PTC THERAPEUTICS, INC.
Form 10-Q
May 02, 2019
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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended March 31, 2019

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 No.)
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 þ No

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted 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 such files). Yes þ 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 þ	Accelerated filer
Non-accelerated filer	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 þ

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.001 par value per share	PTCT	Nasdaq Global Select Market

As of April 29, 2019, there were 58,431,129 shares of Common Stock, $0.001 par value per share, outstanding.

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FORWARD-LOOKING STATEMENTS

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

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

- Our ability to realize the anticipated benefits of our acquisition of Agilis Biotherapeutics, Inc., or Agilis, including the possibility that the expected impact of benefits from the acquisition, including with respect to the business of Agilis and our expectations with respect to the potential achievement of development, regulatory and sales milestones and our contingent payments to the former Agilis equityholders with respect thereto, will not be realized or will not be realized within the expected time period, significant transaction costs, the integration of Agilis's operations and employees into our business, our ability to obtain marketing approval of our gene therapy for the treatment of Aromatic L-Amino Acid Decarboxylase, or AADC, deficiency, or PTC-AADC, and other product candidates we acquired from Agilis, unknown liabilities, the risk of litigation and/or regulatory actions related to the acquisition, and other business effects, including the effects of industry, market, economic, political or regulatory conditions;
- 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;
- our ability to complete the United States Food and Drug Administration, or FDA, post-marketing requirements to the marketing authorization of Emflaza;
- 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 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 in which we are licensed to commercialize them, the potential commercialization of Tegsedi and Waylivra, and 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;

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- our estimates regarding the potential market opportunity for Translarna, Emflaza, PTC-AADC, Tegsedi, Waylivra, risdiplam 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, and Emflaza for the treatment of limb-girdle 2I, as well as studies in our gene therapy, splicing and oncology programs, 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, PTC-AADC, Tegsedi, Waylivra and risdiplam;
- 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, Tegsedi and our other product candidates;
- the ability of Translarna, Emflaza, PTC-AADC, Tegsedi, Waylivra and risdiplam 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 in non-ambulatory patients or otherwise;
- 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 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 increase our manufacturing capabilities for our gene therapy platform;
- 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 and Emflaza for additional indications;
- our ability to advance our earlier stage programs and pursue research and development of other product candidates, including our splicing, gene therapy and oncology programs;
- 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, PTC-AADC, Tegsedi, Waylivra and risdiplam and any other product candidate;
- our intellectual property position;
- the impact of government laws and regulations;

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- the impact of litigation that has been or may be brought against us 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, 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 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, 2018 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.

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PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

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

	March 31, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$201,144	$169,498
Marketable securities	206,018	58,088
Trade receivables, net	46,350	67,907
Inventory, net	16,219	16,117
Prepaid expenses and other current assets	9,348	9,247
Total current assets	479,079	320,857
Fixed assets, net	14,540	12,694
Intangible assets, net	694,955	701,031
Goodwill	82,341	82,341
Deposits and other assets	12,996	2,299
Total assets	$1,283,911	$1,119,222
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$98,450	$128,199
Current portion of long-term debt	16,667	11,667
Deferred revenue	5,497	3,716
Other current liabilities	3,132	3,814
Deferred consideration payable	19,300	19,400
Total current liabilities	143,046	166,796
Deferred revenue	8,853	9,722
Long-term debt	138,468	141,347
Contingent consideration payable	330,900	310,240
Deferred consideration payable	18,900	18,300
Deferred tax liability	122,032	122,032
Other long-term liabilities	8,770	58
Total liabilities	770,969	768,495
Stockholders' equity:		
Common stock, $0.001 par value. Authorized 125,000,000 shares; issued and outstanding 58,418,790 shares at March 31, 2019. Authorized 125,000,000 shares; issued and outstanding 50,606,147 shares at December 31, 2018.	58	51
Additional paid-in capital	1,523,115	1,288,137
Accumulated other comprehensive income	805	1,462
Accumulated deficit	(1,011,036)	(938,923)
Total stockholders' equity	512,942	350,727
Total liabilities and stockholders' equity	$1,283,911	$1,119,222

See accompanying unaudited notes.

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

	Three Months Ended March 31,	
	2019	2018
Revenues:		
Net product revenue	$53,054	$55,981
Collaboration and grant revenue	529	81
Total revenues	53,583	56,062
Operating expenses:		
Cost of product sales, excluding amortization of acquired intangible asset	2,376	3,045
Amortization of acquired intangible asset	6,077	5,428
Research and development	52,566	31,363
Selling, general and administrative	40,544	32,969
Change in the fair value of deferred and contingent consideration	21,160	—
Total operating expenses	122,723	72,805
Loss from operations	(69,140)	(16,743)
Interest expense, net	(2,288)	(3,303)
Other (expense) income, net	(109)	1,004
Loss before income tax expense	(71,537)	(19,042)
Income tax expense	(576)	(221)
Net loss attributable to common stockholders	$(72,113)	$(19,263)
Weighted-average shares outstanding:		
Basic and diluted (in shares)	55,855,111	41,626,617
Net loss per share—basic and diluted (in dollars per share)	$(1.29)	$(0.46)

See accompanying unaudited notes.

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

	Three Months Ended March 31,	
	2019	2018
Net loss	$(72,113)	$(19,263)
Other comprehensive loss:		
Unrealized gain (loss) on marketable securities	59	(123)
Foreign currency translation (loss) gain	(716)	1,107
Comprehensive loss	$(72,770)	$(18,279)

See accompanying unaudited notes.

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PTC Therapeutics, Inc.
Consolidated Statements of Stockholder's Equity (unaudited)
In thousands, except shares

	Common stock		Additional paid-in capital	Accumulated other comprehensive (loss) income	Accumulated deficit	Total stockholders' equity
	Shares	Amount				
Balance, December 31, 2018	50,606,147	$ 51	$1,288,137	$ 1,462	$(938,923)	$ 350,727
Issuance of common stock related to equity offering	7,563,725	7	224,434	—	—	224,441
Exercise of options	80,826	—	1,281	—	—	1,281
Restricted stock vesting and issuance	168,092	—	—	—	—	—
Share-based compensation expense	—	—	9,263	—	—	9,263
Net loss	—	—	—	—	(72,113)	(72,113)
Comprehensive loss	—	—	—	(657)	—	(657)
Balance, March 31, 2019	58,418,790	$ 58	$1,523,115	$ 805	$(1,011,036)	$ 512,942

	Common stock		Additional paid-in capital	Accumulated other comprehensive (loss) income	Accumulated deficit	Total stockholders' equity
	Shares	Amount				
Balance, December 31, 2017	41,612,395	$ 42	$966,534	$ 3,969	$(814,108)	$ 156,437
Adjustment to accumulated deficit	—	—	—	—	3,266	3,266
Exercise of options	77,312	—	1,136	—	—	1,136
Restricted stock vesting and issuance	119,691	—	—	—	—	—
Share-based compensation expense	—	—	7,748	—	—	7,748
Net loss	—	—	—	—	(19,263)	(19,263)
Comprehensive income	—	—	—	984	—	984
Balance, March 31, 2018	41,809,398	$ 42	$975,418	$ 4,953	$(830,105)	$ 150,308

See accompanying unaudited notes.

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

	Three Months Ended March 31,	
	2019	2018
Cash flows from operating activities		
Net loss	$(72,113)	$(19,263)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	7,059	6,022
Change in valuation of deferred and contingent consideration	21,160	—
Non-cash interest expense	1,982	1,780
Amortization of (discounts) premiums on investments, net	(257)	(96)
Amortization of debt issuance costs	139	126
Share-based compensation expense	9,263	7,748
Unrealized foreign currency transaction losses (gains), net	865	(1,300)
Changes in operating assets and liabilities:		
Inventory	(334)	(1,446)
Prepaid expenses and other current assets	(191)	958
Trade receivables, net	20,786	(4,223)
Deposits and other assets	(10,754)	(308)
Accounts payable and accrued expenses	(28,653)	(6,810)
Other liabilities	8,065	(475)
Deferred revenue	574	1,409
Net cash used in operating activities	(42,409)	(15,878)
Cash flows from investing activities		
Purchases of fixed assets	(2,865)	(479)
Purchases of marketable securities	(165,723)	(22,683)
Sale and redemption of marketable securities	18,090	21,514
Net cash used in investing activities	(150,498)	(1,648)
Cash flows from financing activities		
Proceeds from exercise of options	1,281	1,136
Net proceeds from public offerings	224,441	—
Net cash provided by financing activities	225,722	1,136
Effect of exchange rate changes on cash	(1,169)	2,273
Net increase in cash and cash equivalents	31,646	(14,117)
Cash and cash equivalents, beginning of period	169,498	111,792
Cash and cash equivalents, end of period	$201,144	$97,675
Supplemental disclosure of cash information		
Cash paid for interest	$3,111	$3,023
Cash paid for income taxes	$537	$326
Supplemental disclosure of non-cash investing and financing activity		
Change in unrealized gain (loss) on marketable securities, net of tax	$59	$(123)
Right-of-use assets obtained in exchange for lease obligations	$11,314	$—

See accompanying unaudited notes.

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PTC Therapeutics, Inc.
Notes to Consolidated Financial Statements (unaudited)
March 31, 2019
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's ability to globally 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 the 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 has a pipeline of gene therapy product candidates, including PTC-AADC for the treatment of Aromatic L-Amino Acid Decarboxylase, or AADC, deficiency, or AADC deficiency. The Company is preparing a biologics license application, or BLA, for PTC-AADC for the treatment of AADC deficiency in the United States, which it anticipates submitting to the U.S. Food and Drug Administration, or FDA, in late 2019, with anticipated commercial launch in the United States in 2020, subject to approval. The Company is also preparing a marketing authorization application, or MAA, for PTC-AADC for the treatment of AADC deficiency in the European Union, or EU, for submission to the European Medicines Agency, or EMA, which will follow its BLA submission to the FDA.

The Company holds the rights for the commercialization of Tegsedi™ (inotersen) and Waylivra™ (volanesorsen) for the treatment of rare diseases in countries in Latin America and the Caribbean. Tegsedi has received marketing authorization in the U.S., EU and Canada for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hATTR amyloidosis. The Company filed for marketing authorization with ANVISA, the Brazilian health regulatory authority, which granted priority review. It expects approval in Brazil by the end of 2019. Waylivra is currently under regulatory review in the EU for the treatment of familial chylomicronemia syndrome, or FCS. Waylivra has received a positive opinion recommending conditional marketing authorization from the Committee for Medicinal Products for Human Use, or CHMP, of the EMA. The positive opinion will be referred to the European Commission for consideration.

The Company also has a spinal muscular atrophy (SMA) collaboration with F. Hoffman-La Roche Ltd and Hoffman-La Roche Inc., referred to collectively as Roche, and the Spinal Muscular Atrophy Foundation, or SMA Foundation. Currently, its collaboration has two pivotal clinical trials ongoing to evaluate the safety and effectiveness of risdiplam (RG7916, RO7034067), the lead compound in the SMA program. Roche is preparing an NDA and a MAA for risdiplam for the treatment of SMA in the United States and the EU, respectively, which Roche anticipates submitting to the FDA and the EMA in the second half of 2019. In addition, the Company has a pipeline of product candidates and discovery programs that are in early clinical, pre-clinical and research and development stages focused on the development of new treatments for multiple therapeutic areas, including rare diseases and oncology.

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 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. 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. Due to enrollment at a slower pace in certain

countries than initially expected, in its February 2019 marketing authorization renewal request, the Company asked the EMA to extend the timeframe for submission of the results of Study 041 to the EMA to the end of the third quarter of 2022. 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.

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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 March 31, 2019, Translarna was available in over 40 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 2019 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 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, using newer technologies via procedures and methods that the Company designed, such dystrophin data in a new study, Study 045, which the Company initiated in the fourth quarter of 2018. The Company expects that a potential re-submission of an NDA could occur in 2020. 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.

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 is entitled to receive contingent payments from the Company based on annual net sales of Emflaza, 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.

On August 23, 2018, the Company completed its acquisition of Agilis Biotherapeutics, Inc., or Agilis, pursuant to an Agreement and Plan of Merger, dated as of July 19, 2018 (the "Merger Agreement"), by and among the Company, Agility Merger Sub, Inc., a Delaware corporation and the Company's wholly owned, indirect subsidiary, Agilis and, solely in its capacity as the representative, agent and attorney-in-fact of the equityholders of Agilis, Shareholder Representative Services LLC (the "Merger").

Upon the closing of the Merger, the Company paid to Agilis equityholders total upfront consideration comprised of $49.2 million in cash and 3,500,907 shares of the Company's common stock (the "Closing Stock Consideration"). The Closing Stock Consideration was determined by dividing $150.0 million by the volume-weighted average price per share of the Company's common stock on the Nasdaq Global Select Market for the 10 consecutive trading-day period ending on the second trading-day immediately preceding the closing of the Merger. Agilis equityholders may become

entitled to receive contingent payments from the Company based on the achievement of certain development, regulatory and net sales milestones as well as based upon a percentage of net sales of certain products. 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.

As of March 31, 2019, the Company had an accumulated deficit of approximately $1,011.0 million. The Company has financed its operations to date primarily through the private offering in August 2015 of 3.0% convertible senior notes due 2022 (see Note 10), public offerings of common stock in February 2014, October 2014, April 2018 and January 2019, 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 since May 2017, the

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Company has generated revenue from net sales of Emflaza for the treatment of DMD in the United States. The Company expects that 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, 2018 included in the Company's Annual Report on Form 10-K filed with the Securities and Exchange Commission (the "SEC") on March 1, 2019 (the "2018 Form 10-K"). Additional significant accounting policies adopted during the three month period ended March 31, 2019 are discussed in further detail below.

Basis of presentation

The accompanying financial information as of March 31, 2019 and for the three months ended March 31, 2019 and 2018 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, 2018 and notes thereto included in the 2018 Form 10-K.

In the opinion of management, the unaudited financial information as of March 31, 2019 and for the three months ended March 31, 2019 and 2018 reflects all adjustments, which are normal recurring adjustments, necessary to present a fair statement of financial position, results of operations, stockholder's equity, and cash flows. The results of operations for the three month period ended March 31, 2019 are not necessarily indicative of the results to be expected for the year ended December 31, 2019 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, fair value of the contingent consideration, 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:

	March 31, 2019	December 31, 2018
Raw materials	$1,333	$ 1,431
Work in progress	8,378	9,324
Finished goods	6,508	5,362
Total inventory	$16,219	$ 16,117

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. No write downs were recorded for the three

month periods ended March 31, 2019 and 2018. 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. For the three month periods ended March 31, 2019 and 2018, these amounts were immaterial.

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Cost of product sales

Costs of product sales consists of the cost of inventory sold, manufacturing and supply chain costs, including personnel 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.

Net product revenue

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

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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 $0.3 million as of March 31, 2019 and $0.7 million as of December 31, 2018. Bad debt expense was immaterial for the three month periods ended March 31, 2019 and 2018.

Indefinite-lived intangible assets

Indefinite-lived intangible assets consist of in-process research and development (IPR&D). IPR&D acquired directly in a transaction other than a business combination is capitalized if the projects will be further developed or have an alternative future use; otherwise they are expensed. The fair values of IPR&D projects acquired in business combinations are capitalized. Several methods may be used to determine the estimated fair value of the IPR&D acquired in a business combination. The Company utilizes the "income method”, and uses estimated future net cash flows that are derived from projected sales revenues and estimated costs. These projections are based on factors such as relevant market size, patent protection, and expected pricing and industry trends. The estimated future net cash flows are then discounted to the present value using an appropriate discount rate. These assets are treated as indefinite-lived intangible assets until completion or abandonment of the projects, at which time the assets are amortized over the remaining useful life or written off, as appropriate. IPR&D intangible assets that are determined to have had a drop in their fair value are adjusted downward and an impairment is recognized in the statement of operations. These assets are tested at least annually or sooner when a triggering event occurs that could indicate a potential impairment.

Goodwill

Goodwill represents the amount of consideration paid in excess of the fair value of net assets acquired as a result of the Company’s business acquisitions accounted for using the acquisition method of accounting. Goodwill is not amortized and is subject to impairment testing on an annual basis or when a triggering event occurs that may indicate the carrying value of the goodwill is impaired.

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

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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 March 31, 2019.

Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases and net operating loss and credit carryforwards. Deferred tax assets and liabilities are measured at rates expected to apply to taxable income in the years in which those temporary differences and carryforwards are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in the statement of operations in the period that includes the enactment date. A valuation allowance is recorded when it is not more likely than not that all or a portion of the net deferred tax assets will be realized.

The Company recorded a deferred tax liability in conjunction with the Merger of $122.0 million related to the tax basis difference in the IPR&D indefinite-lived intangibles acquired. The Company's policy is to record a deferred tax liability related to acquired IPR&D which may eventually be realized either upon amortization of the asset when the research is completed and a product is successfully launched or the write-off of the asset if it is abandoned or unsuccessful.

Leases

In February 2016, the FASB issued ASU No. 2016-2, “Leases (Topic 842)” along with other amendments issued in 2017 and 2018. Topic 842 supersedes the lease accounting requirements in Accounting Standards Codification Topic 840, Leases (Topic 840). Topic 842 requires organizations to recognize leased assets and liabilities on the balance sheet. The standard also requires disclosures to help investors and other financial statement users better understand the amount, timing and uncertainty of cash flows arising from leases.

The Company determines if an arrangement is a lease at inception. This determination generally depends on whether the arrangement conveys to the Company the right to control the use of an explicitly or implicitly identified fixed asset for a period of time in exchange for consideration. Control of an underlying asset is conveyed to the Company if the Company obtains the rights to direct the use of and to obtain substantially all of the economic benefits from using the underlying asset. The Company has lease agreements which include lease and non-lease components, which the Company accounts for as a single lease component for all leases.

Under the standard, operating leases are classified as right of use ("ROU") assets, short term lease liabilities, and long term lease liabilities. Operating lease ROU assets and lease liabilities are recognized at the commencement date based on the present value of lease payments over the lease term. ROU assets are amortized and lease liabilities accrete to yield straight-line expense over the term of the lease. Lease payments included in the measurement of the lease liability are comprised of fixed payments.

Variable lease payments associated with the Company’s leases are recognized when the event, activity, or circumstance in the lease agreement on which those payments are assessed occurs. Variable lease payments are presented in the Company’s consolidated statements of operations in the same line item as expense arising from fixed lease payments for operating leases.

Leases with an initial term of 12 months or less are not recorded on the consolidated balance sheet and the Company recognizes lease expense for these leases on a straight-line basis over the lease term. The Company applies this policy to all underlying asset categories.

Topic 842 requires a lessee to discount its unpaid lease payments using the interest rate implicit in the lease or, if that rate cannot be readily determined, its incremental borrowing rate. As most of the Company’s leases do not provide an implicit rate, the Company uses its incremental borrowing rate based on the information available at the commencement date in determining the present value of lease payments. The Company gives consideration to its recent debt issuances as well as publicly available data for instruments with similar characteristics when calculating its incremental borrowing rates.

The lease term for all of the Company’s leases includes the non-cancellable period of the lease plus any additional periods covered by either a Company option to extend (or not to terminate) the lease that the Company is reasonably certain to exercise, or an option to extend (or not to terminate) the lease controlled by the lessor. Leasehold

improvements are capitalized and depreciated over the lesser of useful life or lease term.

See the "Impact of recently adopted accounting pronouncements" section within this Note below and Note 3 Leases for additional information. The information presented for periods prior to January 1, 2019 has not been adjusted and is reported under Topic 840.

Recently issued accounting standards

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

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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 August 2018, the FASB issued ASU 2018-13, "Fair Value Measurement (Topic 820), Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement". This standard eliminates certain disclosure requirements for fair value measurements for all entities, requires public entities to disclose certain new information and modifies some disclosure requirements. The new guidance is effective for all entities for fiscal years beginning after December 15, 2019 and for interim periods within those fiscal years. An entity is permitted to early adopt either the entire standard or only the provisions that eliminate or modify requirements. Entities can elect to early adopt in interim periods, including periods for which they have not yet issued financial statements or made their financial statements available for issuance. The Company expects to adopt this guidance when effective and is currently assessing what effect the adoption of ASU No. 2018-13 will have on its consolidated financial statements and accompanying notes.

In August 2018, the FASB issued ASU 2018-15,"Intangibles - Goodwill and Other - Internal-Use Software (Subtopic 350-40): Customer’s Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement That Is a Service Contract". ASU 2018-15 requires a customer in a cloud computing arrangement that is a service contract to follow the internal-use software guidance in Accounting Standards Codification 350-40 to determine which implementation costs to defer and recognize as an asset. For public business entities, the guidance is effective for annual periods, and interim periods within those annual periods, beginning after December 15, 2019. For all other entities, it is effective for annual periods beginning after December 15, 2020 and interim periods in annual periods beginning after December 15, 2021. Early adoption is permitted, including adoption in any interim period for all entities. The Company expects to adopt this guidance when effective and is currently assessing what effect the adoption of ASU No. 2018-13 will have on its consolidated financial statements and accompanying notes.

In November 2018, the FASB issued ASU 2018-18,"Collaborative Arrangements (Topic 808): Clarifying the Interaction between Topic 808 and Topic 606”. ASU 2018-18 provides guidance on whether certain transactions between collaborative arrangement participants should be accounted for with revenue under Topic 606. For public business entities, the guidance is effective for annual periods, and interim periods within those annual periods, beginning after December 15, 2019. For all other entities, it is effective for annual periods beginning after December 15, 2020 and interim periods in annual periods beginning after December 15, 2021. Early adoption is permitted, including adoption in any interim period for all entities. The Company expects to adopt this guidance when effective and is currently assessing what effect the adoption of ASU No. 2018-18 will have on its consolidated financial statements and accompanying notes.

Impact of recently adopted accounting pronouncements

In February 2016, the FASB issued ASU No. 2016-2, “Leases (Topic 842)”. This standard requires 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 also requires 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. Additionally, in March 2019, the FASB issued ASU 2019-01,"Leases (Topic 842): Codification Improvements”. ASU 2019-01 clarifies the transition guidance related to interim disclosures provided in the year of adoption. The Company adopted the new guidance on January 1, 2019 using the modified retrospective method. Prior period results were not adjusted and continue to be presented under Topic 840 based on the accounting standards originally in effect for such periods. As part of the adoption, the Company has elected to utilize practical expedients including the package of practical expedients permitted under the transition guidance within the new standard, which among other things, allowed the Company to: 1) carry forward the historical determination of contracts as leases, lease classification and not reassess initial direct costs for historical lease arrangements, 2) not separate non-lease components from lease components and instead to

account for each separate lease component and the non-lease components associated with that lease component as a single lease component (the Company elected to apply this practical expedient to all underlying asset classes), 3) not apply the recognition requirements in ASC 842 to short-term leases, and 4) not record a right of use asset or right of use liability for leases with an asset or liability balance that would be considered immaterial. Upon adoption, the Company recorded an operating lease liability with a corresponding operating lease ROU asset of $11.3 million. The adoption did not have a material impact on the consolidated results of operations, stockholder's equity, and cash flows for the three-months ended March 31, 2019. As the Company is not a lessor, the aspects of the new guidance pertaining to lessors was not applicable for the Company.

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

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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 adopted this guidance on January 1, 2019 and elected not to reclassify the tax effects in other comprehensive income related to the Act, as these amounts were immaterial. The adoption of the guidance did not have a material impact on the 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 adopted this guidance on January 1, 2019. The adoption of the guidance did not have a material impact on the consolidated financial statements and accompanying notes.

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.

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 the guidance on January 1, 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 on January 1, 2018. The adoption of the guidance did not have a material impact on the consolidated financial statements and accompanying notes.

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 the guidance on January 1, 2018. The adoption of the guidance did not have a material impact on the consolidated financial statements and accompanying notes.

In November 2016, the FASB issued ASU 2016-16, "Intra-Entity Transfers of Assets Other Than Inventory". ASU 2016-16 requires companies to account for the income tax effects of intercompany transfers of assets other than inventory (e.g., intangible assets) when the transfer occurs. The Company adopted the guidance on January 1, 2018. The adoption of the guidance did not have a material impact on the consolidated financial statements and accompanying notes.

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 the guidance on January 1, 2018. The adoption of the guidance did not have a material impact on the consolidated financial statements and accompanying notes.

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 the guidance on January 1, 2018. The adoption of the guidance did not have a material impact on the consolidated financial statements and accompanying notes.

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3. Leases

The Company leases office space in South Plainfield, New Jersey for its principal office under three noncancelable operating leases through May 2022 and August 2024, in addition to office space in various countries for international employees primarily through workspace providers. The Company also leases certain vehicles, lab equipment, and office equipment under operating leases. The Company's operating leases have remaining lease terms ranging from 0.7 years to 7.2 years and certain of the leases include renewal options to extend the lease for up to 10 years.

The components of lease expense were as follows:

	Three Months Ended March 31, 2019
Operating Lease Cost	
Fixed lease cost	$ 812
Variable lease cost	143
Short-term lease cost	53
Total operating lease cost	$ 1,008

Total operating lease cost is a component of operating expenses on the consolidated statements of operations.

Supplemental balance sheet information related to leases was as follows:

	March 31, 2019
Operating lease ROU asset	$10,700
Operating lease liabilities- current	$2,025
Operating lease liabilities- noncurrent	8,770
Total operating lease liability	$10,795

Operating lease ROU asset is a component of Deposits and Other Assets on the consolidated balance sheet. The current portion of operating lease liability is a component of other current liabilities on the consolidated balance sheet. The long term portion of operating lease liabilities is a component of other long term liabilities on the consolidated balance sheet.

Supplemental lease term and discount rate information related to leases was as follows:

	March 31, 2019
Weighted-average remaining lease terms - operating leases (years)	4.71
Weighted-average discount rate - operating leases	7.02%

Supplemental cash flow information related to leases was as follows:

	Three-Months Ended March 31, 2019
Cash paid for amounts included in the measurement of lease liabilities:	

Operating cash flows from operating leases	$ 732
Right-of-use assets obtained in exchange for lease obligations:	
Operating leases	$ 11,314

Future minimum lease payments under non-cancelable leases as of March 31, 2019 were as follows:

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	Operating Leases
2019 (Excludes the three-months ended March 31, 2019)	$ 2,130
2020	2,995
2021	2,478
2022	2,152
2023 and thereafter	2,166
Total lease payments	11,921
Less: Imputed Interest	1,126
Total	$ 10,795

As of March 31, 2019, the Company had no operating leases that had not yet commenced.

4. 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.

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The following represents the fair value using the hierarchy described above for the Company's financial assets and liabilities that are required to be measured at fair value on a recurring basis as of March 31, 2019 and December 31, 2018:

	March 31, 2019			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities	$206,018	$ —	$206,018	$ —
Stock appreciation rights liability	$1,034	$ —	$—	$ 1,034
Deferred consideration payable	$38,200	$ —	$38,200	$ —
Contingent consideration payable- development and regulatory milestones	$270,800	$ —	$—	$ 270,800
Contingent consideration payable- net sales milestones and royalties	$60,100	$ —	$—	$ 60,100

	December 31, 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	$58,088	$ —	$ 58,088	$ —
Stock appreciation rights liability	$3,814	$ —	$—	$ 3,814
Deferred consideration payable	$37,700	$ —	$ 37,700	$ —
Contingent consideration payable- development and regulatory milestones	$257,040	$ —	$—	$ 257,040
Contingent consideration payable- net sales milestones and royalties	$53,200	$ —	$—	$ 53,200

No transfers of assets between Level 1, Level 2, or Level 3 of the fair value measurement hierarchy occurred during the periods ended March 31, 2019 and December 31, 2018.

The following is a summary of marketable securities accounted for as available-for-sale securities at March 31, 2019 and December 31, 2018:

	March 31, 2019			
	Amortized Cost	Gross Unrealized Gains	Losses	Fair Value
Commercial paper	$78,032	$ 46	$ —	$78,078
Corporate debt securities	114,811	68	(46)	114,833
Asset-backed securities	13,104	4	(1)	13,107
Total	$205,947	$ 118	$ (47)	$206,018

	December 31, 2018			
	Amortized Cost	Gross Unrealized Gains	Losses	Fair Value
Commercial paper	$31,657	$ 43	$ (1)	$31,699
Corporate debt securities	26,399	—	(10)	26,389
Total	$58,056	$ 43	$ (11)	$58,088

At March 31, 2019 and December 31, 2018, 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 March 31, 2019 and December 31, 2018, the Company did not have any realized gains/losses from the sale of marketable securities.

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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 March 31, 2019 are as follows:

	March 31, 2019					
	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	$—	$—	$ —	$ —	$—	$—
Corporate debt securities	(46)	61,716	—	—	(46)	61,716
Asset-backed securities	(1)	6,077	—	—	(1)	6,077
Total	$(47)	$67,793	$ —	$ —	$(47)	$67,793

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, 2018 are as follows:

	December 31, 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)	$1,993	$—	$—	$(1)	$1,993
Corporate debt securities	(7)	14,230	(3)	10,087	(10)	24,317
Total	$(8)	$16,223	$(3)	$10,087	$(11)	$26,310

Marketable securities on the balance sheet at March 31, 2019 and December 31, 2018 mature as follows:

	March 31, 2019	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$78,078	$ —
Corporate debt securities	55,621	59,212
Asset-backed securities	11,176	1,931
Total Marketable securities	$144,875	$ 61,143

	December 31, 2018	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 31,699	$ —
Corporate debt securities	26,389	—
Total Marketable securities	$ 58,088	$ —

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 10. 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 March 31, 2019 and December 31, 2018 was $154.3 million and $146.6 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 10) 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.

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Deferred consideration payable

Pursuant to the Merger Agreement, Agilis equityholders may become entitled to receive contingent consideration payments from the Company based on the achievement of certain development milestones up to an aggregate maximum amount of $60.0 million and 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. The Company is required to pay $40.0 million of development milestone payments no later than the second anniversary of the closing of the Merger, regardless of whether the applicable milestones have been achieved. The fair value of the deferred consideration payable at March 31, 2019 was estimated to be $38.2 million by applying a discounted cash flow approach. The discount rates are estimated utilizing Corporate B rated bonds maturing in the years of expected payments based on the Company's estimated development timelines for the acquired product candidates. As of March 31, 2019, $19.3 million of the deferred consideration payable was classified as current on the balance sheet.

Level 3 valuation

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 contingent consideration payable is fair valued each reporting period with the change in fair value recorded as a gain or loss in the consolidated statements of operations. The fair value of the development and regulatory milestones is estimated utilizing a probability adjusted, discounted cash flow approach. The discount rates are estimated utilizing Corporate B rated bonds maturing in the years of expected payments based on the Company's estimated development timelines for the acquired product candidate. The fair value of the net sales milestones and royalties is determined utilizing an option pricing model with Monte Carlo simulation to simulate a range of possible payment scenarios, and the average of the payments in these scenarios is then discounted to calculate present fair value.

The table presented below is a summary of changes in the fair value of the Company's Level 3 valuations for the SARs liability, and the contingent consideration payable for the period ended March 31, 2019. The changes in the fair value of the Company's Level 3 valuations for the period ended March 31, 2018 were immaterial.

	Level 3 liabilities		
	SARs	Contingent consideration payable- development and regulatory milestones	Contingent consideration payable- net sales milestones and royalties
Beginning balance as of December 31, 2018	$3,814	$ 257,040	$ 53,200
Additions	—	—	—
Change in fair value	1,035	13,760	6,900
Payments	(3,815)	$ —	$ —
Ending balance as of March 31, 2019	$1,034	$ 270,800	$ 60,100

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The following significant unobservable inputs were used in the valuation of the SARs liability, and the contingent consideration payable for the periods ended March 31, 2019 and December 31, 2018:

	March 31, 2019			
	Fair Value	Valuation Technique	Unobservable Input	Range
SARs	$1,034	Option-pricing model	Volatility	57.34%
			Risk free interest rate	2.42%
			Strike price	$6.76 - $30.86
			Fair value of common stock	$37.64
			Expected life	0.76 years
Contingent consideration payable-development and regulatory milestones	$270,800	Probability-adjusted discounted cash flow	Potential development and regulatory milestones	$0 - $555 million
			Probabilities of success	25% - 94%
			Discount rates	4.3% - 6.4%
			Projected years of payments	2020 - 2026
Contingent considerable payable-net sales milestones and royalties	$60,100	Option-pricing model with Monte Carlo simulation	Potential net sales milestones	$0 - $150 million
			Probabilities of success	25% - 89%
			Potential percentage of net sales for royalties	2% - 6%
			Discount rate	13.5%
			Projected years of payments	2021 - 2038

	December 31, 2018			
	Fair Value	Valuation Technique	Unobservable Input	Range
SARs	$3,814	Option-pricing model	Volatility	46.53% - 59.59%
			Risk free interest rate	2.44% - 2.63%
			Strike price	$6.76 - $30.86
			Fair value of common stock	$34.32
			Expected life	0.01 - 1.01 years
Contingent consideration payable-development and regulatory milestones	$257,040	Probability-adjusted discounted cash flow	Potential development and regulatory milestones	$0 - $555 million
			Probabilities of success	25% - 94%
			Discount rates	5.8% - 8.0%
			Projected years of payments	2020 - 2026
Contingent considerable payable-net sales milestones and royalties	$53,200	Option-pricing model with Monte Carlo simulation	Potential net sales milestones	$0 - $150 million

Probabilities of success	25% - 89%
Potential percentage of net sales for royalties	2% - 6%
Discount rate	14.0%
Projected years of payments	2021 - 2038

The contingent consideration is classified as a Level 3 liability as its valuation requires substantial judgment and estimation of factors that are not currently observable in the market. If different assumptions were used for the various inputs to the valuation approach, including but not limited to, assumptions involving probability adjusted sales estimates for the Agilis platform and estimated discount rates, the estimated fair value could be significantly higher or lower than the fair value determined.

5. Other comprehensive income (loss) and accumulated other comprehensive items

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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 months ended March 31, 2019:

	Unrealized Gains/(Losses) On Marketable Securities, net of tax	Foreign Currency Translation	Total Accumulated Other Comprehensive Items
Balance at December 31, 2018	$ 31	$ 1,431	$ 1,462
Other comprehensive income (loss) before reclassifications	59	(716)	(657)
Amounts reclassified from other comprehensive items	—	—	—
Other comprehensive income (loss)	59	(716)	(657)
Balance at March 31, 2019	$ 90	$ 715	$ 805

6. Accounts payable and accrued expenses

Accounts payable and accrued expenses at March 31, 2019 and December 31, 2018 consist of the following:

	March 31, 2019	December 31, 2018
Employee compensation, benefits, and related accruals	$ 13,277	$ 27,629
Consulting and contracted research	15,021	11,267
Professional fees	4,631	5,574
Sales allowance and other costs	31,666	29,417
Sales rebates and royalties	20,047	31,874
Accounts payable	9,786	6,001
Other	4,022	16,437
	$ 98,450	$ 128,199

7. Capitalization

In January 2019, 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 7,563,725 shares of common stock under the registration statement at a public offering price of $30.20 per share, including 843,725 shares issued upon exercise by the underwriter of its option to purchase additional shares in February 2019. The Company received net proceeds of $224.4 million after deducting underwriting discounts and commissions and other offering expenses payable by the Company.

Warrants

All of the Company’s outstanding warrants were classified as liabilities as of March 31, 2019 and December 31, 2018 because they contained non-standard antidilution provisions. The fair value of the warrants as of March 31, 2019 and December 31, 2018 was immaterial.

The following is a summary of the Company’s outstanding warrants as of March 31, 2019 and December 31, 2018:

	Warrant shares	Exercise price	Expiration
Common stock	7,030	$128.00	September 2019
Common stock	130	$2,520.00	August 2019

8. 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.

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The following tables set forth the computation of basic and diluted net loss per share:

	Three Months Ended March 31,	
	2019	2018
Numerator		
Net loss	$(72,113)	$(19,263)
Denominator		
Denominator for basic and diluted net loss per share	55,855,111	41,626,617
Net loss per share:		
Basic and diluted	$(1.29)*	$(0.46)*

*In the three months ended March 31, 2019 and 2018, 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 March 31,	
	2019	2018
Stock Options	10,811,383	8,176,777
Unvested restricted stock awards and units	695,339	615,375
Total	11,506,722	8,792,152

9. 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, canceled, 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 March 31, 2019, awards for 323,395 shares of common stock are available for issuance.

From January 1, 2019 through March 31, 2019, the Company issued a total of 2,490,970 stock options to various employees. Of those, 292,550 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.

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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, 2018	8,534,358	$ 28.58		
Granted	2,490,970	$ 33.01		
Exercised	(80,826)	$ 15.85		
Forfeited/Cancelled	(133,119)	$ 29.10		
Outstanding at March 31, 2019	10,811,383	$ 29.67	7.72 years	$ 117,201
Vested or Expected to vest at March 31, 2019	5,364,300	$ 28.40	9.15 years	$ 53,343
Exercisable at March 31, 2019	4,987,460	$ 31.00	6.04 years	$ 60,063

The fair value of grants made in the three months ended March 31, 2019 was contemporaneously estimated on the date of grant using the following assumptions:

	Three months ended March 31, 2019
Risk-free interest rate	2.53 - 2.63%
Expected volatility	62.62 - 63.01%
Expected term	6.11 years

The Company assumed no expected dividends for all grants. The weighted average grant date fair value of options granted during the three-month period ended March 31, 2019 was $19.72 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, 2019	571,479	$ 17.61
Granted	304,549	$ 32.94
Vested	(161,077)	$ 17.60

Forfeited	(19,612)	$ 20.42
Unvested at March 31, 2019	695,339	$ 24.27

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

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the SARs at each reporting period until vesting occurs. For the period ended March 31, 2019, a total of 157,930 SARs vested. For the period ended March 31, 2019, the Company recorded $1.0 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 March 31, 2019, the Company recorded $0.3 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 March 31,	
	2019	2018
Research and development	$4,686	$3,747
Selling, general and administrative	4,577	4,001
Total	$9,263	$7,748

As of March 31, 2019, there was approximately $100.0 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 3.24 years.

10. 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 Company's ability to draw on the remaining $20.0 million under the senior secured term loan facility expired on December 31, 2018. 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 March 31, 2019, 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").

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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 was not permitted to redeem the Convertible Notes prior to August 20, 2018. As of August 20, 2018, the Company may redeem for cash all or any portion of the Convertible Notes, at its option, 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. There have been no redemptions to date.

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.

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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	March 31, 2019	December 31, 2018
Principal	$150,000	$150,000
Less: Debt issuance costs	(1,648)	(1,746)
Less: Debt discount, net(1)	(33,072)	(35,054)
Net carrying amount	$115,280	$113,200

(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 $154.3 million as of March 31, 2019. The Company estimates the fair value of its Convertible Notes utilizing market quotations for debt that have quoted prices in active markets. As of March 31, 2019, the remaining contractual life of the Convertible Notes is approximately 3.4 years.

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

	Three Months Ended March 31,	
	2019	2018
Contractual interest expense	$1,110	$1,110
Amortization of debt issuance costs	99	89
Amortization of debt discount	1,982	1,780
Total	$3,191	$2,979
Effective interest rate of the liability component	11 %	11 %

11. 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.

Pursuant to the Merger Agreement with Agilis, Agilis equityholders may become entitled to receive contingent consideration payments from the Company based on (i) the achievement of certain development milestones up to an aggregate maximum amount

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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%. 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.

The Company also has a Collaboration and License Agreement with Akcea Therapeutics, Inc. ("Akcea") for the commercialization of Tegsedi and Waylivra, and products containing those compounds in countries in Latin America and the Caribbean ("the Akcea Collaboration and License Agreement"). Pursuant to the agreement, the Company paid Akcea an upfront licensing fee, which included an initial payment of $12.0 million. An additional $6.0 million is payable within 30 days after receipt of regulatory approval of Waylivra from the FDA or the 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, subject to a maximum aggregate amount of $8.0 million for all such products. Akcea is also entitled to receive royalty payments subject to certain terms set forth in the Akcea Collaboration and License Agreement. The Company filed a request for marketing authorizations for Tegsedi with ANVISA. Waylivra is currently under regulatory review in the EU.

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.

From time to time in the ordinary course of its business, the Company is subject to claims, legal proceedings and disputes, including as a result of patients seeking to participate in the Company's clinical trials or otherwise gain access to its product candidates. The Company is not currently aware of any material legal proceedings against it.

12. Revenue recognition

Net product sales

The Company views its operations and manages its business in one operating segment. During the three month periods ended March 31, 2019 and 2018, net product sales in the United States were $17.8 million and $19.2 million respectively, consisting solely of Emflaza, and net product sales not in the United States were $35.3 million and $36.8 million, respectively, consisting solely of Translarna.

The following table presents changes in the Company’s contract liabilities from December 31, 2018 to March 31, 2019 and December 31, 2017 to March 31, 2018:

	Balance as of December 31, 2018	Additions	Deductions	ASC 606 Adjustment	Balance as of March 31, 2019
Deferred Revenue	$ 12,938	$ 1,412	$ —	$ —	$ 14,350
	Balance as of December 31, 2017	Additions	Deductions	ASC 606 Adjustment	Balance as of March 31, 2018
Deferred Revenue	$ 11,891	$ 1,346	$ —	$ (3,937)	$ 9,300

The Company did not have any contract assets for the three month periods ended March 31, 2019 and 2018.

During the three month periods ended March 31, 2019 and 2018, the Company recognized revenue in the period from:

	Three Months Ended March 31, 2019	Three Months Ended March 31, 2018

Amounts included in contract liabilities at the beginning of the period	$—	$—
Performance obligations satisfied in previous period	—	—
Performance obligations satisfied in current period	53,054	55,981
Total product revenue	$53,054	$55,981

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The Company has not made significant changes to the judgments made in applying ASC Topic 606 for the three month periods ended March 31, 2019 and 2018.

Remaining performance obligations

Remaining performance obligations represent the transaction price for goods the Company has yet to provide. As of March 31, 2019 and 2018, the aggregate amount of transaction price allocated to remaining performance obligations relating to Translarna net product revenue was $14.4 million and $9.3 million, respectively. The Company expects to recognize revenue over the next one to three years as the specific timing for satisfying the performance obligations is contingent upon a number of factors, including customers' needs and schedules.

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 specified sales events, and up to double digit royalties on worldwide annual net sales of a commercial product.

In August 2013, a lead development compound, RG7800, was selected to move into IND-enabling studies, which triggered a milestone payment to the Company from Roche of $10.0 million. 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, 2013.

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.

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The remaining potential research and development event milestones that can be received as of March 31, 2019 is $87.5 million. The remaining potential sales milestones as of March 31, 2019 is $325.0 million upon achievement of certain sales events. In addition, the Company is eligible to receive up to double digit royalties on worldwide annual net sales of a commercial product.

For the three months ended March 31, 2019 and 2018, 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 three months ended March 31, 2019 and 2018, 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 three months ended March 31, 2019 and 2018, the Company did not recognize any revenue related to early stage collaborations.

13. Intangible assets and goodwill

Definite-lived intangibles

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 and recorded an intangible asset of $148.4 million.

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 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. For the three month periods ended March 31, 2019 and 2018, no milestone payments were recorded.

For the three month periods ended March 31, 2019 and 2018, the Company recognized amortization expense of $6.1 million and $5.4 million, respectively, related to the Emflaza rights intangible asset. The estimated future amortization of the Emflaza rights intangible asset is expected to be as follows:

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	As of March 31, 2019
2019	$ 18,207
2020	24,277
2021	24,277
2022	24,276
2023 and thereafter	27,418
Total	$ 118,455

Indefinite-lived intangibles

In connection with the acquisition of Agilis, the Company acquired rights to PTC-AADC, for the treatment of AADC deficiency. AADC deficiency is a rare CNS disorder arising from reductions in the enzyme AADC that result from mutations in the dopa decarboxylase gene. The Agilis platform also includes a gene therapy asset targeting Friedreich ataxia, a rare and life-shortening neurodegenerative disease caused by a single defect in the FXN gene which causes reduced production of the frataxin protein. An investigational new drug ("IND") submission with the FDA for this program is expected in late 2019. Additionally, the Agilis platform includes two other gene therapy programs targeting CNS disorders, including Angelman syndrome, a rare, genetic, neurological disorder characterized by severe developmental delays.

In accordance with the acquisition method of accounting, the Company allocated the acquisition cost for the Merger to the underlying assets acquired and liabilities assumed, based upon the estimated fair values of those assets and liabilities at the date of acquisition. The Company classified the fair value of the acquired IPR&D as indefinite lived intangible assets until the successful completion or abandonment of the associated research and development efforts. The value allocated to the indefinite lived intangible assets was $576.5 million.

Goodwill

As a result of the Merger on August 23, 2018, the Company recorded $82.3 million of goodwill. There were no changes to the recorded value of goodwill for the three month period ended March 31, 2019.

14. Subsequent events

The Company has evaluated all subsequent events and transactions through the filing date. There were no material events that impacted the unaudited consolidated financial statements or disclosures.

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, 2018 included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 1, 2019, or our 2018 Annual Report. 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 and Part I, Item 1A. (Risk Factors) of our 2018 Annual Report, and 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. Our 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.

Corporate Updates
DMD Franchise

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We have 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. During the quarter ended March 31, 2019, we recognized $35.3 million in net sales of Translarna. Translarna is currently available for the treatment of nmDMD in over 40 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 March 31, 2019, Emflaza achieved net sales of $17.8 million.

Our 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 we refer to as the annual EMA reassessment. In July 2018, the European Commission renewed our marketing authorization, making it effective, unless extended, through August 5, 2019. In February 2019, we submitted a marketing authorization renewal request to the EMA. This marketing authorization is further subject to a specific obligation to conduct and submit the results of an 18-month, placebo-controlled trial, followed by an 18-month open-label extension, which we refer to together as Study 041. The final report on the trial and open-label extension is to be submitted by us to the EMA by the end of the third quarter of 2021. Due to enrollment at a slower pace in certain countries than initially expected, in our February 2019 marketing authorization renewal request, we asked the EMA to extend the timeframe for submission of the results of Study 041 to the EMA to the end of the third quarter of 2022.

In April 2019 we received approval for marketing authorization for Translarna in Brazil from ANVISA, the Brazilian health regulatory authority, for the treatment of nmDMD in ambulatory patients aged five years and older.

Each country, including each member state of the EEA, has its own pricing and reimbursement regulations. 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 launch 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.

There is substantial risk that if we are unable to renew our EEA marketing authorization during any annual renewal cycle, or 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.

Translarna is an investigational new drug in the United States. 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 United States Food and Drug Administration, or 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 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. We intend to follow the FDA's recommendation and will collect, using newer technologies via procedures and methods that we designed, such dystrophin data in a new study, Study 045, which we initiated in the fourth quarter of 2018. We expect that a potential re-submission of an NDA could occur in 2020. 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.

There is substantial risk that Study 045, or any other studies we may use to collect the dystrophin data, will not provide the necessary data to support a marketing approval for Translarna for the treatment of nmDMD in the U.S.

Emflaza is approved in the United States for the treatment of DMD in patients five years and older. In recent interactions with the FDA, we were invited to submit a supplementary NDA, or sNDA, for Emflaza for patients two to five years of age on the basis that existing data support its safety and efficacy in this population. We recently submitted the sNDA for potential approval in 2019, recently received an approval action date of July 4, 2019, and, upon approval, now expect to launch Emflaza in this younger population before the end of 2019.

Gene Therapy Platform

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We have a pipeline of gene therapy product candidates for rare monogenic diseases that affect the central nervous system, or CNS, including PTC-AADC for the treatment of Aromatic L-Amino Acid Decarboxylase, or AADC, deficiency, or AADC deficiency, a rare CNS disorder arising from reductions in the enzyme AADC that result from mutations in the dopa decarboxylase gene. We are preparing a biologics license application, or BLA, for PTC-AADC for the treatment of AADC deficiency in the United States, which we anticipate submitting to the FDA in late 2019, with an anticipated commercial launch in the United States in 2020. We are also preparing a marketing authorization application, or MAA, for PTC-AADC for the treatment of AADC deficiency in the European Union, or EU, for submission to the EMA, which will follow our BLA submission to the FDA.

Akcea Partnership

We hold the rights for the commercialization of Tegsedi™ (inotersen) and Waylivra™ (volanesorsen) for the treatment of rare diseases in countries in Latin America and the Caribbean, or the PTC Territory, pursuant to our collaboration and license agreement with Akcea Therapeutics, Inc, or the Akcea Agreement.

Tegsedi has received marketing authorization in the U.S., EU and Canada for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hATTR amyloidosis. We filed for marketing authorization with ANVISA, the Brazilian health regulatory authority, which granted priority review. We expect approval in Brazil by the end of 2019.

Waylivra is currently under regulatory review in the EU for the treatment of familial chylomicronemia syndrome, or FCS. Waylivra has received a positive opinion recommending conditional marketing authorization from the Committee for Medicinal Products for Human Use, or CHMP, of the EMA. The positive opinion will be referred to the European Commission for consideration.

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

Splicing Platform

We also have a spinal muscular atrophy, or SMA, collaboration 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 two pivotal clinical trials ongoing to evaluate the safety and effectiveness of risdiplam (RG7916, RO7034067), the lead compound in the SMA program. Roche is preparing an NDA and a MAA for risdiplam for the treatment of SMA in the United States and the EU, respectively, which Roche anticipates submitting to the FDA and the EMA in the second half of 2019.

Pre-clinical and other programs

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

In the first quarter of 2019, we initiated a Phase 1 dose escalation study of PTC596, one of our oncology agents, for leiomyosarcoma, or LMS.

Funding

The success of our products, product candidates 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 3.0% convertible senior notes due August 15, 2022, or the Convertible Notes offering, our public offerings of common stock in February 2014, October 2014, April 2018, and January 2019, our initial public offering of common stock in June 2013, private placements of our preferred stock, collaborations, bank debt and convertible debt financings, 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, 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.

The Credit Agreement provides for a senior secured term loan facility of $60 million, of which $40 million was drawn by us on May 5, 2017 and remained outstanding as of March 31, 2019. Our ability to draw on the remaining $20 million under the senior secured term loan facility expired on December 31, 2018. The maturity date of the Credit Agreement is May 1, 2021, unless terminated earlier.

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In January 2019, 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 7,563,725 shares of common stock under the registration statement at a public offering price of $30.20 per share, including 843,725 shares issued upon exercise by the underwriter of its option to purchase additional shares in February 2019. We received net proceeds of approximately $224.4 million after deducting underwriting discounts and commissions and other offering expenses payable by us.

As of March 31, 2019, we had an accumulated deficit of $1,011.0 million. We had a net loss of $72.1 million and $19.3 million for the three month periods ended March 31, 2019 and 2018, respectively.

We anticipate that our expenses will continue to increase in connection with our commercialization efforts in the United States, the EEA, Latin America 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 Study 045 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 and studies for limb-girdle 2I. We also expect to incur ongoing research and development expenses for our other product candidates, including our gene therapy, splicing and oncology programs. 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. In late 2019, we plan to submit a request for marketing authorization for PTC-AADC with the FDA, followed by a request for marketing authorization for PTC-AADC with the EMA and we recently submitted a request for marketing authorization for Tegsedi with ANVISA. 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, 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 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.

We have never been profitable and 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.

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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 fluctuate in connection with our ongoing activities, particularly in connection with Study 041 and other studies for Translarna for the treatment of nmDMD, our studies of Translarna in nonsense mutation aniridia and nonsense mutation Dravet syndrome/CDKL5, our studies of Emflaza in limb-girdle 2I, activities under our gene therapy, splicing and oncology programs, 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 table provides research and development expense for our most advanced principal product development programs, for the three months ended March 31, 2019 and 2018.

	Three Months Ended March 31,	
	2019	2018
	(in thousands)	
Translarna (nmDMD, aniridia and Dravet)	$15,783	$18,323
Gene Therapy	11,241	—
Oncology	7,292	1,111
Emflaza	6,130	3,038
Other research and preclinical	12,120	8,891
Total research and development	$52,566	$31,363

The successful development of our products and 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 and maintain marketing authorizations we currently have or may receive in the future for our products and product candidates;

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·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, commercial, 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, our efforts to commercialize Waylivra and Tegsedi in Latin America and the Caribbean and to support our operations, 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 months ended March 31, 2019, there were no material changes to our critical accounting policies as reported in our 2018 Annual Report on Form 10-K, other than those disclosed below.

Revenue recognition

Net Product Revenue

Our net product revenue consists of sales of Translarna in territories outside of the U.S. for the treatment of nmDMD and sales of Emflaza in the U.S. for the treatment of DMD. 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 have been immaterial to our financial statements.

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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 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.

Leases

In February 2016, the FASB issued ASU No. 2016-2, “Leases (Topic 842)” along with other amendments issued in 2017 and 2018. Topic 842 supersedes the lease accounting requirements in Accounting Standards Codification Topic 840, Leases (Topic 840). Topic 842 requires organizations to recognize leased assets and liabilities on the balance sheet. The standard also requires disclosures to help investors and other financial statement users better understand the

amount, timing and uncertainty of cash flows arising from leases.

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We determine if an arrangement is a lease at inception. This determination generally depends on whether the arrangement conveys to us the right to control the use of an explicitly or implicitly identified fixed asset for a period of time in exchange for consideration. Control of an underlying asset is conveyed to us if we obtain the rights to direct the use of and to obtain substantially all of the economic benefits from using the underlying asset. We have lease agreements which include lease and non-lease components, which we account for as a single lease component for all leases.

Under the standard, operating leases are classified as right of use ("ROU") assets, short term lease liabilities, and long term lease liabilities. Operating lease ROU assets and lease liabilities are recognized at the commencement date based on the present value of lease payments over the lease term. ROU assets are amortized and lease liabilities accrete to yield straight-line expense over the term of the lease. Lease payments included in the measurement of the lease liability are comprised of fixed payments.

Variable lease payments associated with our leases are recognized when the event, activity, or circumstance in the lease agreement on which those payments are assessed occurs. Variable lease payments are presented in our consolidated statements of operations in the same line item as expense arising from fixed lease payments for operating leases.

Leases with an initial term of 12 months or less are not recorded on the consolidated balance sheet and we recognize lease expense for these leases on a straight-line basis over the lease term. We apply this policy to all underlying asset categories.

Topic 842 requires a lessee to discount its unpaid lease payments using the interest rate implicit in the lease or, if that rate cannot be readily determined, its incremental borrowing rate. As most of our leases do not provide an implicit rate, we use our incremental borrowing rate based on the information available at the commencement date in determining the present value of lease payments. We give consideration to our recent debt issuances as well as publicly available data for instruments with similar characteristics when calculating our incremental borrowing rates.

The lease term for all of our leases includes the non-cancellable period of the lease plus any additional periods covered by either our option to extend (or not to terminate) the lease that we are reasonably certain to exercise, or an option to extend (or not to terminate) the lease controlled by the lessor.

Results of operations

Three months ended March 31, 2019 compared to three months ended March 31, 2018

The following table summarizes revenues and selected expense and other income data for the three months ended March 31, 2019 and 2018.

	Three Months Ended March 31,		Change 2019 vs. 2018
(in thousands)	2019	2018	
Net product revenue	$53,054	$55,981	$(2,927)
Collaboration and grant revenue	529	81	448
Cost of product sales, excluding amortization of acquired intangible asset	2,376	3,045	(669)
Amortization of acquired intangible asset	6,077	5,428	649
Research and development expense	52,566	31,363	21,203
Selling, general and administrative expense	40,544	32,969	7,575
Change in the fair value of deferred and contingent consideration	21,160	—	21,160
Interest expense, net	(2,288)	(3,303)	1,015
Other (expense) income, net	(109)	1,004	(1,113)
Income tax expense	(576)	(221)	(355)

Net product revenues. Net product revenues were $53.1 million for the three months ended March 31, 2019, a decrease of $2.9 million, or 5%, from $56.0 million for the three months ended March 31, 2018. The decrease in net product revenue was primarily due to lumpiness in ordering patterns from Latin America for Translarna and first quarter dynamics including seasonality for Emflaza.

Collaboration and grant revenues. Collaboration and grant revenues were $0.5 million for the three months ended March 31, 2019, an increase of $0.4 million, or 553%, from $0.1 million for the three months ended March 31, 2018. The increase in revenues is related to our ongoing collaboration arrangements.

Cost of product sales, excluding amortization of acquired intangible asset. Cost of product sales, excluding amortization of acquired intangible asset, were $2.4 million for the three months ended March 31, 2019, a decrease of $0.7 million, or 22%, from $3.0

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million for the three months ended March 31, 2018. Cost of product sales consist primarily of royalty payments associated with Emflaza and Translarna net product sales, excluding contingent payments to Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC), or Marathon, and costs associated with Emflaza and Translarna product sold during the period.

Amortization of acquired intangible asset. Amortization of our acquired Emflaza intangible asset was $6.1 million for the three months ended March 31, 2019, an increase of $0.6 million, or 12%, from $5.4 million for the three months ended March 31, 2018. These amounts are related to the acquisition of all rights to Emflaza acquired in May 2017 and Marathon contingent payments. The amount allocated to the Emflaza intangible asset is amortized on a straight-line basis over its estimated useful life of approximately seven years from the date of the completion of the acquisition of all rights to Emflaza, the period of estimated future cash flows. The Marathon contingent payments are amortized prospectively as incurred, straight-line, over the remaining useful life of the Emflaza intangible asset.

Research and development expense. Research and development expense was $52.6 million for the three months ended March 31, 2019, an increase of $21.2 million, or 68%, from $31.4 million for the three months ended March 31, 2018. The increase reflects costs associated with advancing the gene therapy platform and increased investment in research programs as well as advancement of the clinical pipeline.

Selling, general and administrative expense. Selling, general and administrative expense was $40.5 million for the three months ended March 31, 2019, an increase of $7.6 million, or 23%, from $33.0 million for the three months ended March 31, 2018. The increase was primarily due to continued investment to support our commercial activities.

Change in the fair value of deferred and contingent consideration. Change in the fair value of deferred and contingent consideration was $21.2 million for the three months ended March 31, 2019, compared to $0.0 million for the three months ended March 31, 2018. The change is related to the fair valuation of the potential future consideration to be paid to former equity holders of Agilis Biotherapeutics, Inc., or Agilis, as a result of our merger with Agilis which closed in August 2018. Changes in the fair value were due to the re-calculation of discounted cash flows for the passage of time and changes to certain other estimated assumptions.

Interest expense, net. Interest expense, net was $2.3 million for the three months ended March 31, 2019, a decrease of $1.0 million, or 31%, from $3.3 million for the three months ended March 31, 2018. The decrease in interest expense, net 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) income, net. Other (expense) income, net was $0.1 million for the three months ended March 31, 2019, a decrease in income of $1.1 million, or 111%, from other income, net of $1.0 million for the three months ended March 31, 2018. The decrease in other income, net resulted primarily from exchange rate changes in the current period.

Income tax expense. Income tax expense was $0.6 million for the three months ended March 31, 2019 and $0.2 million for the three months ended March 31, 2018. We incurred income tax expense in various foreign jurisdictions, and our foreign tax liabilities are largely dependent upon the distribution of pre-tax earnings among these different jurisdictions. We are paying minimum income taxes in the United States because of incurred losses in the various state jurisdictions.

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 related to our products, product candidates and other programs. 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 and 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

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treatment of nmDMD in territories outside of the United States, and since May 2017, we have recognized 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 well as Waylivra and Tegsedi once our commercialization efforts of them commence. As a result, while we expect to continue to generate operating losses in 2019, 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 and remained outstanding as of March 31, 2019. Our ability to draw on the remaining $20 million under the senior secured term loan facility expired on December 31, 2018. 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 January 2019, 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 7,563,725 shares of common stock under the registration statement at a public offering price of $30.20 per share, including 843,725 shares issued upon exercise by the underwriter of its option to purchase additional shares in February 2019. We received net proceeds of approximately $224.4 million after deducting underwriting discounts and commissions and other offering expenses payable by us.

Cash flows

As of March 31, 2019, we had cash, cash equivalents and marketable securities of $407.2 million.

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

	Three Months Ended March 31,	
(in thousands)	2019	2018
Cash (used in) provided by:		
Operating activities	(42,409)	(15,878)
Investing activities	(150,498)	(1,648)
Financing activities	225,722	1,136

Net cash used in operating activities was $42.4 million for the three months ended March 31, 2019 and $15.9 million for the three months ended March 31, 2018. The net cash used in operating activities primarily relates to supporting clinical development and commercial activities.

Net cash used in investing activities was $150.5 million for the three months ended March 31, 2019 and $1.6 million for the three months ended March 31, 2018. Cash used in investing activities for the three months ended March 31, 2019 and the three months ended March 31, 2018 was primarily related to net purchases of marketable securities.

Net cash provided by financing activities was $225.7 million for the three months ended March 31, 2019 and $1.1 million for the three months ended March 31, 2018. Cash provided by financing activities for the three months ended March 31, 2019 was primarily attributable to our equity offering in January 2019 and the exercise of options. Cash

provided by financing activities for the three months ended March 31, 2018 was primarily attributable to exercise of options.

Funding requirements

We anticipate that our expenses will continue to increase in connection with our commercialization efforts in the United States, the EEA, Latin America 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,

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we expect to continue to incur significant costs in connection with Study 041 and Study 045 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 and our studies for limb-girdle 2I. We also expect to incur ongoing research and development expenses for our other product candidates, including our gene therapy, splicing and oncology programs. 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. In late 2019, we plan to submit a request for marketing authorization for PTC-AADC with the FDA, followed by a request for marketing authorization for PTC-AADC with the EMA and we recently submitted a request for marketing authorization for Tegsedi with ANVISA. These efforts may significantly impact the timing and extent of our commercialization expenses.

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

- seek to integrate Agilis's operations and employees into our business and seek to satisfy contractual and regulatory obligations we assumed in connection with the Agilis 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;
- initiate or continue the research and development of Translarna and Emflaza 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 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;
- 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;

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- 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;
- our ability to commercialize and market Tegsedi and Waylivra in the PTC Territory;
- 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, nonsense mutation Dravet syndrome/CDKL5 and limb-girdle 2I and activities under our gene therapy, splicing and oncology programs;
- 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, including those in our gene therapy and oncology programs, 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 and Emflaza for additional indications and for our other product candidates, including those in our gene therapy and oncology programs;
- 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 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 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.

We have never been profitable and 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

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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 March 31, 2019, 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 2018 Annual Report.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

During the period ended March 31, 2019, 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 2018 Annual Report.

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 March 31, 2019. 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 March 31, 2019, 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

In the first quarter of 2019, we implemented an enterprise resource planning (“ERP”) system, Oracle, on a worldwide basis, which is expected to improve the efficiency of certain financial and related transactional processes. We have completed the implementation of certain processes, including the financial consolidation and reporting, sales order to cash, fixed assets, supplier management and indirect procure-to-pay processes, and have revised and updated the related controls. These changes did not materially affect our internal control over financial reporting for the three months ended March 31, 2019. As we implement the remaining functionality under this ERP system over the next several years, we will continue to assess the impact on our internal control over financial reporting for the three months ended March 31, 2019. No other changes in our internal control over financial reporting occurred during the quarter ended March 31, 2019 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

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PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

From time to time in the ordinary course of our business, we are subject to claims, legal proceedings and disputes, including as a result of patients seeking to participate in our clinical trials or otherwise gain access to our product candidates. We are not currently aware of any material legal proceedings to which we are a party or of which any of our property is subject.

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, 2018, 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, 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 March 31, 2019, we issued options to purchase an aggregate of 292,550 shares of common stock to certain new hire employees at a weighted-average exercise price of $33.00 per share. The shares underlying these options will be registered on a Form S-8 registration statement prior to the first vesting event applicable to each such award.

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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.

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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: May 2, 2019 By: /s/ Christine Utter
Christine Utter
Principal Financial Officer
(Principal Financial and Accounting Officer and Duly Authorized Signatory)