PTC THERAPEUTICS, INC.
Form 10-Q
November 05, 2018
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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended 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<br>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.

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FORWARD-LOOKING STATEMENTS

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

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

- Our ability to realize the anticipated benefits of our acquisition of Agilis Biotherapeutics, Inc., or Agilis, including the possibility that the expected impact of benefits from the acquisition, including with respect to the business of Agilis and our expectations with respect to the potential achievement of development, regulatory and sales milestones and our contingent payments to the former Agilis equityholders with respect thereto, will not be realized or will not be realized within the expected time period, significant transaction costs, the integration of Agilis's operations and employees into our business, our ability to obtain marketing approval of our gene therapy for the treatment of Aromatic L-Amino Acid Decarboxylase, or AADC, deficiency, or PTC-AADC, and other product candidates we acquired from Agilis, unknown liabilities, the risk of litigation and/or regulatory actions related to the acquisition, and other business effects, including the effects of industry, market, economic, political or regulatory conditions;
- our ability to negotiate, secure and maintain adequate pricing, coverage and reimbursement terms and processes on a timely basis, or at all, with third-party payors for Emflaza™ (deflazacort) for the treatment of Duchenne muscular dystrophy, or DMD, in the United States and for Translarna™ (ataluren) for the treatment of nonsense mutation DMD, or nmDMD, in the European Economic Area, or EEA, and other countries in which we have or may obtain regulatory approval, or in which there exist significant reimbursed early access programs, or EAP programs;
- our ability to maintain our marketing authorization of Translarna for the treatment of nmDMD in the EEA (which is subject to the specific obligation to conduct and submit the results of Study 041 to the European Medicines Agency, or EMA, and annual review and renewal by the European Commission following reassessment of the benefit-risk balance of the authorization by the EMA);
- our ability to enroll, fund, and complete Study 041, a multicenter, randomized, double-blind, 18-month, placebo-controlled clinical trial of Translarna for the treatment of nmDMD followed by an 18-month open label extension, according to the protocol agreed with the EMA, and by the trial’s deadline;
- the anticipated period of market exclusivity for Emflaza for the treatment of DMD in the United States under the Orphan Drug Act of 1983, or the Orphan Drug Act, the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act 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,

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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;

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- 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.

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PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

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

| | 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.

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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.

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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.

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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.

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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.

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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

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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

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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

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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.

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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.

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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

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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.

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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:

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| | 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

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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.

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The following represents the fair value using the hierarchy described above for the Company's financial assets and liabilities that are required to be measured at fair value on a recurring basis as of 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.

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The unrealized losses and fair values of available-for-sale securities that have been in an unrealized loss position for a period of less than and greater than 12 months as of 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.

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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

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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

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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.

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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.

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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.

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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

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(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, | |