PTC THERAPEUTICS, INC.
Form 10-Q
November 05, 2018
Table of Contents

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 September 30, 2018

OR

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

For the transition period from to

Commission file number: 001-35969

PTC Therapeutics, Inc.
(Exact name of registrant as specified in its charter)

Delaware	04-3416587
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification Number)

100 Corporate Court South Plainfield, NJ	07080
(Address of principal executive offices)	(Zip Code)

(908) 222-7000
(Registrant's telephone number, including area code)

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes 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	Accelerated filer þ
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

As of November 1, 2018, there were 50,454,834 shares of Common Stock, $0.001 par value per share, outstanding.

Table of Contents

TABLE OF CONTENTS
PTC Therapeutics, Inc.

Table of Contents

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 and through any grant of pediatric exclusivity;
- our ability to complete the United States Food and Drug Administration, or FDA, post-marketing requirements to the marketing authorization of Emflaza or any requirements necessary to obtain any grant of pediatric exclusivity;
- our expectations with respect to our acquisition of all rights to Emflaza from Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC), or Marathon, including with respect to our ability to realize the anticipated benefits of the acquisition (including with respect to future revenue generation and contingent payments to Marathon based on annual net sales);
- our ability to complete any dystrophin study necessary in order to resolve the matters set forth in the FDA’s denial of our appeal to the Complete Response Letter we received from the FDA in connection with our New Drug Application, or NDA, for Translarna for the treatment of nmDMD, and our ability to perform additional clinical trials, non-clinical studies or CMC assessments or analyses at significant cost;
- the timing and scope of our continued commercialization of Translarna as a treatment for nmDMD in the EEA or other territories outside of the United States;
- our ability to obtain additional and maintain existing reimbursed named patient and cohort EAP programs for Translarna for the treatment of nmDMD on adequate terms, or at all;

- our 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,

Table of Contents

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;

- our estimates regarding the potential market opportunity for Translarna, Emflaza, PTC-AADC, Tegsedi, Waylivra or any other product candidate, including the size of eligible patient populations and our ability to identify such patients;
- our estimates regarding expenses, future revenues, third-party discounts and rebates, capital requirements and needs for additional financing, including our ability to maintain the level of our expenses consistent with our internal budgets and forecasts and to secure additional funds on favorable terms or at all;
- the timing and conduct of our ongoing, planned and potential future clinical trials and studies of Translarna for the treatment of nmDMD, aniridia, and Dravet syndrome/CDKL5, each caused by nonsense mutations, as well as our studies in spinal muscular atrophy and our oncology program, including the timing of initiation, enrollment and completion of the trials and the period during which the results of the trials will become available;
- the rate and degree of market acceptance and clinical utility of Translarna, Emflaza, PTC-AADC, Tegsedi and Waylivra;
- the ability and willingness of patients and healthcare professionals to access Translarna through alternative means if pricing and reimbursement negotiations in the applicable territory do not have a positive outcome;
- the timing of, and our ability to obtain additional marketing authorizations for, Translarna and our other product candidates;
- the ability of Translarna, Emflaza, PTC-AADC, Tegsedi and Waylivra and our other product candidates to meet existing or future regulatory standards;
- our ability to maintain the current labeling under the marketing authorization in the EEA or expand the approved product label of Translarna for the treatment of nmDMD 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 and any other product candidate in clinically and commercially sufficient quantities and the ability of distributors to process orders in a timely manner and satisfy their other obligations to us;
- our ability to establish and maintain arrangements for the manufacture of Translarna, Emflaza and our other product candidates that are sufficient to meet clinical trial and commercial launch requirements;
- our ability to satisfy our obligations under the terms of the credit and security agreement with MidCap Financial Trust, or MidCap Financial, as administrative agent and MidCap Financial and certain other financial institutions as lenders thereunder;
- our other regulatory submissions, including with respect to timing and outcome of regulatory review;
- our plans to pursue development of Translarna for additional indications;
- our ability to advance our earlier stage programs, including our oncology program;
- our plans to pursue research and development of other product candidates;
- whether we may pursue business development opportunities, including potential collaborations, alliances, and acquisition or licensing of assets and our ability to successfully develop or commercialize any assets to which we may gain rights pursuant to such business development opportunities;
- the potential advantages of Translarna, Emflaza, PTC-AADC, Tegsedi and Waylivra and any other product candidate;
- our intellectual property position;
- the impact of government laws and regulations;

Table of Contents

- the impact of litigation that has 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, 2017, and in Part II, Item 1A. Risk Factors in our Quarterly Reports on Form 10-Q for the periods ended March 31, 2018 and June 30, 2018, and in Exhibit 99.2 to our Current Report on Form 8-K filed on August 24, 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, our Annual Report on Form 10-K for the year ended December 31, 2017 and our Current Report on Form 8-K filed on August 24, 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.

Table of Contents

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

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

	September 30, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 206,913	$ 111,792
Marketable securities	42,491	79,454
Trade receivables, net	42,197	40,394
Inventory, net	13,660	10,754
Prepaid expenses and other current assets	8,020	6,669
Total current assets	313,281	249,063
Fixed assets, net	8,805	8,376
Intangible assets, net	604,612	132,993
Goodwill	100,309	—
Deposits and other assets	1,620	1,221
Total assets	$ 1,028,627	$ 391,653
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 102,788	$ 76,446
Current portion of long-term debt	6,667	—
Deferred revenue	2,004	3,937
Other current liabilities	3,463	1,665
Total current liabilities	114,922	82,048
Deferred revenue - long-term	11,156	7,954
Long-term debt	144,258	144,971
Contingent consideration payable	218,700	—
Deferred consideration payable	38,200	—
Deferred tax liability	115,200	—
Other long-term liabilities	101	243
Total liabilities	642,537	235,216
Stockholders' equity:		
Common stock, $0.001 par value. Authorized 125,000,000 shares; issued and outstanding 50,432,655 shares at September 30, 2018. Authorized 125,000,000 shares; issued and outstanding 41,612,395 shares at December 31, 2017	51	42
Additional paid-in capital	1,275,004	966,534
Accumulated other comprehensive income	1,628	3,969
Accumulated deficit	(890,593)	(814,108)
Total stockholders' equity	386,090	156,437
Total liabilities and stockholders' equity	$ 1,028,627	$ 391,653

See accompanying unaudited notes.

4

Table of Contents

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Revenues:				
Net product revenue	$53,021	$41,780	$177,172	$116,113
Collaboration and grant revenue	570	73	1,224	249
Total revenues	53,591	41,853	178,396	116,362
Operating expenses:				
Cost of product sales, excluding amortization of acquired intangible asset	3,292	1,582	8,909	2,142
Amortization of acquired intangible asset	5,793	9,716	16,815	9,952
Research and development	54,368	30,024	118,337	88,222
Selling, general and administrative	38,368	31,423	104,882	85,788
Total operating expenses	101,821	72,745	248,943	186,104
Loss from operations	(48,230)	(30,892)	(70,547)	(69,742)
Interest expense, net	(3,118)	(3,421)	(9,306)	(8,648)
Other income (expense), net	734	766	1,066	(1,373)
Loss before income tax expense	(50,614)	(33,547)	(78,787)	(79,763)
Income tax expense	(355)	(191)	(964)	(507)
Net loss attributable to common stockholders	$(50,969)	$(33,738)	$(79,751)	$(80,270)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	48,096,521	41,296,740	45,310,690	38,433,749
Net loss per share—basic and diluted (in dollars per share)	$(1.06)	$(0.82)	$(1.76)	$(2.09)

See accompanying unaudited notes.

Table of Contents

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Net loss	$(50,969)	$(33,738)	$(79,751)	$(80,270)
Other comprehensive loss:				
Unrealized gain (loss) on marketable securities	33	31	(50)	—
Foreign currency translation (loss) gain	(260)	983	(2,291)	4,498
Comprehensive loss	$(51,196)	$(32,724)	$(82,092)	$(75,772)

See accompanying unaudited notes.

Table of Contents

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

	Nine Months Ended September 30,	
	2018	2017
Cash flows from operating activities		
Net loss	$(79,751)	$(80,270)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	19,316	11,743
Change in valuation of warrant liability	3	3
Non-cash interest expense	5,563	4,999
Loss on disposal of asset	2	5
Amortization of premiums and accretion of discounts on investments, net	(354)	493
Amortization of debt issuance costs	390	308
Share-based compensation expense	24,773	24,082
Unrealized foreign currency transaction gains	(977)	(364)
Changes in operating assets and liabilities:		
Inventory	(3,252)	(3,625)
Prepaid expenses and other current assets	(1,301)	(570)
Trade receivables, net	(2,681)	(10,994)
Deposits and other assets	(385)	(485)
Accounts payable and accrued expenses	18,606	11,807
Other liabilities	1,617	807
Deferred revenue	5,933	10,710
Net cash used in operating activities	(12,498)	(31,351)
Cash flows from investing activities		
Purchases of fixed assets	(2,489)	(1,058)
Purchases of marketable securities	(28,656)	(19,467)
Sale and redemption of marketable securities	65,923	164,847
Acquisition of product rights	(3,903)	(77,163)
Business acquisition, net of cash acquired	(48,892)	—
Net cash (used in) / provided by investing activities	(18,017)	67,159
Cash flows from financing activities		
Proceeds from exercise of options	8,631	1,437
Net proceeds from public offerings	117,915	—
Proceeds from shares issued under employee stock purchase plan	1,299	1,362
Debt issuance costs related to secured term loan	—	(432)
Proceeds from issuance of secured term loan	—	40,000
Net cash provided by financing activities	127,845	42,367
Effect of exchange rate changes on cash	(2,209)	5,342
Net increase in cash and cash equivalents	95,121	83,517
Cash and cash equivalents, beginning of period	111,792	58,321
Cash and cash equivalents, end of period	$206,913	$141,838
Supplemental disclosure of cash information		
Cash paid for interest	$6,927	$5,496
Cash paid for income taxes	$919	$616
Supplemental disclosure of non-cash investing and financing activity		

Change in unrealized gain (loss) on marketable securities, net of tax	$(50)	$—
Acquisition of product rights and licenses	$(4,530)	$—

See accompanying unaudited notes.

Table of Contents

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

1. The Company

PTC Therapeutics, Inc. (the "Company" or "PTC") is a science-led global biopharmaceutical company focused on the discovery, development and commercialization of clinically-differentiated medicines that provide benefits to patients with rare disorders. The Company'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 2019. The Company is also preparing a marketing authorisation application, or MAA, for PTC-AADC for the treatment of AADC deficiency in the European Union, or EU, which it anticipates submitting to the European Medicines Agency, or EMA, in 2019, as well. 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 plans to file a request for marketing authorization for Tegsedi with ANVISA, the Brazilian Health Regulatory Authority, in the first half of 2019. Waylivra is currently under regulatory review in EU for the treatment of familial chylomicronemia syndrome, or FCS.

The Company also has a spinal muscular atrophy (SMA) collaboration with F. Hoffman-La Roche Ltd and Hoffman-La Roche Inc., which it refers to collectively as Roche, and the Spinal Muscular Atrophy Foundation, or SMA Foundation. Currently, its collaboration has three clinical trials ongoing to evaluate the safety and effectiveness of risdiplam (RG7916, RO7034067), the lead compound in the SMA program. In addition, the Company has a pipeline of product candidates that are in early clinical and pre-clinical development. The Company's pre-clinical and discovery programs are 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 in the approved patient population. The final report on the trial and open-label extension is to be submitted by the Company to the EMA by the end of the third quarter of 2021. The Company refers to the trial and open-label extension together as Study 041.

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

trial within the pre-specified level of statistical significance.

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

Table of Contents

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

The NDA, which seeks approval of Translarna for the treatment of nmDMD in the United States, was initially submitted by the Company in December 2015. In February 2016, following the submission, the Company received a Refuse to File letter from the FDA regarding the NDA. The FDA stated in the Refuse to File letter that the NDA was not sufficiently complete to permit a substantive review. Specifically, the Company was notified in the letter that, in the view of the FDA, both the Phase 2b and Phase 3 ACT DMD trials were negative and do not provide substantial evidence of effectiveness and that the NDA did not contain adequate information regarding the abuse potential of Translarna. Additionally, the FDA stated that the Company had proposed a post-hoc adjustment of ACT DMD that eliminates data from a majority of enrolled patients. During July 2016, the Company appealed the Refuse to File decision via the formal dispute resolution process within FDA's Center for Drug Evaluation and Research; however, this appeal was denied by the FDA's Office of Drug Evaluation I in October 2016.

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

Upon the closing of the Transaction, the Company paid to Marathon total upfront consideration comprised of $75.0 million in cash, funded through cash on hand, and 6,683,598 shares of the Company's common stock. The number of shares of common stock issued at closing was determined by dividing $65.0 million by the volume-weighted average price per share of the Company's common stock on the Nasdaq Stock Market for the 15 trading-day period ending on the third trading day immediately preceding the closing. Marathon 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 September 30, 2018, the Company had an accumulated deficit of approximately $890.6 million. The Company has financed its operations to date primarily through the private offering in August 2015 of 3.00% convertible senior notes due 2022 (see Note 10), public offerings of common stock in February 2014, October 2014 and April 2018, its initial public offering of common stock in June 2013, private placements of its convertible preferred stock, collaborations, bank debt, convertible debt financings, grant funding and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease area addressed by the Company’s product candidates. Since 2014, the Company has also relied on revenue generated from net sales of Translarna for the treatment of nmDMD in territories outside of the United States, and in May 2017, the Company began

Table of Contents

to recognize revenue generated from net sales of Emflaza for the treatment of DMD in the United States. The Company expects that the cash flows from the sales of its products, together with the Company's cash, cash equivalents and marketable securities, will be sufficient to fund its operations for at least the next twelve months.

2. Summary of significant accounting policies

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

Basis of presentation

The accompanying financial information as of September 30, 2018 and for the three and nine months ended September 30, 2018 and 2017 has been prepared by the Company, without audit, pursuant to the rules and regulations of the SEC. Certain information and footnote disclosures normally included in financial statements prepared in accordance with generally accepted accounting principles in the United States (GAAP) have been condensed or omitted pursuant to such rules and regulations. These interim financial statements should be read in conjunction with the Company’s audited financial statements as of December 31, 2017 and notes thereto included in the 2017 Form 10-K.

In the opinion of management, the unaudited financial information as of September 30, 2018 and for the three and nine months ended September 30, 2018 and 2017 reflects all adjustments, which are normal recurring adjustments, necessary to present a fair statement of financial position, results of operations and cash flows. The results of operations for the three and nine month periods ended September 30, 2018 are not necessarily indicative of the results to be expected for the year ended December 31, 2018 or for any other interim period or for any other future year.

Use of estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. Significant estimates in these consolidated financial statements have been made in connection with the calculation of net product sales, certain accruals related to the Company’s research and development expenses, stock-based compensation, valuation procedures for the convertible notes, allowance for doubtful accounts, inventory, acquired intangible assets, 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:

	September 30, 2018	December 31, 2017
Raw materials	$ 399	$ 452
Work in progress	5,997	3,912
Finished goods	7,264	6,390
Total inventory	$ 13,660	$ 10,754

The Company periodically reviews its inventories for excess amounts or obsolescence and writes down obsolete or otherwise unmarketable inventory to its estimated net realizable value. The Company recorded a $1.6 million

inventory write down for the three month period ended September 30, 2018 primarily related to inventory labeling changes. Additionally, though the Company's product is subject to strict quality control and monitoring which it performs throughout the manufacturing processes, certain

Table of Contents

batches or units of product may not meet quality specifications resulting in a charge to cost of product sales. For the three and nine month periods ended September 30, 2018, these amounts were immaterial.

Cost of product sales

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

Revenue recognition

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

Periods prior to January 1, 2018

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

Net product sales

Prior to the second quarter of 2017, the Company's net product sales consisted of sales of Translarna for the treatment of nmDMD in territories outside of the U.S. The Company recognizes revenue from product sales when there is persuasive evidence that an arrangement exists, title to product and associated risk of loss has passed to the customer, the price is fixed or determinable, collectability is reasonably assured and the Company has no further performance obligations in accordance with Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC") Subtopic 605-15, Revenue Recognition—Products.

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

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

known changes in factors and may impact such allowances in the quarter those changes are known.

Collaboration and grant revenue

The terms of these agreements typically include payments to the Company of one or more of the following: nonrefundable, upfront license fees; milestone payments; research funding and royalties on future product sales. In addition, the Company generates

Table of Contents

service revenue through agreements that generally provide for fees for research and development services and may include additional payments upon achievement of specified events.

The Company evaluates all contingent consideration earned, such as a milestone payment, using the criteria as provided by ASC 605-28, Revenue Recognition—Milestone Method. At the inception of a collaboration arrangement, the Company evaluates if a milestone payment is substantive. The criteria requires that (1) the Company determines if the milestone is commensurate with either its performance to achieve the milestone or the enhancement of value resulting from its activities to achieve the milestone; (2) the milestone be related to past performance; and (3) the milestone be reasonable relative to all deliverable and payment terms of the collaboration arrangement. If these criteria are met then the contingent milestones can be considered a substantive milestone and will be recognized as revenue in the period that the milestone is achieved. The Company recognizes royalties as earned in accordance with the terms of various research and collaboration agreements. If not substantive, the contingent consideration is allocated to the existing units of accounting based on relative selling price and recognized following the same basis previously established for the associated unit of accounting.

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

Periods commencing January 1, 2018

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

Net product revenue

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

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

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

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

- Portfolio Approach - the Company applied the Portfolio Approach to contract reviews within its identified revenue streams that have similar characteristics and the Company believes this approach would not differ materially than if applying ASC Topic 606 to each individual contract.
- Significant Financing Component - the Company expects the period between when it transfers a promised good to a customer and when the customer pays for the good or service to be one year or less.
-

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

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

Table of Contents

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

Collaboration revenue

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

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

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

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

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

Allowance for doubtful accounts

The Company maintains an allowance for estimated losses resulting from the inability of its customers to make required payments. The Company estimates uncollectible amounts based upon current customer receivable balances, the age of customer receivable balances, the customer’s financial condition and current economic trends. The allowance for doubtful accounts was $0.6 million as of September 30, 2018 and $0.8 million as of December 31, 2017.

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.

Table of Contents

Income Taxes

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

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

The Company recorded a deferred tax liability in conjunction with the Merger, further discussed in Note 3, of $115.2 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.

Recently issued accounting standards

In February 2016, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") No. 2016-2, "Leases (Topic 842)". This standard will require organizations that lease assets with lease terms of more than 12 months to recognize assets and liabilities for the rights and obligations created by those leases on their balance sheets. The ASU will also require new qualitative and quantitative disclosures to help investors and other financial statement users better understand the amount, timing, and uncertainty of cash flows arising from leases. The standard is effective for public companies for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2018, with early adoption permitted. The Company expects to adopt this guidance when effective and is currently assessing what effect the adoption of ASU No. 2016-2 will have on its consolidated financial statements and accompanying notes, as well as the impact on internal control over financial reporting.

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

In January 2017, the FASB issued ASU 2017-04, "Simplifying the Test for Goodwill Impairment". This standard simplifies the accounting for goodwill impairment by requiring impairment charges to be based on the first step in

today's two-step impairment test under ASC 350. Therefore, entities will record an impairment charge based on the excess of a reporting unit's carrying amount over its fair value. The guidance is effective for annual and interim impairment tests performed in periods beginning after December 15, 2019 for public business entities that meet the definition of an SEC filer, December 15, 2020 for public business entities that are not SEC filers, and December 15, 2021 for all other entities. Early adoption is permitted for all entities for annual and interim goodwill impairment testing dates on or after January 1, 2017. The guidance should be applied on a prospective basis. The Company expects to adopt this guidance when effective and is currently assessing what effect the adoption of ASU No. 2017-04 will have on its consolidated financial statements and accompanying notes.

In February 2018, the FASB issued ASU 2018-02, "Income Statement — Reporting Comprehensive Income (Topic 220): Reclassification of Certain Tax Effects from Accumulated Other Comprehensive Income". This standard permits the reclassification of tax effects stranded in other comprehensive income as a result of tax reform to retained earnings related to the change in federal tax rate in addition to other stranded effects that relate to the Tax Cuts and Job Act ("the Act") but do not directly relate to the

Table of Contents

change in the federal rate. ASU 2018-02 is effective for fiscal years beginning after December 15, 2018, including interim periods within those fiscal years with early adoption permitted for periods for which financial statements have not yet been issued or made available for issuance. The Company expects to adopt this guidance when effective and is currently assessing what effect the adoption of ASU No. 2018-02 will have on its consolidated financial statements and accompanying notes.

In June 2018, the FASB issued ASU 2018-07, "Compensation — Stock Compensation (Topic 718), Improvements to Nonemployee Share-Based Payment Accounting". This standard expands the scope of ASC 718 to include share-based payments granted to nonemployees in exchange for goods or services used or consumed in the entity's own operations and supersedes the guidance in ASC 505-50. The ASU retains the existing cost attribution guidance, which requires entities to recognize compensation cost for nonemployee awards in the same period and in the same manner they would if they paid cash for the goods or services, but it moves the guidance to ASC 718. ASU 2018-07 is effective for fiscal years beginning after December 15, 2018, including interim periods within those fiscal years with early adoption permitted for periods for which financial statements have not yet been issued or made available for issuance. The Company expects to adopt this guidance when effective and is currently assessing what effect the adoption of ASU No. 2018-07 will have on its consolidated financial statements and accompanying notes.

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.

Impact of recently adopted accounting pronouncements

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

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

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

Table of Contents

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

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

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

3. Business combination

On August 23, 2018, the Company completed its acquisition of Agilis pursuant to the Merger Agreement. Agilis was a privately-held biotechnology company advancing an innovative gene therapy platform for rare monogenic diseases that affect the central nervous system. Upon completion of the Merger, the Company acquired Agilis's lead product candidate, PTC-AADC, for the treatment of AADC deficiency, as well as three other gene therapies that were part of the Agilis platform.

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. The fair value of the stock on the acquisition date was determined to be $155.9 million.

Pursuant to the Merger Agreement, 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 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 fair value of the contingent consideration payments at the acquisition date was estimated to be $218.7 million and was determined by applying a probability adjusted, discounted cash flow approach based on development timelines from the acquired product candidates and estimated future sales. Under the Merger Agreement, the Company is required to pay $40.0 million of the development milestone payments mentioned above 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 payments at the closing date was estimated to be $38.2 million. Refer to Footnote 4 for further fair value considerations.

The Company evaluated the acquisition of Agilis under ASU No. 2017-01, Business Combinations: Clarifying the Definition of a Business. Because the business contained both inputs and processes necessary to manage products and

provide economic benefits directly to its owners and substantially all the value of the acquisition did not relate to a similar group of assets, it was determined that the acquisition represents a business combination. Therefore, the transaction has been accounted for using the acquisition method of accounting. Under the acquisition method of accounting, the total purchase price of the acquisition is allocated to the net tangible and identifiable intangible assets acquired and liabilities assumed based on their fair values as of the date of acquisition.

The fair value of consideration totaled approximately $462.0 million summarized as follows:

Table of Contents

	As of August 23, 2018
Cash consideration	$49,221
Fair value of PTC common stock issued	155,860
Estimated fair value of deferred consideration payable	38,200
Estimated fair value of contingent consideration payable	218,700
Total consideration	$461,981

The Company recorded the assets acquired and liabilities assumed as of the date of acquisition based on the information available at that time. As the Company finalizes the fair values of the assets acquired and liabilities assumed, purchase price adjustments may be recorded during the measurement period and such adjustments could be material. The Company will reflect measurement period adjustments, if any, in the period in which the adjustments are recognized. No adjustments have been made as of the acquisition date of August 23, 2018 through the period ended September 30, 2018.

The following table presents the preliminary allocation of the purchase price to the estimated fair values of the assets acquired and liabilities assumed as of the acquisition date of August 23, 2018, and through the period ended September 30, 2018:

	Preliminary Allocation as of the acquisition date and as at September 30, 2018
Cash and cash equivalents	$ 328
Prepaid expenses and other current assets	181
Fixed assets	153
Other assets	38
Intangible assets - in process research and development (“IPRD”)	480,000
Accounts payable and accrued expenses	(3,828)
Deferred tax liability	(115,200)
Fair value of net assets acquired	$ 361,672
Goodwill	100,309
Total purchase price	$ 461,981

The Company incurred approximately $1.5 million in acquisition related expenses as of September 30, 2018, which were included in selling, general and administrative expenses in the consolidated statement of operations. The results of Agilis’s operations have been included in the consolidated statements of operations beginning on the acquisition date of August 23, 2018.

The fair value of the IPR&D will be capitalized as of the acquisition date and subsequently accounted for as indefinite-lived intangible assets until disposition of the assets or completion or abandonment of the associated research and development efforts. Accordingly, during the development period after the completion of the acquisition, these assets will not be amortized into earnings; rather, these assets will be subject to periodic impairment testing. Upon successful completion of the development efforts, the useful lives of the IPR&D assets will be determined and the assets will be considered definite-lived intangible assets and amortized over their expected useful lives.

The goodwill recorded is the excess of the purchase price of the net assets acquired net of any deferred tax adjustments. The Company currently has a deferred tax liability for the indefinite lived IPR&D intangible assets,

which have no tax basis and, therefore, will not result in a future tax deduction. The goodwill is not deductible for income tax purposes.

The net loss of Agilis included in the consolidated statement of operations for the period August 23, 2018 through September 30, 2018 was $1.9 million.

Pro-Forma Financial Information Associated with the Agilis Acquisition (Unaudited)
The following table summarizes certain supplemental pro forma financial information for the three and nine-month periods ended September 30, 2018 and 2017 as if the Merger had occurred as of January 1, 2017. The unaudited pro-forma financial information

Table of Contents

for the three month and nine month periods ended September 30, 2018 reflects adjustments of $0.8 million and $1.5 million, respectively, related to acquisition fees that are non-recurring in nature. There were no adjustments related to the three and nine month periods ended September 30, 2017.

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Revenues	$53,591	$41,853	$178,396	$116,362
Net loss attributable to common stockholders	(52,458)	(41,606)	(89,976)	(90,795)

Bridge Loan

In connection with the Merger Agreement, on July 19, 2018, the Company also entered into a Bridge Loan and Security Agreement, or the Bridge Loan Agreement, by and among the Company, Agilis and certain of Agilis's domestic subsidiaries, as guarantors. Under the Bridge Loan Agreement, the Company made a term loan advance to Agilis on July 23, 2018 in an original principal amount of $10.0 million. In connection with the closing of the Merger, the original principal amount of $10.0 million plus all accrued and unpaid interest thereon was credited against the cash portion of the upfront consideration paid by the Company pursuant to the terms of the Merger Agreement in satisfaction of Agilis's outstanding payment obligations under the Bridge Loan Agreement, and the Company will have no further obligation to extend any further loan amounts under the Bridge Loan Agreement.

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.

Table of Contents

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 September 30, 2018 and December 31, 2017:

	September 30, 2018			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities	$42,491	$ —	$ 42,491	$ —
Warrant liability	$4	$ —	$ —	$ 4
Stock appreciation rights liability	$3,463	$ —	$ —	$ 3,463
Deferred consideration payable	$38,200	$ —	$ 38,200	$ —
Contingent consideration payable	$218,700	$ —	$ —	$ 218,700

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

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

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

	September 30, 2018			
	Amortized Cost	Gross Unrealized Gains	Losses	Fair Value
Commercial paper	$18,441	$ —	$ (6)	$18,435
Corporate debt securities	24,078	2	(24)	24,056
	$42,519	$ 2	$ (30)	$42,491

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

At September 30, 2018 and December 31, 2017, the Company held securities with an unrealized loss position that were not considered to be other-than-temporarily impaired as the Company has the ability to hold such investments until recovery of their fair value. Unrealized gains and losses are reported as a component of accumulated other comprehensive (loss) income in stockholders' equity. As of September 30, 2018 and December 31, 2017, the Company did not have any realized gains/losses from the sale of marketable securities.

Table of Contents

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 September 30, 2018 are as follows:

	September 30, 2018					
	Securities in an unrealized loss position less than 12 months		Securities in an unrealized loss position greater than 12 months		Total	
	Unrealized losses	Fair Value	Unrealized losses	Fair Value	Unrealized losses	Fair Value
Commercial paper	$(6)	$18,435	$—	$—	$(6)	$18,435
Corporate debt securities	(24)	18,067	—	—	(24)	18,067
	$(30)	$36,502	$—	$—	$(30)	$36,502

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

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

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

	September 30, 2018	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$18,435	$—
Corporate debt securities	24,056	—
Total Marketable securities	$42,491	$—
	December 31, 2017	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$13,827	$—
Corporate debt securities	55,550	10,077
Total Marketable securities	$69,377	$10,077

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

Convertible 3.0% senior notes

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

September 30, 2018 and December 31, 2017 was $172.9 million and $115.7 million, respectively.

The carrying amounts reported in the consolidated balance sheets for cash and cash equivalents, accounts receivable, accounts payable and borrowings under the credit and security agreement with MidCap Financial Trust and other financial institutions (as further discussed in Note 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.

Table of Contents

Deferred consideration payable

Pursuant to the Merger Agreement with Agilis, 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 payments at the acquisition date was estimated to be $38.2 million based on calculating the present value utilizing discount rates for BBB rated bonds maturing in the years of expected payments.

Level 3 valuation

The warrant liability is classified in Other long-term liabilities on the Company's consolidated balance sheets. The warrant liability is marked-to-market each reporting period with the change in fair value recorded as a gain or loss within Other expense, net, on the Company's consolidated statements of operations until the warrants are exercised, expire or other facts and circumstances lead the warrant liability to be reclassified as an equity instrument. The fair value of the warrant liability is determined at each reporting period by utilizing the Black-Scholes option pricing model.

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

The 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 Company estimates the fair value of its contingent consideration using a probability weighted discounted cash flow valuation approach based on development timelines and the estimated future sales expected from the Agilis platform.

The table presented below is a summary of changes in the fair value of the Company's Level 3 valuations for the warrant liability, the SARs liability, and the contingent consideration payable for the period ended September 30, 2018:

	Level 3 liabilities		
	Warrants	SARs	Contingent consideration payable
Beginning balance as of December 31, 2017	$1	$1,665	$ —
Additions	—	—	218,700
Change in fair value	3	3,789	—
Payments	—	(1,991)	$ —
Ending balance as of September 30, 2018	$4	$3,463	$ 218,700

Fair value of the warrant liability is estimated using an option-pricing model, which includes variables such as the expected volatility based on guideline public companies, the stock fair value, and the estimated time to a liquidity event. The significant assumptions used in preparing the option pricing model for valuing the Company's warrants as of September 30, 2018 include (i) volatility (51%-54%), (ii) risk free interest rate (2.59%-2.59%), (iii) strike price ($128.00-$2,520.00), (iv) fair value of common stock ($47.00), and (v) expected life (0.8—1.0 years). The significant assumptions used in preparing the option pricing model for valuing the Company's warrants as of December 31, 2017 include (i) volatility (69%-69%), (ii) risk free interest rate (1.89%—1.89%), (iii) strike price ($128.00—$2,520.00), (iv) fair value of common stock ($16.68), and (v) expected life (1.6—1.7 years).

Fair value of the SARs liability is estimated using an option-pricing model, which includes variables such as the expected volatility based on guideline public companies, the stock fair value, and the estimated time to a liquidity event. The significant assumptions used in preparing the option pricing model for valuing the Company's SARs as of September 30, 2018 include (i) volatility (45%—54%), (ii) risk free interest rate (2.19%—2.70%), (iii) strike price ($6.76-$30.86), (iv) fair value of common stock ($47.00), and (v) expected life (0.3—1.3 years). The significant assumptions used in preparing the option pricing model for valuing the Company's SARs as of December 31, 2017 include (i) volatility (31%-70%), (ii) risk free interest rate (1.28%—1.89%), (iii) strike price ($6.76—$30.86), (iv) fair value of common stock ($16.68), and (v) expected life (0.0—2.0 years).

Fair value of the contingent consideration liability is estimated using a probability weighted discounted cash flow approach. Some of the more significant assumptions made in the valuation include (i) the estimated revenue forecasts, (ii) probabilities of success, and (iii) discount periods and rate. The probability of achievement of regulatory and sales milestones ranged from 25% to 89%. The achievement of certain development milestones ranged from zero to an aggregate maximum amount of $20.0 million, the achievement of certain regulatory approval milestones together with a milestone payment following the receipt of a priority review voucher ranged from zero up to an aggregate maximum amount of $535.0 million, the achievement of certain net sales milestones ranged from zero up to an aggregate maximum amount of $150.0 million, and a percentage of annual net sales for Friedreich Ataxia and Angelman Syndrome during specified terms, ranging from 2-6%, in periods which sales occur. The $20.0 million

Table of Contents

development milestones mentioned above do not include $40.0 million in development milestone payments that the Company is required to pay no later than the second anniversary of the closing of the Merger, regardless of whether the applicable milestones have been achieved. Such $40 million development milestones have been recorded as deferred consideration payable on the consolidated balance sheets at its estimated fair value, which was estimated to be $38.2 million.

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

Other comprehensive income (loss) includes changes in equity that are excluded from net income (loss), such as unrealized gains and losses on marketable securities.

The following tables summarize other comprehensive income (loss) and the changes in accumulated other comprehensive items for the three and nine months ended September 30, 2018:

	Unrealized Gains/(Losses) On Marketable Securities, net of tax	Foreign Currency Translation	Total Accumulated Other Comprehensive Items
Balance at June 30, 2018	$ (61)	$ 1,916	$ 1,855
Other comprehensive income (loss) before reclassifications	33	(260)	(227)
Amounts reclassified from other comprehensive items	—	—	—
Other comprehensive income (loss)	33	(260)	(227)
Balance at September 30, 2018	$ (28)	$ 1,656	$ 1,628

	Unrealized Gains/(Losses) On Marketable Securities, net of tax	Foreign Currency Translation	Total Accumulated Other Comprehensive Items
Balance at December 31, 2017	$ 22	$ 3,947	$ 3,969
Other comprehensive loss before reclassifications	(50)	(2,291)	(2,341)
Amounts reclassified from other comprehensive items	—	—	—
Other comprehensive loss	(50)	(2,291)	(2,341)
Balance at September 30, 2018	$ (28)	$ 1,656	$ 1,628

6. Accounts payable and accrued expenses

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

	September 30, 2018	December 31, 2017
Employee compensation, benefits, and related accruals	$ 18,003	$ 17,711
Consulting and contracted research	8,111	5,137
Professional fees	3,541	2,116
Sales allowance and other costs	27,027	22,257
Sales rebates and royalties	26,818	11,657
Accounts payable	6,538	15,282
Other	12,750	2,286
	$ 102,788	$ 76,446

7. Capitalization

22

Table of Contents

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

Warrants

All of the Company's outstanding warrants were classified as liabilities as of September 30, 2018 and December 31, 2017 because they contained non-standard antidilution provisions.

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

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

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.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Numerator				
Net loss	$(50,969)	$(33,738)	$(79,751)	$(80,270)
Denominator				
Denominator for basic and diluted net loss per share	48,096,521	41,296,740	45,310,690	38,433,749
Net loss per share:				
Basic and diluted	$(1.06)*	$(0.82)*	$(1.76)*	$(2.09)*

*In the three and nine months ended September 30, 2018 and 2017, the Company experienced a net loss and therefore did not report any dilutive share impact.

The following table shows historical dilutive common share equivalents outstanding, which are not included in the above historical calculation, as the effect of their inclusion is anti-dilutive during each period.

	As of September 30,	
	2018	2017
Stock Options	9,545,522	6,612,765
Unvested restricted stock awards and units	580,347	402,853
Total	10,125,869	7,015,618

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.

Table of Contents

In May 2013, the Company's Board of Directors and stockholders approved the 2013 Long Term Incentive Plan, which became effective upon the closing of the Company's IPO. The 2013 Long Term Incentive Plan provides for the grant of incentive stock options, nonstatutory stock options, restricted stock awards and other stock-based awards. The number of shares of common stock reserved for issuance under the 2013 Long Term Incentive Plan is the sum of (1) 122,296 shares of common stock available for issuance under the Company's 2009 Equity and Long Term Incentive Plan and 2013 Stock Incentive Plan, (2) the number of shares (up to 3,040,444 shares) equal to the sum of the number of shares of common stock subject to outstanding awards under the Company's 1998 Employee, Director and Consultant Stock Option Plan, 2009 Equity and Long Term Incentive Plan and 2013 Stock Incentive Plan that expire, terminate or are otherwise surrendered, cancelled, forfeited or repurchased by the Company at their original issuance price pursuant to a contractual repurchase right plus (3) an annual increase, to be added on the first day of each fiscal year until the expiration of the 2013 Long Term Incentive Plan, equal to the lowest of 2,500,000 shares of common stock, 4% of the number of shares of common stock outstanding on the first day of the fiscal year and an amount determined by the Company's Board of Directors. As of September 30, 2018, awards for 665,194 shares of common stock are available for issuance.

From January 1, 2018 through September 30, 2018, the Company issued a total of 2,914,139 stock options to various employees. Of those, 1,115,650 were inducement grants for non-statutory stock options. The inducement grant awards were made pursuant to the Nasdaq inducement grant exception as a material component of the Company's new hires' employment compensation and not under the 2013 Long Term Incentive Plan.

A summary of stock option activity is as follows:

	Number of options	Weighted-average exercise price	Weighted-average remaining contractual term	Aggregate intrinsic value (in thousands)
Outstanding at December 31, 2017	6,448,642	$ 29.00		
Granted	2,914,139	$ 25.84		
Exercised	512,145	$ 17.03		
Forfeited/Cancelled	(329,404)	$ 34.20		
Outstanding at September 30, 2018	9,545,522	$ 28.44	7.45 years	$ 172,859
Vested or Expected to vest at September 30, 2018	3,914,413	$ 24.38	8.93 years	$ 89,547
Exercisable at September 30, 2018	4,316,071	$ 32.33	5.99 years	$ 77,003

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

	Nine months ended September 30, 2018
Risk-free interest rate	2.25%—3.03%
Expected volatility	64%—90%
Expected term	5.04 – 10.00 years

The Company assumed no expected dividends for all grants. The weighted average grant date fair value of options granted during the nine-month period ended September 30, 2018 was $17.04 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.

Table of Contents

The following table summarizes information on the Company's restricted stock awards and units:

	Restricted Stock Awards and Units	
	Number of Shares	Weighted Average Grant Date Fair Value
January 1, 2018	393,011	$ 15.64
Granted	354,691	$ 19.09
Vested	(113,795)	$ 16.36
Forfeited	(53,560)	$ 17.24
Unvested at September 30, 2018	580,347	$ 17.60

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

In May 2016, a total of 897,290 SARs were granted to non-executive employees (the 2016 SARs). The 2016 SARs will vest annually in equal installments over four years and will be settled in cash on each vest date, requiring the Company to remeasure the SARs at each reporting period until vesting occurs. For the period ended September 30, 2018, a total of 177,329 SARs vested. For the period ended September 30, 2018, the Company recorded $3.7 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 September 30, 2018, the Company recorded $0.7 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 September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Research and development	$4,431	$3,624	$12,109	$11,986
Selling, general and administrative	4,511	3,544	12,664	12,096
Total	$8,942	$7,168	$24,773	$24,082

As of September 30, 2018, there was approximately $67.5 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 3.00 years.

Table of Contents

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 remaining $20.0 million under the senior secured term loan facility will become available to the Company upon its demonstration (on or prior to December 31, 2018) of net product revenue equaling or exceeding $120.0 million for the trailing 12 month period. The Company capitalized approximately $0.4 million of debt issuance costs, which were netted against the carrying value of the Credit Facility and will be amortized over the term of the Credit Facility.

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

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

Convertible Notes

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

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

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

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

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

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

· upon the occurrence of specified corporate events.

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

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

The Company 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

Table of Contents

(whether or not consecutive) during, any 30 consecutive trading day period ending on, and including, the trading day immediately preceding the date on which the Company provides notice of redemption, at a redemption price equal to 100% of the principal amount of the Convertible Notes to be redeemed, plus accrued and unpaid interest to, but excluding, the redemption date. No sinking fund is provided for the Convertible Notes, which means that the Company is not required to redeem or retire the Convertible Notes periodically.

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

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

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

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

The Convertible Notes consist of the following:

Liability component	September 30, 2018	December 31, 2017
Principal	$150,000	$150,000
Less: Debt issuance costs	(1,844)	(2,121)
Less: Debt discount, net(1)	(37,009)	(42,572)
Net carrying amount	$111,147	$105,307

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

The fair value of the Convertible Notes was approximately $172.9 million as of September 30, 2018. The Company estimates the fair value of its Convertible Notes utilizing market quotations for debt that have quoted prices in active

markets. As of September 30, 2018, the remaining contractual life of the Convertible Notes is approximately 3.9 years.

Table of Contents

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Contractual interest expense	$1,134	$1,134	$3,375	$3,375
Amortization of debt issuance costs	95	86	277	249
Amortization of debt discount	1,919	1,725	5,563	4,999
Total	$3,148	$2,945	$9,215	$8,623
Effective interest rate of the liability component	11 %	11 %	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, 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 Company may also be obligated to pay additional development, regulatory approval, and net sales milestones and net sales royalties. Refer to Note 3 for further details.

The Company also has a Collaboration and License Agreement with Akcea for the commercialization of Tegsedi and Waylivra, and products containing those compounds in countries in Latin America and the Caribbean. 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, the Brazilian Health Regulatory Authority, subject to a maximum aggregate amount of $8.0 million for all such products. Akcea is also entitled to receive royalty payments subject to certain terms set forth in the Akcea Collaboration and License Agreement.

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.

In the period ended September 30, 2018, two lawsuits the Company was involved in were settled and dismissed (refer to Part II, Item 1. Legal Proceedings for further details on the dismissed lawsuits).

12. Revenue recognition

Net product sales

Table of Contents

The Company views its operations and manages its business in one operating segment. During the three and nine months ended September 30, 2018, net product sales in the United States were $22.6 million and $62.2 million, respectively, consisting solely of Emflaza, and net product sales not in the United States were $30.4 million and $115.0 million, respectively, consisting solely of Translarna.

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

	Balance as of December 31, 2017	Additions	Deductions	ASC 606 Adjustment	Balance as of September 30, 2018
Deferred Revenue	$ 11,891	$ 4,706	$ —	$ (3,937)	$ 12,660

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

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

	Three Months Ended September 30, 2018	Nine Months Ended September 30, 2018
Amounts included in contract liabilities at the beginning of the period	$ —	$ —
Performance obligations satisfied in previous period	—	—
Performance obligations satisfied in current period	53,021	177,172
Total product revenue	$ 53,021	$ 177,172

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

Remaining performance obligations

Remaining performance obligations represent the transaction price for goods the Company has yet to provide. As of September 30, 2018, the aggregate amount of transaction price allocated to remaining performance obligations relating to Translarna net product revenue was $12.7 million. 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.

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

Table of Contents

i.Consolidated balance sheets

	As reported September 30, 2018	Impact of changes in accounting policies Adjustments	As reported Balances without adoption of Topic 606
Assets			
Current assets:			
Cash and cash equivalents	$206,913	$ —	$206,913
Marketable securities	42,491	—	42,491
Trade receivables, net	42,197	—	42,197
Inventory	13,660	(84)	13,576
Prepaid expenses and other current assets	8,020	—	8,020
Total current assets	313,281	(84)	313,197
Fixed assets, net	8,805	—	8,805
Intangible assets, net	604,612	—	604,612
Goodwill	100,309	—	100,309
Deposits and other assets	1,620	—	1,620
Total assets	$1,028,627	$ (84)	$1,028,543
Liabilities and stockholders' equity			
Current liabilities:			
Accounts payable and accrued expenses	$102,788	$ (794)	$101,994
Current portion of long-term debt	6,667	—	6,667
Deferred revenue	2,004	5,120	7,124
Other current liabilities	3,463	—	3,463
Total current liabilities	114,922	4,326	119,248
Deferred revenue - long-term	11,156	—	11,156
Long-term debt	144,258	—	144,258
Contingent consideration payable	218,700	—	218,700
Deferred consideration payable	38,200	—	38,200
Deferred tax liability	115,200	—	115,200
Other long-term liabilities	101	—	101
Total liabilities	642,537	4,326	646,863
Stockholders' equity:			
Common stock	51	—	51
Additional paid-in capital	1,275,004	—	1,275,004
Accumulated other comprehensive income	1,628	—	1,628
Accumulated deficit	(890,593)	(4,410)	(895,003)
Total stockholders' equity	386,090	(4,410)	381,680
Total liabilities and stockholders' equity	$1,028,627	$ (84)	$1,028,543

30

Table of Contents

ii.Consolidated statements of operations

	Impact of changes in accounting policies		
	Three Months Ended		
	As reported for the period ended September 30, 2018	Adjustments	As reported Balances without adoption of Topic 606
Revenues:			
Net product revenue	$53,021	$ (834)	$52,187
Collaboration and grant revenue	570	—	570
Total revenues	53,591	(834)	52,757
Operating expenses:			
Cost of product sales, excluding amortization of acquired intangible asset	3,292	(17)	3,275
Amortization of acquired intangible asset	5,793	—	5,793
Research and development	54,368	—	54,368
Selling, general and administrative	38,368	—	38,368
Total operating expenses	101,821	(17)	101,804
Loss from operations	(48,230)	(817)	(49,047)
Interest expense, net	(3,118)	—	(3,118)
Other expense, net	734	—	734
Loss before income tax expense	(50,614)	(817)	(51,431)
Income tax expense	(355)	—	(355)
Net loss attributable to common stockholders	$(50,969)	$ (817)	$(51,786)

	Impact of changes in accounting policies		
	Year to Date		
	As reported for the period ended September 30, 2018	Adjustments	As reported Balances without adoption of Topic 606
Revenues:			
Net product revenue	$177,172	$ (1,059)	$176,113
Collaboration and grant revenue	1,224	—	1,224
Total revenues	178,396	(1,059)	177,337
Operating expenses:			
Cost of product sales, excluding amortization of acquired intangible asset	8,909	(84)	8,825
Amortization of acquired intangible asset	16,815	—	16,815
Research and development	118,337	—	118,337
Selling, general and administrative	104,882	—	104,882
Total operating expenses	248,943	(84)	248,859
Loss from operations	(70,547)	(975)	(71,522)

Interest expense, net	(9,306)	—	(9,306)
Other income, net	1,066	—	1,066
Loss before income tax expense	(78,787)	(975)	(79,762)
Income tax expense	(964)	—	(964)
Net loss attributable to common stockholders	$(79,751)	$ (975)	$(80,726)

Table of Contents

iii.Consolidated statements of comprehensive loss

	Impact of changes in accounting policies		
	Three Months Ended		
	As reported for the period ended September 30, 2018	Adjustments	As reported Balances without adoption of Topic 606
Net loss	$(50,969)	$ (817)	$(51,786)
Other comprehensive loss:			
Unrealized gain on marketable securities, net of tax	33	—	33
Foreign currency translation loss	(260)	—	(260)
Comprehensive loss	$(51,196)	$ (817)	$(52,013)

	Impact of changes in accounting policies		
	Year to Date		
	As reported for the period ended September 30, 2018	Adjustments	As reported Balances without adoption of Topic 606
Net loss	$(79,751)	$ (975)	$(80,726)
Other comprehensive loss:			
Unrealized loss on marketable securities, net of tax	(50)	—	(50)
Foreign currency translation loss	(2,291)	—	(2,291)
Comprehensive loss	$(82,092)	$ (975)	$(83,067)

Table of Contents

iv.Consolidated statements of cash flows

	Impact of changes in accounting policies		
	As reported for the period ended September 30, 2018	Adjustments	Balances without adoption of Topic 606
Cash flows from operating activities			
Net loss	$(79,751)	$ (975)	$(80,726)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	19,316	—	19,316
Change in valuation of warrant liability	3	—	3
Non-cash interest expense	5,563	—	5,563
Loss on disposal of asset	2	—	2
Amortization of premiums and accretion of discounts on investments, net	(354)	—	(354)
Amortization of debt issuance costs	390	—	390
Share-based compensation expense	24,773	—	24,773
Unrealized foreign currency transaction gain	(977)	—	(977)
Changes in operating assets and liabilities:			0
Inventory, net	(3,252)	(84)	(3,336)
Prepaid expenses and other current assets	(1,301)	—	(1,301)
Trade receivables, net	(2,681)	—	(2,681)
Deposits and other assets	(385)	—	(385)
Accounts payable and accrued expenses	18,606	(794)	17,812
Other liabilities	1,617	—	1,617
Deferred revenue	5,933	1,853	7,786
Net cash used in operating activities	(12,498)	—	(12,498)
Cash flows from investing activities			
Purchases of fixed assets	(2,489)	—	(2,489)
Purchases of marketable securities	(28,656)	—	(28,656)
Sale and redemption of marketable securities	65,923	—	65,923
Acquisition of product rights	(3,903)	—	(3,903)
Business acquisition, net of cash acquired	(48,892)	—	(48,892)
Net cash (used in) / provided by investing activities	(18,017)	—	(18,017)
Cash flows from financing activities			
Proceeds from exercise of options	8,631	—	8,631
Net proceeds from public offerings	117,915	—	117,915
Proceeds from shares issued under employee stock purchase plan	1,299	—	1,299
Net cash provided by financing activities	127,845	—	127,845
Effect of exchange rate changes on cash	(2,209)	—	(2,209)
Net increase in cash and cash equivalents	95,121	—	95,121
Cash and cash equivalents, beginning of period	111,792	—	111,792
Cash and cash equivalents, end of period	$206,913	$ —	$206,913
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

Table of Contents

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

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

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

In 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 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.

The remaining potential research and development event milestones that can be received as of September 30, 2018 is $87.5 million.

For the nine months ended September 30, 2018 and 2017, the Company recognized revenue related to the licensing and collaboration agreement with Roche of $0.2 million and $0.2 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

Table of Contents

recognized. For the nine months ended September 30, 2018 and 2017, the Company did not recognize any revenue related to discovery agreements.

The Company is eligible to receive additional payments from its early stage discovery research arrangements if the discovery compounds are ultimately developed and commercialized. The aggregate potential payments the Company is eligible for if all products are developed is $143.0 million and up to $252.0 million in sales milestones upon achievement of specified sales events and up to double digit royalties on worldwide annual net sales of the licensed product. The Company will recognize revenue when it is probable the milestones will be achieved (see Note 2). For the nine months ended September 30, 2018 and 2017, 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 beginning in 2018, up to a specified aggregate maximum amount over the expected commercial life of the asset. In accordance with the guidance for an asset acquisition, the Company will record the milestone payment when it becomes payable to Marathon and increase the cost basis for the Emflaza rights intangible asset.

For the three and nine month periods ended September 30, 2018, the Company recorded $4.5 million and $8.4 million of milestone payments, respectively, which were added to the cost basis for the Emflaza rights intangible asset and will be amortized prospectively on a straight-line basis over the remaining life of the asset. As of September 30, 2018, the $4.5 million milestone payment was recorded on the balance sheet within accrued expenses as a contingent payment payable to Marathon.

For the three and nine months ended September 30, 2018, the Company recognized amortization expense of $5.8 million and $16.8 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:

	As of September 30, 2018
2018(1)	$ 5,794
2019	23,172
2020	23,172
2021	23,172
2022 and thereafter	49,302
Total	$ 124,612

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

Indefinite-lived intangibles

In connection with the acquisition of Agilis (Note 3), 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 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.

Table of Contents

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 preliminary value allocated to the indefinite lived intangible assets was $480 million.

Goodwill

As a result of the Merger on August 23, 2018, the Company recorded $100.3 million of goodwill. There were no changes to the recorded value of goodwill for the three and nine month periods ended September 30, 2018.

Collaboration and Licensing Agreement

On August 1, 2018, the Company entered into a Collaboration and License Agreement with Akcea for the commercialization of Tegsedi and Waylivra, and products containing those compounds in countries in Latin America and the Caribbean. 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 United States Food and Drug Administration or the European Medicines Agency, whichever occurs earlier. The Company evaluated the agreement under the guidance in ASC 730 and concluded that the acquired rights to commercialize the products had no alternative future use as of the date of the Merger. Accordingly, the $12.0 million was charged to research and development expense in the consolidated statements of operations for the three and nine month periods ended September 30, 2018. The Company plans to a file a request for marketing authorizations for Tegsedi with ANVISA in the first half of 2019. Waylivra is currently under regulatory review in the EU.

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, 2017 included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 6, 2018. This discussion contains forward-looking statements that involve significant risks and uncertainties. As a result of many factors, such as those set forth in Part II, Item 1A. (Risk Factors) of this Quarterly Report on Form 10-Q, Part I, Item 1A. (Risk Factors) of our Annual Report on Form 10-K for the year ended December 31, 2017, Part II, Item 1A. (Risk Factors) of our Quarterly Reports on Form 10-Q for the periods ended March 31, 2018 and June 30, 2018, and Exhibit 99.2 to our Current Report on Form 8-K filed on August 24, 2018 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 globally commercialize products is the foundation that drives our continued investment in a robust pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need. Our strategy is to bring best-in-class therapies with differentiated clinical benefit to patients affected by rare disorders and to leverage our global commercial infrastructure to maximize value for our patients and other stakeholders.

We have two products, Translarna™ (ataluren) and Emflaza™ (deflazacort), for the treatment of Duchenne muscular dystrophy, or DMD, a rare, life threatening disorder. During the quarter ended September 30, 2018, we recognized $30.4 million in sales of Translarna. Translarna is currently available for the treatment of nmDMD in over 25 countries on a commercial basis or through a reimbursed early access program, or EAP program. We hold worldwide commercialization rights to Translarna for all indications in all territories. Emflaza is approved in the United States for

the treatment of DMD in patients five years and older. During the quarter ended September 30, 2018, Emflaza achieved sales of $22.6 million.

We have 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. 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 U.S. Food and Drug

Table of Contents

Administration, or FDA, in 2019. We are also preparing a marketing authorisation application, or MAA, for PTC-AADC for the treatment of AADC deficiency in the European Union, or EU, which we anticipate submitting to the European Medicines Agency, or EMA, in 2019, as well. 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. 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 hereditary transthyretin amyloidosis, or hATTR amyloidosis. We plan to file a request for marketing authorization for Tegsedi with ANVISA, the Brazilian Health Regulatory Authority, in the first half of 2019. Waylivra is currently under regulatory review in the EU for the treatment of familial chylomicronemia syndrome, or FCS.

We also have a spinal muscular atrophy (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 three clinical trials ongoing to evaluate the safety and effectiveness of risdiplam (RG7916, RO7034067), the lead compound in the SMA program. In addition, we have a pipeline of product candidates that are in early clinical and pre-clinical development. Our pre-clinical and discovery programs are focused on the development of new treatments for multiple therapeutic areas, including rare diseases and oncology.

Corporate Updates

Acquisition of Agilis Biotherapeutics, Inc.

On August 23, 2018, we completed our acquisition of Agilis Biotherapeutics, Inc., or Agilis, pursuant to an Agreement and Plan of Merger, dated as of July 19, 2018, or the Merger Agreement, by and among us, Agility Merger Sub, Inc., a Delaware corporation and our 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, or the Merger.

Upon the closing of the Merger, we paid to Agilis equityholders total upfront consideration comprised of $49.2 million in cash and 3,500,907 shares of our common stock, or the Closing Stock Consideration. The Closing Stock Consideration was determined by dividing $150.0 million by the volume-weighted average price per share of our 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 us 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, we are 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 completion of the Merger gives us a gene therapy platform focused on the development of innovative therapies for rare, debilitating diseases of the central nervous system, or CNS. Our lead gene therapy product candidate is 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. AADC is the enzyme responsible for the conversion of L-dopa to dopamine. Dopamine is a key neurotransmitter that acts within the striatum (caudate and putamen), a component of the brain’s deep grey matter, to modulate output of neurons that project to the motor and premotor cortices of the brain that plan and execute normal motor function and is required to be present in the brain for humans to develop and maintain proper motor function.

AADC deficiency is a monogenic disorder of neurotransmitter synthesis that manifests in young children and most commonly results in profound developmental delay, often seen as complete arrest of motor development. AADC deficiency generally causes the inability to develop motor control (global muscular hypotonia/dystonia), resulting in breathing, feeding, and swallowing problems, frequent hospitalizations, and the need for life-long care. On average, patients with AADC deficiency die in the first decade of life due to profound motor dysfunction and secondary complications such as choking, hypoxia, and pneumonia. Currently, no treatment options are available for the underlying cause of the disorder, and care is limited to palliative options with significant burden on caregivers.

The prevalence of AADC deficiency has been estimated to be approximately 5,000 patients worldwide, with a live-birth incidence of approximately 1 in 40,000 worldwide. While several diagnostic tests for AADC deficiency are available, the condition remains largely misdiagnosed or undiagnosed.

PTC-AADC is a large molecule, adeno-associated virus (AAV) gene therapy, which has been assessed in two completed clinical trials, and one trial in which enrollment and dosing is ongoing. The two completed trials include a total of 18 children with severe AADC deficiency who were treated with a one-time total dose of 1.8×10^{11} vg of PTC-AADC during a single procedure in which the gene therapy was administered directly to the region of the brain where dopamine is made, called the putamen. The targeted micro-dosing approach administering small amounts of gene therapy directly to focal regions of affected cells in the putamen has the benefit of keeping the supply requirements for materials low, improving access of the therapeutic gene to key cells, potentially limiting immune and complement-mediated responses and reducing the risk of off-target uptake and secretion and excretion of

Table of Contents

the gene therapy by the liver and kidneys. To date, results from these trials suggest that patients may have a gain of motor functions and improvement in cognitive scales following gene therapy administration and have shown significant increases in motor function, which contrasts with the published natural history.

The two completed trials, AADC-1601, a trial in which patients were enrolled under individual compassionate use consents, and AADC-010, were both single-arm, open-label, interventional trials that enrolled a total of 18 patients. The primary and secondary endpoints of these trials were to assess the safety and efficacy of PTC-AADC administered via bilateral putaminal infusions in patients with severe AADC deficiency at a total one-time dose of 1.8 x 10^{11} vg. Study enrollment required a diagnosis of AADC deficiency, defined as decreased homovanilic acid, or HVA, and 5-hydroxyindoleacetic acid, or 5 HIAA, and elevated L-Dopa cerebrospinal fluid, or CSF, levels, presence of more than one DDC gene mutation, and presence of clinical symptoms of AADC deficiency (including developmental delay, hypotonia, dystonia, and oculogyric crisis), and patient age of older than 2 years.

Patients were evaluated monthly for safety assessments and every three months for efficacy assessments that included tests of motor developmental testing (Peabody Developmental Motor Scale, Second Edition, or PDMS-2, and Alberta Infant Motor Scale, or AIMS) through the first year after treatment with PTC-AADC and at periodic intervals thereafter through five years following treatment. The PDMS-2 and AIMS are validated scales used to assess motor skills in young children. Pharmacodynamic testing of CNS AADC activity over time included analyses of CSF neurotransmitter metabolites and FDOPA PET imaging intervals, also through five years.

8 patients were enrolled in the AADC-1601 study. 10 patients were enrolled in the AADC-010 study. In both studies, the average age of patients was less than 5 years of age.

At baseline, patients had no functional movement and failed to achieve any motor milestones, including head control, sitting or standing capabilities, consistent with the published natural history of severe AADC deficiency. Compared to baseline, at one-year and at five-years after PTC-AADC administration, patients had objective evidence of de novo dopamine production as visualized by F-DOPA PET imaging of the brain, consistent with successful and stable gene expression and enzyme activity over time.

Based on preliminary analysis, following administration of PTC-AADC, the combined group of patients showed significant changes from baseline capabilities at one-year post-treatment in functional motor skills assessed with the PDMS-2 total score, as well as locomotion, grasping, visual-motor integration and stationary subscales. Significant changes from baseline at one-year post-treatment were also observed for the combined group of patients on the AIMS total score and prone, supine, sit and stand subscales.

Compared to published natural history data, patients in these trials showed statistically significant improvements at both two- and five-years post-treatment in achievement of motor milestones of full head control (at 2 and 5 years), sitting unassisted (at 2 and 5 years) and standing with support (at 5 years), reinforcing the clinical benefit and sustainability of functional motor improvements.

Surgical injection of PTC-AADC in both completed trials was well tolerated, with no adverse events occurring during the surgical procedure. Adverse events were generally associated with the disease state. The most frequent adverse event associated with PTC-AADC was dyskinesia and these events completely resolved over time. No serious adverse events have been attributed to PTC-AADC.

The ongoing clinical trial, AADC-011, is a single-center, open-label trial to assess the efficacy and safety of PTC-AADC in patients with AADC deficiency. The primary outcomes for this trial include assessing a change in the PDMS-2 score and measuring the change in the neurotransmitter metabolite homovanillic acid (HVA) or 5-hydroxyindoleacetic acid (HIAA) in the cerebrospinal fluid.

An end-of-phase 2 meeting was held with the FDA in July 2017, and the clinical, non-clinical and manufacturing data available to date from the two completed clinical trials was reviewed. The FDA provided feedback indicating that the clinical and non-clinical data available to date was sufficient to support the submission of a BLA without undertaking additional trials or studies at this time. Based on the FDA input, including with respect to manufacturing, we are preparing a BLA for PTC-AADC for the treatment of AADC deficiency in the United States, which we anticipate submitting to the FDA in 2019. PTC-AADC for the treatment of AADC deficiency has orphan drug designation in the United States and European Union, and rare pediatric disease designation in the United States, and upon BLA approval the FDA may grant us a priority review voucher.

In April 2018, a protocol assistance meeting was held with the Scientific Advice Working Party of the European Medicines Agency, or EMA, in anticipation of the expected submission of a marketing authorisation application, or MAA, in the European Union and feedback was received indicating that the clinical and non-clinical data available to date was sufficient to support the submission of an MAA without undertaking additional trials or studies at this time. We expect to prepare and submit to the EMA an MAA for the treatment of AADC deficiency with PTC-AADC in the European Union during 2019. Based on the FDA input and feedback from the EMA, we do not deem necessary and do not plan to conduct a Phase 3 trial for PTC-AADC for the treatment of AADC deficiency.

Table of Contents

There is no guarantee that we will be able to make the BLA or MAA submissions within our expected timelines or that following such submissions, the FDA or EMA would not have additional comments or requirements with respect to the respective submissions that we would be required to address before obtaining regulatory approval, or that the FDA, the EMA or any other regulatory authority will approve PTC-AADC for treatment of AADC deficiency at all. If PTC-AADC for the treatment of AADC deficiency receives FDA approval, we expect that PTC-AADC would have a twelve-year exclusive marketing period in the United States for the approved indication, commencing on the date of FDA approval, under the provisions of the Biologics Price Competition and Innovation Act of 2009, or BPCIA, as well as a concurrent seven-year exclusive marketing period, which would commence on the date of FDA approval, under the provisions of the Orphan Drug Act of 1983, or the Orphan Drug Act. We are pursuing patent protection for PTC-AADC, and, in the meantime, we expect to rely on the twelve-year BPCIA regulatory exclusivity and concurrent seven-year Orphan Drug Act exclusivity to commercialize PTC-AADC in the United States, if it is approved.

Our gene therapy 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, or IND, submission with the FDA for this program is expected in 2019. Additionally, the gene therapy platform includes two other gene therapy programs targeting CNS disorders, including Angelman syndrome, a rare, genetic, neurological disorder characterized by severe developmental delays.

Bridge Loan and Security Agreement

In connection with the Merger Agreement, on July 19, 2018, we entered into a Bridge Loan and Security Agreement, or the Bridge Loan Agreement, by and among us, Agilis and certain of Agilis's domestic subsidiaries, as guarantors. Under the Bridge Loan Agreement, we made a term loan advance to Agilis on July 23, 2018 in an original principal amount of $10.0 million. In connection with the closing of the Merger, the original principal amount of $10.0 million plus all accrued and unpaid interest thereon was credited against the cash portion of the upfront consideration paid by us pursuant to the terms of the Merger Agreement in satisfaction of Agilis's outstanding payment obligations under the Bridge Loan Agreement, and we will have no further obligation to extend any further loan amounts under the Bridge Loan Agreement.

Akcea Collaboration and Licensing Agreement

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

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

PTC International agreed to pay to Akcea an upfront licensing fee of $18.0 million, consisting of an initial payment of $12.0 million paid in connection with entering into the Akcea Agreement, and $6.0 million to be paid 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, the Brazilian Health Regulatory Authority, subject to a maximum aggregate amount of $8.0 million for all such Products. Akcea is also entitled to receive royalty payments in the mid-twenty percent range of net sales on a country-by-country and Product-by-Product basis, commencing on the earlier to occur of (1) 12 months after the first commercial sale of such Product in Brazil or (2) the date when PTC International, its affiliates or sublicensees have recognized revenue of $10.0 million or more in cumulative net sales

for such Product in the PTC Territory. The royalty payments are subject to reduction in certain circumstances as set forth in the Akcea Agreement.

Tegsedi, a product of Ionis' proprietary antisense technology, is an antisense oligonucleotide, or ASO, inhibitor of human transthyretin, or TTR, production. Tegsedi is the world's first RNA-targeted therapeutic to treat patients with hereditary transthyretin amyloidosis (hATTR amyloidosis). It has received marketing authorization in the U.S., EU and Canada for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hATTR amyloidosis. We plan to file a request for marketing authorization with ANVISA in the first half of 2019.

Table of Contents

hATTR amyloidosis is a progressive, systemic and fatal inherited disease caused by the abnormal formation of the TTR protein and aggregation of TTR amyloid deposits in various tissues and organs throughout the body, including in peripheral nerves, heart, intestinal tract, eyes, kidneys, central nervous system, thyroid and bone marrow. The progressive accumulation of TTR amyloid deposits in these tissues and organs leads to sensory, motor and autonomic dysfunction often having debilitating effects on multiple aspects of a patient's life. Patients with hATTR amyloidosis often present with a mixed phenotype and experience overlapping symptoms of polyneuropathy and cardiomyopathy. Ultimately, hATTR amyloidosis generally results in death within three to fifteen years of symptom onset. Therapeutic options for the treatment of patients with hATTR amyloidosis are limited and there are currently no disease-modifying drugs approved for the disease. There are an estimated 50,000 patients with hATTR amyloidosis worldwide, including approximately 6,000 patients with polyneuropathic hATTR amyloidosis in Latin America.

Waylivra, is under regulatory review in the EU for the treatment of familial chylomicronemia syndrome, or FCS. The U.S. and EU regulatory agencies have granted Orphan Drug Designation to Waylivra for the treatment of FCS. In August 2018, Waylivra received a Complete Response Letter from the FDA's Division of Metabolism and Endocrinology Products. In the coming weeks, Waylivra will be receiving a notice of noncompliance withdrawal letter from Health Canada. Additionally, Waylivra is currently in Phase 3 clinical development for the treatment of people with familial partial lipodystrophy, or FPL. The EMA has granted orphan drug designation to Waylivra for the treatment of patients with FPL.

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

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

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

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

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

European Economic Area. In July 2018, the European Commission renewed our marketing authorization for Translarna for the treatment of nmDMD in ambulatory patients aged two years and older in the 31 member states of the European Economic Area, or EEA, and it is effective, unless extended, through August 5, 2019. We received initial marketing authorization from the European Commission in August 2014 for the treatment of nmDMD in ambulatory patients aged five years and older. In July 2018, the European Commission approved a label-extension request to our marketing authorization for Translarna in the EEA to include patients from two to up to five years of age. In September 2018, we submitted to the EMA a label-extension request to our marketing authorization in the

EEA to include patients who are non-ambulatory. However, there can be no assurances that we will successfully obtain such label extension.

The marketing authorization is subject to annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of continued authorization, which we refer to as the annual EMA reassessment, as well as our satisfaction of any specific obligation or other requirement placed upon the marketing authorization, including Study 041. Study 041 is a three-year clinical trial to confirm the efficacy and safety of Translarna in the approved patient population. The trial is comprised of two stages: an 18-month randomized, double-blind, placebo controlled clinical trial followed by an 18-month open label extension period. We expect to submit the results of Study 041 to the EMA by the end of the third quarter of 2021. We expect that as part of the annual EMA assessment, the EMA will consider the ongoing status of Study 041. There is substantial risk that

Table of Contents

if we are unable to renew our EEA marketing authorization during any annual renewal cycle, if our product label is materially restricted, or if Study 041 does not provide the data necessary to maintain our marketing authorization, we would lose all, or a significant portion of, our ability to generate revenue from sales of Translarna in the EEA and other territories.

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

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

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

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

Translarna for additional indications

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

Spinal muscular atrophy program

Our spinal muscular atrophy (SMA) collaboration is with F. Hoffman-La Roche Ltd and Hoffman-La Roche Inc., which we refer to collectively as Roche, and the Spinal Muscular Atrophy Foundation, or SMA Foundation. Currently, our collaboration has three clinical trials ongoing to evaluate the safety and effectiveness of risdiplam (RG7916, RO7034067), the lead compound in the SMA program. Sunfish, a two-part clinical study in pediatric and adult type 2 and type 3 SMA patients initiated in the fourth quarter of 2016, followed by the initiation of Firefish in the fourth quarter of 2016, a two-part clinical study in infants with type 1 SMA. In October 2017, Sunfish transitioned into the pivotal second part of its study, which triggered a $20.0 million milestone payment to us from Roche. In March 2018, Firefish transitioned into the pivotal second part of its study, the primary endpoint of which is the proportion of patients sitting without support after 12 months on risdiplam treatment.

Data from the open label extension of part 1 of the Sunfish trial were presented in October 2018 at the 23rd International Annual Congress of the World Muscle Society, or the World Muscle conference. Risdiplam was well tolerated at all doses and there have been no drug-related safety findings leading to withdrawal. In Sunfish, the data demonstrate that the previously reported median greater than 2-fold SMN protein level increase is maintained over 52 weeks of treatment indicating the durability of the pharmacodynamic effect. Interim clinical data from the Firefish

trial were also presented in October 2018 at the World Muscle conference. The median age of first dose was 6.7 months and babies have received risdiplam for a duration of up to 20.3 months. Risdiplam has been well tolerated at all doses and there have been no drug-related safety findings leading to withdrawal. At day 245 of treatment, over 90% of the babies achieved a greater than 4-point increase in CHOP-INTEND score compared to baseline, a rating to evaluate the motor skills of patients with type 1 SMA developed by the Children's Hospital of Philadelphia. The CHOP-INTEND data were further supported by video footage presented by a principal investigator in the trial, who showed a video of an additional type 1 SMA baby sitting unassisted, bringing the total to 4 babies sitting unassisted as shown in patient videos. Natural history indicates that type 1 SMA babies never achieve this milestone. The video also showed type 1 SMA babies from the FIREFISH trial demonstrating head control and rolling. Moreover, no babies have required a tracheostomy or permanent

Table of Contents

ventilation since study initiation and no baby has lost the ability to swallow. Previously published natural history data indicate that in comparable historic cohorts the median age of event-free survival for type 1 SMA infants is between 8 and 10.5 months. In addition, SMN protein level increases of up to 6.5-fold were observed after 28 days of dosing and the increase was sustained.

Jewelfish, an open-label study investigating the safety, tolerability, PK, and PK/pharmacodynamic relationship of risdiplam in type 2 and type 3 SMA patients who have been previously treated with a survival of motor neuron 2 (SMN2)-targeting therapy, initiated in the first quarter of 2017. Preliminary PD data from ten Jewelfish patients presented at the annual meeting of the Academy of American Neurology in April 2018 and at the 22nd Annual SMA Reasercher meeting organized by CureSMA in June 2018 demonstrate increases in SMN2 FL/SMN7 mRNA ratio and SMN protein level increases of up to 4-fold.

Pre-clinical and other programs

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

In September 2018, our IND submission for PTC 299 for the treatment of acute myeloid leukemia, or AML, became effective. We are currently enrolling patients for a phase 1 AML study.

Funding

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

To date, we have financed our operations primarily through our offering of 3.00% convertible senior notes due August 15, 2022, or the Convertible Notes offering, our public offerings of common stock in February 2014, October 2014 and April 2018, our initial public offering of common stock in June 2013, private placements of our preferred stock, collaborations, bank debt and convertible debt financings, the Credit Agreement and grants and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease areas addressed by our product candidates. Since 2014, we have also relied on revenue generated from net sales of Translarna for the treatment of nmDMD in territories outside of the United States, and in May 2017, we began to recognize revenue generated from net sales of Emflaza for the treatment of DMD in the United States.

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

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

As of September 30, 2018, we had an accumulated deficit of $890.6 million. We had a net loss of $79.8 million and $80.3 million for the nine month periods ended September 30, 2018 and 2017, respectively.

We anticipate that our expenses will continue to increase in connection with our commercialization efforts in the United States, the EEA, 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 our open label extension trials of Translarna for the treatment of nmDMD as well as our studies

for nonsense mutation aniridia and nonsense mutation Dravet syndrome/CDKL5 and our FDA post-marketing requirements with respect to Emflaza in the United States. We also expect to incur ongoing research and development expenses for our other product candidates, including our gene therapy 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 2019, we plan to file requests for marketing

Table of Contents

authorizations for PTC-AADC with the FDA and EMA and 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 will require total funding of $4.5 million annually. Additionally, under the terms of our Credit Agreement cash interest payments are payable monthly in arrears. Furthermore, as a result of our initial public offering in June 2013, we have incurred and expect to continue to incur additional costs associated with operating as a public company including significant legal, accounting, investor relations and other expenses.

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

Financial operations overview

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

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

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

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

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

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

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

Research and development expense

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

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

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

Table of Contents

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

We use our employee and infrastructure resources across multiple research projects, including our drug development programs. We track expenses related to our clinical programs and certain preclinical programs on a per project basis. We expect our research and development expenses to increase in connection with our ongoing activities, particularly in connection with Study 041 for Translarna for the treatment of nmDMD, our studies of Translarna in nonsense mutation aniridia and nonsense mutation Dravet syndrome/CDKL5, activities under our gene therapy 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 tables provide research and development expense for our most advanced principal product development programs, for the three and nine months ended September 30, 2018 and 2017.

	Three Months Ended September 30,	
	2018	2017
	(in thousands)	
Translarna (nmDMD, nmCF, nmMPS I, aniridia and Dravet)	$33,878	$20,834
Oncology	3,495	555
Next generation nonsense readthrough	1,653	1,365
Emflaza	4,670	1,859
Other research and preclinical	10,672	5,411
Total research and development	$54,368	$30,024

	Nine Months Ended September 30,	
	2018	2017
	(in thousands)	
Translarna (nmDMD, nmCF, nmMPS I, aniridia and Dravet)	$68,552	$61,276
Oncology	8,467	2,735
Next generation nonsense readthrough	4,905	4,145
Emflaza	10,676	4,303
Other research and preclinical	25,737	15,763
Total research and development	$118,337	$88,222

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

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

· the scope, rate of progress and expense of our clinical trials and other research and development activities;
· the potential benefits of our products and product candidates over other therapies;
· our ability to market, commercialize and achieve market acceptance for any of our products or product candidates that we are developing or may develop in the future, including our ability to negotiate pricing and reimbursement terms acceptable to us and to obtain or maintain marketing authorizations we have or may receive for our products and product candidates;

·clinical trial results;
·the terms and timing of regulatory approvals; and

Table of Contents

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

Revenue recognition

Periods prior to January 1, 2018

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

Net product sales

Prior to the second quarter of 2017, our net product sales consisted of sales of Translarna for the treatment of nmDMD in territories outside of the U.S. We recognize revenue from product sales when there is persuasive evidence that an arrangement exists, title to product and associated risk of loss has passed to the customer, the price is fixed or determinable, collectability is reasonably assured and we have no further performance obligations in accordance with Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC") Subtopic 605-15, Revenue Recognition—Products.

We have recorded revenue on sales where Translarna is available either on a commercial basis or through a reimbursed EAP program. Orders for Translarna are generally received from hospital and retail pharmacies and our

third-party partner distributors. Revenue is recognized when risk of ownership has transferred. Our third-party partner distributors act as intermediaries between us and end users and do not typically stock significant quantities of Translarna. The ultimate payor for Translarna is typically a government authority or institution or a third-party health insurer.

Table of Contents

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

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

Collaboration and grant revenue

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

We evaluate all contingent consideration earned, such as a milestone payment, using the criteria as provided by ASC 605-28, Revenue Recognition—Milestone Method. At the inception of a collaboration arrangement, we evaluate if a milestone payment is substantive. The criteria requires that (1) we determine if the milestone is commensurate with either its performance to achieve the milestone or the enhancement of value resulting from our activities to achieve the milestone; (2) the milestone be related to past performance; and (3) the milestone be reasonable relative to all deliverable and payment terms of the collaboration arrangement. If these criteria are met then the contingent milestones can be considered a substantive milestone and will be recognized as revenue in the period that the milestone is achieved. We recognize royalties as earned in accordance with the terms of various research and collaboration agreements. If not substantive, the contingent consideration is allocated to the existing units of accounting based on relative selling price and recognized following the same basis previously established for the associated unit of accounting.

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

Periods commencing January 1, 2018

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

Net Product Revenue

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

We record product sales net of any variable consideration, which includes discounts, allowances, rebates and distribution fees. We use the expected value or most likely amount method when estimating variable consideration, unless discount or rebate terms are specified within contracts. Historically, returns of Translarna and Emflaza were

immaterial to our financial statements. The identified variable consideration is recorded as a reduction of revenue at the time revenues from product sales are recognized. These estimates for variable consideration are adjusted to reflect known changes in factors and may impact such estimates in the quarter those changes are known. Revenue recognized does not include amounts of variable consideration that are constrained.

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

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

Table of Contents

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

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

Collaboration Revenue

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

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

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

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

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

Inventory and cost of product sales

Inventory

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

We periodically review inventory for excess amounts or obsolescence and write down obsolete or otherwise unmarketable inventory to its estimated net realizable value. We recorded a $1.6 million writedown of inventory for the three month period ended September 30, 2018 primarily related to inventory labeling changes. Additionally,

though our products are subject to strict quality control and monitoring which is performed throughout the manufacturing processes, certain batches or units of product may not meet quality specifications resulting in a charge to cost of product sales.

Cost of product sales

Table of Contents

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

Accrued expenses

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

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

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. We utilize the "income method", and use 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 our 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.

Results of operations

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

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

	Three Months Ended September 30,		Change 2018 vs. 2017
(in thousands)	2018	2017	
Net product revenue	$53,021	$41,780	$11,241
Collaboration and grant revenue	570	73	497
Cost of product sales, excluding amortization of acquired intangible asset	3,292	1,582	1,710
Amortization of acquired intangible asset	5,793	9,716	(3,923)
Research and development expense	54,368	30,024	24,344
Selling, general and administrative expense	38,368	31,423	6,945
Interest expense, net	(3,118)	(3,421)	303
Other income, net	734	766	(32)
Income tax expense	(355)	(191)	(164)

48

Table of Contents

Net product revenues. Net product revenues were $53.0 million for the three months ended September 30, 2018, an increase of $11.2 million, or 27%, from $41.8 million for the three months ended September 30, 2017. The increase in net product revenue was primarily due to the increase in revenue of Emflaza in the United States, which launched in May 2017.

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

Cost of product sales, excluding amortization of acquired intangible asset. Cost of product sales, excluding amortization of acquired intangible asset, were $3.3 million for the three months ended September 30, 2018 and $1.6 million for the three months ended September 30, 2017. Cost of product sales, excluding amortization of acquired intangible asset, consist primarily of royalty payments associated with Emflaza and Translarna net product sales and costs associated with Emflaza and Translarna product sold during the period. For Translarna sold in 2017, the majority of related manufacturing costs incurred had previously been expensed prior to January 1, 2017 as research and development expenses.

Amortization of acquired intangible asset. Amortization of the acquired intangible asset was $5.8 million for the three months ended September 30, 2018 and $9.7 million for the three months ended September 30, 2017, resulting from the acquisition of all rights to Emflaza. The amount allocated to the Emflaza intangible asset will be amortized on a straight-line basis over its estimated useful life of approximately seven years from the date of the completion of our acquisition of all rights to Emflaza, the period of estimated future cash flows.

Research and development expense. Research and development expense was $54.4 million for the three months ended September 30, 2018, an increase of $24.3 million, or 81%, from $30.0 million for the three months ended September 30, 2017. The increase was primarily due to increased investment in research programs and the advancement of the clinical pipeline, as well as the Akcea upfront licensing fee of $12.0 million paid during the current quarter.

Selling, general and administrative expense. Selling, general and administrative expense was $38.4 million for the three months ended September 30, 2018, an increase of $6.9 million, or 22%, from $31.4 million for the three months ended September 30, 2017. The increase resulted primarily from the continued investment in commercial activities for Emflaza, which launched in May 2017, and Translarna, as well as $1.5 million in Agilis acquisition related expenses.

Interest expense, net. Interest expense, net was $3.1 million for the three months ended September 30, 2018, a decrease of $0.3 million, or 9%, from $3.4 million for the three months ended September 30, 2017. 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 income, net. Other income, net was $0.7 million for the three months ended September 30, 2018, a decrease in income of $0.03 million, or 4%, from other income, net of $0.8 million for the three months ended September 30, 2017. The decrease in other income, net resulted primarily from exchange rate changes in the current period.

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

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

Nine months ended September 30, 2018 compared to nine months ended September 30, 2017

Table of Contents

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

	Nine Months Ended September 30,		Change 2018 vs.
(in thousands)	2018	2017	2017
Net product revenue	$177,172	$116,113	$61,059
Collaboration and grant revenue	1,224	249	975
Cost of product sales, excluding amortization of acquired intangible asset	8,909	2,142	6,767
Amortization of acquired intangible asset	16,815	9,952	6,863
Research and development expense	118,337	88,222	30,115
Selling, general and administrative expense	104,882	85,788	19,094
Interest expense, net	(9,306)	(8,648)	(658)
Other income (expense), net	1,066	(1,373)	2,439
Income tax expense	(964)	(507)	(457)

Net product revenues. Net product revenues were $177.2 million for the nine months ended September 30, 2018, an increase of $61.1 million, or 53%, from $116.1 million for the nine months ended September 30, 2017. The increase in net product revenue was primarily due to the increase in net product revenue in existing markets where Translarna is available as well as continued geographic expansion into new territories, in addition to net product revenue of Emflaza in the United States, which launched in May 2017.

Collaboration and grant revenues. Collaboration and grant revenues were $1.2 million for the nine months ended September 30, 2018, an increase of $1.0 million, or 392%, from $0.2 million for the nine months ended September 30, 2017. These revenues are from ongoing collaboration arrangements.

Cost of product sales, excluding amortization of acquired intangible asset. Cost of product sales, excluding amortization of acquired intangible asset, were $8.9 million for the nine months ended September 30, 2018 and $2.1 million for the nine months ended September 30, 2017. Cost of product sales, excluding amortization of acquired intangible asset, consist primarily of royalty payments associated with Emflaza and Translarna net product sales and costs associated with Emflaza and Translarna product sold during the period. For Translarna sold in 2017, the majority of related manufacturing costs incurred had previously been expensed prior to January 1, 2017 as research and development expenses.

Amortization of acquired intangible asset. Amortization of the acquired intangible asset was $16.8 million for the nine months ended September 30, 2018 and $10.0 million for the nine months ended September 30, 2017, resulting from the acquisition of Emflaza. The amount allocated to the Emflaza intangible asset will be amortized on a straight-line basis over its estimated useful life of approximately seven years from the date of the completion of our acquisition of all rights to Emflaza, the period of estimated future cash flows.

Research and development expense. Research and development expense was $118.3 million for the nine months ended September 30, 2018, an increase of $30.1 million, or 34%, from $88.2 million for the nine months ended September 30, 2017. The increase was primarily due to increased investment in research programs and the advancement of the clinical pipeline, as well as the Akcea upfront licensing fee of $12.0 million paid during the current period.

Selling, general and administrative expense. Selling, general and administrative expense was $104.9 million for the nine months ended September 30, 2018, an increase of $19.1 million, or 22%, from $85.8 million for the nine months ended September 30, 2017. The increase resulted primarily from the continued investment in commercial activities for Emflaza, which launched in May 2017, and Translarna, as well as $1.5 million in Agilis acquisition related expenses.

Interest expense, net. Interest expense, net was $9.3 million for the nine months ended September 30, 2018, an increase in expense of $0.7 million, or 8%, from interest expense of $8.6 million for the nine months ended September 30, 2017. The increase in interest expense was primarily due to current year interest expense recorded from the Convertible Notes and the Credit Agreement, partially offset by interest income from investments.

Other income (expense), net. Other income, net was $1.1 million for the nine months ended September 30, 2018, and other expense, net was $1.4 million for nine months ended September 30, 2017. The change in other income

(expense), net was primarily from foreign currency fluctuations in exchange rates in the current period.

Income tax expense. Income tax expense was $1.0 million for the nine months ended September 30, 2018 and $0.5 million for the nine months ended September 30, 2017. We are subject to income taxes in the United States, although currently not a tax payer,

Table of Contents

and various foreign jurisdictions, and our foreign tax liabilities are largely dependent upon the distribution of pre-tax earnings among these different jurisdictions.

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

Liquidity and capital resources

Sources of liquidity

Since inception, we have incurred significant operating losses.

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

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

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

On May 5, 2017, we entered into the Credit Agreement with MidCap Financial, which provides for a senior secured term loan facility of $60 million, of which $40 million was drawn by us on May 5, 2017. The remaining $20 million under the senior secured term loan facility would become available to us upon our demonstration (on or prior to December 31, 2018) of net product revenue equaling or exceeding $120 million for the trailing 12 month period. The

maturity date of the Credit Agreement is May 1, 2021, unless terminated earlier. The facility is structured to require only monthly interest payments for the initial two years with principal amortization beginning in years three and four. The facility bears interest at a rate per annum equal to LIBOR (with a LIBOR floor rate of 1.00%) plus 6.15%, as well as additional upfront and administrative fees and expenses.

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

Table of Contents

Cash flows

As of September 30, 2018, we had cash, cash equivalents and marketable securities of $249.4 million.

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

	Nine Months Ended September 30,	
(in thousands)	2018	2017
Cash provided by (used in):		
Operating activities	(12,498)	(31,351)
Investing activities	(18,017)	67,159
Financing activities	127,845	42,367

Net cash used in operating activities was $12.5 million for the nine months ended September 30, 2018 and $31.4 million for the nine months ended September 30, 2017. The net cash used in operating activities primarily relates to supporting clinical development and commercial activities, partially offset by increased cash receipts resulting from higher net product revenues.

Net cash used in investing activities was $18.0 million for the nine months ended September 30, 2018 and net cash provided by investing activities was $67.2 million for the nine months ended September 30, 2017. Cash used in investing activities for the nine months ended September 30, 2018 was primarily related to the acquisition of Agilis and royalty payments related to Emflaza product rights, partially offset by the net redemption of marketable securities. Cash provided by investing activities for the nine months ended September 30, 2017 was primarily related to the net redemption of marketable securities, partially offset by acquisition costs associated with the Emflaza asset acquisition.

Net cash provided by financing activities was $127.8 million for the nine months ended September 30, 2018 and $42.4 million for the nine months ended September 30, 2017. Cash provided by financing activities for the nine months ended September 30, 2018 is primarily attributable to the April 2018 equity offering and the exercise of options and issuance of stock under the ESPP. Cash provided by financing activities for the nine months ended September 30, 2017 is primarily attributable to borrowings under the Credit Agreement and the exercise of options and issuance of stock under the ESPP.

Funding requirements

We anticipate that our expenses will increase in connection with our commercialization efforts in the United States, the EEA, Latin America and other territories, including expansion of our infrastructure and corresponding sales and marketing, legal and regulatory, distribution and manufacturing and administrative and employee-based expenses. In addition to the foregoing, we expect to continue to incur significant costs in connection with Study 041 and our open label extension trials of Translarna for the treatment of nmDMD as well as our studies for nonsense mutation aniridia and nonsense mutation Dravet syndrome/CDKL5 and our FDA post-marketing requirements with respect to Emflaza in the United States. We also expect to incur ongoing research and development expenses for our other product candidates, including our gene therapy and oncology program. In addition, we may incur substantial costs in connection with our efforts to advance our regulatory submissions. We have begun seeking and intend to continue to seek marketing authorization for Translarna for the treatment of nmDMD in territories outside of the EEA and we may also seek marketing authorization for Translarna for other indications. In 2019, we plan to file requests for marketing authorizations for PTC-AADC with the FDA and EMA and 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;

Table of Contents

- are required to take other steps, in addition to Study 041, to maintain our current marketing authorization in the EEA for Translarna for the treatment of nmDMD or to obtain further marketing authorizations for Translarna for the treatment of nmDMD or other indications in the EEA or elsewhere;
- initiate or continue the research and development of Translarna for additional indications and of our other product candidates, including for our gene therapy and oncology programs;
- seek to discover and develop additional product candidates;
- seek to expand and diversify our product pipeline through strategic transactions;
- maintain, expand and protect our intellectual property portfolio; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and commercialization efforts.

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

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

- our ability to commercialize and market Tegsedi and Waylivra in the PTC Territory;
- our ability to commercialize and market Emflaza for the treatment of DMD in the United States;
- our ability to negotiate, secure and maintain adequate pricing, coverage and reimbursement terms, on a timely basis, with third-party payors for Emflaza for the treatment of DMD in the United States and for Translarna for the treatment of nmDMD in the EEA and other territories outside of the United States;
- our ability to maintain orphan exclusivity for, and successfully complete all FDA post-marketing requirements with respect to, Emflaza, or to obtain an additional six-month period of pediatric exclusivity;
- our ability to maintain the marketing authorization in the EEA for Translarna for the treatment of nmDMD, including whether the EMA determines on an annual basis that the benefit-risk balance of Translarna supports renewal of our marketing authorization in the EEA, on the current approved label;
- the costs, timing and outcome of Study 041;
- the costs, timing and outcome of our efforts to advance Translarna for the treatment of nmDMD in the United States, including, whether we will be required to perform additional clinical trials, non-clinical studies or CMC assessments or analyses at significant cost which, if successful, may enable FDA review of an NDA submission by us and, ultimately, may support approval of Translarna for nmDMD in the United States;
- the progress and results of our open label extension clinical trials of Translarna for the treatment of nmDMD as well as our studies for nonsense mutation aniridia and nonsense mutation Dravet syndrome/CDKL5 and activities under our gene therapy 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 for additional indications and for our other product candidates, including those in our gene therapy and oncology programs;

Table of Contents

- 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 will require total funding of $4.5 million annually. Furthermore, as a result of our initial public offering in June 2013, we have incurred and expect to continue to incur additional costs associated with operating as a public company. These costs include significant legal, accounting, investor relations and other expenses that we did not incur as a private company.

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

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

Off-Balance Sheet Arrangements

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

Contractual obligations

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

(in thousands)	Total	Less than 1 year	1 - 3 years	4 - 5 years	More than 5 years
Deferred consideration payable (1)	$40,000		40,000	—	—

(1) Pursuant to the Merger Agreement with Agilis, we are required to pay $40.0 million of development milestone payments, or deferred consideration payments, no later than the second anniversary of the closing of the Merger, regardless of

Table of Contents

whether the applicable milestones have been achieved. The payment schedule above reflects our expected timing of when the payments will be made as of September 30, 2018. The fair value of the deferred consideration payments at the acquisition date was estimated to be $38.2 million.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

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

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

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

Changes in Internal Control over Financial Reporting

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

Table of Contents

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

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

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

Item 1A. Risk Factors

We have set forth in Item 1A to our Annual Report on Form 10-K for the year ended December 31, 2017, risk factors relating to our business, our industry, our structure and our common stock. Readers of this Quarterly Report on Form 10-Q are referred to such Item 1A for a more complete understanding of risks concerning us. There have been no material changes in our risk factors since those published in such Form 10-K for the year ended December 31, 2017, other than as reported in Item Part II Item 1A on our Form 10-Q for the periods ended March 31, 2018 and June 30, 2018, and in Exhibit 99.2 to our Current Report on Form 8-K filed on August 24, 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 September 30, 2018, we issued options to purchase an aggregate of 653,900 shares of common stock to certain new hire employees at a weighted-average exercise price of $43.36 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.

Table of Contents

Item 6. Exhibits.

Exhibit Number	Description of Exhibit
2.1†	Agreement and Plan of Merger, dated July 19, 2018, by and among PTC Therapeutics, Inc., Agility Merger Sub, Inc., Agilis Biotherapeutics, Inc., and solely in its capacity as equityholder representative, Shareholder Representative Services LLC (incorporated by reference to Exhibit 2.1 to the Current Report on Form 8-K filed by the Registrant on July 19, 2018)
10.1	Bridge Loan and Security Agreement, dated as July 19, 2018, by and among PTC Therapeutics, Inc., Agilis Biotherapeutics, Inc., and the Guarantors as defined therein (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed by the Registrant on July 19, 2018)
10.2	Amendment No. 1 and Limited Consent to Credit and Security Agreement, dated as of July 19, 2018, by and among PTC Therapeutics, Inc., MidCap Financial trust and the Lenders as defined therein (incorporated by reference to Exhibit 10.2 to the Current Report on Form 8-K filed by the Registrant on July 19, 2018)
10.3††	Collaborative Research Agreement, dated September 30, 2015, as amended, by and between National Taiwan University and Agilis Biotherapeutics, Inc. (formerly Agilis Biotherapeutics, LLC)
10.4††	License and Technolgy Transfer Agreement, dated December 23, 2015, by and between National Taiwan University and Agilis Biotherapeutics, Inc. (formerly Agilis Biotherapeutics, LLC)
10.5††	Collaboration and License Agreement, dated August 1, 2018, by and between PTC Therapeutics International Limited and Akcea Therapeutics, Inc.
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*

† Confidential treatment has been granted for certain portions that are omitted from this exhibit. The omitted information has been filed separately with the U.S. Securities and Exchange Commission (the “SEC”) pursuant to the registrant’s application for confidential treatment. In addition, schedules have been omitted from this exhibit pursuant to Item 601(b)(2) of Regulation S-K. A copy of any omitted schedule will be furnished supplementally to the SEC upon request; provided, however, that the registrant may request confidential treatment for any document so furnished.

†† Confidential treatment has been requested for certain portions that are omitted from this exhibit. The omitted information has been filed separately with the U.S. Securities and Exchange Commission (the “SEC”) pursuant to the registrant’s application for confidential treatment.

* Submitted electronically herewith.

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

57

Table of Contents

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: November 5, 2018 By: /s/ Christine Utter
Christine Utter
Principal Financial Officer
(Principal Financial and Accounting Officer and Duly Authorized Signatory)