,454	$ —	$ 79,454	$ —
Warrant Liability	$ 1	$ —	$ —	$ 1
Stock appreciation rights liability	$ 1,665	$ —	$ —	$ 1,665

No transfers of assets between Level 1 and Level 2 of the fair value measurement hierarchy occurred during the periods ended June 30, 2018 and December 31, 2017.

The following is a summary of marketable securities accounted for as available-for-sale securities at June 30, 2018 and December 31, 2017:

	June 30, 2018			
	Amortized Cost	Gross Unrealized		Fair Value
		Gains	Losses	
Commercial paper	$ 26,322	$ 7	$ (1)	$ 26,328
Corporate debt securities	46,057	—	(67)	45,990
	$ 72,379	$ 7	$ (68)	$ 72,318

	December 31, 2017			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 13,775	$ 52	$ —	$ 13,827
Corporate debt securities	65,657	—	(30)	65,627
	$ 79,432	$ 52	$ (30)	$ 79,454

At June 30, 2018 and December 31, 2017, 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, 2018 and December 31, 2017, the Company did not have any realized gains/losses from the sale of marketable securities.

The unrealized losses and fair values of available-for-sale securities that have been in an unrealized loss position for a period of less than and greater than 12 months as of June 30, 2018 are as follows:

	June 30, 2018					
	Securities in an unrealized loss position less than 12 months		Securities in an unrealized loss position greater than 12 months		Total	
	Unrealized losses	Fair Value	Unrealized losses	Fair Value	Unrealized losses	Fair Value
Commercial paper	$ (1)	$ 10,457	$ —	$ —	$ (1)	$ 10,457
Corporate debt securities	(67)	45,990	—	—	(67)	45,990
	$ (68)	$ 56,447	$ —	$ —	$ (68)	$ 56,447

The unrealized losses and fair values of available-for-sale securities that have been in an unrealized loss position for a period of less than and greater than 12 months as of December 31, 2017 are as follows:

	December 31, 2017					
	Securities in an unrealized loss position less than 12 months		Securities in an unrealized loss position greater than 12 months		Total	
	Unrealized losses	Fair Value	Unrealized losses	Fair Value	Unrealized losses	Fair Value
Corporate debt securities	$ (28)	$ 59,108	$ (2)	$ 6,519	$ (30)	$ 65,627

Marketable securities on the balance sheet at June 30, 2018 and December 31, 2017 mature as follows:

	June 30, 2018	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 26,328	$ —
Corporate debt securities	45,990	—
Total Marketable securities	$ 72,318	$ —

	December 31, 2017	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 13,827	$ —
Corporate debt securities	55,550	10,077
Total Marketable securities	$ 69,377	$ 10,077

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

Convertible 3.0% senior notes

In August 2015, the Company issued $150.0 million of 3.0% convertible senior notes due August 15, 2022 (the "Convertible Notes"). Interest is payable semi-annually on February 15 and August 15 of each year, beginning on February 15, 2016. The Company separately accounted for the liability and equity components of the Convertible Notes by allocating the proceeds between

the liability component and equity component, as further discussed in Note 9. The fair value of the Convertible Notes, which differs from their carrying values, is influenced by interest rates, the Company's stock price and stock price volatility and is determined by prices for the Convertible Notes observed in market trading which are Level 2 inputs. The estimated fair value of the Convertible Notes at June 30, 2018 and December 31, 2017 was $148.2 million and $115.7 million, respectively.

The carrying amounts reported in the consolidated balance sheets for cash and cash equivalents, accounts receivable, accounts payable and borrowings under the credit and security agreement with MidCap Financial Trust and other financial institutions (as further discussed in Note 9) approximate fair value because of the immediate or short-term maturity of these financial instruments. The carrying amounts for the credit and security agreement approximate fair value based on market activity for other debt instruments with similar characteristics and comparable risk.

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 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, 2018:

	Level 3 liabilities	
	Warrants	SARs
Beginning balance as of December 31, 2017	$ 1	$ 1,665
Change in fair value	—	2,600
Payments	—	(1,991)
Ending balance as of June 30, 2018	$ 1	$ 2,274

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, 2018 include (i) volatility (54%-53%), (ii) risk free interest rate (2.43%-2.43%), (iii) strike price ($128.00-$2,520.00), (iv) fair value of common stock ($33.73), and (v) expected life (1.2—1.1 years). The significant assumptions used in preparing the option pricing model for valuing the Company's warrants as of December 31, 2017 include (i) volatility (69%-69%), (ii) risk free interest rate (1.89%—1.89%), (iii) strike price ($128.00—$2,520.00), (iv) fair value of common stock ($16.68), and (v) expected life (1.6—1.7 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, 2018 include (i) volatility (55%—56%), (ii) risk free interest rate (2.22%—2.43%), (iii) strike price ($6.76-$30.86), (iv) fair value of common stock ($33.73), and (v) expected life (0.5—1.5 years). The significant assumptions used in preparing the option pricing model for valuing the Company's SARs as of December 31, 2017 include (i) volatility (31%-70%), (ii) risk free interest rate (1.28%—1.89%), (iii) strike price ($6.76—$30.86), (iv) fair value of common stock ($16.68), and (v) expected life (0.0—2.0 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, 2018:

	Unrealized Gains/(Losses) On Marketable Securities, net of tax	Foreign Currency Translation	Total Accumulated Other Comprehensive Items
Balance at March 31, 2018	$ (101)	$ 5,054	$ 4,953
Other comprehensive income (loss) before reclassifications	40	(3,138)	(3,098)
Amounts reclassified from other comprehensive items	—	—	—
Other comprehensive income (loss)	40	(3,138)	(3,098)
Balance at June 30, 2018	$ (61)	$ 1,916	$ 1,855

	Unrealized Gains/(Losses) On Marketable Securities, net of tax	Foreign Currency Translation	Total Accumulated Other Comprehensive Items
Balance at December 31, 2017	$ 22	$ 3,947	$ 3,969
Other comprehensive loss before reclassifications	(83)	(2,031)	(2,114)
Amounts reclassified from other comprehensive items	—	—	—
Other comprehensive loss	(83)	(2,031)	(2,114)
Balance at June 30, 2018	$ (61)	$ 1,916	$ 1,855

5. Accounts payable and accrued expenses

Accounts payable and accrued expenses at June 30, 2018 and December 31, 2017 consist of the following:

	June 30, 2018	December 31, 2017
Employee compensation, benefits, and related accruals	$ 12,959	$ 17,711
Consulting and contracted research	5,562	5,137
Professional fees	4,494	2,116
Sales allowance and other costs	26,167	22,257
Sales rebates and royalties	24,028	11,657
Accounts payable	4,364	15,282
Other	4,960	2,286
	$ 82,534	$ 76,446

6. Capitalization

In April 2018, the Company closed an underwritten public offering of its common stock pursuant to a registration statement on Form S-3. The Company issued and sold an aggregate of 4,600,000 shares of common stock under the registration statement at a public offering price of $27.04 per share, including 600,000 shares issued upon exercise by the underwriters of their option to purchase additional shares. The Company received net proceeds of approximately $117.9 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, 2018 and December 31, 2017 because they contained non-standard antidilution provisions.

The following is a summary of the Company's outstanding warrants as of June 30, 2018 and December 31, 2017:

	Warrant shares	Exercise price	Expiration
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 loss by the weighted-average number of common shares outstanding. Diluted earnings per share is computed by dividing net loss by the weighted-average number of common shares plus the effect of any 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,	
	2018	2017	2018	2017
Numerator				
Net loss	$ (9,520)	$ (17,475)	$ (28,782)	$ (46,532)
Denominator				
Denominator for basic and diluted net loss per share	46,137,833	39,621,738	46,257,397	36,978,528
Net loss per share:				
Basic and diluted	$ (0.21) *	$ (0.44) *	$ (0.62) *	$ (1.26) *

*In the three and six months ended June 30, 2018 and 2017, 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,	
	2018	2017
Stock Options	8,166,403	7,124,052
Unvested restricted stock awards and units	584,181	423,986
Total	8,750,584	7,548,038

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 2009, the Company's shareholders approved 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, subject to certain adjustments and annual increases. 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. There are no additional shares available for issuance under this plan.

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, 2018, awards for 654,499 shares of common stock are available for issuance.

From January 1, 2018 through June 30, 2018, the Company issued a total of 2,247,739 stock options to various employees. Of those, 461,750 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 the Company's 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, 2017	6,448,642	$ 29.00		
Granted	2,247,739	$ 20.67		
Exercised	(260,879)	$ 13.77		
Forfeited/Cancelled	(269,099)	$ 33.12		
Outstanding at June 30, 2018	8,166,403	$ 27.06	7.55 years	$ 90,372
Vested or Expected to vest at June 30, 2018	3,586,552	$ 21.60	8.90 years	$ 48,673
Exercisable at June 30, 2018	4,313,766	$ 32.03	6.32 years	$ 37,911

The fair value of grants made in the six months ended June 30, 2018 was contemporaneously estimated on the date of grant using the following assumptions:

	Six months ended June 30, 2018
Risk-free interest rate	2.25%—2.89%
Expected volatility	64%—90%
Expected term	5.04 – 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, 2018 was $13.87 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 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, 2018	393,011	$ 15.64
Granted	345,991	$ 18.38
Vested	(112,545)	$ 16.31
Forfeited	(42,276)	$ 17.06
Unvested at June 30, 2018	584,181	$ 17.11

Stock Appreciation Rights—Stock appreciation rights (SARs) entitle the holder to receive, upon exercise, an amount of the Company's common stock or cash (or a combination thereof) determined by reference to appreciation, from and after the date of grant, in the fair market value of a share of the Company's common stock over the measurement price based on the exercise date.

In May 2016, a total of 897,290 SARs were granted to non-executive employees (the 2016 SARs). The 2016 SARs will vest annually in equal installments over four years and will be settled in cash on each vest date, requiring the Company to remeasure

the SARs at each reporting period until vesting occurs. For the period ended June 30, 2018, a total of 177,329 SARs vested. For the period ended June 30, 2018, the Company recorded $2.6 million in compensation expense related to the 2016 SARs.

Employee Stock Purchase Plan—In June 2016, the Company established an Employee Stock Purchase Plan ("ESPP" or "the Plan") for certain eligible employees. The Plan is administered by the Company's Board of Directors or a committee appointed by the Board. The total number of shares available for purchase under the Plan is one million shares of the Company's common stock. Employees may participate over a six-month period through payroll withholdings and may purchase, at the end of the six-month period, the Company's common stock at a purchase price of at least 85% of the closing price of a share of the Company's common stock on the first business day of the offering period or the closing price of a share of the Company's common stock on the last business day of the offering period, whichever is lower. No participant will be granted a right to purchase the Company's common stock under the Plan if such participant would own more than 5% of the total combined voting power of the Company or any subsidiary of the Company after such purchase. For the period ended June 30, 2018, the Company recorded $0.5 million in compensation expense related to the ESPP.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Research and development	$ 3,932	$ 3,895	$ 7,678	$ 8,362
Selling, general and administrative	4,152	3,990	8,153	8,552
Total	$ 8,084	$ 7,885	$ 15,831	$ 16,914

As of June 30, 2018, there was approximately $57.8 million of total unrecognized compensation cost related to unvested share-based compensation arrangements granted under the 2009 Equity and Long Term Incentive Plan, the 2013 Long Term Incentive Plan and equity awards made pursuant to the Nasdaq inducement grant exception for new hires. This cost is expected to be recognized as share-based compensation expense over the weighted average remaining service period of approximately 2.69 years.

9. Debt

2017 Credit Facility

In May 2017, the Company entered into a credit and security agreement (the "Credit Facility") with MidCap Financial Trust, a Delaware statutory trust ("MidCap"), as administrative agent and MidCap and certain other financial institutions as lenders thereunder (the "Credit Agreement") that provides for a senior secured term loan facility of $60.0 million, of which $40.0 million was drawn by the Company on May 5, 2017. The remaining $20.0 million under the senior secured term loan facility will become available to the Company upon its demonstration (on or prior to December 31, 2018) of net product revenue equaling or exceeding $120.0 million for the trailing 12 month period. The Company capitalized approximately $0.4 million of debt issuance costs, which were netted against the carrying value of the Credit Facility and will be amortized over the term of the Credit Facility.

Borrowings under the Credit Agreement bear interest at a rate per annum equal to LIBOR (with a LIBOR floor rate of 1.00%) plus 6.15%. The Company is obligated to make interest only payments (payable monthly in arrears) through April 30, 2019. Commencing on May 1, 2019 and continuing for the remaining twenty-four months of the facility, the Company will be required to make monthly interest payments and monthly principal payments. The principal payments are to be made based on straight-line amortization of the principal over the twenty-four month period. The maturity date of the Credit Agreement is May 1, 2021, unless terminated earlier.

The Credit Facility is subject to certain financial covenants. As of June 30, 2018, the Company was in compliance with all required covenants.

Convertible Notes

In August 2015, the Company issued, at par value, $150.0 million aggregate principal amount of 3.0% convertible senior notes due 2022 (the "Convertible Notes"). The Convertible Notes bear cash interest at a rate of 3.0% per year, payable semi-annually on February 15 and August 15 of each year, beginning on February 15, 2016. The Convertible Notes will mature on August 15, 2022, unless earlier repurchased or converted. The net proceeds to the Company from the offering were $145.4 million after deducting the initial purchasers' discounts and commissions and the offering expenses payable by the Company.

The Convertible Notes are governed by an indenture (the Convertible Notes Indenture) with U.S Bank National Association as trustee (the Convertible Notes Trustee).

Holders may convert their Convertible Notes at their option at any time prior to the close of business on the business day immediately preceding February 15, 2022 only under the following circumstances:

- during any calendar quarter commencing on or after September 30, 2015 (and only during such calendar quarter), if the last reported sale price of the Company's common stock for at least 20 trading days (whether or not consecutive) during a period of 30 consecutive trading days ending on the last trading day of the immediately preceding calendar quarter is greater than or equal to 130% of the conversion price on each applicable trading day;

- during the five business day period after any five consecutive trading day period (the "measurement period") in which the trading price (as defined in the Convertible Notes Indenture) per $1,000 principal amount of Convertible Notes for each trading day of the measurement period was less than 98% of the product of the last reported sale price of the Company's common stock and the conversion rate on each such trading day;

- during any period after the Company has issued notice of redemption until the close of business on the scheduled trading day immediately preceding the relevant redemption date; or

- upon the occurrence of specified corporate events.

On or after February 15, 2022, until the close of business on the business day immediately preceding the maturity date, holders may convert their Convertible Notes at any time, regardless of the foregoing circumstances. Upon conversion, the Company will pay cash up to the aggregate principal amount of the Convertible Notes to be converted and deliver shares of its common stock in respect of the remainder, if any, of its conversion obligation in excess of the aggregate principal amount of Convertible Notes being converted.

The conversion rate for the Convertible Notes was initially, and remains, 17.7487 shares of the Company's common stock per $1,000 principal amount of the Convertible Notes, which is equivalent to an initial conversion price of approximately $56.34 per share of the Company's common stock.

The Company may not redeem the Convertible Notes prior to August 20, 2018. The Company may redeem for cash all or any portion of the Convertible Notes, at its option, on or after August 20, 2018 if the last reported sale price of its common stock has been at least 130% of the conversion price then in effect on the last trading day of, and for at least 19 other trading days (whether or not consecutive) during, any 30 consecutive trading day period ending on, and including, the trading day immediately preceding the date on which the Company provides notice of redemption, at a redemption price equal to 100% of the principal amount of the Convertible Notes to be redeemed, plus accrued and unpaid interest to, but excluding, the redemption date. No sinking fund is provided for the Convertible Notes, which means that the Company is not required to redeem or retire the Convertible Notes periodically.

If the Company undergoes a "fundamental change" (as defined in the Indenture governing the Convertible Notes Indenture), subject to certain conditions, holders of the Convertible Notes may require the Company to repurchase for cash all or part of their Convertible Notes at a repurchase price equal to 100% of the principal amount of the Convertible Notes to be repurchased, plus accrued and unpaid interest to, but excluding, the fundamental change repurchase date.

The Convertible Notes Indenture contains customary events of default with respect to the Convertible Notes, including that upon certain events of default (including the Company's failure to make any payment of principal or interest on the Convertible Notes when due and payable) occurring and continuing, the Convertible Notes Trustee by notice to the Company, or the holders of at least 25% in principal amount of the outstanding Convertible Notes by notice to the Company and the Convertible Notes Trustee, may, and the Convertible Notes Trustee at the request of such holders (subject to the provisions of the Convertible Notes Indenture) shall, declare 100% of the principal of and accrued and unpaid interest, if any, on all the Convertible Notes to be due and payable. In case of certain events of bankruptcy, insolvency or reorganization, involving the Company or a significant subsidiary, 100% of the principal of and accrued and unpaid interest on the Convertible Notes will automatically become due and payable. Upon such a declaration of acceleration, such principal and accrued and unpaid interest, if any, will be due and payable immediately.

The Company accounts for the Convertible Notes as a liability and equity component where the carrying value of the liability component will be valued based on a similar instrument. In accounting for the issuance of the Convertible Notes, the Company separated the Convertible Notes into liability and equity components. The carrying amount of the liability component was calculated by measuring the fair value of a similar liability that does not have an associated convertible feature. The carrying amount of the equity component representing the conversion option was determined by deducting the fair value of the liability component from the par value of the Convertible Notes as a whole. The excess of the principal amount of the liability component over its carrying amount, referred to as the debt discount, is amortized to interest expense over the seven-year term of the Convertible Notes. The equity component is not re-measured as long as it continues to meet the conditions for equity classification. The equity component recorded at issuance related to the Convertible Notes is $57.5 million and was recorded in additional paid-in capital.

In accounting for the transaction costs related to the issuance of the Convertible Notes, the Company allocated the total costs incurred to the liability and equity components of the Convertible Notes based on their relative values. Transaction costs attributable to the liability component are amortized to interest expense over the seven-year term of the Convertible Notes, and transaction costs attributable to the equity component are netted with the equity components in stockholders' equity. Additionally, the Company initially recorded a net deferred tax liability of $22.3 million in connection with the Notes.

The Convertible Notes consist of the following:

Liability component	June 30, 2018	December 31, 2017
Principal	$ 150,000	$ 150,000
Less: Debt issuance costs	(1,939)	(2,121)
Less: Debt discount, net(1)	(38,929)	(42,572)
Net carrying amount	$ 109,132	$ 105,307

(1) Included in the consolidated balance sheets within convertible senior notes (due 2022) and amortized to interest expense over the remaining life of the Convertible Notes using the effective interest rate method.

The fair value of the Convertible Notes was approximately $148.2 million as of June 30, 2018. The Company estimates the fair value of its Convertible Notes utilizing market quotations for debt that have quoted prices in active markets. As of June 30, 2018, the remaining contractual life of the Convertible Notes is approximately 4.1 years.

The following table sets forth total interest expense recognized related to the Convertible Notes:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Contractual interest expense	$ 1,131	$ 1,131	$ 2,241	$ 2,241
Amortization of debt issuance costs	93	83	182	163
Amortization of debt discount	1,863	1,674	3,644	3,274
Total	$ 3,087	$ 2,888	$ 6,067	$ 5,678
Effective interest rate of the liability component	11%	11%	11%	11%

10. Commitments and contingencies

Under various agreements, the Company will be required to pay royalties and milestone payments upon the successful development and commercialization of products. The Company has entered into funding agreements with The Wellcome Trust Limited ("Wellcome Trust") for the research and development of small molecule compounds in connection with the Company's oncology and antibacterial programs. As the Company has discontinued development under its antibacterial program, it no longer expects that milestone and royalty payments from the Company to Wellcome Trust will apply under that agreement, resulting in a change to the total amount of development and regulatory milestone payments the Company may become obligated to pay for this program. Under the oncology program funding agreement, to the extent that the Company develops and commercializes program intellectual property on a for-profit basis itself or in collaboration with a partner (provided the Company retains overall control of worldwide commercialization), the Company may become obligated to pay to Wellcome Trust development and regulatory milestone payments and single-digit royalties on sales of any research program product. The Company's obligation to pay such royalties would continue on a country-by-country basis until the longer of the expiration of the last patent in the program intellectual property in such country covering the research program product and the expiration of market exclusivity of such product in such country. The Company's first such milestone payment of $0.8 million payable to Wellcome Trust occurred in the second quarter of 2016. Additional milestone payments of up to an aggregate of $22.4 million may become payable by the Company to Wellcome Trust under this agreement.

The Company has also entered into a collaboration agreement with the SMA Foundation. The Company may become obligated to pay the SMA Foundation single- digit royalties on worldwide net product sales of any collaboration product that is successfully developed and subsequently commercialized or, if the Company outlicenses rights to a collaboration product, a specified percentage of certain payments the Company receives from its licensee. The Company is not obligated to make such payments unless and until annual sales of a collaboration product exceed a designated threshold. The Company's obligation to make such payments would end upon the Company's payment to the SMA Foundation of a specified amount.

The Company has employment agreements with certain employees which require the funding of a specific level of payments, if certain events, such as a change in control or termination without cause, occur. Additionally, the Company has royalty payments

associated with Translarna and Emflaza product net sales, payable quarterly or annually in accordance with the terms of the related agreements.

The Company is currently involved in various legal proceedings (refer to Part II, Item 1. Legal Proceedings for further details on the lawsuits filed). The Company denies any allegations of wrongdoing and intends to vigorously defend against these lawsuits. The Company is unable, however, to predict the outcome of these matters at this time. Moreover, any conclusion of this matter in a manner adverse to the Company and for which it incurs substantial costs or damages not covered by the Company's directors' and officers' liability insurance would have a material adverse effect on its financial condition and business. In addition, the litigation could adversely impact the Company's reputation and divert management's attention and resources from other priorities, including the execution of business plans and strategies that are important to the Company's ability to grow its business, any of which could have a material adverse effect on the Company's business.

11. Revenue recognition

Net product sales

The Company views its operations and manages its business in one operating segment. During the three and six months ended June 30, 2018, net product sales in the United States were $20.3 million and $39.5 million, respectively, consisting solely of Emflaza, and net product sales not in the United States were $47.8 million and $84.6 million, respectively, consisting solely of Translarna.

The following table presents changes in the Company's contract liabilities from December 31, 2017 to June 30, 2018:

	Balance as of December 31, 2017	Additions	Deductions	ASC 606 Adjustment	Balance as of June 30, 2018
Deferred Revenue	$ 11,891	$ 2,586	$ —	$ (3,937)	$ 10,540

The Company did not have any contract assets for the three and six months ended June 30, 2018.

During the three and six months ended June 30, 2018, the Company recognized revenue in the period from:

	Three Months Ended June 30, 2018	Six Months Ended June 30, 2018
Amounts included in contract liabilities at the beginning of the period	$ —	$ —
Performance obligations satisfied in previous period	—	—
Performance obligations satisfied in current period	68,170	124,151
Total product revenue	$ 68,170	$ 124,151

The Company has not made significant changes to the judgments made in applying ASC Topic 606 for the three and six months ended June 30, 2018.

Remaining performance obligations

Remaining performance obligations represent the transaction price for goods the Company has yet to provide. As of June 30, 2018, the aggregate amount of transaction price allocated to remaining performance obligations relating to Translarna net product revenue was $10.5 million. The Company expects to recognize revenue over the next two to four years as the specific timing for satisfying the performance obligations is contingent upon a number of factors, including customers' needs and schedules.

The impact of adoption using the modified retrospective method on the Company's consolidated financial statements is as follows:

i.Consolidated balance sheets

	Impact of changes in accounting policies		
	As reported June 30, 2018	Adjustments	As reported Balances without adoption of Topic 606
Assets			
Current assets:			
Cash and cash equivalents	$ 223,788	$ —	$ 223,788
Marketable securities	72,318	—	72,318
Trade receivables, net	59,383	—	59,383
Inventory	13,852	(67)	13,785
Prepaid expenses and other current assets	6,305	—	6,305
Total current assets	375,646	(67)	375,579
Fixed assets, net	8,217	—	8,217
Intangible assets, net	126,290	—	126,290
Deposits and other assets	1,620	—	1,620
Total assets	$ 511,773	$ (67)	$ 511,706
Liabilities and stockholders' equity			
Current liabilities:			
Accounts payable and accrued expenses	$ 82,534	$ (649)	$ 81,885
Current portion of long-term debt	1,666	—	1,666
Deferred revenue	—	4,141	4,141
Other current liabilities	2,274	—	2,274
Total current liabilities	86,474	3,492	89,966
Deferred revenue - long-term	10,540	—	10,540
Long-term debt	147,204	—	147,204
Other long-term liabilities	153	—	153
Total liabilities	244,371	3,492	247,863
Stockholders' equity:			
Common stock	47	—	47
Additional paid-in capital	1,105,124	—	1,105,124
Accumulated other comprehensive income	1,855	—	1,855
Accumulated deficit	(839,624)	(3,559)	(843,183)
Total stockholders' equity	267,402	(3,559)	263,843
Total liabilities and stockholders' equity	$ 511,773	$ (67)	$ 511,706

ii.Consolidated statements of operations

	Impact of changes in accounting policies Three Months Ended		
	As reported for the period ended June 30, 2018	Adjustments	As reported Balances without adoption of Topic 606
Revenues:			
Net product revenue	$ 68,170	$ 604	$ 68,774
Collaboration and grant revenue	573	—	573
Total revenues	68,743	604	69,347
Operating expenses:			
Cost of product sales, excluding amortization of acquired intangible asset	2,572	13	2,585
Amortization of acquired intangible asset	5,593	—	5,593
Research and development	32,607	—	32,607
Selling, general and administrative	33,545	—	33,545
Total operating expenses	74,317	13	74,330
Loss from operations	(5,574)	591	(4,983)
Interest expense, net	(2,884)	—	(2,884)
Other expense, net	(673)	—	(673)
Loss before income tax expense	(9,131)	591	(8,540)
Income tax expense	(389)	—	(389)
Net loss attributable to common stockholders	$ (9,520)	$ 591	$ (8,929)

	Impact of changes in accounting policies Year to Date		
	As reported for the period ended June 30, 2018	Adjustments	As reported Balances without adoption of Topic 606
Revenues:			
Net product revenue	$ 124,151	$ (225)	$ 123,926
Collaboration and grant revenue	654	—	654
Total revenues	124,805	(225)	124,580
Operating expenses:			
Cost of product sales, excluding amortization of acquired intangible asset	5,616	(67)	5,549
Amortization of acquired intangible asset	11,022	—	11,022
Research and development	63,970	—	63,970
Selling, general and administrative	66,514	—	66,514
Total operating expenses	147,122	(67)	147,055
Loss from operations	(22,317)	(158)	(22,475)
Interest expense, net	(6,187)	—	(6,187)
Other income, net	332	—	332
Loss before income tax expense	(28,172)	(158)	(28,330)
Income tax expense	(610)	—	(610)
Net loss attributable to common stockholders	$ (28,782)	$ (158)	$ (28,940)

iii.Consolidated statements of comprehensive loss

	Impact of changes in accounting policies Three Months Ended		
	As reported for the period ended June 30, 2018	Adjustments	As reported Balances without adoption of Topic 606
Net loss	$ (9,520)	$ 591	$ (8,929)
Other comprehensive loss:			
Unrealized gain on marketable securities, net of tax	40	—	40
Foreign currency translation loss	(3,138)	—	(3,138)
Comprehensive loss	$ (12,618)	$ 591	$ (12,027)

	Impact of changes in accounting policies Year to Date		
	As reported for the period ended June 30, 2018	Adjustments	As reported Balances without adoption of Topic 606
Net loss	$ (28,782)	$ (158)	$ (28,940)
Other comprehensive loss:			
Unrealized loss on marketable securities, net of tax	(83)	—	(83)
Foreign currency translation loss	(2,031)	—	(2,031)
Comprehensive loss	$ (30,896)	$ (158)	$ (31,054)

iv.Consolidated statements of cash flows

	Impact of changes in accounting policies		
	As reported for the period ended June 30, 2018	Adjustments	Balances without adoption of Topic 606
Cash flows from operating activities			
Net loss	$ (28,782)	$ (158)	$ (28,940)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	12,243	—	12,243
Change in valuation of warrant liability	—	—	—
Non-cash interest expense	3,644	—	3,644
Loss on disposal of asset	—	—	—
Amortization of premiums and accretion of discounts on investments, net	(230)	—	(230)
Amortization of debt issuance costs	256	—	256
Share-based compensation expense	15,831	—	15,831
Benefit for deferred income taxes	—	—	—
Unrealized foreign currency transaction losses, net	(764)	—	(764)
Changes in operating assets and liabilities:			
Inventory, net	(3,393)	(67)	(3,460)
Prepaid expenses and other current assets	254	—	254
Trade receivables, net	(20,429)	—	(20,429)
Deposits and other assets	(419)	—	(419)
Accounts payable and accrued expenses	2,225	(649)	1,576
Other liabilities	485	—	485
Deferred revenue	3,204	874	4,078
Net cash used in operating activities	(15,875)	—	(15,875)
Cash flows from investing activities			
Purchases of fixed assets	(1,187)	—	(1,187)
Purchases of marketable securities	(28,656)	—	(28,656)
Sale and redemption of marketable securities	35,939	—	35,939
Acquisition, including transaction costs	—	—	—
Net cash provided by investing activities	6,096	—	6,096
Cash flows from financing activities			
Proceeds from exercise of options	3,592	—	3,592
Net proceeds from public offerings	117,874	—	117,874
Proceeds from shares issued under employee stock purchase plan	1,299	—	1,299
Debt issuance costs related to secured term loan	—	—	—
Proceeds from issuance of secured term loan	—	—	—
Debt issuance costs related to convertible notes	—	—	—
Proceeds from issuance of convertible notes	—	—	—
Net cash provided by financing activities	122,765	—	122,765
Effect of exchange rate changes on cash	(990)	—	(990)
Net increase in cash and cash equivalents	111,996	—	111,996
Cash and cash equivalents, beginning of period	111,792	—	111,792
Cash and cash equivalents, end of period	$ 223,788	$ —	$ 223,788

Collaboration revenue

The Company has ongoing collaborations with the Spinal Muscular Atrophy Foundation (SMA Foundation) and F. Hoffman-La Roche Ltd and Hoffman- La Roche Inc. (collectively, Roche) and early stage discovery arrangements with other institutions. The

following are the key terms to the Company's (i) ongoing collaborations and (ii) early stage discovery and development arrangements.

Roche and SMA Foundation

In November 2011, the Company and the SMA Foundation entered into a licensing and collaboration agreement with Roche for a spinal muscular atrophy program. Under the terms of the agreement, Roche acquired an exclusive worldwide license to the Company's spinal muscular atrophy program, which includes three compounds currently in preclinical development, as well as potential back-up compounds. The Company received a nonrefundable upfront cash payment of $30.0 million during the research term, which was terminated effective December 31, 2014, after which Roche provided the Company with funding, based on an agreed- upon full-time equivalent rate, for an agreed-upon number of full- time equivalent employees that the Company contributed to the research program.

The Company identified two material promises in the collaboration agreement, the license and the research activities. The Company evaluated whether these material promises are distinct and determined that the license does not have standalone functionality and there is a significant integration of the license and research activities. As such, both promises were bundled into one distinct performance obligation. As a result, the Company deferred the $30.0 million upfront payment which was recognized over the estimated performance period of two years, which was the contracted research period. As of adoption of ASC Topic 606 on January 1, 2018, all performance obligations had been satisfied and the balance of the remaining deferred upfront payment was fully recognized.

Under the agreement, the Company is eligible to receive additional payments from Roche if specified events are achieved with respect to each licensed product, including up to $135.0 million in research and development event milestones, up to $325.0 million in sales milestones upon achievement of sales events, and up to double digit royalties on worldwide annual net sales of a commercial product.

In January 2014, the Company announced the initiation of a Phase 1 clinical program in its spinal muscular atrophy collaboration with Roche and the SMA Foundation which triggered a $7.5 million milestone payment from Roche. Under ASC Topic 605, the Company considered this milestone event substantive because the applicable criteria of its revenue recognition policy would be satisfied and recorded it as collaboration revenue for the year ended December 31, 2014.

In November 2014, the Company announced the initiation of a Phase 2 study in adult and pediatric patients in its spinal muscular atrophy collaboration with Roche and the SMA Foundation which triggered a $10 million payment from Roche. Under ASC Topic 605, the Company considered this milestone event substantive because the applicable criteria of its revenue recognition policy would be satisfied and recorded it as collaboration revenue for the year ended December 31, 2014.

In October 2017, the Company announced that the Sunfish, a two-part clinical trial in pediatric and adult type 2 and type 3 spinal muscular atrophy initiated in the fourth quarter of 2016 with Roche and SMA Foundation, had transitioned into the pivotal second part of its study. The achievement of this milestone triggered a $20.0 million payment to the Company from Roche. Under ASC Topic 605, the Company considered this milestone event substantive because the applicable criteria of its revenue recognition policy would be satisfied and recorded it as collaboration revenue for the year ended December 31, 2017.

For the six months ended June 30, 2018 and 2017, the Company recognized revenue related to the licensing and collaboration agreement with Roche of $0.1 million and $0.1 million, respectively.

Early stage collaboration and discovery agreements

From time to time, the Company has arrangements with several organizations pursuant to which the Company uses its discovery technologies to help identify potential drug candidates. The Company does not take ownership of the potential compounds, but rather provides research services to the collaborator using its specialized technology platform.

Generally, these arrangements are structured such that the collaborator and the Company work together to jointly select targets from which to apply its discovery technologies. The research period for the Company to apply its technology is generally three to four years. The Company will typically receive a nonrefundable, upfront cash payment and the collaborator agrees to provide funding for research activities performed on its behalf.

Generally, the two material promises in these arrangements are the license and the research activities. The Company evaluated whether these material promises are distinct and determined that the license does not have standalone functionality and there is a significant integration of the license and research activities. As such, both promises are bundled into one distinct performance obligation. As of adoption of ASC Topic 606 on January 1, 2018, all deferred revenue related to these arrangements had been recognized. For the six months ended June 30, 2018 and 2017, the Company did not recognize any revenue related to discovery agreements.

The Company is eligible to receive additional payments from its early stage discovery research arrangements if the discovery compounds are ultimately developed and commercialized. The aggregate potential payments the Company is eligible for if all products are developed is $143.0 million and up to $252.0 million in sales milestones upon achievement of specified sales events and up to double digit royalties on worldwide annual net sales of the licensed product. The Company will recognize revenue when it is probable the milestones will be achieved (see Note 2). For the six months ended June 30, 2018 and 2017, the Company did not recognize any revenue related to early stage collaborations.

12. Intangible assets

On April 20, 2017, the Company completed its previously announced acquisition of all rights to Emflaza pursuant to the Asset Purchase Agreement, dated March 15, 2017, and amended on April 20, 2017, by and between the Company and Marathon. The assets acquired by the Company in the Transaction include intellectual property rights related to Emflaza, inventories of Emflaza, and certain contractual rights related to Emflaza. In accordance with ASU No. 2017-01, the Company determined that substantially all of the fair value is concentrated in the Emflaza rights intangible asset and as such accounted for the transaction as an asset acquisition under ASC 805-50.

The Emflaza rights intangible asset is being amortized to cost of product sales over its expected useful life of approximately seven years on a straight line basis.

Marathon is entitled to receive contingent payments from the Company based on annual net sales of Emflaza beginning in 2018, up to a specified aggregate maximum amount over the expected commercial life of the asset. In accordance with the guidance for an asset acquisition, the Company will record the milestone payment when it becomes payable to Marathon and increase the cost basis for the Emflaza rights intangible asset.

As of June 30, 2018, the Company recorded a contingent payment of $3.9 million payable to Marathon as an increase to the cost of the Emflaza rights intangible asset and that will be amortized prospectively on a straight-line basis over the remaining life of the Emflaza rights intangible asset.

For the six months ended June 30, 2018, the Company recognized amortization expense of $11.0 million with respect to the Emflaza rights intangible asset. The estimated future amortization of the Emflaza rights intangible asset is expected to be as follows:

	As of June 30, 2018
2018(1)	$ 11,238
2019	22,477
2020	22,477
2021	22,477
2022 and thereafter	47,621
Total	$ 126,290

(1) For the six months ended December 31, 2018.

13. Subsequent events

Merger Agreement

On July 19, 2018, the Company entered into an Agreement and Plan of Merger (the "Merger Agreement" by and among the Company, Agility Merger Sub, Inc., a Delaware corporation and a wholly owned, indirect subsidiary of the Company ("Transitory Subsidiary"), Agilis Biotherapeutics, Inc., a Delaware corporation ("Agilis") and, solely in its capacity as the representative, agent and attorney-in-fact of the equityholders of Agilis, Shareholder Representative Services LLC, a Colorado limited liability company. The Merger Agreement provides for the acquisition of Agilis by the Company through the merger of Transitory Subsidiary into Agilis, with Agilis surviving as a wholly owned, indirect subsidiary of the Company (the "Merger"). Agilis is a privately-held biotechnology company advancing an innovative gene therapy platform for rare monogenic diseases that affect the central nervous system.

At the effective time of the Merger, by virtue of the Merger and without any action on the part of the holders of capital stock of Agilis, all issued and outstanding shares of the capital stock and outstanding vested options and warrants of Agilis will be converted into the right to receive, subject to customary adjustments, an aggregate, of (i) $50.0 million in cash and (ii) a number of shares

of the Company's common stock (the "Closing Stock Consideration"), equal to $150.0 million divided by the volume-weighted average price per share of the Company's common stock on the Nasdaq Global Select Market for the ten consecutive trading day period ending on the second trading day immediately preceding the closing of the Merger and will be subject to reduction such that the number of shares issuable will not equal or exceed 20% of the issued and outstanding shares of the Company's common stock immediately prior to the closing date, which is expected to be a maximum of approximately 9.34 million shares of the Company's common stock. Agilis equityholders would receive additional cash consideration in lieu of any such reduction in Closing Stock Consideration.

In addition, pursuant to the Merger Agreement, Agilis equityholders will be entitled to receive contingent payments from the Company based on (i) the achievement of certain development milestones up to an aggregate maximum amount of $60.0 million, (ii) the achievement of certain regulatory approval milestones together with a milestone payment following the receipt of a priority review voucher up to an aggregate maximum amount of $535.0 million, (iii) the achievement of certain net sales milestones up to an aggregate maximum amount of $150.0 million, and (iv) a percentage of annual net sales for Friedreich ataxia and Angelman Syndrome during specified terms, ranging from 2-6%. Under the Merger Agreement, the Company is required to pay $40.0 million of the development milestone payments no later than the second anniversary of the closing of the Merger, regardless of whether the applicable milestones have been achieved.

Pursuant to the Merger Agreement, the Company has agreed (i) in the event that Agilis provides certain required information at least 10 business days prior to the closing of the Merger, on the closing date of the Merger or, (ii) alternatively, following the closing of the Merger, to use commercially reasonable efforts to file a registration statement on Form S-3 with respect to the resale of the shares of the Closing Stock Consideration to be issued to Agilis equityholders as Merger consideration and to maintain the effectiveness of such registration statement until the six month anniversary date of the closing of the Merger or such earlier time as all shares of Company common stock covered by the registration statement have been sold, subject to certain exceptions and the provision of certain information by Agilis.

Bridge Loan and Security Agreement

In connection with the Merger Agreement, on July 19, 2018, the Company entered into a Bridge Loan and Security Agreement (the "Bridge Loan Agreement"), by and among the Company, Agilis and certain of Agilis's domestic subsidiaries, as guarantors. Under the Bridge Loan Agreement, the Company made a term loan advance to Agilis on July 23, 2018 in an original principal amount of $10.0 million and, in the event that the Merger does not close prior to September 2, 2018, the Company is obligated to make an additional term loan advance of $10.0 million, in each case, subject to the satisfaction of certain conditions. Each domestic subsidiary of Agilis has agreed to jointly and severally guarantee Agilis's payment obligations under the Bridge Loan Agreement.

The term loans will accrue interest at a fixed rate per annum equal to 3.0%, subject to increase to 8.0% in the event of a default. Upon closing of the Merger or an event of default, Agilis must repay all outstanding term loans plus accrued and unpaid interest thereon, plus any other sums that have then become due and payable under the Bridge Loan Agreement; provided, however, that only the second term loan advance (if any) would be accelerated for payment in an event of default arising due to Agilis's failure to obtain stockholder approval for the Merger or a material breach of the Merger Agreement by Agilis. To the extent not previously repaid, all outstanding credit extensions plus accrued and unpaid interest thereon, plus any other sums that have then become due and payable under the Bridge Loan Agreement, must be repaid by July 19, 2020. Agilis may elect to prepay any or all of the term loan advances at any time without penalty or premium.

Under the Bridge Loan Agreement, Agilis granted the Company a continuing, first priority perfected security interest (subject to certain permitted liens) to all right, title and interest in, to and under all present and future real and personal property of Agilis and any guarantor, whether tangible or intangible, subject to certain exceptions in the Bridge Loan Agreement, but not including Agilis's intellectual property.

The Bridge Loan Agreement contains certain customary representations and warranties, affirmative covenants and conditions. The Bridge Loan Agreement also contains a number of negative covenants that, among other things and subject to certain exceptions, restrict Agilis's and its subsidiaries' ability to engage in certain actions or undergo certain changes from and after the termination of the Merger Agreement. The Bridge Loan Agreement also contains customary events of default (subject to grace periods in specified circumstances). In an event of default occurring as a result of termination of the Merger Agreement by the Company for Agilis's material breach or failure to perform or failure to timely obtain the required stockholder approval, the Company has a limited remedy to declare the second term loan advance (if any) immediately due and payable, but the first term loan advance would remain outstanding and accrue interest at 8.0% per annum.

Consent under Credit Agreement

In connection with the execution of the Merger Agreement and Bridge Loan Agreement, on July 19, 2018, the Company entered into an amendment and consent agreement (the "Credit Agreement Amendment") to the Credit Agreement with MidCap. Pursuant to the Credit Agreement Amendment, MidCap and the requisite lenders agreed to, among other things, amend and modify certain

covenants and other provisions in the Credit Agreement to permit the entering into of the Merger Agreement and the Bridge Loan Agreement, and the consummation and performance of the transactions contemplated thereby, in each case, subject to certain terms and conditions.

Collaboration and Licensing Agreement

On August 1, 2018 ("the Effective Date") PTC Therapeutics International Limited ("PTC International"), a subsidiary of the Company, entered into a Collaboration and License Agreement (the "Akcea Agreement") with Akcea Therapeutics, Inc. ("Akcea") for the commercialization by PTC International of Tegsedi™ (inotersen), Waylivra™ (volanesorsen) and products containing those compounds (collectively the "Products"), in countries in Latin America and the Caribbean (the "PTC Territory"). Tegsedi is an antisense oligonucleotide inhibitor of human transthyretin production ("TTR protein") for the treatment of patients with hereditary transthyretin amyloidosis (hATTR amyloidosis), a severe, rare and fatal genetic disease. Waylivra is an antisense drug candidate in development for two rare metabolic disorders: familial chylomicronemia syndrome (FCS) and familial partial lipodystrophy (FPL). Neither Tegsedi nor Waylivra is currently approved for marketing in the PTC Territory. In addition, Akcea has granted to PTC International a right of first negotiation (a "ROFN") to commercialize AKCEA-TTR-Lrx, a follow-on product candidate to inotersen, on an exclusive basis in the PTC Territory.

Under the terms of the Akcea Agreement, Akcea has granted to PTC International an exclusive right and license, with the right to grant certain sublicenses, under Akcea's product-specific intellectual property to develop, manufacture and commercialize the Products in the PTC Territory. In addition, Akcea has granted to PTC International a non-exclusive right and license, with the right to grant certain sublicenses, under Akcea's core intellectual property and manufacturing intellectual property to develop, manufacture and commercialize the Products in the PTC Territory and to manufacture the Products worldwide in accordance with a supply agreement with Akcea. Akcea has in-licensed certain of the Akcea intellectual property from its affiliate, Ionis Pharmaceuticals, Inc. ("Ionis"). Each party has agreed not to, independently or with any third party, commercialize any competing oligonucleotide product in the PTC Territory for the same gene target as inotersen.

Within 30 days after the effective date, Akcea has agreed to assign and transfer to PTC International the ownership and sponsorship of applicable regulatory approvals in countries in the PTC Territory, after which PTC International has agreed to prepare, file and maintain regulatory filings and approvals for the applicable Products in such countries. After the Effective Date, PTC International is responsible for all meetings, communications and other interactions with regulatory authorities in the PTC Territory.

PTC International has agreed to pay to Akcea an upfront licensing fee of $18.0 million, consisting of a payment of $12.0 million within ten business days after the Effective Date, and $6.0 million within 30 days after receipt of regulatory approval of Waylivra from the United States Food and Drug Administration or the European Medicines Agency, whichever occurs earlier. In addition, Akcea is eligible to receive milestone payments, on a Product-by-Product basis, of $4.0 million upon receipt of regulatory approval for a Product from ANVISA, the Brazilian Health Regulatory Authority, subject to a maximum aggregate amount of $8.0 million for all such Products. Akcea is also entitled to receive royalty payments in the mid-twenty percent range of net sales on a country-by-country and Product-by-Product basis, commencing on the earlier to occur of (1) 12 months after the first commercial sale of such product in Brazil or (2) the date when PTC International, its affiliates or sublicensees have recognized revenue of $10.0 million or more in cumulative net sales for such product in the PTC Territory. The royalty payments are subject to reduction in certain circumstances as set forth in the Agreement.

Akcea has granted to PTC International a ROFN to commercialize AKCEA-TTR-Lrx, a follow-on product candidate to inotersen, on an exclusive basis in the PTC Territory, subject to negotiation of the terms of a definitive agreement and certain other terms and conditions. Such a definitive agreement would provide for a royalty rate to be paid by PTC International for AKCEA-TTR-Lrx equal to the royalty rate PTC International has agreed to pay for Tegsedi under the Akcea Agreement, or in the mid-twenty percent range of net sales, and the term of such royalty payments would be the same as the term of the Tegsedi royalty payments. During a specified period in the Akcea Agreement, neither Akcea nor Ionis may enter into an agreement or grant any license to AKCEA-TTR-Lrx that is inconsistent with PTC International's ROFN. The activities of the parties pursuant to the Akcea Agreement will be overseen by a Joint Steering Committee, to be composed of an equal number of representatives appointed by each of PTC International and Akcea.

The Akcea Agreement continues until the expiration of the last to expire royalty term with respect to all Products in all countries in the PTC Territory. Either party may terminate the Akcea Agreement on written notice to the other party if such other party is in material breach of its obligations thereunder and has not cured such breach within 30 days after notice in the case of a payment breach or 60 days after notice in the case of any other breach.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations.

The following discussion of our financial condition and results of operations should be read in conjunction with our financial statements and the notes to those financial statements appearing elsewhere in this Quarterly Report on Form 10-Q and the

audited consolidated financial statements and notes thereto and management's discussion and analysis of financial condition and results of operations for the year ended December 31, 2017 included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 6, 2018. This discussion contains forward-looking statements that involve significant risks and uncertainties. As a result of many factors, such as those set forth in Part II, Item 1A. (Risk Factors) of this Quarterly Report on Form 10-Q, Part I, Item 1A. (Risk Factors) of our Annual Report on Form 10-K for the year ended December 31, 2017, and Part II, Item 1A. (Risk Factors) of our Quarterly Report on Form 10-Q for the period ended March 31, 2018, our actual results may differ materially from those anticipated in these forward-looking statements.

Our Company

We are a science-led global biopharmaceutical company focused on the discovery, development and commercialization of clinically-differentiated medicines that provide benefits to patients with rare disorders. We have launched two rare disease products and have a global commercial footprint. Our recent ability to commercialize products is the foundation that drives our continued investment in a robust pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need. Our strategy is to bring best-in-class therapies with differentiated clinical benefit to patients affected by rare disorders and to leverage our global commercial infrastructure to maximize value for our patients and other stakeholders.

We have two products, Translarna™ (ataluren) and Emflaza™ (deflazacort), for the treatment of Duchenne muscular dystrophy, or DMD, a rare, life threatening disorder. During the quarter ended June 30, 2018, we recognized $47.8 million in sales of Translarna. Translarna is currently available for the treatment of nmDMD in over 25 countries on a commercial basis or through a reimbursed early access program, or EAP program. We hold worldwide commercialization rights to Translarna for all indications in all territories. Emflaza is approved in the United States for the treatment of DMD in patients five years and older. During the quarter ended June 30, 2018, Emflaza achieved sales of $20.3 million.

Corporate Updates

Acquisition of Agilis Biotherapeutics, Inc.

On July 19, 2018, we announced that we had entered into an Agreement and Plan of Merger, or the Merger Agreement, by and among us, Agility Merger Sub, Inc., a Delaware corporation and our wholly owned, indirect subsidiary, or the Transitory Subsidiary, Agilis Biotherapeutics, Inc., a Delaware corporation, or Agilis, and, solely in its capacity as the representative, agent and attorney-in-fact of the equityholders of Agilis, Shareholder Representative Services LLC, a Colorado limited liability company. The Merger Agreement provides for the acquisition of Agilis by us through the merger of Transitory Subsidiary into Agilis, with Agilis surviving as our wholly owned, indirect subsidiary, subject to the satisfaction or waiver of certain conditions. Agilis is a privately-held biotechnology company advancing an innovative gene therapy platform for rare monogenic diseases that affect the central nervous system. Additional information concerning the planned acquisition is discussed in Note 12. Subsequent Events in the consolidated financial statements and in Part II Item 1A. Risk Factors, each appearing elsewhere in this Quarterly Report on Form 10-Q. Unless otherwise stated or the context otherwise requires, we have not reflected in this Quarterly Report on Form 10-Q the changes to our business that may occur if we consummate the planned acquisition.

Bridge Loan and Security Agreement

In connection with the Merger Agreement, on July 19, 2018, we entered into a Bridge Loan and Security Agreement, or the Bridge Loan Agreement, by and among us, Agilis and certain of Agilis's domestic subsidiaries, as guarantors. Under the Bridge Loan Agreement, we made a term loan advance to Agilis on July 23, 2018 in an original principal amount of $10.0 million and, in the event that the Merger does not close prior to September 2, 2018, we will be obligated to make an additional term loan advance of up to $10.0 million, in each case, subject to the satisfaction of certain conditions. Each domestic subsidiary of Agilis has agreed to jointly and severally guarantee Agilis's payment obligations under the Bridge Loan Agreement.

The term loans will accrue interest at a fixed rate per annum equal to 3.0%, subject to increase to 8.0% in the event of a default. Upon closing of the Merger or an event of default, Agilis must repay all outstanding term loans plus accrued and unpaid interest thereon, plus any other sums that have then become due and payable under the Bridge Loan Agreement; provided, however, that only the second term loan advance (if any) would be accelerated for payment in an event of default arising due to Agilis's failure to obtain stockholder approval for the Merger or a material breach of the Merger Agreement by Agilis. To the extent not previously repaid, all outstanding credit extensions plus accrued and unpaid interest thereon, plus any other sums that have then become due and payable under the Bridge Loan Agreement, must be repaid by July 19, 2020. Agilis may elect to prepay any or all of the term loan advances at any time without penalty or premium.

Akcea Collaboration and Licensing Agreement

On August 1, 2018, or the Effective Date, PTC Therapeutics International Limited, or PTC International, our subsidiary, entered into a Collaboration and License Agreement, or the Akcea Agreement, with Akcea Therapeutics, Inc., or Akcea, for the commercialization by PTC International of Tegsedi™ (inotersen), WAYLIVR™ (volanesorsen) and products containing those compounds, which we refer to collectively as the Products, in countries in Latin America and the Caribbean, or the PTC Territory.

Under the terms of the Akcea Agreement, Akcea has granted to PTC International an exclusive right and license, with the right to grant certain sublicenses, under Akcea's product-specific intellectual property to develop, manufacture and commercialize the Products in the PTC Territory. In addition, Akcea has granted to PTC International a non-exclusive right and license, with the right to grant certain sublicenses, under Akcea's core intellectual property and manufacturing intellectual property to develop, manufacture and commercialize the Products in the PTC Territory and to manufacture the Products worldwide in accordance with a supply agreement with Akcea. Akcea has in-licensed certain of the Akcea intellectual property from its affiliate, Ionis Pharmaceuticals, Inc., or Ionis. Each party has agreed not to, independently or with any third party, commercialize any competing oligonucleotide product in the PTC Territory for the same gene target as inotersen.

PTC International has agreed to pay to Akcea an upfront licensing fee of $18.0 million, consisting of a payment of $12.0 million within ten business days after the Effective Date, and $6.0 million within 30 days after receipt of regulatory approval of Waylivra from the United States Food and Drug Administration, or FDA, or the European Medicines Agency, or EMA, whichever occurs earlier. In addition, Akcea is eligible to receive milestone payments, on a Product-by-Product basis, of $4.0 million upon receipt of regulatory approval for a Product from ANVISA, the Brazilian Health Regulatory Authority, subject to a maximum aggregate amount of $8.0 million for all such Products. Akcea is also entitled to receive royalty payments in the mid-twenty percent range of net sales on a country-by-country and Product-by-Product basis, commencing on the earlier to occur of (1) 12 months after the first commercial sale of such Product in Brazil or (2) the date when PTC International, its affiliates or sublicensees have recognized revenue of $10.0 million or more in cumulative net sales for such Product in the PTC Territory. The royalty payments are subject to reduction in certain circumstances as set forth in the Akcea Agreement.

Tegsedi, a product of Ionis' proprietary antisense technology, is an antisense oligonucleotide, or ASO, inhibitor of human transthyretin, or TTR, production. Tegsedi is the world's first RNA-targeted therapeutic to treat patients with hereditary transthyretin amyloidosis (hATTR amyloidosis). It has received marketing authorization approval from the European Commission for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hATTR amyloidosis. Tegsedi is also the subject of a pending new drug application in the U.S. and Canada. Tegsedi has a PDUFA date of October 6, 2018. We plan on filing requests for marketing authorizations in various Latin American countries in the next 12 months.

hATTR amyloidosis is a progressive, systemic and fatal inherited disease caused by the abnormal formation of the TTR protein and aggregation of TTR amyloid deposits in various tissues and organs throughout the body, including in peripheral nerves, heart, intestinal tract, eyes, kidneys, central nervous system, thyroid and bone marrow. The progressive accumulation of TTR amyloid deposits in these tissues and organs leads to sensory, motor and autonomic dysfunction often having debilitating effects on multiple aspects of a patient's life. Patients with hATTR amyloidosis often present with a mixed phenotype and experience overlapping symptoms of polyneuropathy and cardiomyopathy.

Ultimately, hATTR amyloidosis generally results in death within three to fifteen years of symptom onset. Therapeutic options for the treatment of patients with hATTR amyloidosis are limited and there are currently no disease-modifying drugs approved for the disease. There are an estimated 50,000 patients with hATTR amyloidosis worldwide, including approximately 6,000 patients with polyneuropathic hATTR amyloidosis in Latin America

Waylivra, is under regulatory review in the U.S., EU and Canada for the treatment familial chylomicronemia syndrome, or FCS. The U.S. and EU regulatory agencies have granted Orphan Drug Designation to Waylivra for the treatment of FCS. Waylivra recently received a positive vote from the FDA's Division of Metabolism and Endocrinology Products Advisory Committee and has a PDUFA date of August 30, 2018. Additionally, Waylivra is currently in Phase 3 clinical development for the treatment of people with familial partial lipodystrophy, or FPL. The EMA has granted orphan drug designation to Waylivra for the treatment of patients with FPL.

FCS is an ultra-rare disease caused by impaired function of the enzyme lipoprotein lipase (LPL) and characterized by severe hypertriglyceridemia (>880mg/dL) and a risk of unpredictable and potentially fatal acute pancreatitis. Because of limited LPL function, people with FCS cannot break down chylomicrons, lipoprotein particles that are 90% triglycerides. In addition to pancreatitis, FCS patients are at risk of chronic complications due to permanent organ damage. They can experience daily symptoms including abdominal pain, generalized fatigue and impaired cognitions that affect their ability to work. People with FCS also report major emotional and psychosocial effects including anxiety, social withdrawal, depression and brain fog. There is no effective therapy for FCS currently available.

Neither Tegsedi nor Waylivra is currently approved for marketing in the PTC Territory.

Regulatory, clinical and marketing authorization matters for Translarna in nonsense mutation Duchenne muscular dystrophy

United States. Translarna is an investigational new drug in the U.S. During the first quarter of 2017, we filed a New Drug Application, or NDA, for Translarna for the treatment of nmDMD over protest with the FDA. In October 2017, the Office of Drug Evaluation I of the FDA issued a Complete Response Letter for the NDA, stating that it was unable to approve the application in its current form. In response, we filed a formal dispute resolution request with the Office of New Drugs of the FDA. In February 2018, the Office of New Drugs of the FDA denied our appeal of the Complete Response Letter. In its response, the Office of New Drugs recommended a possible path forward for our ataluren NDA submission based on the accelerated approval pathway. This would involve a re-submission of an NDA containing the current data on effectiveness of ataluren with new data to be generated on dystrophin production in nmDMD patients' muscles. We intend to follow the FDA's recommendation and will collect such dystrophin data using newer technologies via procedures and methods that we are currently designing and expect to initiate such a study by the end of 2018. Additionally, should a re-submission of an NDA receive accelerated approval, the Office of New Drugs stated that Study 041, which is currently enrolling, could serve as the confirmatory post-approval trial required in connection with the accelerated approval pathway.

There is substantial risk that the studies we use to collect the dystrophin data will not provide the necessary data to support a marketing approval for Translarna for the treatment of nmDMD.

European Economic Area. In July 2018, the European Commission renewed our marketing authorization for Translarna for the treatment of nmDMD in ambulatory patients aged two years and older in the 31 member states of the European Economic Area, or EEA, and it is effective, unless extended, through August 5, 2019. We received initial marketing authorization from the European Commission in August 2014 for the treatment of nmDMD in ambulatory patients aged five years and older. In July 2018, the European Commission approved a label-extension request to our marketing authorization for Translarna in the EEA to include patients from two to up to five years of age. The marketing authorization is subject to annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of continued authorization, which we refer to as the annual EMA reassessment, as well as our satisfaction of any specific obligation or other requirement placed upon the marketing authorization, including Study 041. Study 041 is a three-year clinical trial to confirm the efficacy and safety of Translarna in the approved patient population. The trial is comprised of two stages: an 18-month randomized, double-blind, placebo controlled clinical trial followed by an 18-month open label extension period. We expect to submit the results of Study 041 to the EMA by the end of the third quarter of 2021. We expect that as part of the annual EMA assessment, the EMA will consider the ongoing status of Study 041. There is substantial risk that if we are unable to renew our EEA marketing authorization during any annual renewal cycle, if our product label is materially restricted, or if Study 041 does not provide the data necessary to maintain our marketing authorization, we would lose all, or a significant portion of, our ability to generate revenue from sales of Translarna in the EEA and other territories.

Each country, including each member state of the EEA, has its own pricing and reimbursement regulations and system. In order to commence commercial sale of product pursuant to our Translarna marketing authorization in any particular country in the EEA, we must finalize pricing and reimbursement negotiations with the applicable government body in such country. As a result, our commercial activities will continue to be on a country-by-country basis. We also have made, and expect to continue to make, product available under EAP programs, both in countries in the EEA and other territories. Our ability to negotiate, secure and maintain reimbursement for product under commercial and EAP programs can be subject to challenge in any particular country and can also be affected by political, economic and regulatory developments in such country.

Emflaza for the treatment of Duchenne muscular dystrophy in the United States

Emflaza, both in tablet and suspension form, received approval from the FDA in February 2017 as a treatment for DMD in patients five years of age and older in the United States. We estimate that there are approximately 10,000 DMD patients in the United States aged five years or older. We are obligated to complete certain post-marketing requirements in connection with the FDA's approval, including pre-clinical and clinical safety studies.

We expect that Emflaza will have a seven-year exclusive marketing period in the United States for the approved indication, commencing on the date of FDA approval, under the provisions of the Orphan Drug Act of 1983, or the Orphan Drug Act, as well as a concurrent five-year exclusive marketing period in the United States for the active ingredient in Emflaza under the provisions of the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act. Additionally, because the FDA has requested that we conduct a pediatric study of Emflaza, we expect to be granted a term of pediatric exclusivity upon completion of an agreed-upon study. This additional exclusivity would provide for an additional six months of marketing protection beginning as of the end of the term of any existing regulatory exclusivity, including the seven-year orphan exclusivity period. As we presently have no patent rights to protect the approved use of Emflaza, we expect to rely on both the five-year Hatch-Waxman Act and seven-year Orphan Drug Act exclusivity periods to commercialize Emflaza for the approved

indication in the U.S. As the holder of orphan exclusivity, we are required to ensure the availability of sufficient quantities of Emflaza to meet the needs of patients. Failure to do so could result in loss of orphan exclusivity in the U.S.

Translarna for additional indications

Based on its understood mechanism of action, we believe that Translarna may have benefit in the treatment of patients with genetic disorders that arise as a result of a nonsense mutation. We are pursuing studies for Translarna in additional indications including nonsense mutation aniridia, and nonsense mutation Dravet syndrome/CDKL5. We have completed enrollment for our aniridia study and anticipate results during 2019.

Spinal muscular atrophy program

Our spinal muscular atrophy (SMA) collaboration is with F. Hoffman-La Roche Ltd and Hoffman-La Roche Inc., which we refer to collectively as Roche, and the Spinal Muscular Atrophy Foundation, or SMA Foundation. Currently, our collaboration has three clinical trials ongoing to evaluate the safety and effectiveness of risdiplam (RG7916, RO7034067), the lead compound in the SMA program. Sunfish, a two-part clinical study in pediatric and adult type 2 and type 3 SMA patients initiated in the fourth quarter of 2016, followed by the initiation of Firefish in the fourth quarter of 2016, a two-part clinical study in infants with type 1 SMA. In October 2017, Sunfish transitioned into the pivotal second part of its study, which triggered a $20.0 million milestone payment to us from Roche. In March 2018, Firefish transitioned into the pivotal second part of its study, the primary endpoint of which is the proportion of patients sitting without support after 12 months on risdiplam treatment. Data from the open label extension of part 1 of the Sunfish trial were presented in June 2018 at the 22nd Annual SMA Researcher Meeting organized by CureSMA, or the CureSMA meeting. Risdiplam was well tolerated at all doses and there have been no drug-related safety findings leading to withdrawal. In Sunfish, the data demonstrate that the previously reported SMN protein increase is maintained over 35 weeks of treatment indicating the durability of the pharmacodynamic effect. Interim clinical data from the Firefish trial were also presented in June 2018 at the CureSMA meeting. The median age of first dose was 6.7 months and babies have received risdiplam for a duration of up to 16.9 months. Risdiplam has been well tolerated at all doses and there have been no drug-related safety findings leading to withdrawal. At Day 182, over 90% of the babies achieved a greater than 4-point increase in CHOP-INTEND score, a rating to evaluate the motor skills of patients with SMA-I developed by the Children's Hospital of Philadelphia, compared to baseline. The CHOP-INTEND data were further supported by video footage presented by an independent investigator demonstrating antigravity movements, the ability to control their head, roll, or sit in three babies participating in Firefish. We understand that the number of babies having the ability to sit has increased since the presentation at the CureSMA meeting. Moreover, no babies have required a tracheostomy or permanent ventilation since study initiation and no baby has lost the ability to swallow. Previously published natural history data indicate that in comparable historic cohorts the median age of event-free survival for SMA Type 1 infants is between 8 and 10.5 months. In addition, SMN protein level increases of up to 6.5-fold were observed after 28 days of dosing and the increase was sustained. Jewelfish, an open-label study investigating the safety, tolerability, PK, and PK/pharmacodynamic relationship of risdiplam in type 2 and type 3 SMA patients who have been previously treated with a survival of motor neuron 2 (SMN2)-targeting therapy, initiated in the first quarter of 2017. Preliminary PD data from ten Jewelfish patients presented at the annual meeting of the Academy of American Neurology in April 2018 and at the CureSMA meeting demonstrate increases in SMN2 FL/SMN7 mRNA ratio and SMN protein level increases of up to 4-fold.

Pre-clinical and other programs

In addition, we have a pipeline of product candidates that are in early clinical and pre-clinical development. Our pre-clinical and discovery programs are focused on the development of new treatments for multiple therapeutic areas, including rare diseases and oncology.

Funding

The success of Translarna, Emflaza and any other product candidates we may develop, depends largely on obtaining and maintaining reimbursement from governments and third-party insurers. Our revenues are primarily generated from sales of Translarna for the treatment of nmDMD in territories where we are permitted to distribute Translarna under our EAP programs and in countries in the EEA where we were able to obtain acceptable commercial pricing and reimbursement terms, and from sales of Emflaza for the treatment of DMD in the United States.

To date, we have financed our operations primarily through our offering of 3.00% convertible senior notes due August 15, 2022, or the Convertible Notes offering, our public offerings of common stock in February 2014, October 2014 and April 2018, our initial public offering of common stock in June 2013, private placements of our preferred stock, collaborations, bank debt and convertible debt financings, the Credit Agreement and grants and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease areas addressed by our product candidates. Since 2014, we have also relied on revenue generated from net sales of Translarna for the treatment of nmDMD in territories outside of the United States,

and in May 2017, we began to recognize revenue generated from net sales of Emflaza for the treatment of DMD in the United States.

We have a credit and security agreement, or the Credit Agreement, with MidCap Financial Trust, or MidCap Financial, as administrative agent and MidCap Financial and other certain institutions as lenders thereto, that provides for a senior secured term loan facility of $60 million, of which $40 million was drawn by us on May 5, 2017. The remaining $20 million under the senior secured term loan facility would become available to us upon our demonstration (on or prior to December 31, 2018) of net product revenue equaling or exceeding $120 million for the trailing 12 month period. The maturity date of the Credit Agreement is May 1, 2021, unless terminated earlier.

In April 2018, we closed an underwritten public offering of our common stock pursuant to a registration statement on Form S-3. We issued and sold an aggregate of 4,600,000 shares of common stock under the registration statement at a public offering price of $27.04 per share, including 600,000 shares issued upon exercise by the underwriters of their option to purchase additional shares. We received net proceeds of approximately $117.9 million after deducting underwriting discounts and commissions and other offering expenses payable by us.

As of June 30, 2018, we had an accumulated deficit of $839.6 million. We had a net loss of $28.8 million and $46.5 million for the six month periods ended June 30, 2018 and 2017, respectively.

We anticipate that our expenses will continue to increase in connection with our commercialization efforts in the United States, the EEA and other territories, including the expansion of our infrastructure and corresponding sales and marketing, legal and regulatory, distribution and manufacturing and administrative and employee-based expenses. In addition to the foregoing, we expect to continue to incur significant costs in connection with Study 041 and our open label extension trials of Translarna for the treatment of nmDMD as well as our studies for nonsense mutation aniridia and nonsense mutation Dravet syndrome/CDKL5 and our FDA post-marketing requirements with respect to Emflaza in the United States. We also expect to incur ongoing research and development expenses for our other product candidates, including our oncology program. In addition, we may incur substantial costs in connection with our efforts to advance our regulatory submissions. We have begun seeking and intend to continue to seek marketing authorization for Translarna for the treatment of nmDMD in territories outside of the EEA and we may also seek marketing authorization for Translarna for other indications. These efforts may significantly impact the timing and extent of our commercialization expenses.

We may seek to continue to expand and diversify our product pipeline through opportunistically in-licensing or acquiring the rights to products, product candidates or technologies and we may incur expenses, including with respect to transaction costs, subsequent development costs or any upfront, milestone or other payments or other financial obligations associated with any such transaction, including our planned acquisition of Agilis, which would increase our future capital requirements.

With respect to our outstanding Convertible Notes, cash interest payments are payable on a semi-annual basis in arrears, which will require total funding of $4.5 million annually. Additionally, under the terms of our Credit Agreement cash interest payments are payable monthly in arrears. Furthermore, as a result of our initial public offering in June 2013, we have incurred and expect to continue to incur additional costs associated with operating as a public company including significant legal, accounting, investor relations and other expenses. Additionally, we could be forced to expend significant resources in the defense of the pending securities class action lawsuits brought against us and certain of our current and former executive officers, as described under Part II, Item 1. Legal Proceedings in this Quarterly Report on Form 10-Q.

We will need to generate significant revenues to achieve and sustain profitability, and we may never do so. Accordingly, we may need to obtain substantial additional funding in connection with our continuing operations. Adequate additional financing may not be available to us on acceptable terms, or at all. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or our commercialization efforts.

Financial operations overview

To date, our net product sales have consisted solely of sales of Translarna for the treatment of nmDMD in territories outside of the United States and sales of Emflaza for the treatment of DMD in the United States. Our process for recognizing revenue is described below under “Critical accounting policies and significant judgments and estimates—Revenue recognition”.

Roche and the SMA Foundation Collaboration. In November 2011, we entered into a license and collaboration agreement, or licensing agreement, with Roche and the SMA Foundation pursuant to which we are collaborating with Roche and the SMA Foundation to further develop and commercialize compounds identified under our spinal muscular atrophy program with the SMA Foundation. The research component of this agreement terminated effective December 31, 2014. The licensing agreement included a $30 million upfront payment made in 2011 which was recognized on a deferred basis over the research term, and the potential for up to $460 million in milestone payments and royalties on net sales.

In August 2013, we announced the selection of a development candidate. The achievement of this milestone triggered a $10.0 million payment to us from Roche, which we recorded as collaboration revenue for the year ended December 31, 2013.

In January 2014, we initiated a Phase 1 clinical program, which triggered a $7.5 million milestone payment to us from Roche which we recorded as collaboration revenue for the year ended December 31, 2014.

In November 2014, we announced that our joint development program in SMA with Roche and the SMA Foundation (SMAF) had started a Phase 2 study in adult and pediatric patients. The achievement of this milestone triggered a $10.0 million payment to us from Roche which we recorded as collaboration revenue for the year ended December 31, 2014.

In October 2017, we announced that the joint development program in SMA with Roche and SMAF had transitioned into the pivotal second part of its study evaluating the efficacy and safety of RG7916 in pediatric and adult Type 2/3 SMA patients. The achievement of this milestone triggered a $20.0 million payment to us from Roche which we recorded as collaboration revenue at time of achievement.

Grant revenue. From time to time, we receive grant funding from various institutions and governmental bodies. The grants are typically for early discovery research, and generally such grant programs last from two to five years.

Research and development expense

Research and development expenses consist of the costs associated with our research activities, as well as the costs associated with our drug discovery efforts, conducting preclinical studies and clinical trials, manufacturing development efforts and activities related to regulatory filings. Our research and development expenses consist of:

- external research and development expenses incurred under agreements with third-party contract research organizations and investigative sites, third-party manufacturing organizations and consultants;

- employee-related expenses, which include salaries and benefits, including share-based compensation, for the personnel involved in our drug discovery and development activities; and

- facilities, depreciation and other allocated expenses, which include direct and allocated expenses for rent and maintenance of facilities, IT, human resources and other support functions, depreciation of leasehold improvements and equipment, and laboratory and other supplies.

We use our employee and infrastructure resources across multiple research projects, including our drug development programs. We track expenses related to our clinical programs and certain preclinical programs on a per project basis.

We expect our research and development expenses to increase in connection with our ongoing activities, particularly in connection with Study 041 for Translarna for the treatment of nmDMD, our studies of Translarna in nonsense mutation aniridia and nonsense mutation Dravet syndrome/CDKL5, activities under our oncology program, and performance of our FDA post-marketing requirements with respect to Emflaza in the United States. The timing and amount of these expenses will depend upon the outcome of our ongoing clinical trials and the costs associated with our planned clinical trials. The timing and amount of these expenses will also depend on the costs associated with potential future clinical trials of our products or product candidates and the related expansion of our research and development organization, regulatory requirements, advancement of our preclinical programs, and product and product candidate manufacturing costs.

The following tables provide research and development expense for our most advanced principal product development programs, for the three and six months ended June 30, 2018 and 2017.

	Three Months Ended June 30,	
	2018	2017
	(in thousands)	
Translarna (nmDMD, nmCF, nmMPS I, aniridia and Dravet)	$ 16,942	$ 19,379
Oncology	3,081	2,105
Next generation nonsense readthrough	1,540	1,331
Emflaza	2,754	2,446
Other research and preclinical	8,290	5,574
Total research and development	$ 32,607	$ 30,835

	Six Months Ended June 30,	
	2018	2017
	(in thousands)	
Translarna (nmDMD, nmCF, nmMPS I, aniridia and Dravet)	$ 34,470	$ 37,423
Oncology	5,314	4,516
Next generation nonsense readthrough	3,190	2,758
Emflaza	5,823	2,519
Other research and preclinical	15,173	10,982
Total research and development	$ 63,970	$ 58,198

We discontinued our clinical studies for nonsense mutation cystic fibrosis (nmCF) and nonsense mutation mucopolysaccharidosis type I (nmMPS I) in 2017 and we expect the research and development costs for those programs to continue to decline as we complete the wind down of those programs.

The successful development of Translarna and our other product candidates is highly uncertain. This is due to the numerous risks and uncertainties associated with developing drugs, including the uncertainty of:

- the scope, rate of progress and expense of our clinical trials and other research and development activities;
- the potential benefits of our products and product candidates over other therapies;
- our ability to market, commercialize and achieve market acceptance for any of our products or product candidates that we are developing or may develop in the future, including our ability to negotiate pricing and reimbursement terms acceptable to us and to obtain or maintain marketing authorizations we have or may receive for our products and product candidates;
- clinical trial results;
- the terms and timing of regulatory approvals; and
- the expense of filing, prosecuting, defending and enforcing patent claims and other intellectual property rights.

A change in the outcome of any of these variables with respect to the development of our products or product candidates could mean a significant change in the costs and timing associated with the development of that product or product candidate. For example, if the EMA or FDA or other regulatory authority were to require us to conduct clinical trials beyond those which we currently anticipate will be required for the completion of clinical development of Translarna or any other product candidate or if we experience significant delays in enrollment in any of our clinical trials, we could be required to expend significant additional financial resources and time on the completion of clinical development.

Selling, general and administrative expense

Selling, general and administrative expenses consist primarily of salaries and other related costs for personnel, including share-based compensation expenses, in our executive, legal, business development, finance, accounting, information technology and human resource functions. Other selling, general and administrative expenses include facility-related costs not otherwise included in research and development expense; advertising and promotional expenses; costs associated with industry and trade shows; and professional fees for legal services, including patent-related expenses, accounting services and miscellaneous selling costs.

We expect that selling, general and administrative expenses will increase in future periods in connection with our continued efforts to commercialize Emflaza in the United States, our continued efforts to commercialize Translarna for the treatment of nmDMD in territories outside the United States, and to support our operations as a combined company assuming successful completion of the planned acquisition of Agilis, including increased payroll, expanded infrastructure, commercial operations, increased consulting, legal, accounting and investor relations expenses.

Interest (expense) income, net

Interest (expense) income, net consists of interest income earned on investments and interest expense from the Convertible Notes outstanding and interest expense from the Credit Agreement.

Critical accounting policies and significant judgments and estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our financial statements, which we have prepared in accordance with generally accepted accounting principles in the United States. The preparation of

these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported revenues and expenses during the reporting periods. Actual results may differ from these estimates under different assumptions or conditions.

During the three and six months ended June 30, 2018, there were no material changes to our critical accounting policies as reported in our Annual Report on Form 10-K for the year ended December 31, 2017, which was filed with the Securities and Exchange Commission, or SEC, on March 6, 2018, or the 2017 Annual Report on Form 10-K, other than those disclosed below.

Revenue recognition

Periods prior to January 1, 2018

We recognize 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

Prior to the second quarter of 2017, our net product sales consisted of sales of Translarna for the treatment of nmDMD in territories outside of the U.S. We recognize 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 we have no further performance obligations in accordance with Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC") Subtopic 605-15, Revenue Recognition—Products.

We have 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 our third-party partner distributors. Revenue is recognized when risk of ownership has transferred. Our third-party partner distributors act as intermediaries between us 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.

In May 2017, we began the commercialization of Emflaza in the U.S. We recorded product revenue related to the sales of Emflaza in the U.S. in accordance with ASC 605-15, when persuasive evidence of an arrangement exists, delivery has occurred and title of the product and associated risk of loss has passed to the customer, the price is fixed or determinable and collection from the customer has been reasonably assured. Due to the early stage of the product launch, we determined that we were not able to reliably make certain estimates, including returns, necessary to recognize product revenue upon shipment to distributors. As a result, we recorded net product revenue for Emflaza using a deferred revenue recognition model (sell-through). Under the deferred revenue model, we did not recognize revenue until Emflaza was shipped to the specialty pharmacy.

We record 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 us of one or more of the following: nonrefundable, upfront license fees; milestone payments; research funding and royalties on future product sales. In addition, we generate service revenue through agreements that generally provide for fees for research and development services and may include additional payments upon achievement of specified events.

We evaluate all contingent consideration earned, such as a milestone payment, using the criteria as provided by ASC 605-28, Revenue Recognition—Milestone Method. At the inception of a collaboration arrangement, we evaluate if a milestone payment is substantive. The criteria requires that (1) we determine 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. We recognize 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.

We recognize revenue for reimbursements of research and development costs under collaboration agreements as the services are performed. We record these reimbursements as revenue and not as a reduction of research and development expenses as we have the risks and rewards as the principal in the research and development activities.

Periods commencing January 1, 2018

Our net product revenue consists of sales of Translarna in territories outside of the U.S. and sales of Emflaza in the U.S., both for the treatment of DMD.

Net Product Revenue

We recognize revenue when performance obligations with customers have been satisfied. Our performance obligations are to provide Translarna or Emflaza based on customer orders from distributors, hospitals, specialty pharmacies or retail pharmacies. The performance obligations are satisfied at a point in time when our customer obtains control of either Translarna or Emflaza, which is typically upon delivery. We invoice customers after the products have been delivered and invoice payments are generally due within 30 to 90 days of invoice date. We determine the transaction price based on fixed consideration in its contractual agreements. Contract liabilities arise in certain circumstances when consideration is due for goods not yet provided. As we have identified only one distinct performance obligation, the transaction price is allocated entirely to either product sales of Translarna or Emflaza. In determining the transaction price, a significant financing component does not exist since the timing from when we deliver product to when the customers pay for the product is typically less than one year. Customers in certain countries pay in advance of product delivery. In those instances, payment and delivery typically occur in the same month.

We record product sales net of any variable consideration, which includes discounts, allowances, rebates and distribution fees. We use the expected value or most likely amount method when estimating variable consideration, unless discount or rebate terms are specified within contracts. Historically, returns of Translarna and Emflaza were immaterial to our financial statements. The identified variable consideration is recorded as a reduction of revenue at the time revenues from product sales are recognized. These estimates for variable consideration are adjusted to reflect known changes in factors and may impact such estimates in the quarter those changes are known. Revenue recognized does not include amounts of variable consideration that are constrained.

In relation to customer contracts, we incur costs to fulfill a contract but do not incur costs to obtain a contract. These costs to fulfill a contract do not meet the criteria for capitalization and are expensed as incurred.

Upon adoption of ASC Topic 606 on January 1, 2018, we have elected the following practical expedients:

- Portfolio Approach - We applied the Portfolio Approach to contract reviews within identified revenue streams that have similar characteristics and we believe this approach would not differ materially than if applying ASC Topic 606 to each individual contract.
- Significant Financing Component - We expect the period between when an we transfer a promised good or service to a customer and when the customer pays for the good or service to be one year or less.
- Immaterial Performance Obligations - We disregard promises deemed to be immaterial in the context of the contract.
- Shipping and Handling Activities - We consider any shipping and handling costs that are incurred after the customer has obtained control of the product as a cost to fulfill a promise.

Shipping and handling costs associated with finished goods delivered to customers are recorded as a selling expense.

Collaboration Revenue

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

At the inception of a collaboration arrangement, we need to first evaluate if the arrangement meets the criteria in ASC Topic 808 “Collaborative Arrangements” to then determine if ASC Topic 606 is applicable by considering whether the collaborator meets the definition of a customer. If the criteria are met, we assess the promises in the arrangement to identify distinct performance obligations.

For licenses of intellectual property, we assess, at contract inception, whether the intellectual property is distinct from other performance obligations identified in the arrangement. If the licensing of intellectual property is determined to be distinct, revenue is recognized for nonrefundable, upfront license fees when the license is transferred to the customer and the customer can use and benefit from the license. If the licensing of intellectual property is determined not to be distinct, then the license will be bundled with other promises in the arrangement into one distinct performance obligation. We determine if the bundled performance obligation is satisfied over time or at a point in time. If we conclude that the nonrefundable, upfront license fees will be recognized over time, we assess the appropriate method of measuring proportional performance.

For milestone payments, we assess, at contract inception, whether the development or sales-based milestones are considered probable of being achieved. If it is probable that a significant revenue reversal will occur, we will not record revenue until the uncertainty has been resolved. Milestone payments that are contingent upon regulatory approval are not considered probable of being achieved until the applicable regulatory approvals or other external conditions are obtained as such conditions are not within our control. If it is probable that a significant revenue reversal will not occur, we will estimate the milestone payments using the most likely amount method. We will re-assess the development and sales-based milestones each reporting period to determine the probability of achievement.

We recognize revenue for reimbursements of research and development costs under collaboration agreements as the services are performed. We record these reimbursements as revenue and not as a reduction of research and development expenses as we have the risks and rewards as the principal in the research and development activities.

Inventory and cost of product sales

Inventory

Inventories are stated at the lower of cost and net realizable value with cost determined on a first-in, first-out basis by product. We capitalize inventory costs associated with products following regulatory approval when future commercialization is considered probable and the future economic benefit is expected to be realized. Translarna and Emflaza product which may be used in clinical development programs are included in inventory and charged to research and development expense when the product enters the research and development process and no longer can be used for commercial purposes. Inventory used for marketing efforts are charged to selling, general and administrative expense.

We periodically review inventory for excess amounts or obsolescence and write down obsolete or otherwise unmarketable inventory to its estimated net realizable value. We have not recorded any inventory write downs as of the current period. Additionally, though our products are subject to strict quality control and monitoring which is performed throughout the manufacturing processes, certain batches or units of product may not meet quality specifications resulting in a charge to cost of product sales.

Cost of product sales

Cost of product sales consists of the cost of inventory sold, manufacturing and supply chain costs, storage costs, amortization of the acquired intangible asset and royalty payments associated with net product sales.

Accrued expenses

As part of the process of preparing our financial statements, we are required to estimate accrued expenses. This process involves communicating with our applicable personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual cost. The majority of our service providers invoice us monthly in arrears for services performed. We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on facts and circumstances known to us. Examples of estimated accrued expenses include:

- fees paid to contract research organizations in connection with preclinical and toxicology studies and clinical trials;
- fees paid to investigative sites in connection with clinical trials;
- fees paid to contract manufacturers in connection with the production of clinical trial materials; and
- professional service fees.

Results of operations

Three months ended June 30, 2018 compared to three months ended June 30, 2017

The following table summarizes revenues and selected expense and other income data for the three months ended June 30, 2018 and 2017.

(in thousands)	Three Months Ended June 30, 2018	2017	Change 2018 vs. 2017
Net product revenue	$ 68,170	$ 47,891	$ 20,279
Collaboration and grant revenue	573	71	502
Cost of product sales, excluding amortization of acquired intangible asset	2,572	758	1,814
Amortization of acquired intangible asset	5,593	—	5,593
Research and development expense	32,607	30,835	1,772
Selling, general and administrative expense	33,545	28,866	4,679
Interest expense, net	(2,884)	(3,008)	124
Other expense, net	(673)	(1,820)	1,147
Income tax expense	(389)	(150)	(239)

Net product revenues. Net product revenues were $68.2 million for the three months ended June 30, 2018, an increase of $20.3 million, or 42%, from $47.9 million for the three months ended June 30, 2017. The increase in net product revenue was primarily due to the increase in revenue in existing markets where Translarna is available as well as continued geographic expansion into new territories, in addition to revenue of Emflaza in the United States, which launched in May 2017.

Collaboration and grant revenues. Collaboration and grant revenues were $0.6 million for the three months ended June 30, 2018 and $0.1 million for the three months ended June 30, 2017. Revenues are primarily from ongoing collaboration arrangements.

Cost of product sales, excluding amortization of acquired intangible asset. Cost of product sales, excluding amortization of acquired intangible asset, were $2.6 million for the three months ended June 30, 2018 and $0.8 million for the three months ended June 30, 2017. Cost of product sales, excluding amortization of acquired intangible asset, consist primarily of royalty payments associated with Emflaza and Translarna net product sales and costs associated with Emflaza and Translarna product sold during the period. For Translarna sold in 2017, the majority of related manufacturing costs incurred had previously been expensed prior to January 1, 2017 as research and development expenses.

Amortization of acquired intangible asset. Amortization of the acquired intangible asset was $5.6 million for the three months ended June 30, 2018 resulting from the acquisition of all rights to Emflaza. The amount allocated to the Emflaza intangible asset will be amortized on a straight-line basis over its estimated useful life of approximately seven years from the date of the completion of our acquisition of all rights to Emflaza, the period of estimated future cash flows.

Research and development expense. Research and development expense was $32.6 million for the three months ended June 30, 2018, an increase of $1.8 million, or 6%, from $30.8 million for the three months ended June 30, 2017. The increase was primarily due to increased investment in research programs and the advancement of the clinical pipeline.

Selling, general and administrative expense. Selling, general and administrative expense was $33.5 million for the three months ended June 30, 2018, an increase of $4.7 million, or 16%, from $28.9 million for the three months ended June 30, 2017. The increase resulted primarily from the continued investment in commercial activities for Emflaza and Translarna.

Interest expense, net. Interest expense, net was $2.9 million for the three months ended June 30, 2018, a decrease of $0.1 million, or 4%, from $3.0 million for the three months ended June 30, 2017. The decrease in interest expense was primarily due to increased interest income from investments, which partially offset current year interest expense recorded from the Convertible Notes and the Credit Agreement.

Other expense, net. Other expense, net was $0.7 million for the three months ended June 30, 2018, a decrease in expense of $1.1 million, or 63%, from other expense, net of $1.8 million for the three months ended June 30, 2017. The decrease in other expense, net resulted primarily from exchange rate changes in the current period.

Income tax expense. Income tax expense was $0.4 million for the three months ended June 30, 2018 and $0.2 million for the three months ended June 30, 2017. We are subject to income taxes in the United States, although currently not a tax payer, and various foreign jurisdictions, and our foreign tax liabilities are largely dependent upon the distribution of pre-tax earnings among these different jurisdictions.

The income tax expense for the three months ended June 30, 2018 differed from the amounts computed by applying the U.S. federal income tax rate of 21% to loss before tax expense as a result of a favorable change in the jurisdictional mix of profits in jurisdictions which have lower tax rates, as well as by having a full valuation allowance in jurisdictions where we have net operating

losses. We review the expected annual effective income tax rate and make changes on a quarterly basis as necessary based on certain factors such as changes in forecasted annual operating income, changes to the actual and permanent book-to-tax differences, and changes resulting from the impact of tax law changes.

Six months ended June 30, 2018 compared to six months ended June 30, 2017

The following table summarizes revenues and selected expense and other income data for the six months ended June 30, 2018 and 2017.

(in thousands)	Six Months Ended June 30, 2018	Six Months Ended June 30, 2017	Change 2018 vs. 2017
Net product revenue	$ 124,151	$ 74,334	$ 49,817
Collaboration and grant revenue	654	176	478
Cost of product sales, excluding amortization of acquired intangible asset	5,616	797	4,819
Amortization of acquired intangible asset	11,022	—	11,022
Research and development expense	63,970	58,198	5,772
Selling, general and administrative expense	66,514	54,365	12,149
Interest expense, net	(6,187)	(5,227)	(960)
Other income (expense), net	332	(2,139)	2,471
Income tax expense	(610)	(316)	(294)

Net product revenues. Net product revenues were $124.2 million for the six months ended June 30, 2018, an increase of $49.8 million, or 67%, from $74.3 million for the six months ended June 30, 2017. The increase in net product revenue was primarily due to the increase in net product revenue in existing markets where Translarna is available as well as continued geographic expansion into new territories, in addition to net product revenue of Emflaza in the United States, which launched in May 2017.

Collaboration and grant revenues. Collaboration and grant revenues were $0.7 million for the six months ended June 30, 2018, an increase of $0.5 million, or 272%, from $0.2 million for the six months ended June 30, 2017. These revenues are primarily from ongoing collaboration arrangements.

Cost of product sales, excluding amortization of acquired intangible asset. Cost of product sales, excluding amortization of acquired intangible asset, were $5.6 million for the six months ended June 30, 2018 and $0.8 million for the six months ended June 30, 2017. Cost of product sales, excluding amortization of acquired intangible asset, consist primarily of royalty payments associated with Emflaza and Translarna net product sales and costs associated with Emflaza and Translarna product sold during the period. For Translarna sold in 2017, the majority of related manufacturing costs incurred had previously been expensed prior to January 1, 2017 as research and development expenses.

Amortization of acquired intangible asset. Amortization of the acquired intangible asset was $11.0 million for the six months ended June 30, 2018 resulting from the acquisition of Emflaza. The amount allocated to the Emflaza intangible asset will be amortized on a straight-line basis over its estimated useful life of approximately seven years from the date of the completion of our acquisition of all rights to Emflaza, the period of estimated future cash flows.

Research and development expense. Research and development expense was $64.0 million for the six months ended June 30, 2018, an increase of $5.8 million, or 10%, from $58.2 million for the six months ended June 30, 2017. The increase was primarily due to increased investment in research programs and the advancement of the clinical pipeline.

Selling, general and administrative expense. Selling, general and administrative expense was $66.5 million for the six months ended June 30, 2018, an increase of $12.1 million, or 22%, from $54.4 million for the six months ended June 30, 2017. The increase resulted primarily from the continued investment in commercial activities for Emflaza and Translarna.

Interest expense, net. Interest expense, net was $6.2 million for the six months ended June 30, 2018, an increase in expense of $1.0 million, or 18%, from interest expense of $5.2 million for the six months ended June 30, 2017. The increase in interest expense was primarily due to current year interest expense recorded from the Convertible Notes and the Credit Agreement, partially offset by interest income from investments.

Other income (expense), net. Other income, net was $0.3 million for the six months ended June 30, 2018, and other expense, net was $2.1 million for six months ended June 30, 2017. The change in other income (expense), net was primarily from foreign currency fluctuations in exchange rates in the current period.

Income tax expense. Income tax expense was $0.6 million for the six months ended June 30, 2018 and $0.3 million for the six months ended June 30, 2017. We are subject to income taxes in the United States, although currently not a tax payer, and various foreign jurisdictions, and our foreign tax liabilities are largely dependent upon the distribution of pre-tax earnings among these different jurisdictions.

The income tax expense for the six months ended June 30, 2018 differed from the amounts computed by applying the U.S. federal income tax rate of 21% to loss before tax expense as a result of a favorable change in the jurisdictional mix of profits in jurisdictions which have lower tax rates, as well as by having a full valuation allowance in jurisdictions where we have net operating losses. We review the expected annual effective income tax rate and make changes on a quarterly basis as necessary based on certain factors such as changes in forecasted annual operating income, changes to the actual and permanent book-to-tax differences, and changes resulting from the impact of tax law changes.

Liquidity and capital resources

Sources of liquidity

Since inception, we have incurred significant operating losses.

As a growing commercial-stage biopharmaceutical company, we are engaging in significant commercialization efforts for Translarna for nmDMD and Emflaza for the treatment of DMD while also devoting a substantial portion of our efforts on research and development programs related to Translarna and our other product candidates.To date, almost all of our product revenue has been attributable to sales of Translarna for the treatment of nmDMD in territories outside of the United States. Since May 2017, we have also begun to generate product revenue from Emflaza for the treatment of DMD in the United States. Our ongoing ability to generate revenue from sales of Translarna for the treatment of nmDMD is dependent upon our ability to maintain our marketing authorization in the EEA and secure market access through commercial programs following the conclusion of pricing and reimbursement terms at sustainable levels in the member states of the EEA or through EAP programs in the EEA and other territories. The marketing authorization requires annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of the authorization and is subject to the specific obligation to conduct Study 041. Our ability to generate product revenue from Emflaza will largely depend on the coverage and reimbursement levels set by governmental authorities, private health insurers and other third-party payors.

We have historically financed our operations primarily through the issuance and sale of our common stock in public offerings, the private placements of our preferred stock, collaborations, bank debt, convertible debt financings, the Credit Agreement and grants and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease areas addressed by our product candidates. Since 2014, we have also relied on revenues generated from net sales of Translarna for the treatment of nmDMD in territories outside of the United States, and in May 2017, we began to recognize revenue generated from net sales of Emflaza for the treatment of DMD in the United States. Based on our current commercial, research and development plans, we expect to continue to incur significant operating expenses for the foreseeable future, which we anticipate will be partially offset by revenues generated from the sale of both Translarna and Emflaza. As a result, while we expect to continue to generate operating losses in 2018, we anticipate that operating losses generated in future periods should decline versus prior periods. The net losses we incur may fluctuate significantly from quarter to quarter.

In August 2015, we closed a private offering of $150 million in aggregate principal amount of 3.00% convertible senior notes due 2022 including the exercise by the initial purchasers of an option to purchase an additional $25 million in aggregate principal amount of the Convertible Notes. The Convertible Notes bear cash interest payable on February 15 and August 15 of each year, beginning on February 15, 2016. The Convertible Notes are senior unsecured obligations of ours and will mature on August 15, 2022, unless earlier converted, redeemed or repurchased in accordance with their terms prior to such date. We received net proceeds from the offering of approximately $145.4 million, after deducting the initial purchasers' discounts and commissions and the estimated offering expenses payable by us.

On May 5, 2017, we entered into the Credit Agreement with MidCap Financial, which provides for a senior secured term loan facility of $60 million, of which $40 million was drawn by us on May 5, 2017. The remaining $20 million under the senior secured term loan facility would become available to us upon our demonstration (on or prior to December 31, 2018) of net product revenue equaling or exceeding $120 million for the trailing 12 month period. The maturity date of the Credit Agreement is May 1, 2021, unless terminated earlier. The facility is structured to require only monthly interest payments for the initial two years with principal amortization beginning in years three and four. The facility bears interest at a rate per annum equal to LIBOR (with a LIBOR floor rate of 1.00%) plus 6.15%, as well as additional upfront and administrative fees and expenses.

In April 2018, we closed an underwritten public offering of 4,600,000 shares of common stock under the registration statement at a public offering price of $27.04 per share, including 600,000 shares issued upon exercise by the underwriters of their option to purchase additional shares. We received net proceeds of approximately $117.9 million after deducting underwriting discounts and commissions and other offering expenses payable by us.

Cash flows

As of June 30, 2018, we had cash, cash equivalents and marketable securities of $296.1 million.

The following table provides information regarding our cash flows and our capital expenditures for the periods indicated.

	Six Months Ended June 30,	
(in thousands)	**2018**	**2017**
Cash provided by (used in):		
Operating activities	(15,875)	(15,582)
Investing activities	6,096	47,887
Financing activities	122,765	40,660

Net cash used in operating activities was $15.9 million for the six months ended June 30, 2018 and $15.6 million for the six months ended June 30, 2017. The net cash used in operating activities primarily relates to supporting clinical development and commercial activities, partially offset by increased cash receipts resulting from higher net product revenues.

Net cash provided by investing activities was $6.1 million for the six months ended June 30, 2018 and net cash provided by investing activities was $47.9 million for the six months ended June 30, 2017. Cash provided by investing activities for both periods was primarily related to the net redemption of marketable securities partially offset by purchases of fixed assets for the six months ended June 30, 2018 and acquisition costs associated with the Emflaza asset acquisition for the six months ended June 30, 2017.

Net cash provided by financing activities was $122.8 million for the six months ended June 30, 2018 and $40.7 million for the six months ended June 30, 2017. Cash provided by financing activities for the six months ended June 30, 2018 is primarily attributable to the April 2018 equity offering and the exercise of options and issuance of stock under the ESPP. Cash provided by financing activities for the six months ended June 30, 2017 is primarily attributable to borrowings under the Credit Agreement and the exercise of options and issuance of stock under the ESPP.

Funding requirements

We anticipate that our expenses will increase in connection with our commercialization efforts in the United States, the EEA and other territories, including expansion of our infrastructure and corresponding sales and marketing, legal and regulatory, distribution and manufacturing and administrative and employee-based expenses. In addition to the foregoing, we expect to continue to incur significant costs in connection with Study 041 and our open label extension trials of Translarna for the treatment of nmDMD as well as our studies for nonsense mutation aniridia and nonsense mutation Dravet syndrome/CDKL5 and our FDA post-marketing requirements with respect to Emflaza in the United States. We also expect to incur ongoing research and development expenses for our other product candidates, including our oncology program. In addition, we may incur substantial costs in connection with our efforts to advance our regulatory submissions. We have begun seeking and intend to continue to seek marketing authorization for Translarna for the treatment of nmDMD in territories outside of the EEA and we may also seek marketing authorization for Translarna for other indications. These efforts may significantly impact the timing and extent of our commercialization expenses.

In addition, our expenses will increase if and as we:

- complete our planned acquisition of Agilis, subject to satisfying closing conditions and obtaining applicable regulatory approvals, and seek to integrate Agilis's operations and employees into our business and seek to satisfy contractual and regulatory obligations following closing of the planned acquisition;
- seek to satisfy contractual and regulatory obligations in conjunction with the Akcea Agreement, including the potential commercialization of Tegsedi and Waylivra in the PTC Territory;
- execute our strategy for Emflaza in the United States, including commercialization and integration efforts;
- satisfy contractual and regulatory obligations that we assumed through the Emflaza acquisition;
- are required to complete any additional clinical trials, non-clinical studies or CMC assessments or analyses in order to advance Translarna for the treatment of nmDMD in the United States or elsewhere;
- are required to take other steps, in addition to Study 041, to maintain our current marketing authorization in the EEA for Translarna for the treatment of nmDMD or to obtain further marketing authorizations for Translarna for the treatment of nmDMD or other indications in the EEA or elsewhere;

- initiate or continue the research and development of Translarna for additional indications and of our other product candidates;
- seek to discover and develop additional product candidates;
- seek to expand and diversify our product pipeline through strategic transactions;
- maintain, expand and protect our intellectual property portfolio; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and commercialization efforts.

We believe that our cash flows from product sales, together with existing cash and cash equivalents, including the net proceeds from our term loan facility with MidCap Financial, our offering of the Convertible Notes, public offerings of common stock, marketable securities and research funding that we expect to receive under our collaborations, will be sufficient to fund our operating expenses and capital expenditure requirements for at least the next twelve months. We have based this estimate on assumptions that may prove to be wrong, and we could use our capital resources sooner than we currently expect.

Our future capital requirements will depend on many factors, including:

- our ability to commercialize and market Tegsedi and Waylivra in the PTC Territory;
- our ability to commercialize and market Emflaza for the treatment of DMD in the United States;
- our ability to negotiate, secure and maintain adequate pricing, coverage and reimbursement terms, on a timely basis, with third-party payors for Emflaza for the treatment of DMD in the United States and for Translarna for the treatment of nmDMD in the EEA and other territories outside of the United States;
- our ability to maintain orphan exclusivity for, and successfully complete all FDA post-marketing requirements with respect to, Emflaza, or to obtain an additional six-month period of pediatric exclusivity;
- our ability to maintain the marketing authorization in the EEA for Translarna for the treatment of nmDMD, including whether the EMA determines on an annual basis that the benefit-risk balance of Translarna supports renewal of our marketing authorization in the EEA, on the current approved label;
- the costs, timing and outcome of Study 041;
- the costs, timing and outcome of our efforts to advance Translarna for the treatment of nmDMD in the United States, including, whether we will be required to perform additional clinical trials, non-clinical studies or CMC assessments or analyses at significant cost which, if successful, may enable FDA review of an NDA submission by us and, ultimately, may support approval of Translarna for nmDMD in the United States;
- the progress and results of our open label extension clinical trials of Translarna for the treatment of nmDMD as well as our studies for nonsense mutation aniridia and nonsense mutation Dravet syndrome/CDKL5 and activities under our oncology program;
- the scope, costs and timing of our commercialization activities, including product sales, marketing, legal, regulatory, distribution and manufacturing, for both Emflaza for the treatment of DMD and Translarna for the treatment of nmDMD, for Tegsedi, for Waylivra and for any of our other product candidates that may receive marketing authorization or any additional indications or territories in which we receive authorization to market Translarna;
- the costs, timing and outcome of regulatory review of our other product candidates and Translarna in other territories or for indications other than nmDMD;
- our ability to satisfy our obligations under the terms of the Credit Agreement with MidCap Financial;
- the timing and scope of growth in our employee base;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for Translarna for additional indications and for our other product candidates;
- revenue received from commercial sales of Translarna, Emflaza, Tegsedi, Waylivra, or any of our other product candidates;
- our ability to obtain additional and maintain existing reimbursed named patient and cohort EAP programs for Translarna for the treatment of nmDMD on adequate terms, or at all;

- 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;

- the costs of preparing, filing and prosecuting patent applications, maintaining, and protecting our intellectual property rights and defending against intellectual property-related claims;

- the extent to which we acquire or invest in other businesses, products, product candidates, and technologies, including the success of any acquisition, in-licensing or other strategic transaction we may pursue, and the costs of subsequent development requirements and commercialization efforts, including with respect to our acquisition of Emflaza, our planned acquisition of Agilis, and our licensing of Tegsedi and Waylivra; and

- our ability to establish and maintain collaborations, including our collaborations with Roche and the SMA Foundation, and our ability to obtain research funding and achieve milestones under these agreements.

With respect to our outstanding Convertible Notes, cash interest payments are payable on a semi-annual basis in arrears, which will require total funding of $4.5 million annually. Furthermore, as a result of our initial public offering in June 2013, we have incurred and expect to continue to incur additional costs associated with operating as a public company. These costs include significant legal, accounting, investor relations and other expenses that we did not incur as a private company. Additionally, we could be forced to expend significant resources in the defense of the pending securities class action lawsuits brought against us and certain of our current and former executive officers, as described under Part II, Item 1. Legal Proceedings in this Quarterly Report on Form 10-Q.

We will need to generate significant revenues to achieve and sustain profitability, and we may never do so. We may need to obtain substantial additional funding in connection with our continuing operations. Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs primarily through a combination of equity offerings, debt financings, collaborations, strategic alliances, grants and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease areas addressed by our product and product candidates and marketing, distribution or licensing arrangements. Adequate additional financing may not be available to us on acceptable terms, or at all. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our shareholders ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our common stockholders. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us.

If we are unable to raise additional funds through equity or debt financings when needed or on attractive terms, we may be required to delay, limit, reduce or terminate our product development or commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined under Securities and Exchange Commission rules.

Contractual obligations

During the period ended June 30, 2018, there were no material changes to our contractual obligations and commitments outside the ordinary course of business from those disclosed under the heading “Management’s Discussion and Analysis of Financial Condition and Results of Operations-Contractual Obligations” in our Annual Report on Form 10-K for the year ended December 31, 2017.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

During the period ended June 30, 2018, there were no material changes in our market risk or how our market risk is managed, compared to those disclosed under the heading “Quantitative and Qualitative Disclosures about Market Risk” in our Annual Report on Form 10-K for the year ended December 31, 2017.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and our Principal Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of June 30, 2018. The term "disclosure controls and procedures", as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the Securities and Exchange Commission's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of June 30, 2018, our Chief Executive Officer and Principal Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

No change in our internal control over financial reporting occurred during the quarter ended June 30, 2018 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

In March 2016, three purported securities class action lawsuits were commenced in the United States District Court for the District of New Jersey (one each on March 3, 10, and 11), naming as defendants the Company, our Chief Executive Officer, and our former Chief Financial Officer. The lawsuits have been consolidated into one action captioned *In re PTC Therapeutics, Inc. Securities Litigation*, No. 16-1224 (KM) (the "Securities Class Action"). A consolidated amended complaint was filed on January 13, 2017. The complaint alleges violations of Sections 10(b) and 20(a) and Rule 10b-5 of the Securities Exchange Act of 1934 in connection with allegedly false and misleading statements made by the Company about its business, operations, and prospects as it relates to the NDA for Translarna for the treatment of nmDMD that the Company submitted to the FDA in December 2015. The plaintiffs seek, among other things, compensatory damages for purchasers of the Company's common stock between November 6, 2014 and February 23, 2016, as well as attorneys' fees and costs. On February 14, 2017, the defendants filed a motion to dismiss the consolidated amended complaint. On August 28, 2017, the motion to dismiss was granted in part and denied in part. On September 25, 2017, defendants filed an answer and affirmative defenses to the consolidated amended complaint. On January 10, 2018, the parties agreed to a settlement in principle of all legal claims, subject to court approval, which will be funded by the Company's insurance subject to the applicable deductible. The Court preliminarily approved the settlement on May 15, 2018.

On September 19, 2017, a purported stockholder of the Company filed a derivative lawsuit in the United States District Court for the District of New Jersey against our Chief Executive Officer, our former Chief Financial Officer, and current or former directors (Michael Schmertzler; Richard Aldrich; Allan Jacobson; Adam Koppel; Michael Kranda; C. Geoffrey McDonough; Ronald C. Renaud, Jr.; David P. Southwell; Jerome Zeldis; and Glenn D. Steele, Jr.), with the caption *Choi v. Peltz, et al.*, No. 17-cv-07216. The Company is named as a nominal defendant. On October 10, 2017, another purported stockholder of the Company filed a derivative lawsuit in the United States District Court for the District of New Jersey against the same defendants and nominal defendant, with the caption *Kim v. Peltz, et al.*, No. 17-cv-08062. On January 17, 2018, a third purported stockholder of the Company filed a derivative lawsuit in the United States District Court for the District of New Jersey against the same defendants and nominal defendant, with the caption *Lee v. Peltz, et al.*, No. 2:18-cv-00730. The *Choi, Kim* and *Lee* actions have been consolidated and captioned *In re PTC Therapeutics, Inc. Derivative Litigation*, No. 17-cv-07216 (the "Consolidated Derivative Action"). The Consolidated Derivative Action alleges violations of Section 14(a) of the Securities Exchange Act of 1934, breaches of defendants' fiduciary duties, unjust enrichment, abuse of control, and gross mismanagement based on allegations that defendants made or approved improper statements regarding the NDA for Translarna for the treatment of nmDMD that the Company submitted to the FDA in December 2015. The Consolidated Derivative Action seeks, among other things, any damages sustained by the Company as a result of the defendants' alleged wrongdoing (including fees associated with the Securities Class Action), an order directing the Company to take all necessary actions to reform and improve its corporate governance and internal procedures, restitution from the defendants, and attorneys' fees and costs. On February 12, 2018, the defendants moved to dismiss the Consolidated Derivative Action. On March 20, 2018, the parties agreed to a settlement in principle of all legal claims, comprising payment of plaintiffs' attorneys' fees and certain corporate governance reforms. The Court approved the settlement and dismissed the case on July 27, 2018.

Item 1A. Risk Factors

We have set forth in Item 1A to our Annual Report on Form 10-K for the year ended December 31, 2017, risk factors relating to our business, our industry, our structure and our common stock. Readers of this Quarterly Report on Form 10-Q are referred to such Item 1A for a more complete understanding of risks concerning us. There have been no material changes in our risk factors since those published in such Form 10-K for the year ended December 31, 2017, other than as reported in Item Part II Item 1A on our Form 10-Q for the period ended March 31, 2018, and as reported below.

Risks Related to Our Planned Acquisition of Agilis

Consummation of our planned acquisition of Agilis is subject to satisfaction of closing conditions, including antitrust approval, which, if delayed or not granted or granted with unacceptable conditions, may prevent, delay or impair the consummation of the transaction, result in additional expenditures of money and resources, subject us to business uncertainties that could adversely affect our business and operations, and/or delay, reduce or eliminate the anticipated benefits of the transaction.

On July 19, 2018, we announced that we have entered into a Merger Agreement with Agilis, providing for our acquisition of Agilis. Completion of the planned acquisition is subject to certain closing conditions, including, among others, the clearance of the transaction by certain governmental and regulatory authorities, including the expiration or termination of applicable waiting periods under the Hart-Scott-Rodino Antitrust Improvements Act. The governmental and regulatory agencies with which we and Agilis have made and will make required filings and seek certain approvals and consents have broad discretion in

administering applicable governing regulations. We can provide no assurance that all required approvals and consents will be obtained. Moreover, as a condition to their approval of the transaction, certain governmental agencies may impose requirements, limitations or costs or place restrictions on the conduct of our business after the closing of the transaction. Any one of these requirements, limitations, costs or restrictions could jeopardize or delay the effective time of the transaction or delay, reduce or eliminate the anticipated benefits of the transaction. Further, no assurance can be given that the required closing conditions will be satisfied and, if all required consents and approvals are obtained and the closing conditions are satisfied, no assurance can be given as to the terms, conditions and timing of the approvals or clearances. The occurrence of any of the foregoing could result in a failure to close the transaction and have a material adverse effect on our business, financial condition and results of operations.

In addition, while the transaction is pending, we will be subject to business uncertainties that could adversely affect our business and operations. These uncertainties may impair our ability to attract, retain and motivate key personnel until the transaction is consummated and for a period of time thereafter. We may also experience negative reactions from our stockholders, patients, the medical community, vendors, payors, and employees, among others. Further, if the transaction is not completed for any reason, the price of our common stock may decline to the extent that current market prices reflect a market assumption that the transaction will be completed and the perception of the effectiveness of our management and our company may suffer in the marketplace. In addition, some costs related to the transaction must be paid whether or not it is completed, including our transaction expenses and, under certain circumstances, we would be required to make an additional term loan advance of up to $10.0 million to Agilis under the Bridge Loan Agreement.

Even if we successfully consummate our planned acquisition of Agilis, we may fail to realize all of the anticipated benefits of the transaction, those benefits may take longer to realize than expected, or we may encounter significant integration difficulties.

Our ability to realize the anticipated benefits of the planned acquisition will depend, to a large extent, on our ability to integrate Agilis's operation and employees into our business, and any product candidates we may acquire from Agilis into our business strategy, and realize anticipated growth opportunities and synergies. We will be required to devote significant management attention and resources to integrating Agilis's operations and employees into our business and any product candidates we may acquire from Agilis into our business strategy. The process may be disruptive to our business and the expected benefits may not be achieved within the anticipated time frame, or at all. The failure to meet the challenges involved and to realize the anticipated benefits of the transaction could cause an interruption of, or a loss of momentum in, our development and commercialization efforts and could adversely affect our business, financial condition and results of operations.

Our ability to realize the anticipated benefits of the transaction is expected to entail numerous material potential difficulties, including, among others:

- the diversion of management attention to integration matters;
- difficulties in achieving anticipated business opportunities and growth prospects from the acquisition;
- challenges related to public and market perception of our acquisition of Agilis;
- difficulties in managing the expanded operations of a larger and more complex company following the acquisition;
- difficulties in assimilating employees and in attracting and retaining key personnel; and
- potential unknown liabilities, adverse consequences, unforeseen increased expenses or other unanticipated problems associated with the transaction.

Many of these factors are outside of our control, and any one of them could result in increased costs, decreased expected revenues and further diversion of management time and energy, which could materially impact our business, financial condition and results of operations.

All of these factors could decrease or delay the expected accretive effect of the transaction and negatively impact our stock price. As a result, it cannot be assured that the pending acquisition of Agilis will result in the full realization of the benefits anticipated from the transaction within the anticipated time frames or at all.

In addition, upfront consideration for the planned acquisition is comprised of approximately $50 million in cash and approximately $150 million in our common stock, subject to an estimated maximum of 9.34 million share limit (with any shortfall to be made whole with additional cash consideration). In addition, pursuant to the Merger Agreement, Agilis equityholders will be entitled to receive contingent payments from us based on (i) the achievement of certain development milestones up to an aggregate maximum amount of $60.0 million, (ii) the achievement of certain regulatory approval milestones together with a milestone payment following the receipt of a priority review voucher up to an aggregate maximum

amount of $535.0 million, (iii) the achievement of certain net sales milestones up to an aggregate maximum amount of $150.0 million, and (iv) a percentage of annual net sales for Friedreich ataxia and Angelman Syndrome during specified terms, ranging from 2-6%. Under the Merger Agreement, we are required to pay $40.0 million of the development milestone payments no later than the second anniversary of the closing of the acquisition, regardless of whether the applicable milestones have been achieved.

Following completion of the planned acquisition, we will become responsible for Agilis's liabilities and obligations, including with respect to financial, regulatory and compliance matters. These obligations will result in additional cost and investment by us and, if we have underestimated the amount of these costs and investments or if we fail to satisfy any such obligations, we may not realize the anticipated benefits of the transaction. Further, it is possible that there may be undisclosed, contingent or other liabilities or problems that may arise in the future, the existence and/or magnitude of which we were previously unaware. Any such liabilities or problems could have an adverse effect on our business, financial condition or results of operations.

The issuance of our common stock to complete this transaction will be dilutive to our existing stockholders and because we have limited financial resources, by investing in this transaction, we may forego or delay pursuit of other opportunities that may have proven to have greater commercial potential.

In addition, the Merger Agreement obligates us to register for resale the Closing Stock Consideration, and the sale or resale of these shares in the public market, or the market's expectation of such sales, may result in a decline in our stock price. Such a decline would adversely affect our investors and also might make it difficult for us to sell equity securities in the future at a time and at a price that we deem appropriate.

Risks Related to Our Collaboration and Licensing Agreement with Akcea

If Tegsedi or Waylivra fail to obtain or maintain regulatory approval, we will not be able to commercialize them in the PTC Territory.

Tegsedi has only received marketing authorization from the EMA, and Waylivra has not yet received regulatory approval from the FDA, EMA or any other regulatory agency. Should Tegsedi or Waylivra not receive marketing authorization in the territories in which we have obtained the rights to commercialize such product candidates, or should Tegsedi lose its marketing authorization from the EMA, we would not be able to commercialize Tegsedi or Waylivra, as applicable, successfully, and we would fail to realize the anticipated benefit of our licensing rights to Tegsedi and WAYLIVERA or those benefits may take longer to realize than expected, any of which could have a material adverse effect on our business.

If we or our partners fail to compete effectively, Tegsedi or Waylivra will not contribute significant revenue.

Our competitors engage in drug discovery throughout the world, are numerous and include, among others, major pharmaceutical companies and specialized biopharmaceutical firms. Our competitors may succeed in developing drugs that are:

- safer than our drugs;
- more effective than our drugs;
- priced lower than our drugs;
- reimbursed more favorably by government and other third-party payors than our drugs; or
- more convenient to use than our drugs.

These competitive developments could make Tegsedi or Waylivra obsolete or non-competitive. Further, Tegsedi and Waylivra are delivered by injection, which may render them less attractive to patients than non-injectable products offered by our current or future competitors.

There are several pharmaceutical and biotechnology companies engaged in the development or commercialization of products against targets that are also targets of Tegsedi and Waylivra. For example, if approved, Waylivra could face competition from drugs like metreleptin. Metreleptin, produced by Novelion Therapeutics, Inc., is currently approved for use in generalized lipodystrophy patients. Additionally, Tegsedi could face competition from drugs like patisiran and ALN-TTRsc02 in development by Alnylam, tafamidis commercialized in some countries in LATAM by Pfizer and tolcapone in development by SOM Biotech. If Tegsedi or Waylivra cannot compete effectively with these and other products with common or similar indications, we may not be able to generate substantial revenue from our product sales.

We may fail to realize all of the anticipated benefits of the Akcea Agreement or those benefits may take longer to realize than expected.

Our ability to realize the anticipated benefits of the Akcea Agreement is subject to general business risk, including risks related to:

- serious adverse or undesirable side effects or other unexpected characteristics that may be identified with respect to Tegsedi or Waylivra;
- the rate and degree of market acceptance and clinical utility of Tegsedi and Waylivra;
- our sales, marketing and distribution capabilities;
- doing business internationally;
- pricing regulations and reimbursement practices in various jurisdictions;
- potential product liability lawsuits;
- our limited resources and opportunity costs;
- our intellectual property;
- our dependence on third-parties; and
- legislative and regulatory changes in the pharmaceutical industry or healthcare systems in various jurisdictions.

In addition, our ability to realize the anticipated benefits of the Akcea Agreement is subject to additional risks and potential difficulties similar to the risks with respect to Translarna, Emflaza and our other products and product candidates and other risks relating to our business as set forth in our Form 10-K for the year ended December 31, 2017 and our Form 10-Q for the period ended March 31, 2018.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.

Recent Sales of Unregistered Securities

Inducement grant awards. Pursuant to the Nasdaq inducement grant exception, during the quarter ended June 30, 2018, we issued i) options to purchase an aggregate of 306,600 shares of common stock to certain new hire employees at a weighted-average exercise price of $32.04 per share, and ii) restricted stock units for 7,000 shares of our common stock to certain new hire employees. The shares underlying these option and restricted stock awards will be registered on a Form S-8 registration statement prior to the first vesting event applicable to each such award.

Item 6. Exhibits.

Exhibit Number	Description of Exhibit
31.1	Certification of Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2	Certification of Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1	Certification of Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
32.2	Certification of Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.INS	XBRL Instance Document*
101.SCH	XBRL Taxonomy Extension Schema Document*
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document*
101.LAB	XBRL Taxonomy Extension Label Linkbase Database*
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document*
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document*

* Submitted electronically herewith.

In accordance with SEC Release 33-8238, Exhibits 32.1 and 32.2 are being furnished and not filed.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

PTC THERAPEUTICS, INC.

Date: August 7, 2018

By: /s/ Christine Utter

Christine Utter

Principal Financial Officer

(Principal Financial and Accounting Officer and Duly Authorized Signatory)

EXHIBIT INDEX

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* Submitted electronically herewith.

In accordance with SEC Release 33-8238, Exhibits 32.1 and 32.2 are being furnished and not filed.

Exhibit 31.1

CERTIFICATIONS

I, Stuart W. Peltz, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of PTC Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 7, 2018

By: /s/ STUART W. PELTZ

Stuart W. Peltz

Chief Executive Officer

(Principal Executive Officer)

Exhibit 31.2

CERTIFICATIONS

I, Christine Utter, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of PTC Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 7, 2018

By: /s/ CHRISTINE UTTER

Christine Utter

Principal Financial Officer

(Principal Financial and Accounting Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report on Form 10-Q of PTC Therapeutics, Inc. (the "Company") for the period ended June 30, 2018 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Stuart W. Peltz, Chief Executive Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: August 7, 2018

By: /s/ STUART W. PELTZ

Stuart W. Peltz
Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report on Form 10-Q of PTC Therapeutics, Inc. (the "Company") for the period ended June 30, 2018 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Christine Utter, Principal Financial Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: August 7, 2018

By: /s/ CHRISTINE UTTER

Christine Utter
Principal Financial Officer
(Principal Financial and Accounting Officer)