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 f