UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended March 31, 2020

or

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

For the transition period from to

Commission file number: 001-35969

PTC Therapeutics, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**04-3416587**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
100 Corporate Court **South Plainfield, NJ**	**07080**
(Address of principal executive offices)	(Zip Code)

(908) 222-7000

(Registrant's telephone number, including area code)

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

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

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☑ No ☐

Indicate by check mark whether the registrant has submitted electronically 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 ☑ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer", "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☑	Accelerated filer	☐
Non-accelerated filer	☐	Smaller reporting company	☐
		Emerging growth company	☐

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

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☑

As of April 28, 2020, there were 62,875,395 shares of Common Stock, $0.001 par value per share, outstanding.

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PTC Therapeutics, Inc.

FORWARD-LOOKING STATEMENTS

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

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

- expectations with respect to our gene therapy platform, including any potential regulatory submissions and potential approvals, including those related to our gene therapy for the treatment of Aromatic L-Amino Acid Decarboxylase, or AADC deficiency, or PTC-AADC, our manufacturing capabilities and the potential financial impact and benefits of our leased biologics manufacturing facility and the potential achievement of development, regulatory and sales milestones and contingent payments that we may be obligated to make;

- 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 our products or product candidates that we commercialize or may commercialize in the future;

- our ability to maintain our marketing authorization of Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, in the European Economic Area, or 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 EMA'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 Orphan Drug Act, the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act;

- our ability to complete any dystrophin study necessary in order to resolve the matters set forth in the United States Food and Drug Administration's, or the FDA, 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 commercialization of our products and product candidates;

- our expectations with respect to the COVID-19 pandemic and related response measures and their effects on our business, operations, clinical trials, potential regulatory submissions and approvals, our collaborators, contract research organizations, suppliers and manufacturers;

- our ability to obtain additional and maintain existing reimbursed named patient and cohort early access programs, or EAP programs, for our products on adequate terms, or at all;

- our expectations with respect to the development, regulatory and commercial 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;

- our expectations and the potential financial impact and benefits related to our Collaboration and Licensing Agreement with Akcea Therapeutics, Inc., or Akcea, including with respect to the timing of regulatory approval of Tegsedi™ (inotersen) and Waylivra™ (volanesorsen) in countries in which we are licensed to commercialize them, the 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 our products or product candidates, 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 in our gene therapy, splicing, Bio-e and oncology programs as well as studies in our products for maintaining authorizations, label extensions and additional indications, including the timing of initiation, enrollment and completion of the trials and the period during which the results of the trials will become available;

- our ability to realize the anticipated benefits of our acquisitions or other strategic transactions, including the possibility that the expected impact of benefits from the acquisitions or strategic transactions will not be realized or will not be realized within the expected time period, significant transaction costs, the integration of operations and employees into our business, our ability to obtain marketing approval of our product candidates we acquired from the acquisitions or other strategic transactions and unknown liabilities;

- the rate and degree of market acceptance and clinical utility of any of our products or product candidates;

- the ability and willingness of patients and healthcare professionals to access our product and product candidates through alternative means if pricing and reimbursement negotiations in the applicable territory do not have a positive outcome;

- our ability to complete the FDA post-marketing requirements to the marketing authorization of Emflaza and any other post-marketing requirements for our products;

- the timing of, and our ability to obtain additional marketing authorizations for our products and product candidates;

- the ability of our products and our product candidates to meet existing or future regulatory standards;

- our ability to maintain the current labeling under the marketing authorization in the EEA or expand the approved product label of Translarna for the treatment of nmDMD;

- the potential receipt of revenues from future sales of our products or product candidates;

- 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 our products and product candidates 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 our products and product candidates that are sufficient to meet clinical trial and commercial launch requirements;

- our ability to establish and grow our manufacturing capabilities for our gene therapy platform;

- our expectations with respect to the potential financial impact and benefits of our leased biologics manufacturing facility and our ability to satisfy our obligations under the terms of the lease agreement for such facility;

- 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 ability to satisfy our obligations under the indenture governing our 3.00% convertible senior notes due August 15, 2022 and under the indenture governing our 1.50% convertible senior notes due September 15, 2026;

- our regulatory submissions, including with respect to timing and outcome of regulatory review;

- our plans to advance our earlier stage programs and pursue research and development of other product candidates, including our splicing, gene therapy, Bio-e and oncology programs;

- whether we may pursue business development opportunities, including potential collaborations, alliances, and acquisition or licensing of assets and our ability to successfully develop or commercialize any assets to which we may gain rights pursuant to such business development opportunities;

- the potential advantages of our products and any product candidate;

- our intellectual property position;

- the impact of government laws and regulations;
- the impact of litigation that has been or may be brought against us or of litigation that we are pursuing against others; and
- our competitive position.

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, 2019, that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make.

You should read this Quarterly Report on Form 10-Q and the documents that we have filed as exhibits to this Quarterly Report on Form 10-Q and our Annual Report on Form 10-K for the year ended December 31, 2019 completely and with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward-looking statements whether as a result of new information, future events or otherwise, except as required by applicable law.

In this Quarterly Report on Form 10-Q, unless otherwise stated or the context otherwise requires, references to “PTC,” “PTC Therapeutics,” “the Company,” “we,” “us,” “our,” and similar references refer to PTC Therapeutics, Inc. and, where appropriate, its subsidiaries. The trademarks, trade names and service marks appearing in this Quarterly Report on Form 10-Q are the property of their respective owners.

All website addresses given in this Quarterly Report on Form 10-Q are for information only and are not intended to be an active link or to incorporate any website information into this document.

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

PTC Therapeutics, Inc.
Consolidated Balance Sheets (unaudited)
In thousands (except shares)

	March 31, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 123,287	$ 288,028
Marketable securities	472,572	398,535
Trade receivables, net	52,623	55,538
Inventory, net	19,133	19,285
Prepaid expenses and other current assets	24,307	17,898
Total current assets	691,922	779,284
Fixed assets, net	26,169	21,549
Intangible assets, net	705,294	710,500
Goodwill	82,341	82,341
Deposits and other assets	39,866	30,108
Total assets	$ 1,545,592	$ 1,623,782
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 145,386	$ 159,276
Current portion of long-term debt	20,000	20,000
Deferred revenue	8,638	8,242
Other current liabilities	3,644	8,339
Deferred consideration payable	40,000	40,000
Total current liabilities	217,668	235,857
Deferred revenue- long term	1,768	3,415
Long-term debt	294,574	293,859
Contingent consideration payable	357,200	356,300
Deferred tax liability	130,862	130,862
Other long-term liabilities	14,786	9,159
Total liabilities	1,016,858	1,029,452
Stockholders' equity:		
Common stock, $0.001 par value. Authorized 125,000,000 shares; issued and outstanding 62,758,520 shares at March 31, 2020. Authorized 125,000,000 shares; issued and outstanding 61,935,870 shares at December 31, 2019.	62	62
Additional paid-in capital	1,834,061	1,795,351
Accumulated other comprehensive income	(1,985)	(10,584)
Accumulated deficit	(1,303,404)	(1,190,499)
Total stockholders' equity	528,734	594,330
Total liabilities and stockholders' equity	$ 1,545,592	$ 1,623,782

See accompanying unaudited notes.

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

	Three Months Ended March 31,	
	2020	2019
Revenues:		
Net product revenue	$ 68,196	$ 53,054
Collaboration and grant revenue	63	529
Total revenues	68,259	53,583
Operating expenses:		
Cost of product sales, excluding amortization of acquired intangible assets	4,085	2,376
Amortization of acquired intangible assets	7,949	6,077
Research and development	90,107	52,566
Selling, general and administrative	58,209	40,544
Change in the fair value of deferred and contingent consideration	900	21,160
Total operating expenses	161,250	122,723
Loss from operations	(92,991)	(69,140)
Interest expense, net	(5,642)	(2,288)
Other expense, net	(13,832)	(109)
Loss before income tax expense	(112,465)	(71,537)
Income tax expense	(222)	(576)
Net loss attributable to common stockholders	$ (112,687)	$ (72,113)
Weighted-average shares outstanding:		
Basic and diluted (in shares)	62,389,158	55,855,111
Net loss per share—basic and diluted (in dollars per share)	$ (1.81)	$ (1.29)

See accompanying unaudited notes.

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

	Three Months Ended March 31,	
	2020	**2019**
Net loss	$ (112,687)	$ (72,113)
Other comprehensive (loss) income:		
Unrealized (loss) gain on marketable securities, net of tax	(63)	59
Foreign currency translation gain (loss), net of tax	8,662	(716)
Comprehensive loss	$ (104,088)	$ (72,770)

See accompanying unaudited notes.

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

Three months ended March 31, 2020	Common stock		Additional paid-in capital	Accumulated other comprehensive (loss) income	Accumulated deficit	Total stockholders' equity
	Shares	Amount				
Balance, December 31, 2019	61,935,870	$ 62	$ 1,795,351	$ (10,584)	$ (1,190,499)	$ 594,330
Issuance of common stock related to equity offerings	262,205	—	13,503	—	—	13,503
Exercise of options	379,684	—	9,987	—	—	9,987
Restricted stock vesting and issuance, net	180,761	—	—	—	—	—
Share-based compensation expense	—	—	15,220	—	—	15,220
Other	—	—	—	—	(218)	(218)
Net loss	—	—	—	—	(112,687)	(112,687)
Comprehensive income	—	—	—	8,599	—	8,599
Balance, March 31, 2020	62,758,520	$ 62	$ 1,834,061	$ (1,985)	$ (1,303,404)	$ 528,734

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

See accompanying unaudited notes.

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

	Three Months Ended March 31,	
	2020	2019
Cash flows from operating activities		
Net loss	$ (112,687)	$ (72,113)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	9,235	7,059
Non-cash lease expense	1,330	—
Change in valuation of deferred and contingent consideration	900	21,160
Unrealized loss on equity investment in Clearpoint	1,574	—
Unrealized loss on Clearpoint convertible debt security	2,799	—
Non-cash interest expense	5,459	1,982
Amortization of discounts on investments, net	(333)	(257)
Amortization of debt issuance costs	256	139
Share-based compensation expense	15,220	9,263
Unrealized foreign currency transaction losses, net	8,675	865
Changes in operating assets and liabilities:		
Inventory	102	(334)
Prepaid expenses and other current assets	(6,675)	(191)
Trade receivables, net	2,346	20,786
Deposits and other assets	(5,819)	(10,754)
Accounts payable and accrued expenses	(4,000)	(28,653)
Other liabilities	1,264	8,065
Deferred revenue	(1,200)	574
Net cash used in operating activities	(81,554)	(42,409)
Cash flows from investing activities		
Purchases of fixed assets	(6,023)	(2,865)
Purchase of convertible debt security	(10,000)	—
Purchases of marketable securities	(298,814)	(165,723)
Sale and redemption of marketable securities	224,997	18,090
Acquisition of product rights and licenses	(11,434)	—
Net cash used in investing activities	(101,274)	(150,498)
Cash flows from financing activities		
Proceeds from exercise of options	9,987	1,281
Net proceeds from public offerings	13,503	224,441
Repayment of senior secured term loan	(5,000)	—
Net cash provided by financing activities	18,490	225,722
Effect of exchange rate changes on cash	(403)	(1,169)
Net increase in cash and cash equivalents	(164,741)	31,646
Cash and cash equivalents, and restricted cash beginning of period	295,528	169,498
Cash and cash equivalents, and restricted cash end of period	$ 130,787	$ 201,144
Supplemental disclosure of cash information		
Cash paid for interest	$ 4,878	$ 3,111
Cash paid for income taxes	$ 507	$ 537
Supplemental disclosure of non-cash investing and financing activity		
Unrealized (loss) gain on marketable securities, net of tax	$ (63)	$ 59
Right-of-use assets obtained in exchange for lease obligations	$ 22,642	$ 11,314
Acquisition of product rights and licenses	$ 2,775	$ —

See accompanying unaudited notes.

PTC Therapeutics, Inc.
Notes to Consolidated Financial Statements (unaudited)
March 31, 2020
In thousands (except per share data unless otherwise noted)

1. The Company

PTC Therapeutics, Inc. (the "Company" or "PTC") is a science-driven 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 diversified 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's strategy is to bring best-in-class therapies with differentiated clinical benefit to patients affected by rare disorders and to leverage its global commercial infrastructure to maximize value for its patients and other stakeholders.

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 has marketing authorization in the European Economic Area (the "EEA") for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, in ambulatory patients aged 2 years and older and in Brazil for the treatment of nmDMD in ambulatory patients aged 5 years and older, subject to annual renewal and other conditions. Emflaza is approved in the United States for the treatment of DMD in patients two years and older.

The Company has a pipeline of gene therapy product candidates for rare monogenic diseases that affect the central nervous system ("CNS") including PTC-AADC for the treatment of Aromatic L-Amino Acid Decarboxylase, or AADC deficiency, a rare CNS disorder arising from reductions in the enzyme AADC that results from mutations in the dopa decarboxylase gene. 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 United States Food and Drug Administration, or FDA, in the second half of 2020. In January 2020, the Company submitted a marketing authorization application, or MAA, to the European Medicines Agency ("EMA") for PTC-AADC for the treatment of AADC deficiency in the EEA, and the Company expects an opinion from the Committee for Medicinal Products for Human Use by the end of 2020.

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 pursuant to the Company's Collaboration and License Agreement with Akcea Therapeutics, Inc. ("Akcea"). Tegsedi has received marketing authorization in the United States, the European Union (the "EU") and Brazil for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis, or hATTR amyloidosis. Waylivra has received marketing authorization in the EU for the treatment of familial chylomicronemia syndrome, or FCS. The Company anticipates filing for marketing authorization for Waylivra with ANVISA, the Brazilian health regulatory authority, in the second half of 2020.

The Company also has a spinal muscular atrophy ("SMA") collaboration with F. Hoffman-La Roche Ltd and Hoffman-La Roche Inc., referred to collectively as Roche, and the Spinal Muscular Atrophy Foundation, or SMA Foundation. The lead compound in the SMA program is risdiplam (RG7916, RO7034067). Roche submitted an NDA for risdiplam to the FDA in the fourth quarter of 2019. In April 2020, the FDA extended the Prescription Drug User Fee Act ("PDUFA") date for a decision from May 24, 2020 to August 24, 2020 as a result of additional data that Roche submitted, including comprehensive data from the Sunfish part 2 study. Risdiplam is expected to be indicated in the United States for SMA type 1, 2 and 3 patients, if approved. Roche anticipates submitting an MAA for risdiplam in the EEA in mid-year 2020.

On October 25, 2019, the Company completed the acquisition of substantially all of the assets of BioElectron Technology Corporation ("BioElectron"), a Delaware corporation, including certain compounds that the Company has begun to develop as part of its Bio-e platform, (the "Asset Acquisition") pursuant to an Asset Purchase Agreement by and between the Company and BioElectron, dated October 1, 2019 (the "Asset Acquisition Agreement"). The transaction was accounted for as an asset acquisition. In 2020, the Company plans to initiate three trials in this platform with two unique compounds that regulate inflammation and oxidative stress.

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

The Company's marketing authorization for Translarna in the EEA is subject to annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of the authorization, which the Company refers to

as the annual EMA reassessment. This marketing authorization is further subject to the specific obligation to conduct and submit the results of a multi-center, randomized, double-blind, 18-month, placebo-controlled trial, followed by an 18-month open-label extension, according to an agreed protocol, in order to confirm the efficacy and safety of Translarna. The final report on the trial and open-label extension is to be submitted by the Company to the EMA by the end of the third quarter of 2022. The Company refers to the trial and open-label extension together as Study 041.

The marketing authorization in the EEA was last renewed in July 2019 and is effective, unless extended, through August 5, 2020. 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 February 2020, the Company submitted a marketing authorization renewal request to the EMA.

Translarna is an investigational new drug in the United States. During the first quarter of 2017, the Company filed a New Drug Application, or NDA, over protest with the FDA, for which the FDA granted a standard review. In October 2017, the Office of Drug Evaluation I of the FDA issued a complete response letter for the NDA, stating that it was unable to approve the application in its current form. In response, the Company filed a formal dispute resolution request with the Office of New Drugs of the FDA. In February 2018, the Office of New Drugs of the FDA denied the Company's appeal of the Complete Response Letter. In its response, the Office of New Drugs recommended a possible path forward for the ataluren NDA submission based on the accelerated approval pathway. This would involve a re-submission of an NDA containing the current data on effectiveness of ataluren with new data to be generated on dystrophin production in nmDMD patients' muscles. The Company intends to follow the FDA's recommendation and will collect, using newer technologies via procedures and methods that the Company designed, such dystrophin data in a new study, Study 045, which the Company initiated in the fourth quarter of 2018. The Company expects that data for Study 045 will be available in the third quarter of 2020 followed by a potential re-submission of an NDA thereafter. Additionally, should a re-submission of an NDA receive accelerated approval, the Office of New Drugs stated that Study 041, which is currently enrolling, could serve as the confirmatory post-approval trial required in connection with the accelerated approval framework.

On April 20, 2017, the Company completed its acquisition of all rights to Emflaza, or the Transaction. 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 upon the passing of the second anniversary of the closing of the Merger, regardless of whether the applicable milestones have been achieved.

Upon the closing of the Asset Acquisition, the Company paid to BioElectron total upfront consideration of $10.0 million, funded with cash on hand, less (i) transaction expenses incurred by BioElectron, (ii) the amount of outstanding indebtedness of BioElectron

including a $4.0 million loan advance to BioElectron plus accrued and unpaid interest thereon and (iii) $1.5 million held in an escrow account to secure potential indemnification obligations owed to the Company. Subject to the terms and conditions of the Asset Acquisition Agreement, BioElectron may become entitled to receive contingent milestone payments of up to $200.0 million (in cash or in shares of the Company's common stock, as determined by the Company) from the Company based on the achievement of certain regulatory and net sales milestones. Subject to the terms and conditions of the Asset Acquisition Agreement, BioElectron may also become entitled to receive contingent payments based on a percentage of net sales of certain products.

As of March 31, 2020, the Company had an accumulated deficit of approximately $1,303.4 million. The Company has financed its operations to date primarily through the private offerings in September 2019 of 1.50% convertible senior notes due 2026 and in August 2015 of 3.00% convertible senior notes due 2022 (see Note 10), public offerings of common stock in February 2014, October 2014, April 2018, January 2019, and September 2019, "at the market offerings" of its common stock, its initial public offering of common stock in June 2013, private placements of its convertible preferred stock, collaborations, bank debt, grant funding and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease area addressed by the Company's product candidates. Since 2014, the Company has also relied on revenue generated from net sales of Translarna for the treatment of nmDMD in territories outside of the United States, and since May 2017, the Company has generated revenue from net sales of Emflaza for the treatment of DMD in the United States. The Company expects that cash flows from the sales of its products, together with the Company's cash, cash equivalents and marketable securities, will be sufficient to fund its operations for at least the next twelve months.

2. Summary of significant accounting policies

The Company's complete listing of significant accounting policies is set forth in Note 2 of the notes to the Company's audited financial statements as of December 31, 2019 included in the Company's Annual Report on Form 10-K filed with the Securities and Exchange Commission (the "SEC") on March 2, 2020 (the "2019 Form 10-K"). Additional significant accounting policies adopted during the three month period ended March 31, 2020 are discussed in further detail below.

Basis of presentation

The accompanying financial information as of March 31, 2020 and for the three months ended March 31, 2020 and 2019 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, 2019 and notes thereto included in the 2019 Form 10-K.

In the opinion of management, the unaudited financial information as of March 31, 2020 and for the three months ended March 31, 2020 and 2019 reflects all adjustments, which are normal recurring adjustments, necessary to present a fair statement of financial position, results of operations, stockholders' equity, and cash flows. The results of operations for the three month period ended March 31, 2020 are not necessarily indicative of the results to be expected for the year ended December 31, 2020 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, valuation procedures for convertible notes, 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.

Restricted cash

Restricted cash included in deposits and other assets on the consolidated balance sheet relates to an unconditional, irrevocable and transferable letter of credit that was entered into during the twelve-month period ended December 31, 2019 in connection with obligations under a facility lease for our leased biologics manufacturing facility in Hopewell Township, New Jersey. The amount of the letter of credit is $7.5 million, is to be maintained for a term of not less than five years and has the potential to be reduced to $3.8 million if after five years the Company is not in default of its lease. The amount is classified within deposits and other assets on the consolidated balance sheet due to the long-term nature of the letter of credit.

The following table provides a reconciliation of cash, cash equivalents, and restricted cash reported within the consolidated balance sheet that sum to the total of the same amounts shown in the statement of cash flows:

	Beginning of period- December 31, 2019	End of period- March 31, 2020
Cash and cash equivalents	$ 288,028	$ 123,287
Restricted cash included in deposits and other assets	7,500	7,500
Total Cash, cash equivalents and restricted cash per statement of cash flows	$ 295,528	$ 130,787

Marketable securities

The Company considers securities with original maturities of greater than 90 days to be available for sale securities. Securities under this classification are recorded at fair value and unrealized gains and losses within accumulated other comprehensive income. The estimated fair value of the available for sale securities is determined based on quoted market prices or rates for similar instruments. In addition, the cost of debt securities in this category is adjusted for amortization of premium and accretion of discount to maturity. For available for sale debt securities in an unrealized loss position, the Company assesses whether it intends to sell or if it is more likely than not that the Company will be required to sell the security before recovery of its amortized cost basis. If either of the criteria regarding intent or requirement to sell is met, the security's amortized cost basis is written down to fair value. If the criteria are not met, the Company evaluates whether the decline in fair value has resulted from a credit loss or other factors. In making this assessment, management considers, among other factors, the extent to which fair value is less than amortized cost, any changes to the rating of the security by a rating agency, and adverse conditions specifically related to the security. If this assessment indicates that a credit loss exists, the present value of cash flows expected to be collected from the security are compared to the amortized cost basis of the security. If the present value of the cash flows expected to be collected is less than the amortized cost basis, a credit loss exists and an allowance for credit losses is recorded for the credit loss, limited by the amount that the fair value is less than the amortized costs basis. Any impairment that has not been recorded through an allowance for credit losses is recognized in other comprehensive income. For the three-month period ended March 31, 2020, no allowance was recorded for credit losses.

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. Products 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. Amounts related to clinical development programs and marketing efforts are immaterial.

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

	March 31, 2020	December 31, 2019
Raw materials	$ 871	$ 874
Work in progress	9,482	9,652
Finished goods	8,780	8,759
Total inventory	$ 19,133	$ 19,285

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. For the three month period ended March 31, 2020, the Company recorded a $0.2 million inventory write-down, primarily related to product approaching expiration. No write downs were recorded for the three month period ended March 31, 2019. Additionally, though the Company’s product is subject to strict quality control and monitoring which it performs throughout the manufacturing processes, certain batches or units of product may not meet quality specifications resulting in a charge to cost of product sales. For the three month periods ended March 31, 2020 and 2019, 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. Production costs are expensed as cost of product sales when the related products are sold.

Revenue recognition

Net product revenue

The Company's net product revenue primarily consists of sales of Translarna in territories outside of the U.S. for the treatment of nmDMD and sales of Emflaza in the U.S. for the treatment of DMD. The Company recognizes revenue when its performance obligations with its customers have been satisfied. The Company's performance obligations are to provide products 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 the product, 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 the 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 product sales. 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 related to Medicaid and other government pricing programs, 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. 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. For the three months ended March 31, 2020 and 2019, net product sales outside of the United States were $40.7 million and $35.3 million respectively, and net product sales in the United States were $27.5 million and $17.8 million respectively.

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. The Company considers any shipping and handling costs that are incurred after the customer has obtained control of the product as a cost to fulfill a promise. Shipping and handling costs associated with finished goods delivered to customers are recorded as a selling expense.

Collaboration revenue

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

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

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

For milestone payments, the Company assesses, at contract inception, whether the development or sales-based milestones are considered probable of being achieved. If it is probable that a significant revenue reversal will occur, the Company will not record revenue until the uncertainty has been resolved. Milestone payments that are contingent upon regulatory approval are not considered probable of being achieved until the applicable regulatory approvals or other external conditions are obtained as such conditions are not within the Company's control. If it is probable that a significant revenue reversal will not occur, the Company will estimate

the milestone payments using the most likely amount method. The Company will re-assess the development and sales-based milestones each reporting period to determine the probability of achievement.

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

Allowance for doubtful accounts

The Company maintains an allowance for estimated losses resulting from the inability of its customers to make required payments. The Company estimates uncollectible amounts based upon current customer receivable balances, the age of customer receivable balances, the customer's financial condition and current economic trends. The allowance for doubtful accounts was $0.2 million as of March 31, 2020 and $0.3 million as of December 31, 2019. Bad debt expense was immaterial for the three-month periods ended March 31, 2020 and 2019. For the three-month period ended March 31, 2020, no allowance was recorded for credit losses.

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 and license agreement assets acquired in business combinations are capitalized. Several methods may be used to determine the estimated fair value of the IPR&D and license agreement asset 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. Intangible assets with indefinite lives, including IPR&D, are tested for impairment if impairment indicators arise and, at a minimum, annually. However, an entity is permitted to first assess qualitative factors to determine if a quantitative impairment test is necessary. Further testing is only required if the entity determines, based on the qualitative assessment, that it is more likely than not that an indefinite-lived intangible asset's fair value is less than its carrying amount. Otherwise, no further impairment testing is required. The indefinite-lived intangible asset impairment test consists of a one-step analysis that compares the fair value of the intangible asset with its carrying amount. If the carrying amount of an intangible asset exceeds its fair value, an impairment loss is recognized in an amount equal to that excess. The Company considers many factors in evaluating whether the value of its intangible assets with indefinite lives may not be recoverable, including, but not limited to, expected growth rates, the cost of equity and debt capital, general economic conditions, the Company's outlook and market performance of the Company's industry and recent and forecasted financial performance.

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 at a reporting unit level on an annual basis or when a triggering event occurs that may indicate the carrying value of the goodwill is impaired. The Company reassess its reporting units as part of its annual segment review. An entity is permitted to first assess qualitative factors to determine if a quantitative impairment test is necessary. Further testing is only required if the entity determines, based on the qualitative assessment, that it is more likely than not that the fair value of the reporting unit is less than its carrying amount.

Income Taxes

On March 27, 2020, the United States enacted the Coronavirus Aid, Relief, and Economic Security Act, referred to herein as the CARES Act, as a response to the economic uncertainty resulting from a strain of novel coronavirus, COVID-19. The CARES Act includes modifications for net operating loss carryovers and carrybacks, limitations of business interest expense for tax, immediate refund of alternative minimum tax (AMT) credit carryovers as well as a technical correction to the 2017 Tax Cuts and Jobs Act ("the 2017 Tax Act"), referred to herein as the U.S. Tax Act, for qualified improvement property. As of March 31, 2020, the Company expects that these provisions will not have a material impact. Tax provisions of the Act also include the deferral of certain payroll taxes, relief for retaining employees, and other provisions. The Company is evaluating the impact of the Act and currently expects to benefit from the deferral of certain payroll taxes and retention credit through the end of calendar year 2020. The ultimate impact of the CARES Act may differ from this estimate due to changes in interpretations and guidance that may be issued and actions the Company may take in response to the CARES Act. The Company will continue to assess the impact that various provisions will have on its business.

On December 22, 2017, the U.S. government enacted 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 March 31, 2020.

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

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

Leases

The Company determines if an arrangement is a lease at inception. This determination generally depends on whether the arrangement conveys to the Company the right to control the use of an explicitly or implicitly identified fixed asset for a period of time in exchange for consideration. Control of an underlying asset is conveyed to the Company if the Company obtains the rights to direct the use of and to obtain substantially all of the economic benefits from using the underlying asset. The Company has lease agreements which include lease and non-lease components, which the Company accounts for as a single lease component for all leases. Operating leases are classified as right of use ("ROU") assets, short term lease liabilities, and long term lease liabilities. Operating lease ROU assets and lease liabilities are recognized at the commencement date based on the present value of lease payments over the lease term. ROU assets are amortized and lease liabilities accrete to yield straight-line expense over the term of the lease. Lease payments included in the measurement of the lease liability are comprised of fixed payments.

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

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

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

The lease term for all of the Company's leases includes the non-cancellable period of the lease plus any additional periods covered by either a Company option to extend (or not to terminate) the lease that the Company is reasonably certain to exercise, or an option to extend (or not to terminate) the lease controlled by the lessor. Leasehold improvements are capitalized and depreciated over the lesser of useful life or lease term. See Note 3 Leases for additional information.

Impact of recently adopted accounting pronouncements

In December 2019, the FASB issued ASU 2019-12,"Income Taxes (Topic 740): Simplifying the Accounting for Income Taxes". ASU 2019-12 simplifies the accounting for income taxes by removing certain exceptions to the general principals in Topic 740. The amendments also improve consistent application of and simplify GAAP for other areas of Topic 740 by clarifying and amending the existing guidance. For public business entities, the guidance is effective for annual periods, and interim periods within those annual periods, beginning after December 15, 2020. For all other entities, it is effective for annual periods beginning after December 15, 2021 and interim periods in annual periods beginning after December 15, 2022. Early adoption is permitted, including adoption in any interim period. The Company early adopted this guidance January 1, 2020. The adoption of the guidance did not have a material impact on the consolidated financial statements and accompanying notes.

In June 2016, the FASB issued ASU No. 2016-13, “Financial Instruments — Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments”. This standard requires financial assets measured at amortized cost basis to be presented at the net amount expected to be collected. This standard is effective for public companies who are SEC filers for fiscal years beginning after December 15, 2019, including interim periods within those years. In November 2019, the FASB issued ASU 2019-11, Codification Improvements to Topic 326, Financial Instruments - Credit Losses, which expands the scope of the practical expedient that allows entities to exclude the accrued interest component of amortized cost from various disclosures required by ASC 326 to also include certain disclosures required by ASC 320. Entities that elect to apply the practical expedient must disclose the total amount of accrued interest that they exclude from their disclosures of amortized cost. The amendments have the same effective dates as ASU 2016-13 (Topic 326) for entities that have not yet adopted that standard. The Company adopted ASU 2016-13 and ASU 2019-11 effective January 1, 2020. The adoption of the guidance did not have a material impact on the consolidated financial statements. The Company has updated its accounting policy for marketable securities within this footnote as well as its fair value footnote (Note 4) with additional disclosures as required by the standard upon adoption.

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 adopted this guidance January 1, 2020. The adoption of the guidance did not have a material impact on the consolidated financial statements. The Company has updated its fair value footnote (Note 4) with additional and modified disclosures as required by the standard upon adoption.

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 adopted this guidance January 1, 2020. The adoption of the guidance did not have a material impact on the consolidated financial statements and accompanying notes.

In November 2018, the FASB issued ASU 2018-18,"Collaborative Arrangements (Topic 808): Clarifying the Interaction between Topic 808 and Topic 606”. ASU 2018-18 provides guidance on whether certain transactions between collaborative arrangement participants should be accounted for with revenue under Topic 606. For public business entities, the guidance is effective for annual periods, and interim periods within those annual periods, beginning after December 15, 2019. For all other entities, it is effective for annual periods beginning after December 15, 2020 and interim periods in annual periods beginning after December 15, 2021. Early adoption is permitted, including adoption in any interim period for all entities. The Company adopted this guidance January 1, 2020. The adoption of the guidance did not have a material impact on the consolidated financial statements and accompanying notes.

3. Leases

The Company leases office space in South Plainfield, New Jersey for its principal office under three noncancelable operating leases through May 2022 and August 2024, in addition to office space in various countries for international employees primarily through workspace providers. The Company also leases certain vehicles, lab equipment, and office equipment under operating leases. The Company’s operating leases have remaining lease terms ranging from 0.1 years to 7.2 years and certain of the leases include renewal options to extend the lease for up to 10 years. Rent expense was approximately $2.4 million and $1.0 million for the three month periods ended March 31, 2020 and 2019.

The components of lease expense were as follows:

	Three Months Ended March 31, 2020	Three Months Ended March 31, 2019
Operating Lease Cost		
Fixed lease cost	$ 2,118	$ 812
Variable lease cost	228	143
Short-term lease cost	77	53
Total operating lease cost	$ 2,423	$ 1,008

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

Supplemental balance sheet information related to leases was as follows:

	March 31, 2020	December 31, 2019
Operating lease ROU asset	$ 17,671	$ 13,693
Operating lease liabilities- current	$ 3,589	$ 5,153
Operating lease liabilities- noncurrent	14,645	9,018
Total operating lease liability	$ 18,234	$ 14,171

Operating lease ROU asset is a component of deposits and other assets on the consolidated balance sheets. The current portion of operating lease liability is a component of other current liabilities on the consolidated balance sheets. The long term portion of operating lease liabilities is a component of other long term liabilities on the consolidated balance sheets.

Supplemental lease term and discount rate information related to leases was as follows as of March 31, 2020 and 2019:

	March 31, 2020	March 31, 2019
Weighted-average remaining lease terms - operating leases (years)	5.35	4.71
Weighted-average discount rate - operating leases	7.34%	7.02%

Supplemental cash flow information related to leases was as follows as of March 31, 2020 and 2019:

	Three Months Ended March 31, 2020	Three Months Ended March 31, 2019
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows from operating leases	$ 1,971	$ 732
Right-of-use assets obtained in exchange for lease obligations:		
Operating leases	$ 22,642	$ 11,314

Future minimum lease payments under non-cancelable leases as of March 31, 2020 were as follows:

	Operating Leases
2020 (excludes the three-months ended March 31, 2020)	$ 3,543
2021	4,372
2022	3,846
2023	3,435
2024 and thereafter	6,900
Total lease payments	22,096
Less: Imputed Interest	3,862
Total	$ 18,234

In conjunction with the Asset Acquisition, the Company acquired BioElectron’s lease in Mountainview, California. As substantially all of the fair value of the gross assets acquired was related to PTC743, the relative fair value allocated to the right of use asset and corresponding lease liability for the Mountainview lease was determined to be immaterial, and accordingly is not included in the tables above. The future minimum lease payments for the Mountainview lease as of March 31, 2020 are $1.3 million, $1.8 million, and $1.4 million for 2020, 2021 and 2022, respectively.

As of March 31, 2020, the Company had two operating leases that had not yet commenced, and accordingly, are not reflected in the tables above. On March 20, 2020, the Company entered into a lease agreement with COE Bridgewater LLC relating to the

lease of office and laboratory space located in Bridgewater, New Jersey. This lease will replace the Company's existing lease on the property beginning on May 1, 2020 and will include additional rental property of approximately 59,000 square feet. Additional obligations stem from the occupancy of the additional space which has not yet commenced as of March 31, 2020.

On August 4, 2019, the Company and Bristol-Myers Squibb Company, (the “Landlord”), entered into a Lease Agreement (the “Lease”), relating to the lease of approximately 185,000 square feet of office, manufacturing and laboratory space at a facility located in Hopewell Township, New Jersey (the “Campus”). On March 25, 2020, the Company entered into an amendment increasing the rented space to approximately 220,500 square feet. The term of occupancy has not yet commenced as of March 31, 2020.

The rental term of the Lease is estimated to commence on July 1, 2020 (the “Commencement Date”). Upon the Commencement Date, the Lease has an initial term of fifteen years (the “Initial Term”), with two consecutive five year renewal periods, each at the Company’s option.

The aggregate rent for the Initial Term will be approximately $111.5 million. The rental rate for the renewal periods will be 95% of the Prevailing Market Rate (as defined in the Lease) and determined at the time of the exercise of the renewal. The Company is also responsible for maintaining certain insurance and the payment of proportional taxes, utilities and common area operating expenses. The Lease contains customary events of default, representations, warranties and covenants.

Subject to the terms of the Lease, the Company has a right of first refusal to rent certain other space of the Campus, which would be triggered upon the Landlord’s issuance of a second round proposal or letter of intent to another tenant for such space. The Company also may seek to build a new separate building on the Campus, which may not contain less than 75,000 square feet (the “New Building”). Upon receipt of notice of the Company’s intention to build the New Building, the Landlord may, in its sole discretion, construct and lease the New Building to the Company or enter into a ground lease with the Company permitting the Company to construct the New Building. Rent terms for the New Building would be determined based on the land value, construction and project costs subject to whether the Landlord or Company constructs the New Building.

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 marketable securities are reflected in the accompanying financial statements at fair value. The carrying amount of receivables and accounts payable and accrued expenses approximates fair value due to the short-term nature of those instruments. The carrying amounts for borrowings under the credit and security agreement with MidCap Financial approximate fair value based on market activity for other debt instruments with similar characteristics and comparable risk.

In May 2019, the Company purchased $4.0 million of shares of ClearPoint Neuro, Inc.'s ("ClearPoint"), formerly known as MRI Interventions, Inc., common stock, at a purchase price of $3.10 per share, in connection with a securities purchase agreement that the Company entered into with ClearPoint, a publicly traded medical device company. The Company determined that the equity investment represents a financial instrument and therefore, recorded it at fair value, which is readily determinable. The equity investment is a component of deposits and other assets on the consolidated balance sheet. During the three month period ended March 31, 2020, the Company recorded an unrealized loss of $1.6 million, which is a component of other expense, net within the consolidated statement of operations. The fair value of the equity investment was $4.6 million as of March 31, 2020. The Company classifies its equity investment in ClearPoint as a Level 1 asset within the fair value hierarchy, as the value is based on a quoted market price in an active market, which is not adjusted.

In January 2020, the Company purchased a $10.0 million convertible note from Clearpoint that the Company can convert into Clearpoint shares at a conversion rate of $6.00 per share at any point throughout the term of the loan, which matures five years from the purchase date. The Company determined that the convertible note represents an available for sale debt security and the Company has elected to record it at fair value under ASC 825. The Company classifies its Clearpoint convertible debt security as a Level 2 asset within the fair value hierarchy, as the value is based on inputs other than quoted prices that are observable. The fair value of the Clearpoint convertible debt security is determined at each reporting period by utilizing a Black-Scholes option pricing model, as well as a present value of expected cash flows from the debt security utilizing the risk free rate and the estimated credit spread as of the valuation date as the discount rate. During the three month period ended March 31, 2020, the Company recorded an unrealized loss of $2.8 million, which is a component of other expense, net within the consolidated statement of operations. The fair value of the convertible debt security was $7.2 million as of March 31, 2020. The convertible debt security is considered to be long term and is included as a component of deposits and other assets on the consolidated balance sheet. Other than the equity investment and the convertible debt security, no other items included in deposits and other assets on the consolidated balance sheets are fair valued.

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 following represents the fair value using the hierarchy described above for the Company's financial assets and liabilities that are required to be measured at fair value on a recurring basis as of March 31, 2020 and December 31, 2019:

	March 31, 2020			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities	$ 472,572	$ —	$ 472,572	$ —
Equity investment in ClearPoint	$ 4,619	$ 4,619	$ —	$ —
ClearPoint convertible debt security	$ 7,201	$ —	$ 7,201	$ —
Deferred consideration payable	$ 40,000	$ —	$ 40,000	$ —
Contingent consideration payable- development and regulatory milestones	$ 273,900	$ —	$ —	$ 273,900
Contingent consideration payable- net sales milestones and royalties	$ 83,300	$ —	$ —	$ 83,300

	December 31, 2019			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities	$ 398,535	$ —	$ 398,535	$ —
Equity investment in ClearPoint	$ 6,194	$ 6,194	$ —	$ —
Stock appreciation rights liability	$ 3,186	$ —	$ —	$ 3,186
Deferred consideration payable	$ 40,000	$ —	$ 40,000	$ —
Contingent consideration payable- development and regulatory milestones	$ 290,500	$ —	$ —	$ 290,500
Contingent consideration payable- net sales milestones and royalties	$ 65,800	$ —	$ —	$ 65,800

No transfers of assets between Level 1, Level 2, or Level 3 of the fair value measurement hierarchy occurred during the periods ended March 31, 2020 and December 31, 2019.

The following is a summary of marketable securities accounted for as available-for-sale securities at March 31, 2020 and December 31, 2019:

	March 31, 2020			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 76,324	$ 149	$ —	$ 76,473
Corporate debt securities	259,525	1,086	(649)	259,962
Asset-backed securities	45,702	111	(114)	45,699
Government obligations	90,412	28	(2)	90,438
Total	$ 471,963	$ 1,374	$ (765)	$ 472,572

	December 31, 2019			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 157,936	$ 162	$ —	$ 158,098
Corporate debt securities	188,778	576	(20)	189,334
Asset-backed securities	51,062	49	(8)	51,103
Total	$ 397,776	$ 787	$ (28)	$ 398,535

For available for sale debt securities in an unrealized loss position, the Company assesses whether it intends to sell or if it is more likely than not that the Company will be required to sell the security before recovery of its amortized cost basis. If either of the criteria regarding intent or requirement to sell is met, the security's amortized cost basis is written down to fair value. For the three-month period ended March 31, 2020, no write downs occurred. The Company does not intend to sell the investments and it is not more likely than not that the Company will be required to sell the investments before recovery of their amortized cost bases, which may be maturity. The Company also reviews its available for sale debt securities in an unrealized loss position and evaluates whether the decline in fair value has resulted from credit losses or other factors. This review is subjective, as it requires management to evaluate whether an event or change in circumstances has occurred in that period that may be related to credit issues. For the three-month period ended March 31, 2020, no allowance was recorded for credit losses. Unrealized gains and losses are reported as a component of accumulated other comprehensive (loss) income in stockholders' equity.

As of March 31, 2020 and December 31, 2019, the Company had no realized gains/losses from the sale of marketable securities.

The unrealized losses and fair values of available-for-sale securities that have been in an unrealized loss position for a period of less than and greater than 12 months as of March 31, 2020 are as follows:

	March 31, 2020					
	Securities in an unrealized loss position less than 12 months		Securities in an unrealized loss position greater than 12 months		Total	
	Unrealized losses	Fair Value	Unrealized losses	Fair Value	Unrealized losses	Fair Value
Commercial paper	$ —	$ —	$ —	$ —	$ —	$ —
Corporate debt securities	(649)	168,871	—	—	(649)	168,871
Asset-backed securities	(114)	28,750	—	—	(114)	28,750
Government obligations	(2)	9,998	—	—	(2)	9,998
Total	$ (765)	$ 207,619	$ —	$ —	$ (765)	$ 207,619

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, 2019 are as follows:

	December 31, 2019					
	Securities in an unrealized loss position less than 12 months		Securities in an unrealized loss position greater than 12 months		Total	
	Unrealized losses	Fair Value	Unrealized losses	Fair Value	Unrealized losses	Fair Value
Corporate debt securities	$ (20)	$ 71,779	$ —	$ —	$ (20)	$ 71,779
Asset-backed securities	(8)	24,211	—	—	(8)	24,211
Total	$ (28)	$ 95,990	$ —	$ —	$ (28)	$ 95,990

Marketable securities on the balance sheet at March 31, 2020 and December 31, 2019 mature as follows:

	March 31, 2020	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 76,473	$ —
Corporate debt securities	196,826	63,136
Asset-backed securities	36,574	9,125
Government obligations	70,439	19,999
Total Marketable securities	$ 380,312	$ 92,260

	December 31, 2019	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 158,098	$ —
Corporate debt securities	139,596	49,738
Asset-backed securities	44,724	6,379
Total Marketable securities	$ 342,418	$ 56,117

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

Convertible senior notes

In August 2015, the Company issued $150.0 million of 3.00% convertible senior notes due August 15, 2022 (the “2022 Convertible Notes”). In September 2019, the Company issued $287.5 million of 1.50% convertible senior notes due September 15, 2026 (the “2026 Convertible Notes,” together with the “2022 Convertible Notes,” the “Convertible Notes”). 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 2022 Convertible Notes at March 31, 2020 and December 31, 2019 was $161.6 million and $171.2 million, respectively. The estimated fair value of the 2026 Convertible Notes at March 31, 2020 and December 31, 2019 was $305.3 million and $335.0 million, respectively.

Deferred consideration payable

Pursuant to the Merger Agreement, Agilis equityholders may become entitled to receive contingent consideration payments from the Company based on the achievement of certain development milestones up to an aggregate maximum amount of $60.0 million and the achievement of certain regulatory approval milestones together with a milestone payment following the receipt of a priority review voucher up to an aggregate maximum amount of $535.0 million. The Company is required to pay $40.0 million of development milestone payments upon the passing of the second anniversary of the closing of the Merger, regardless of whether the applicable milestones have been achieved. The fair value of the deferred consideration payable at March 31, 2020 was estimated to be $40.0 million. The Company did not apply a discount, as the milestones will be paid within one calendar year. Accordingly, as of March 31, 2020, the $40.0 million of the deferred consideration payable was classified as current on the balance sheet.

Level 3 valuation

The stock appreciation rights ("SARs") liability is classified in other liabilities on the Company’s consolidated balance sheets. The SARs liability is marked-to-market each reporting period with the change in fair value recorded as compensation expense on the Company’s consolidated statements of operations until the SARs vest. The fair value of the SARs liability is determined at each reporting period by utilizing the Black-Scholes option pricing model. The last payment of the SARs liability was made in the three-month period ended March 31, 2020, and accordingly, the balance of the SARS liability as of March 31, 2020 was $0.

The contingent consideration payable is fair valued each reporting period with the change in fair value recorded as a gain or loss within the change in the fair value of deferred and contingent consideration on the consolidated statements of operations. The fair value of the development and regulatory milestones is estimated utilizing a probability adjusted, discounted cash flow approach. The discount rates are estimated utilizing Corporate B rated bonds maturing in the years of expected payments based on the Company’s estimated development timelines for the acquired product candidate. At March 31, 2020, the weighted average discount rate for the development and regulatory milestones was 6.0% and the weighted average probability of success was 57%. The fair value of the net sales milestones and royalties is determined utilizing an option pricing model with Monte Carlo simulation to

simulate a range of possible payment scenarios, and the average of the payments in these scenarios is then discounted to calculate present fair value. At March 31, 2020, the weighted average discount rate for the net sales milestones and royalties was 11.5% and the weighted average probability of success for the net sales milestones was 50%.

The table presented below is a summary of changes in the fair value of the Company's Level 3 valuations for the SARs liability, and the contingent consideration payable for the period ended March 31, 2020 and March 31, 2019 .

	Level 3 liabilities		
	SARs	Contingent consideration payable- development and regulatory milestones	Contingent consideration payable- net sales milestones and royalties
Beginning balance as of December 31, 2019	$ 3,186	$ 290,500	$ 65,800
Additions	—	—	—
Change in fair value	—	(16,600)	17,500
Payments	(3,186)	—	—
Ending balance as of March 31, 2020	$ —	$ 273,900	$ 83,300

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

The following significant unobservable inputs were used in the valuation of the contingent consideration payable for the periods ended March 31, 2020 and December 31, 2019 and of the SARs liability for the period ended December 31, 2019:

	March 31, 2020			
	Fair Value	Valuation Technique	Unobservable Input	Range
Contingent consideration payable- development and regulatory milestones	$273,900	Probability-adjusted discounted cash flow	Potential development and regulatory milestones	$0 - $555 million
			Probabilities of success	25% - 94%
			Discount rates	5.2% - 7.1%
			Projected years of payments	2020 - 2026
Contingent considerable payable- net sales milestones and royalties	$83,300	Option-pricing model with Monte Carlo simulation	Potential net sales milestones	$0 - $150 million
			Probabilities of success	25% - 94%
			Potential percentage of net sales for royalties	2% - 6%
			Discount rate	11.5%
			Projected years of payments	2022 - 2038

	December 31, 2019			
	Fair Value	**Valuation Technique**	**Unobservable Input**	**Range**
SARs	$3,186	Option-pricing model	Volatility	28.93%
			Risk free interest rate	0.19%
			Strike price	$6.76 - $30.86
			Fair value of common stock	$48.03
			Expected life	0.01 years
Contingent consideration payable- development and regulatory milestones	$290,500	Probability-adjusted discounted cash flow	Potential development and regulatory milestones	$0 - $555 million
			Probabilities of success	25% - 94%
			Discount rates	2.2% - 4.7%
			Projected years of payments	2020 - 2026
Contingent considerable payable- net sales milestones and royalties	$65,800	Option-pricing model with Monte Carlo simulation	Potential net sales milestones	$0 - $150 million
			Probabilities of success	25% - 89%
			Potential percentage of net sales for royalties	2% - 6%
			Discount rate	14.5%
			Projected years of payments	2021 - 2038

The contingent consideration payables are classified Level 3 liabilities as their 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 approaches, including but not limited to, assumptions involving probability adjusted sales estimates for the gene therapy 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 months ended March 31, 2020:

	Unrealized Gains/(Losses) On Marketable Securities, net of tax	Foreign Currency Translation	Total Accumulated Other Comprehensive Items
Balance at December 31, 2019	$ 755	$ (11,339)	$ (10,584)
Other comprehensive (loss) income before reclassifications	(63)	8,662	8,599
Amounts reclassified from other comprehensive items	—	—	—
Other comprehensive (loss) income	(63)	8,662	8,599
Balance at March 31, 2020	$ 692	$ (2,677)	$ (1,985)

6. Accounts payable and accrued expenses

Accounts payable and accrued expenses at March 31, 2020 and December 31, 2019 consist of the following:

	March 31, 2020	December 31, 2019
Employee compensation, benefits, and related accruals	$ 21,770	$ 38,889
Consulting and contracted research	15,227	12,969
Professional fees	5,026	3,562
Sales allowance and other costs	43,229	41,155
Sales rebates and royalties	37,511	42,997
Accounts payable	16,074	10,324
Other	6,549	9,380
Total	$ 145,386	$ 159,276

7. Capitalization

In August 2019, the Company entered into an At the Market Offering Sales Agreement (the "Sales Agreement") with Cantor Fitzgerald and RBC Capital Markets, LLC (together, the "Sales Agents"), pursuant to which, the Company may offer and sell shares of its common stock, having an aggregate offering price of up to $125.0 million from time to time through the Sales Agents by any method that is deemed to be an "at the market offering" as defined in Rule 415(a)(4) promulgated under the Securities Act of 1933, as amended. During the three month period ending March 31, 2020, the Company issued and sold an aggregate of 262,205 shares of common stock pursuant to the Sales Agreement at a weighted average public offering price of $52.81 per share. The Company received net proceeds of $13.5 million after deducting underwriting discounts and commissions and other offering expenses payable by the Company.

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 March 31,	
	2020	2019
Numerator		
Net loss	$ (112,687)	$ (72,113)
Denominator		
Denominator for basic and diluted net loss per share	62,389,158	55,855,111
Net loss per share:		
Basic and diluted	$ (1.81) *	$ (1.29) *

*In the three months ended March 31, 2020 and 2019, the Company experienced a net loss and therefore did not report any dilutive share impact.

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

	As of March 31,	
	2020	2019
Stock Options	12,670,068	10,811,383
Unvested restricted stock awards and units	927,151	695,339
Total	13,597,219	11,506,722

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 Company's Board of Directors approved a grant of 735,324 shares of restricted stock and 4,613 stock options. There are no additional shares available for issuance under this plan.

In 2009, the Company's shareholders approved the 2009 Equity and Long-Term Incentive Plan, which provides for the granting of stock option awards, restricted stock awards, and other stock-based and cash-based awards, subject to certain adjustments and annual increases. In May 2013, the Company's Board of Directors and stockholders increased by 2,500,000 the number of shares authorized under the 2009 Equity and Long Term Incentive Plan, which provides for the granting of stock option awards, restricted stock awards, and other stock-based and cash-based awards. There are no additional shares available for issuance under this plan.

In May 2013, the Company's Board of Directors and stockholders approved the 2013 Long Term Incentive Plan, which became effective upon the closing of the Company's initial public offering. The 2013 Long Term Incentive Plan provides for the grant of incentive stock options, nonstatutory stock options, restricted stock awards and other stock-based awards. The number of shares of common stock reserved for issuance under the 2013 Long Term Incentive Plan is the sum of (1) 122,296 shares of common stock available for issuance under the Company's 2009 Equity and Long Term Incentive Plan and 2013 Stock Incentive Plan, (2) the number of shares (up to 3,040,444 shares) equal to the sum of the number of shares of common stock subject to outstanding awards under the Company's 1998 Employee, Director and Consultant Stock Option Plan, 2009 Equity and Long Term Incentive Plan and 2013 Stock Incentive Plan that expire, terminate or are otherwise surrendered, canceled, forfeited or repurchased by the Company at their original issuance price pursuant to a contractual repurchase right plus (3) an annual increase, to be added on the first day of each fiscal year until the expiration of the 2013 Long Term Incentive Plan, equal to the lowest of 2,500,000 shares of common stock, 4% of the number of shares of common stock outstanding on the first day of the fiscal year and an amount determined by the Company's Board of Directors. As of March 31, 2020, awards for 677,561 shares of common stock are available for issuance under the 2013 Long Term Incentive Plan.

In January 2020, the Company's Board of Directors approved the 2020 Inducement Stock Incentive Plan. The 2020 Inducement Stock Incentive Plan provides for the grant of incentive stock options, nonstatutory stock options, restricted stock awards and other stock-based awards for up to an aggregate of 1,000,000 shares of common stock. Any grants made under the 2020 Inducement Stock Incentive Plan must be made pursuant to the Nasdaq Listing Rule 5635(c)(4) inducement grant exception as a material component of the Company's new hires' employment compensation. As of March 31, 2020, awards for 714,100 shares of common stock are available for issuance under the 2020 Inducement Stock Incentive Plan.

From January 1, 2020 through March 31, 2020, the Company issued a total of 2,152,400 stock options to various employees. Of those, 285,900 were inducement grants for non-statutory stock options, all of which were made pursuant to the 2020 Inducement Stock 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, 2019	11,043,939	$ 31.67		
Granted	2,152,400	$ 51.35		
Exercised	(379,684)	$ 26.47		
Forfeited/Cancelled	(146,587)	$ 50.45		
Outstanding at March 31, 2020	12,670,068	$ 34.95	7.56 years	$ 150,312
Vested or Expected to vest at March 31, 2020	6,097,752	$ 37.81	9.00 years	$ 54,437
Exercisable at March 31, 2020	5,716,856	$ 30.96	5.78 years	$ 90,702

The fair value of grants made in the three months ended March 31, 2020 was contemporaneously estimated on the date of grant using the following assumptions:

	Three months ended March 31, 2020
Risk-free interest rate	0.62 - 1.45%
Expected volatility	87.50 - 89.31%
Expected term	5.75 years

The Company assumed no expected dividends for all grants. The weighted average grant date fair value of options granted during the three-month period ended March 31, 2020 was $37.31 per share.

The expected term of options was estimated based on the Company's historical exercise data and the expected volatility of options was estimated based on the Company's historical stock volatility. The risk-free rate of the options was 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 and Restricted Stock Units—Restricted stock awards and 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 awards and restricted stock units, which have been determined based upon the market value of the Company’s shares on the grant date, are expensed over the vesting period.

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

	Restricted Stock Awards and Units	
	Number of Shares	Weighted Average Grant Date Fair Value
January 1, 2020	642,419	$ 24.50
Granted	535,450	$ 51.16
Vested	(220,361)	$ 22.67
Forfeited	(30,357)	$ 32.07
Unvested at March 31, 2020	927,151	$ 40.23

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 vested annually in equal installments over four years and were settled in cash on each vest date, which required the Company to remeasure the SARs at each reporting period until vesting occurs. For the three month period ended March 31, 2020, a total of 132,136 SARs vested. The last payment of the SARs liability was made in the three-month period ended March 31, 2020, and accordingly, the balance of the SARS liability as of March 31, 2020 was $0.

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 Company's Board of Directors. 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 three month period ended March 31, 2020, the Company recorded $0.4 million in compensation expense related to the ESPP.

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

	Three Months Ended March 31,	
	2020	2019
Research and development	$ 8,179	$ 4,686
Selling, general and administrative	7,041	4,577
Total	$ 15,220	$ 9,263

As of March 31, 2020, there was approximately $200.7 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 Listing Rule 5635(c)(4) 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 3.23 years.

10. Debt

2017 Credit Facility

In May 2017, the Company entered into a credit and security agreement (the "Credit Facility") with MidCap Financial, a Delaware statutory trust, as administrative agent and MidCap Financial and certain other financial institutions as lenders thereunder (the “Credit Agreement”) that provides for a senior secured term loan facility of $60.0 million, of which $40.0 million was drawn by the Company on May 5, 2017. The Company's ability to draw on the remaining $20.0 million under the senior secured term loan facility expired on December 31, 2018. The Company capitalized approximately $0.4 million of debt issuance costs, which were netted against the carrying value of the Credit Facility and will be amortized over the term of the Credit Facility. As of March 31, 2020, the Company had made loan repayments of $16.7 million on the Credit Facility. The remaining balance of the Credit Facility as of March 31, 2020 was $23.3 million, $20.0 million of which was classified as current portion of long term debt and $3.3 million was included within long term debt on the consolidated balance sheet.

Borrowings under the Credit Agreement bear interest at a rate per annum equal to the London Interbank Offered Rate, or LIBOR, (with a LIBOR floor rate of 1.00%) plus 6.15%. The Company was 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 is required to make monthly interest payments and monthly principal payments. The principal payments are to be made based on straight-line amortization of the principal over the twenty-four month period. The maturity date of the Credit Agreement is May 1, 2021, unless terminated earlier.

The Credit Facility is subject to certain financial covenants. As of March 31, 2020, the Company was in compliance with all required covenants.

2026 Convertible Notes

In September 2019, the Company issued, at par value, $287.5 million aggregate principal amount of 1.50% convertible senior notes due 2026, which included an option to purchase up to an additional $37.5 million in aggregate principal amount of the 2026 Convertible Notes. The 2026 Convertible Notes bear cash interest at a rate of 1.50% per year, payable semi-annually on March 15 and September 15 of each year, beginning on March 15, 2020. The 2026 Convertible Notes will mature on September 15, 2026, unless earlier repurchased or converted. The net proceeds to the Company from the offering were $279.3 million after deducting the initial purchasers’ discounts and commissions and the offering expenses payable by the Company.

The 2026 Convertible Notes are governed by an indenture (the "2026 Convertible Notes Indenture") with U.S Bank National Association as trustee (the "2026 Convertible Notes Trustee").

Holders may convert their 2026 Convertible Notes at their option at any time prior to the close of business on the business day immediately preceding March 15, 2026 only under the following circumstances:

- during any calendar quarter commencing on or after December 31, 2019 (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 2026 Convertible Notes Indenture) per $1,000 principal amount of 2026 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 March 15, 2026, until the close of business on the business day immediately preceding the maturity date, holders may convert their 2026 Convertible Notes at any time, regardless of the foregoing circumstances. Upon conversion, the Company will pay or deliver, as the case may be, cash, shares of the Company's common stock or any combination thereof at the Company's election.

The conversion rate for the 2026 Convertible Notes was initially, and remains, 19.0404 shares of the Company's common stock per $1,000 principal amount of the 2026 Convertible Notes, which is equivalent to an initial conversion price of approximately $52.52 per share of the Company's common stock. The conversion rate may be subject to adjustment in some events but will not be adjusted for any accrued and unpaid interest.

The Company is not permitted to redeem the 2026 Convertible Notes prior to September 20, 2023. The Company may redeem for cash all or any portion of the 2026 Convertible Notes, at its option, if the last reported sale price of its common stock has been at least 130% of the conversion price then in effect on the last trading day of, and for at least 19 other trading days (whether or not consecutive) during, any 30 consecutive trading day period ending on, and including, the trading day immediately preceding the date on which the Company provides notice of redemption, at a redemption price equal to 100% of the principal amount of the 2026 Convertible Notes to be redeemed, plus accrued and unpaid interest to, but excluding, the redemption date. No sinking fund is provided for the 2026 Convertible Notes, which means that the Company is not required to redeem or retire the 2026 Convertible Notes periodically.

If the Company undergoes a "fundamental change" (as defined in the 2026 Convertible Notes Indenture), subject to certain conditions, holders of the 2026 Convertible Notes may require the Company to repurchase for cash all or part of their 2026 Convertible Notes at a repurchase price equal to 100% of the principal amount of the 2026 Convertible Notes to be repurchased, plus accrued and unpaid interest to, but excluding, the fundamental change repurchase date.

The 2026 Convertible Notes represent senior unsecured obligations and will rank senior in right of payment to the Company's future indebtedness that is expressly subordinated in right of payment to the notes, equal in right of payment to the Company's existing and future unsecured indebtedness that is not so subordinated, effectively junior in right of payment to any of the Company's secured indebtedness to the extent of the value of the assets securing such indebtedness, and structurally subordinated to all existing and future indebtedness and other liabilities (including trade payables) incurred by the Company's subsidiaries. The 2026 Convertible Notes Indenture contains customary events of default with respect to the 2026 Convertible Notes, including that upon certain events of default (including the Company's failure to make any payment of principal or interest on the 2026 Convertible Notes when due and payable) occurring and continuing, the 2026 Convertible Notes Trustee by notice to the Company, or the holders of at least 25% in principal amount of the outstanding 2026 Convertible Notes by notice to the Company and the Convertible Notes Trustee, may, and the 2026 Convertible Notes Trustee at the request of such holders (subject to the provisions of the 2026 Convertible Notes Indenture) shall, declare 100% of the principal of and accrued and unpaid interest, if any, on all the 2026 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 2026 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 2026 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 2026 Convertible Notes, the Company separated the 2026 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 2026 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 2026 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 2026 Convertible Notes was $123.0 million and was recorded in additional paid-in capital.

In accounting for the transaction costs related to the issuance of the 2026 Convertible Notes, the Company allocated the total costs incurred to the liability and equity components of the 2026 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 2026 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 $25.3 million in connection with the 2026 Convertible Notes.

The 2026 Convertible Notes consist of the following:

Liability component	March 31, 2020	December 31, 2019
Principal	$ 287,500	$ 287,500
Less: Debt issuance costs	(4,443)	(4,567)
Less: Debt discount, net(1)	(116,116)	(119,350)
Net carrying amount	$ 166,941	$ 163,583

(1) Included in the consolidated balance sheets within convertible senior notes (due 2026) and amortized to interest expense over the remaining life of the 2026 Convertible Notes using the effective interest rate method.

As of March 31, 2020, the remaining contractual life of the 2026 Convertible Notes is approximately 6.5 years.

The following table sets forth total interest expense recognized related to the 2026 Convertible Notes:

	Three Months Ended March 31, 2020
Contractual interest expense	$ 1,076
Amortization of debt issuance costs	124
Amortization of debt discount	3,234
Total	$ 4,434
Effective interest rate of the liability component	10.2%

2022 Convertible Notes

In August 2015, the Company issued, at par value, $150.0 million aggregate principal amount of 3.00% convertible senior notes due 2022. The 2022 Convertible Notes bear cash interest at a rate of 3.00% per year, payable semi-annually on February 15 and August 15 of each year, beginning on February 15, 2016. The 2022 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 2022 Convertible Notes are governed by an indenture (the "2022 Convertible Notes Indenture") with U.S Bank National Association as trustee (the "2022 Convertible Notes Trustee").

Holders may convert their 2022 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 2022 Convertible Notes Indenture) per $1,000 principal amount of 2022 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 2022 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 2022 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 2022 Convertible Notes being converted.

The conversion rate for the 2022 Convertible Notes was initially, and remains, 17.7487 shares of the Company's common stock per $1,000 principal amount of the 2022 Convertible Notes, which is equivalent to an initial conversion price of approximately $56.34 per share of the Company's common stock. The conversion rate may be subject to adjustment in some events but will not be adjusted for any accrued and unpaid interest.

The Company was not permitted to redeem the 2022 Convertible Notes prior to August 20, 2018. As of August 20, 2018, the Company may redeem for cash all or any portion of the 2022 Convertible Notes, at its option, if the last reported sale price of its common stock has been at least 130% of the conversion price then in effect on the last trading day of, and for at least 19 other trading days (whether or not consecutive) during, any 30 consecutive trading day period ending on, and including, the trading day immediately preceding the date on which the Company provides notice of redemption, at a redemption price equal to 100% of the principal amount of the 2022 Convertible Notes to be redeemed, plus accrued and unpaid interest to, but excluding, the redemption date. No sinking fund is provided for the 2022 Convertible Notes, which means that the Company is not required to redeem or retire the 2022 Convertible Notes periodically. There have been no redemptions to date.

If the Company undergoes a "fundamental change" (as defined in the 2022 Convertible Notes Indenture), subject to certain conditions, holders of the 2022 Convertible Notes may require the Company to repurchase for cash all or part of their 2022 Convertible Notes at a repurchase price equal to 100% of the principal amount of the 2022 Convertible Notes to be repurchased, plus accrued and unpaid interest to, but excluding, the fundamental change repurchase date.

The 2022 Convertible Notes represent senior unsecured obligations and will rank senior in right of payment to the Company's future indebtedness that is expressly subordinated in right of payment to the notes, equal in right of payment to the Company's existing and future unsecured indebtedness that is not so subordinated, effectively junior in right of payment to any of the Company's secured indebtedness to the extent of the value of the assets securing such indebtedness, and structurally subordinated to all existing and future indebtedness and other liabilities (including trade payables) incurred by the Company's subsidiaries. The 2022 Convertible Notes Indenture contains customary events of default with respect to the 2022 Convertible Notes, including that upon certain events of default (including the Company's failure to make any payment of principal or interest on the 2022 Convertible Notes when due and payable) occurring and continuing, the 2022 Convertible Notes Trustee by notice to the Company, or the holders of at least 25% in principal amount of the outstanding 2022 Convertible Notes by notice to the Company and the Convertible Notes Trustee, may, and the 2022 Convertible Notes Trustee at the request of such holders (subject to the provisions of the 2022 Convertible Notes Indenture) shall, declare 100% of the principal of and accrued and unpaid interest, if any, on all the 2022 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 2022 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 2022 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 2022 Convertible Notes, the Company separated the 2022 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 2022 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 2022 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 2022 Convertible Notes was $57.5 million and was recorded in additional paid-in capital.

In accounting for the transaction costs related to the issuance of the 2022 Convertible Notes, the Company allocated the total costs incurred to the liability and equity components of the 2022 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 2022 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 2022 Convertible Notes.

The 2022 Convertible Notes consist of the following:

Liability component	**March 31, 2020**	**December 31, 2019**
Principal	$ 150,000	$ 150,000
Less: Debt issuance costs	(1,219)	(1,329)
Less: Debt discount, net(1)	(24,461)	(26,686)
Net carrying amount	$ 124,320	$ 121,985

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

As of March 31, 2020, the remaining contractual life of the 2022 Convertible Notes is approximately 2.4 years.

The following table sets forth total interest expense recognized related to the 2022 Convertible Notes:

	Three Months Ended March 31,	
	2020	2019
Contractual interest expense	$ 1,119	$ 1,110
Amortization of debt issuance costs	111	99
Amortization of debt discount	2,225	1,982
Total	$ 3,455	$ 3,191
Effective interest rate of the liability component	11.0%	11.0%

11. Commitments and contingencies

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

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

Pursuant to the Asset Purchase Agreement with Marathon, Marathon is entitled to receive contingent payments from the Company based on annual net sales of Emflaza up to a specified aggregate maximum amount over the expected commercial life of the asset. In addition, Marathon has the opportunity to receive a single $50.0 million sales-based milestone.

Pursuant to the Merger Agreement with Agilis, Agilis equityholders may become entitled to receive contingent consideration payments from the Company based on (i) the achievement of certain development milestones up to an aggregate maximum amount of $60.0 million, (ii) the achievement of certain regulatory approval milestones together with a milestone payment following the receipt of a priority review voucher up to an aggregate maximum amount of $535.0 million, (iii) the achievement of certain net sales milestones up to an aggregate maximum amount of $150.0 million, and (iv) a percentage of annual net sales for Friedreich ataxia and Angelman syndrome during specified terms, ranging from 2%-6%. The Company is required to pay $40.0 million of the development milestone payments upon the passing of the second anniversary of the closing of the Merger, regardless of whether the applicable milestones have been achieved.

Subject to the terms and conditions of the Asset Acquisition Agreement, BioElectron may become entitled to receive contingent milestone payments of up to $200.0 million (in cash or in shares of the Company's common stock, as determined by the Company) from the Company based on the achievement of certain regulatory and net sales milestones. Subject to the terms and conditions of the Asset Acquisition Agreement, BioElectron may also become entitled to receive contingent payments based on a percentage of net sales of certain products.

The Company also has a Collaboration and License Agreement with Akcea Therapeutics, Inc. ("Akcea") for the commercialization of Tegsedi and Waylivra, and products containing those compounds in countries in Latin America and the Caribbean (the "Akcea Collaboration and License Agreement"). Pursuant to the agreement, the Company paid Akcea an upfront licensing fee, which included an initial payment of $12.0 million. In 2019, a $6.0 million milestone was paid upon receipt of regulatory approval of Waylivra from the EMA and a $4.0 million milestone was paid upon regulatory approval of Tegsedi from ANVISA, the Brazilian health regulatory authority, upon receipt of regulatory approval for Waylivra from ANVISA. In addition, Akcea is eligible to receive an additional milestone payment of $4.0 million upon receipt of regulatory approval for Waylivra from ANVISA. Akcea is also entitled to receive royalty payments subject to certain terms set forth in the Akcea Collaboration and License Agreement.

The Company has employment agreements with certain employees which require the funding of a specific level of payments, if certain events, such as a change in control or termination without cause, occur. Additionally, the Company has royalty payments associated with Translarna and Emflaza product net sales, payable quarterly or annually in accordance with the terms of the related agreements.

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

12. Revenue recognition

Net product sales

The Company views its operations and manages its business in one operating segment. During the three months ended March 31, 2020 and 2019, net product sales in the United States were $27.5 million and $17.8 million, respectively, consisting solely of Emflaza, and net product sales not in the United States were $40.7 million and $35.3 million, respectively, consisting of Translarna and Tegsedi. For the three months ended March 31, 2020, two of the Company's distributors each accounted for over 10% of the Company's net product sales. For the three months ended March 31, 2019, three of the Company's distributors each accounted for over 10% of the Company's net product sales.

The Company's contract liabilities balances as of March 31, 2020 and as of December 31, 2019 were $10.4 million and $11.7 million, respectively. The Company did not have any contract assets as of March 31, 2020 and as of December 31, 2019. During the three months ended March 31, 2020, the Company recognized $2.0 million of revenue related to the amounts included in the contract liability balance at the beginning of the period. For the three months ended March 31, 2019, the Company did not recognize any revenues relating to the contract liability balance at the beginning of the period. The Company has not made significant changes to the judgments made in applying ASC Topic 606 for the three months ended March 31, 2020 and 2019.

Remaining performance obligations

Remaining performance obligations represent the transaction price for goods the Company has yet to provide. As of March 31, 2020 and December 31, 2019, the aggregate amount of the transaction price allocated to the remaining performance obligations relating to Translarna net product revenue was $10.4 million and $11.7 million, respectively. The Company expects to recognize revenue over the next one to two years as the specific timing for satisfying the performance obligations is contingent upon a number of factors, including customers' needs and schedules.

Collaboration revenue

In November 2011, the Company and the Spinal Muscular Atrophy Foundation (SMA Foundation) entered into a licensing and collaboration agreement with F. Hoffman-La Roche Ltd and Hoffman- La Roche Inc. (collectively, Roche) for a spinal muscular atrophy program. Under the terms of the agreement, Roche acquired an exclusive worldwide license to the Company's spinal muscular atrophy program.

The Company is eligible to receive additional payments from Roche if specified events are achieved with respect to each licensed product, including up to $135.0 million in research and development event milestones, up to $325.0 million in sales milestones upon achievement of specified sales events, and up to double digit royalties on worldwide annual net sales of a commercial product. As of March 31, 2020, the remaining potential research and development event milestones that can be received is $72.5 million. The remaining potential sales milestones as of March 31, 2020 is $325.0 million upon achievement of certain sales events. In addition, the Company is eligible to receive up to double digit royalties on worldwide annual net sales of a commercial product.

For the three months ended March 31, 2020 and 2019, the Company recognized revenue related to the licensing and collaboration agreement with Roche of $0.1 million and $0.1 million, respectively.

13. Intangible assets and goodwill

Definite-lived intangibles

On April 20, 2017, the Company completed its previously announced acquisition of all rights to Emflaza pursuant to the Asset Purchase Agreement, dated March 15, 2017, and amended on April 20, 2017, by and between the Company and Marathon. The assets acquired by the Company in the Transaction include intellectual property rights related to Emflaza, inventories of Emflaza, and certain contractual rights related to Emflaza. In accordance with ASU 2017-01, the Company determined that substantially all of the fair value is concentrated in the Emflaza rights intangible asset and as such accounted for the transaction as an asset acquisition under ASC 805-50 and recorded an intangible asset of $148.4 million, which is being amortized to cost of product sales over its expected useful life of approximately seven years on a straight line basis.

Marathon is entitled to receive contingent payments from the Company based on annual net sales of Emflaza beginning in 2018, up to a specified aggregate maximum amount over the expected commercial life of the asset. In accordance with the guidance for an asset acquisition, the Company will record the milestone payment when it becomes payable to Marathon and increase the cost basis for the Emflaza rights intangible asset. For the three month period ended March 31, 2020, a milestone payment of $2.8 million was recorded and is included on the balance sheet within accounts payable and accrued expenses. For the three months ended March 31, 2019, no milestone payment was recorded. These payments are being amortized over the remaining useful life of the Emflaza rights asset on a straight line basis.

Pursuant to the Akcea Collaboration and License Agreement, in May 2019 the Company made a $6.0 million milestone payment to Akcea upon regulatory approval of Waylivra from the EMA. The payment was recorded as an intangible asset and is being amortized to cost of product sales over its expected useful life of approximately ten years on a straight line basis. Additionally, in December 2019, the Company made a $4.0 million milestone payment to Akcea upon regulatory approval of Tegsedi from ANVISA. The payment was recorded as an intangible asset and is being amortized to cost of product sales over its expected useful life of approximately ten years on a straight line basis.

Akcea is also entitled to receive royalty payments subject to certain terms set forth in the Akcea Collaboration and License Agreement related to sales of Waylivra. In accordance with the guidance for an asset acquisition, the Company will record royalty payments when they become payable to Akcea and increase the cost basis for the Waylivra intangible asset.

For the three month periods ended March 31, 2020 and 2019, the Company recognized amortization expense of $7.9 million and $6.1 million, respectively, related to the Emflaza rights, Waylivra, and Tegsedi intangible assets. The estimated future amortization of the Emflaza rights, Waylivra, and Tegsedi intangible assets is expected to be as follows:

	As of March 31, 2020
2020	$ 23,851
2021	31,801
2022	31,801
2023	31,801
2024 and thereafter	9,540
Total	$ 128,794

The weighted average remaining amortization period of the definite-lived intangibles as of March 31, 2020 is 4.2 years.

Indefinite-lived intangibles

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

In accordance with the acquisition method of accounting, the Company allocated the acquisition cost for the Merger to the underlying assets acquired and liabilities assumed, based upon the estimated fair values of those assets and liabilities at the date of acquisition. The Company classified the fair value of the acquired IPR&D as indefinite lived intangible assets until the successful completion or abandonment of the associated research and development efforts. The value allocated to the indefinite lived intangible assets was $576.5 million. There have been no changes to the balance of the indefinite-lived intangibles since the Merger.

Goodwill

As a result of the Merger on August 23, 2018, the Company recorded $82.3 million of goodwill. There were no changes to the recorded value of goodwill for the three month periods ended March 31, 2020.

14. Subsequent events

On April 29, 2020, the Company, certain of the former equityholders of Agilis, and, for the limited purposes set forth in the agreement, Shareholder Representative Services LLC, entered into a Rights Exchange Agreement (the "Rights Exchange Agreement"), pursuant to which the Company agreed to issue 2,821,176 shares of its common stock (the "Common Stock Consideration") and paid $36.9 million (the "Cash Consideration"), in the aggregate, to such former equityholders of Agilis (the "Participating Rightholders") in exchange for the cancellation and forfeiture by the Participating Rightholders of their rights to receive certain milestone-based contingent payments under the Merger Agreement, pursuant to which the Company completed the Merger. Also on April 29, 2020, the Company issued 2,723,826 shares of the Common Stock Consideration to certain of the Participating Rightholders pursuant to the Rights Exchange Agreement, with the issuance of the remaining 97,350 shares of the Common Stock Consideration to certain of the Participating Rightholders who are former employees of Agilis or current or former employees of the Company to be deferred until the earlier of (i) December 15, 2020 or (ii) the date on which the Company files a registration statement on Form S-3 with respect to the resale of the shares of the Common Stock Consideration pursuant to the terms of the Rights Exchange Agreement.

Pursuant to the terms of the Rights Exchange Agreement, the Participating Rightholders have canceled and forfeited their rights under the Merger Agreement to receive (i) $174.0 million, in the aggregate, of potential milestone payments based on the achievement of certain regulatory milestones and (ii) $37.6 million, in the aggregate, of $40.0 million in development milestone payments due upon the passing of the second anniversary of the closing of the Merger, regardless of whether the milestones are achieved.

The Rights Exchange Agreement has no effect on the Merger Agreement other than to provide for the cancellation and forfeiture of the Participating Rightholders' rights to receive $211.6 million, in the aggregate, of the milestone payments described above. As a result, all other rights and obligations under the Merger Agreement remain in effect pursuant to their terms, including the Company's obligation to pay up to an aggregate maximum amount of $22.4 million upon the achievement of certain development milestones (representing the remaining portion of potential development milestone payments after deducting the $37.6 million for which rights were canceled and forfeited pursuant to the Rights Exchange Agreement from the $40.0 million in development milestone payments that are due upon the passing of the second anniversary of the closing of the Acquisition), up to an aggregate maximum amount of $361.0 million upon the achievement of certain regulatory milestones (representing the remaining portion of potential regulatory milestone payments for which rights were not canceled and forfeited pursuant to the Rights Exchange Agreement), up to a maximum aggregate amount of $150.0 million upon the achievement of certain net sales milestones and a percentage of annual net sales for Friedreich ataxia and Angelman syndrome during specified terms, ranging from 2% to 6%, pursuant to the terms of the Merger Agreement.

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

The following discussion of our financial condition and results of operations should be read in conjunction with our financial statements and the notes to those financial statements appearing elsewhere in this Quarterly Report on Form 10-Q and the audited consolidated financial statements and notes thereto and management's discussion and analysis of financial condition and results of operations for the year ended December 31, 2019 included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 2, 2020, or our 2019 Annual Report. This discussion contains forward-looking statements that involve significant risks and uncertainties. As a result of many factors, such as those set forth in Part II, Item 1A. (Risk Factors) of this Quarterly Report on Form 10-Q and Part I, Item 1A. (Risk Factors) of our 2019 Annual Report, and our actual results may differ materially from those anticipated in these forward-looking statements.

Our Company

We are a science-driven global biopharmaceutical company focused on the discovery, development and commercialization of clinically-differentiated medicines that provide benefits to patients with rare disorders. Our ability to commercialize products is the foundation that drives our continued investment in a robust diversified pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need. Our strategy is to bring best-in-class therapies with differentiated clinical benefit to patients affected by rare disorders and to leverage our global commercial infrastructure to maximize value for our patients and other stakeholders. We have a portfolio pipeline that includes commercial products as well as product candidates in various stages of development, including clinical, pre-clinical and research and discovery stages, focused on the development of new treatments for multiple therapeutic areas, including rare diseases and oncology.

Corporate Updates

COVID-19 Impact

The global pandemic caused by a strain of novel coronavirus, COVID-19, has impacted the timing of certain of our clinical trials and regulatory submissions as well as other aspects of our business operations. The following expectations have been revised as a result of the impact or expected impact of the COVID-19 pandemic:

- As a result of the COVID-19 pandemic, our clinical trial site for Study 045 at the University of California, Los Angeles is temporarily closed to elective procedures, resulting in the delay of Study 045's expected completion as certain patients still require final study muscle biopsies. During the delay, patients are remaining on drug until the biopsies can be performed. We now expect that data from Study 045 will be available in the third quarter of 2020 followed by a potential resubmission of the New Drug Application, or NDA, for Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, thereafter.

- As previously disclosed, in response to discussions with the United States Food and Drug Administration, or the FDA, we intend to provide additional information concerning the use of the commercial cannula for PTC-AADC in young patients. However, because hospitals have generally canceled elective surgeries due to the COVID-19 pandemic, we have been delayed in our ability to gather such information. We now anticipate submitting a biologics license application, or BLA, for PTC-AADC for the treatment of Aromatic L-Amino Acid Decarboxylase, or AADC, deficiency in the United States in the second half of 2020.

- As a result of the COVID-19 pandemic, certain of our Bio-e platform trials have been delayed until study sites reopen and are available for clinical trials and we can ensure that patients will be able to travel to the study sites safely. We expect to initiate a potential registrational Phase 2 placebo-controlled trial of PTC743 in approximately 60 children with mitochondrial disease and associated refractory epilepsy in the third quarter of 2020. We now expect to initiate a potential registrational Phase 3 trial of PTC743 in approximately 100 patients with Friedrich ataxia in the fourth quarter of 2020.

- The COVID-19 pandemic has impacted multiple investigational new drug application, or IND, enabling activities for our gene therapy programs targeting Friedrich ataxia and Angelman syndrome. We now anticipate the IND filings for each of these programs to be delayed at least one quarter.

- To date, our ability to generate revenue has not been significantly affected by the COVID-19 pandemic. However, due to travel restrictions, social distancing and the continued global uncertainty resulting from the COVID-19 pandemic, we may have difficulty identifying and accessing new patients, supporting existing patients and meeting with regulatory authorities or other governmental entities, which may negatively affect our future revenue. We continue to remotely connect with our existing patient base and have not encountered any material issues in supplying those patients.

- In response to the global uncertainty caused by the COVID-19 pandemic, we are prioritizing our expenses where we deem appropriate and strategically positioning our capital allocation. For example, we have deferred certain capital expenditures related to our leased biologics facility in Hopewell Township, New Jersey, or the Hopewell Facility, and we now expect GMP manufacturing of clinical material at this facility to begin in early 2021.

The COVID-19 pandemic and responsive measures thereto may result in further negative impacts, including additional delays in our clinical and regulatory activities. We cannot be certain what the overall impact of the COVID-19 pandemic will be on our business and it has the potential to materially adversely affect our business, financial condition, results of operations, and prospects. For additional information, see “Item 1A. Risk Factors - We face risks related to health epidemics and other widespread outbreaks of contagious disease, which are, and may continue to, delay our ability to complete our ongoing clinical trials and initiate future clinical trials, disrupt regulatory activities and have other adverse effects on our business and operations, including the novel coronavirus (COVID-19) pandemic, which could significantly disrupt our operations and impact our operating results. In addition, this pandemic has caused substantial disruption in the financial markets and economies, which could result in adverse effects on our business and operations.”

Agilis Rights Exchange Agreement

On April 29, 2020, we, certain of the former equityholders of Agilis Biotherapeutics, Inc., or Agilis, and, for the limited purposes set forth in the agreement, Shareholder Representative Services LLC, entered into a Rights Exchange Agreement, or the Rights Exchange Agreement, pursuant to which we agreed to issue 2,821,176 shares of our common stock, or the Common Stock Consideration and paid $36.9 million or the Cash Consideration, in the aggregate, to such former equityholders of Agilis, or the “Participating Rightholders, in exchange for the cancellation and forfeiture by the Participating Rightholders of their rights to receive certain milestone-based contingent payments under the Agreement and Plan of Merger, or the Merger Agreement, by and among us, Agility Merger Sub, Inc., Agilis, and, solely in its capacity as the representative, agent and attorney-in-fact of the equityholders of Agilis, Shareholder Representative Services LLC, dated as of July 19, 2018, pursuant to which we acquired Agilis in 2018, or the Acquisition. Also on April 29, 2020,we issued 2,723,826 shares of the Common Stock Consideration to certain of the Participating Rightholders pursuant to the Rights Exchange Agreement, with the issuance of the remaining 97,350 shares of the Common Stock Consideration to certain of the Participating Rightholders who are former employees of Agilis or current or

former employees of the Company to be deferred until the earlier of (i) December 15, 2020 or (ii) the date on which the we file a registration statement on Form S-3 with respect to the resale of the shares of the Common Stock Consideration pursuant to the terms of the Rights Exchange Agreement, as further described below.

Pursuant to the terms of the Rights Exchange Agreement, the Participating Rightholders have canceled and forfeited their rights under the Merger Agreement to receive (i) $174.0 million, in the aggregate, of potential milestone payments based on the achievement of certain regulatory milestones and (ii) $37.6 million, in the aggregate, of $40.0 million in development milestone payments due upon the passing of the second anniversary of the closing of the Acquisition, regardless of whether the milestones are achieved.

The Rights Exchange Agreement has no effect on the Merger Agreement other than to provide for the cancellation and forfeiture of the Participating Rightholders' rights to receive $211.6 million, in the aggregate, of the milestone payments described above. As a result, all other rights and obligations under the Merger Agreement remain in effect pursuant to their terms, including the our obligation to pay up to an aggregate maximum amount of $22.4 million upon the achievement of certain development milestones (representing the remaining portion of potential development milestone payments after deducting the $37.6 million for which rights were canceled and forfeited pursuant to the Rights Exchange Agreement from the $40.0 million in development milestone payments that are due upon the passing of the second anniversary of the closing of the Acquisition), up to an aggregate maximum amount of $361.0 million upon the achievement of certain regulatory milestones (representing the remaining portion of potential regulatory milestone payments for which rights were not canceled and forfeited pursuant to the Rights Exchange Agreement), up to a maximum aggregate amount of $150.0 million upon the achievement of certain net sales milestones and a percentage of annual net sales for Friedreich ataxia and Angelman syndrome during specified terms, ranging from 2% to 6%, pursuant to the terms of the Merger Agreement.

Pursuant to the Rights Exchange Agreement, we have agreed to use commercially reasonable efforts to file as promptly as practicable after the date of the Rights Exchange Agreement, but in no event earlier than the date on of this Quarterly Report on Form 10-Q, a registration statement on Form S-3 with respect to the resale of the shares of the Common Stock Consideration issued to the Participating Rightholders pursuant to the Rights Exchange Agreement and to maintain the effectiveness of such registration statement until the six-month anniversary date of the Rights Exchange Agreement or such earlier time as all shares of the Common Stock Consideration covered by the registration statement have been sold, subject to certain exceptions and the provision of certain information by the Participating Rightholders. The Rights Exchange Agreement contains customary indemnification obligations of the Company and the former Agilis equityholders with respect to the registration statement.

Global Commercial Footprint

Global DMD Franchise

We have two products, Translarna™ (ataluren) and Emflaza™ (deflazacort), for the treatment of Duchenne muscular dystrophy, or DMD, a rare, life threatening disorder. Translarna has marketing authorization in the European Economic Area, or EEA, for the treatment of nmDMD in ambulatory patients aged two years and older and in Brazil for the treatment of nmDMD in ambulatory patients aged five years and older. During the quarter ended March 31, 2020, we recognized $40.5 million in net sales from Translarna. We hold worldwide commercialization rights to Translarna for all indications in all territories. Emflaza is approved in the United States for the treatment of DMD in patients two years and older. During the quarter ended March 31, 2020, we recognized $27.5 million in net sales from Emflaza.

Our marketing authorization for Translarna in the EEA is subject to annual review and renewal by the European Commission following reassessment by the European Medicines Agency, or EMA, of the benefit-risk balance of the authorization, which we refer to as the annual EMA reassessment. In July 2019, the European Commission renewed our marketing authorization, making it effective, unless extended, through August 5, 2020. In February 2020, we submitted a marketing authorization renewal request to the EMA. This marketing authorization is further subject to a specific obligation to conduct and submit the results of an 18-month, placebo-controlled trial, followed by an 18-month open-label extension, which we refer to together as Study 041. The final report on the trial and open-label extension is to be submitted by us to the EMA by the end of the third quarter of 2022.

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

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

Translarna is an investigational new drug in the United States. During the first quarter of 2017, we filed an NDA for Translarna for the treatment of nmDMD over protest with the FDA. In October 2017, the Office of Drug Evaluation I of the FDA issued a Complete Response Letter for the NDA, stating that it was unable to approve the application in its current form. In response, we filed a formal dispute resolution request with the Office of New Drugs of the FDA. In February 2018, the Office of New Drugs of the FDA denied our appeal of the Complete Response Letter. In its response, the Office of New Drugs recommended a possible path forward for the ataluren NDA submission based on the accelerated approval pathway. This would involve a re-submission of an NDA containing the current data on effectiveness of ataluren with new data to be generated on dystrophin production in nmDMD patients' muscles. We intend to follow the FDA's recommendation and will collect, using newer technologies via procedures and methods that we designed, such dystrophin data in a new study, Study 045, which we initiated in the fourth quarter of 2018. We expect that data from Study 045 will be available in the third quarter of 2020 followed by a potential re-submission of an NDA thereafter. Additionally, should a re-submission of an NDA receive accelerated approval, the Office of New Drugs stated that Study 041, which is currently enrolling, could serve as the confirmatory post-approval trial required in connection with the accelerated approval framework.

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

LATAM Commercialization

We hold the rights for the commercialization of Tegsedi™ (inotersen) and Waylivra™ (volanesorsen) for the treatment of rare diseases in countries in Latin America and the Caribbean pursuant to our Collaboration and License Agreement with Akcea Therapeutics, Inc., or Akcea. Tegsedi has received marketing authorization in the United States, EU and Brazil for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis, or hATTR amyloidosis. Waylivra has received marketing authorization in the European Union, or EU, for the treatment of familial chylomicronemia syndrome, or FCS. We anticipate filing for marketing authorization with ANVISA in the second half of 2020.

Diversified Development Pipeline

Gene Therapy Platform

We have a pipeline of gene therapy product candidates for rare monogenic diseases that affect the central nervous system, or CNS, including PTC-AADC for the treatment of AADC deficiency, a rare CNS disorder arising from reductions in the enzyme AADC that result from mutations in the dopa decarboxylase gene. We are preparing a BLA for PTC-AADC for the treatment of AADC deficiency in the United States, which we anticipate submitting to the FDA in the second half of 2020. In January 2020, we submitted a marketing authorization application, or MAA, for PTC-AADC for the treatment of AADC deficiency in the EEA to the EMA and we expect an opinion from the Committee for Medicinal Products for Human Use, or CHMP, by the end of 2020.

Splicing Platform

We also have a spinal muscular atrophy, or SMA, collaboration with F. Hoffman-La Roche Ltd. and Hoffman-La Roche Inc., which we refer to collectively as Roche, and the Spinal Muscular Atrophy Foundation, or SMA Foundation. The lead compound in the SMA program is risdiplam (RG7916, RO7034067). Roche submitted an NDA for risdiplam to the FDA in the fourth quarter of 2019. In April 2020, we announced that the FDA extended the Prescription Drug User Fee Act, or PDUFA, date for a decision from May 24, 2020 to August 24, 2020. Roche submitted additional data to the FDA including comprehensive data from the SUNFISH part 2 study to help provide access to risdiplam for a broad range of people living with SMA, triggering this extension. Risdiplam is expected to be indicated in the United States for SMA type 1, 2 and 3 patients, if approved. Roche anticipates submitting an MAA for risdiplam for the treatment of SMA in the EEA in mid-year 2020.

Bio-e Platform

In 2019, we acquired substantially all of the assets of BioElectron Technology Corporation, or BioElectron, including certain compounds that we have begun to develop as part of our Bio-e platform. In 2020, we plan to initiate three trials in this platform with two unique compounds that regulate inflammation and oxidative stress.

Multi-Platform Discovery

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

A clinical study assessing Translarna in nonsense mutation Dravet syndrome/CDKL5 was initiated in the first quarter of 2017. This study did not meet its primary endpoint. Given the results of the study, we have discontinued this program.

Funding

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

To date, we have financed our operations primarily through our offering of 3.00% convertible senior notes due August 15, 2022, or the 2022 Convertible Notes, our offering of 1.50% convertible senior notes due September 15, 2026, or the 2026 Convertible Notes, and, together with the 2022 Convertible Notes, the Convertible Notes, our public offerings of common stock in February 2014, in October 2014, in April 2018, in January 2019, and in September 2019, the common stock issued in our “at the marketing offering”, our initial public offering of common stock in June 2013, private placements of our preferred stock, collaborations, bank debt and convertible debt financings, our credit and security agreement, or the Credit Agreement, with MidCap Financial Trust, or MidCap Financial, as administrative agent and MidCap Financial and other certain institutions as lenders thereto, and grants and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease areas addressed by our product candidates. Since 2014, we have also relied on revenue generated from net sales of Translarna for the treatment of nmDMD in territories outside of the United States, and since May 2017, we have generated revenue from net sales of Emflaza for the treatment of DMD in the United States.

The 2022 Convertible Notes consist of $150.0 million in aggregate principal amount of 3.00% convertible senior notes due 2022. The 2022 Convertible Notes bear cash interest payable on February 15 and August 15 of each year, beginning on February 15, 2016. The 2022 Convertible Notes are senior unsecured obligations of ours and will mature on August 15, 2022, unless earlier converted, redeemed or repurchased in accordance with their terms prior to such date. We received net proceeds from the offering of approximately $145.4 million, after deducting the initial purchasers’ discounts and commissions and the estimated offering expenses payable by us.

The Credit Agreement provides for a senior secured term loan facility of $60.0 million, of which $40.0 million was drawn by us on May 5, 2017. Our ability to draw on the remaining $20.0 million under the senior secured term loan facility expired on December 31, 2018. The maturity date of the Credit Agreement is May 1, 2021, unless terminated earlier. As of March 31, 2020, we made loan repayments of $16.7 million on the Credit Facility.

In August 2019, we entered into an At the Market Offering Sales Agreement, or the Sales Agreement, with Cantor Fitzgerald and RBC Capital Markets, LLC, or together, the Sales Agents, pursuant to which, we may offer and sell shares of our common stock, having an aggregate offering price of up to $125.0 million from time to time through the Sales Agents by any method that is deemed to be an “at the market offering” as defined in Rule 415(a)(4) promulgated under the Securities Act of 1933, as amended, or the Securities Act. During the twelve month period ending December 31, 2019, we issued and sold an aggregate of 63,926 shares of common stock pursuant to the Sales Agreement at a weighted average public offering price of $46.60 per share. We received net proceeds of $2.6 million after deducting agent discounts and commissions and other offering expenses payable by us. During the three month period ending March 31, 2020, we issued and sold an aggregate of 262,205 shares of common stock pursuant to the Sales Agreement at a weighted average public offering price of $52.81 per share. We received net proceeds of $13.5 million after deducting underwriting discounts and commissions and other offering expenses payable by us.

The 2026 Convertible Notes consist of $287.5 million aggregate principal amount of 1.50% convertible senior notes due 2026. The 2026 Convertible Notes bear cash interest at a rate of 1.50% per year, payable semi-annually on March 15 and September 15 of each year, beginning on March 15, 2020. The 2026 Convertible Notes will mature on September 15, 2026, unless earlier repurchased or converted. We received net proceeds of $279.3 million after deducting the initial purchasers’ discounts and commissions and the offering expenses payable by us.

As of March 31, 2020, we had an accumulated deficit of $1,303.4 million. We had a net loss of $112.7 million and $72.1 million for the three month periods ended March 31, 2020 and 2019, respectively.

We anticipate that our expenses will continue to increase in connection with our commercialization efforts in the United States, the EEA, Latin America and other territories, including the expansion of our infrastructure and corresponding sales and marketing, legal and regulatory, distribution and manufacturing, including expanding our direct manufacturing capabilities at our new biologics manufacturing facility and administrative and employee-based expenses. In addition to the foregoing, we expect to continue to incur ongoing research and development expenses for our products and product candidates, including our gene therapy, splicing, Bio-e and oncology programs as well as studies in our products for maintaining authorizations, including Study 041, label extensions and additional indications. In addition, we may incur substantial costs in connection with our efforts to advance our regulatory submissions. We continue to seek marketing authorization for Translarna for the treatment of nmDMD in territories that we do not currently have marketing authorization in and we may also seek marketing authorization for Translarna for other indications. We submitted an MAA to the EMA for the treatment of AADC deficiency with PTC-AADC in the EEA and we are preparing a BLA for PTC-AADC for the treatment of AADC deficiency in the United States, which we anticipate submitting to the FDA in the second half of 2020. We also anticipate filing for marketing authorization for Waylivra with ANVISA in the second half of 2020. These efforts may significantly impact the timing and extent of our commercialization expenses.

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

With respect to our outstanding 2022 Convertible Notes, cash interest payments are payable on a semi-annual basis in arrears, which require total funding of $4.5 million annually. With respect to our outstanding 2026 Convertible Notes, cash interest payments are payable on a semi-annual basis in arrears, which will require total funding of $4.3 million annually. Additionally, under the terms of our Credit Agreement cash interest payments are payable monthly in arrears. On April 29, 2020 pursuant to the Rights Exchange Agreement, we paid $37.6 million as partial consideration to the Participating Rightholders in exchange for the cancellation and forfeiture by the Participating Rightholders of their rights to receive certain milestone-based contingent payments under the Merger Agreement. We are still required to pay an additional $2.4 million in development milestone payments upon the passing of the second anniversary of the closing of the Agilis acquisition, August 23, 2020, regardless of whether the applicable milestones have been achieved. In addition, Akcea is eligible to receive from us an additional milestone payment of $4.0 million upon receipt of regulatory approval for Waylivra from

ANVISA. Furthermore, since we are a public company, we have incurred and expect to continue to incur additional costs associated with operating as such including significant legal, accounting, investor relations and other expenses.

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

Financial operations overview

Revenues

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

We record product sales net of any variable consideration, which includes discounts, allowances, rebates related to Medicaid and other government pricing programs, and distribution fees. We use the expected value or most likely amount method when estimating variable consideration, unless discount or rebate terms are specified within contracts. 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. For the three months ended March 31, 2020 and 2019, net product sales outside of the United States were $40.7 million and $35.3 million respectively, and net product sales in the United States were $27.5 million and $17.8 million respectively.

In relation to customer contracts, we incur costs to fulfill a contract but do not incur costs to obtain a contract. These costs to fulfill a contract do not meet the criteria for capitalization and are expensed as incurred. We consider any shipping and handling costs that are incurred after the customer has obtained control of the product as a cost to fulfill a promise. Shipping and handling costs associated with finished goods delivered to customers are recorded as a selling expense.

Roche and the SMA Foundation Collaboration. In November 2011, we entered into a license and collaboration agreement, or licensing agreement, with Roche and the SMA Foundation pursuant to which we are collaborating with Roche and the SMA Foundation to further develop and commercialize compounds identified under our SMA program with the SMA Foundation. The research component of this agreement terminated effective December 31, 2014. The licensing agreement included a $30.0 million upfront payment made in 2011 which was recognized on a deferred basis over the research term, and the potential for up to $460.0 million in milestone payments and royalties on net sales.

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

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

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

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

In November 2019, we announced that the FDA accepted the filing of and granted priority review for the NDA for risdiplam for the treatment of SMA. The filing acceptance by the FDA triggered a $15.0 million payment to us from Roche which we recorded as collaboration revenue at time of achievement.

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

Research and development expense

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

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

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

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

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

We expect our research and development expenses to fluctuate in connection with our ongoing activities, particularly in connection with Study 041 and other studies for Translarna for the treatment of nmDMD, our activities under our gene therapy, splicing, Bio-e and oncology programs, our studies of Translarna for additional indications, and performance of our FDA post-marketing requirements with respect to Emflaza in the United States. The timing and amount of these expenses will depend upon the outcome of our ongoing clinical trials and the costs associated with our planned clinical trials. The timing and amount of these expenses

will also depend on the costs associated with potential future clinical trials of our products or product candidates and the related expansion of our research and development organization, regulatory requirements, advancement of our preclinical programs, and product and product candidate manufacturing costs.

The following tables provide research and development expense for our most advanced principal product development programs, for the three months ended March 31, 2020 and 2019.

	Three Months Ended March 31,	
	2020	2019
	(in thousands)	
Translarna (nmDMD, aniridia and Dravet)	$ 32,370	$ 15,783
Gene Therapy	30,899	11,241
Oncology	4,755	7,292
Emflaza	4,114	6,130
Other research and preclinical	17,969	12,120
Total research and development	$ 90,107	$ 52,566

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

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

A change in the outcome of any of these variables with respect to the development of our products or product candidates could mean a significant change in the costs and timing associated with the development of that product or product candidate. For example, if the EMA or FDA or other regulatory authority were to require us to conduct clinical trials beyond those which we currently anticipate will be required for the completion of clinical development of any of our products or product candidates or if we experience significant delays in enrollment in any of our clinical trials, we could be required to expend significant additional financial resources and time on the completion of clinical development. In addition, the uncertainty with respect to the duration, nature and extent of negative impacts of the COVID-19 pandemic and responsive measures relating thereto on our ability to successfully enroll our current and future clinical trials, is causing us to experience delays, and may cause us to experience further delays, in our clinical trials and regulatory submissions.

Selling, general and administrative expense

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

We expect that selling, general and administrative expenses will increase in future periods in connection with our continued efforts to commercialize our products, including increased payroll, expanded infrastructure, commercial operations, increased consulting, legal, accounting and investor relations expenses.

Interest expense, net

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

Critical accounting policies and significant judgments and estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our financial statements, which we have prepared in accordance with generally accepted accounting principles in the United States. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported revenues and expenses during the reporting periods. Actual results may differ from these estimates under different assumptions or conditions.

During the three months ended March 31, 2020, there were no material changes to our critical accounting policies as reported in our 2019 Annual Report on Form 10-K.

Results of operations

Three months ended March 31, 2020 compared to three months ended March 31, 2019

The following table summarizes revenues and selected expense and other income data for the three months ended March 31, 2020 and 2019.

(in thousands)	Three Months Ended March 31, 2020	2019	Change 2020 vs. 2019
Net product revenue	$ 68,196	$ 53,054	$ 15,142
Collaboration and grant revenue	63	529	(466)
Cost of product sales, excluding amortization of acquired intangible asset	4,085	2,376	1,709
Amortization of acquired intangible asset	7,949	6,077	1,872
Research and development expense	90,107	52,566	37,541
Selling, general and administrative expense	58,209	40,544	17,665
Change in the fair value of deferred and contingent consideration	900	21,160	(20,260)
Interest expense, net	(5,642)	(2,288)	(3,354)
Other expense, net	(13,832)	(109)	(13,723)
Income tax expense	(222)	(576)	354

Net product revenues. Net product revenues were $68.2 million for the three months ended March 31, 2020, an increase of $15.1 million, or 29%, from $53.1 million for the three months ended March 31, 2019. The increase in net product revenue was primarily due to the increase in net product sales in existing markets where Translarna is available as well as continued geographic expansion into new territories, in addition to an increase in net product sales of Emflaza. The increase in net product sales of Emflaza was due to new patient growth driven in part by diagnostic and educational efforts as well as ongoing operational improvements.

Collaboration and grant revenues. Collaboration and grant revenues were $0.1 million for the three months ended March 31, 2020, a decrease of $0.5 million, or 88%, from $0.5 million for the three months ended March 31, 2019. The decrease in collaboration and grant revenues is related to our ongoing collaboration arrangements.

Cost of product sales, excluding amortization of acquired intangible asset. Cost of product sales, excluding amortization of acquired intangible asset, were $4.1 million for the three months ended March 31, 2020, an increase of $1.7 million, or 72%, from $2.4 million for the three months ended March 31, 2019. Cost of product sales consist primarily of royalty payments associated with Emflaza and Translarna net product sales, excluding contingent payments to Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC), and costs associated with Emflaza and Translarna product sold during the period.

Amortization of acquired intangible asset. Amortization of our intangible assets was $7.9 million for the three months ended March 31, 2020, an increase of $1.9 million, or 31%, from $6.1 million for the three months ended March 31, 2019. These amounts are related to the acquisition of all rights to Emflaza acquired in May 2017, Marathon contingent payments, and our Waylivra and Tegsedi intangible assets. The increase is primarily related to additional Marathon contingent payments. The amount allocated to the Emflaza intangible asset is amortized on a straight-line basis over its estimated useful life of approximately seven years from the date of the completion of the acquisition of all rights to Emflaza, the period of estimated future cash flows. The Marathon contingent payments are amortized prospectively as incurred, straight-line, over the remaining useful life of the Emflaza intangible asset. The Waylivra and Tegsedi assets are amortized on a straight-line basis over their estimated useful life of approximately ten years, respectively.

Research and development expense. Research and development expense was $90.1 million for the three months ended March 31, 2020, an increase of $37.5 million, or 71%, from $52.6 million for the three months ended March 31, 2019. The increase reflects costs associated with advancing the gene therapy and Bio-e platforms and increased investment in research programs as well as advancement of the clinical pipeline.

Selling, general and administrative expense. Selling, general and administrative expense was $58.2 million for the three months ended March 31, 2020, an increase of $17.7 million, or 44%, from $40.5 million for the three months ended March 31, 2019. The

increase was primarily due to continued investment to support our commercial activities including our expanding commercial portfolio.

Change in the fair value of deferred and contingent consideration. The change in the fair value of deferred and contingent consideration was $0.9 million for the three months ended March 31, 2020, a decrease of $20.3 million, or 96% from $21.2 million for the three months ended March 31, 2019. The change is related to the fair valuation of the potential future consideration to be paid to former equity holders of Agilis as a result of our merger with Agilis which closed in August 2018. Changes in the fair value were due to the re-calculation of discounted cash flows for the passage of time and changes to certain other estimated assumptions.

Interest expense, net. Interest expense, net was $5.6 million for the three months ended March 31, 2020, a increase of $3.4 million, or 147%, from $2.3 million for the three months ended March 31, 2019. The increase in interest expense, net was primarily due to interest expense recorded from the 2022 and 2026 Convertible Notes and the Credit Agreement, partially offset by interest income from our investments.

Other expense net. Other expense, net was $13.8 million for the three months ended March 31, 2020, an increase of $13.7 million, or over 100%, from other expense, net of $0.1 million for the three months ended March 31, 2019. The increase in other expense, net resulted primarily from a foreign exchange loss from the remeasurement of our intercompany loan, unrealized losses on our equity investment and convertible debt security in ClearPoint Neuro, Inc. of $1.6 million and $2.8 million, respectively, and exchange rate changes.

Income tax expense. Income tax expense was $0.2 million for the three months ended March 31, 2020 compared to income tax expense of $0.6 million for the three months ended March 31, 2019. We incurred income tax expense in various foreign jurisdictions, and our foreign tax liabilities are largely dependent upon the distribution of pre-tax earnings among these different jurisdictions. We are paying minimum income taxes in the United States because of incurred losses in the various state jurisdictions.

Liquidity and capital resources

Sources of liquidity

Since inception, we have incurred significant operating losses.

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

We have historically financed our operations primarily through the issuance and sale of our common stock in public offerings, our “at the market offering” of our common stock, the private placements of our preferred stock, collaborations, bank debt, convertible debt financings, the Credit Agreement and grants and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease areas addressed by our product candidates. We expect to continue to incur significant expenses and operating losses for at least the next several years. The net losses we incur may fluctuate significantly from quarter to quarter.

In August 2015, we closed a private offering of $150.0 million in aggregate principal amount of 3.00% convertible senior notes due 2022 including the exercise by the initial purchasers of an option to purchase an additional $25.0 million in aggregate principal amount of the 2022 Convertible Notes. The 2022 Convertible Notes bear cash interest payable on February 15 and August 15 of each year, beginning on February 15, 2016. The 2022 Convertible Notes are senior unsecured obligations of ours and will mature on August 15, 2022, unless earlier converted, redeemed or repurchased in accordance with their terms prior to such date. We received net proceeds from the offering of approximately $145.4 million, after deducting the initial purchasers’ discounts and commissions and the estimated offering expenses payable by us.

On May 5, 2017, we entered into the Credit Agreement with MidCap Financial, which provides for a senior secured term loan facility of $60.0 million, of which $40.0 million was drawn by us on May 5, 2017. Our ability to draw on the remaining $20.0 million under the senior secured term loan facility expired on December 31, 2018. The maturity date of the Credit Agreement is

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

In August 2019, we entered into the Sales Agreement, pursuant to which, we may offer and sell shares of our common stock, having an aggregate offering price of up to $125.0 million from time to time through the Sales Agents by any method that is deemed to be an "at the market offering" as defined in Rule 415(a)(4) promulgated under the Securities Act. During the twelve month period ending December 31, 2019, we issued and sold an aggregate of 63,926 shares of common stock pursuant to the Sales Agreement at a weighted average public offering price of $46.60 per share. We received net proceeds of $2.6 million after deducting agent discounts and commissions and other offering expenses payable by us. During the three month period ending March 31, 2020, we issued and sold an aggregate of 262,205 shares of common stock pursuant to the Sales Agreement at a weighted average public offering price of $52.81 per share. We received net proceeds of $13.5 million after deducting underwriting discounts and commissions and other offering expenses payable by us.

In September 2019, we closed a private offering of $287.5 million aggregate principal amount of 1.50% convertible senior notes due 2026 including the exercise by the initial purchasers of an option to purchase an additional $37.5 million in aggregate principal amount of the 2026 Convertible Notes. The 2026 Convertible Notes bear cash interest at a rate of 1.50% per year, payable semi-annually on March 15 and September 15 of each year, beginning on March 15, 2020. The 2026 Convertible Notes will mature on September 15, 2026, unless earlier repurchased or converted. We received net proceeds of $279.3 million after deducting the initial purchasers' discounts and commissions and the offering expenses payable by us.

Cash flows

As of March 31, 2020, we had cash, cash equivalents and marketable securities of $595.9 million.

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

	Three Months Ended March 31,	
(in thousands)	**2020**	**2019**
Cash (used in) provided by:		
Operating activities	(81,554)	(42,409)
Investing activities	(101,274)	(150,498)
Financing activities	18,490	225,722

Net cash used in operating activities was $81.6 million for the three months ended March 31, 2020 and $42.4 million for the three months ended March 31, 2019. The net cash used in operating activities primarily relates to supporting clinical development and commercial activities.

Net cash used in investing activities was $101.3 million for the three months ended March 31, 2020 and $150.5 million for the three months ended March 31, 2019. Cash used in investing activities for the three months ended March 31, 2020 and for the three months ended March 31, 2019 were primarily related to net purchases of marketable securities.

Net cash provided by financing activities was $18.5 million for the three months ended March 31, 2020 and $225.7 million for the three months ended March 31, 2019. Cash provided by financing activities for the three months ended March 31, 2020 was primarily attributable to proceeds from our at the market equity offering and the exercise of options. Cash provided by financing activities for the three months ended March 31, 2019 was primarily attributable to our equity offering in January 2019 and the exercise of options.

Funding requirements

We anticipate that our expenses will continue to increase in connection with our commercialization efforts in the United States, the EEA, Latin America and other territories, including the expansion of our infrastructure and corresponding sales and marketing, legal and regulatory, distribution and manufacturing and administrative and employee-based expenses. In addition to the foregoing, we expect to continue to incur significant costs in connection with the research and development of our gene therapy, splicing, Bio-e and oncology programs as well as studies in our products for maintaining authorizations, including Study 041, label extensions and additional indications. In addition, we may incur substantial costs in connection with our efforts to advance our regulatory submissions. We continue to seek marketing authorization for Translarna for the treatment of nmDMD in territories that we do not currently have marketing authorization in and we may also seek marketing authorization for Translarna for other indications. We recently submitted an MAA to the EMA for the treatment of AADC deficiency with PTC-AADC in the EEA and we are preparing a BLA for PTC-AADC for the treatment of AADC deficiency in the United States, which we anticipate submitting to

the FDA in the second half of 2020. We also anticipate filing for marketing authorization for Waylivra with ANVISA in the second half of 2020. These efforts may significantly impact the timing and extent of our commercialization expenses.

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

- seek to satisfy contractual and regulatory obligations we assumed in connection with the Agilis acquisition;
- seek to satisfy contractual and regulatory obligations in conjunction with the Akcea Agreement;
- satisfy contractual and regulatory obligations that we assumed through our other acquisitions and collaborations;
- execute our commercialization strategy for our products and product candidates that may receive marketing authorization;
- are required to complete any additional clinical trials, non-clinical studies or Chemistry, Manufacturing and Controls, or CMC, assessments or analyses in order to advance Translarna for the treatment of nmDMD in the United States or elsewhere;
- utilize the Hopewell Facility to begin manufacturing program materials for certain of our gene therapy product candidates;
- initiate or continue the research and development of our gene therapy, splicing, Bio-e and oncology programs as well as studies in our products for maintaining authorizations, including Study 041, label extensions and additional indications;
- seek to discover and develop additional product candidates;
- seek to expand and diversify our product pipeline through strategic transactions;
- maintain, expand and protect our intellectual property portfolio; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and commercialization efforts.

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

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

- our ability to commercialize and market our products and product candidates that may receive marketing authorization;
- our ability to negotiate, secure and maintain adequate pricing, coverage and reimbursement terms, on a timely basis, with third-party payors for our products and products candidates;
- our ability to maintain the marketing authorization for our products, including in the EEA for Translarna for the treatment of nmDMD and whether the EMA determines on an annual basis that the benefit-risk balance of Translarna supports renewal of our marketing authorization in the EEA, on the current approved label;
- the costs, timing and outcome of Study 041;
- the costs, timing and outcome of our efforts to advance Translarna for the treatment of nmDMD in the United States, including, whether we will be required to perform additional clinical trials, non-clinical studies or CMC assessments or analyses at significant cost which, if successful, may enable FDA review of an NDA submission by us and, ultimately, may support approval of Translarna for nmDMD in the United States;
- our ability to maintain orphan exclusivity in the United States for Emflaza and successfully completing all post-marketing requirements with respect to Emflaza and any other products;
- the progress and results of activities under our gene therapy, splicing, Bio-e and oncology programs as well as studies in our products for maintaining authorizations, label extensions and additional indications;
- the scope, costs and timing of our commercialization activities, including product sales, marketing, legal, regulatory, distribution and manufacturing, for any of our products and for any of our other product candidates that may receive

marketing authorization or any additional indications or territories in which we receive authorization to market Translarna;

- the costs, timing and outcome of regulatory review of our gene therapy, splicing, Bio-e and oncology programs and Translarna for additional indications and in other territories;
- unexpected decreases in revenue or increase in expenses resulting from the COVID-19 pandemic;
- our ability to utilize the Hopewell Facility to begin manufacturing program materials for certain of our gene therapy product candidates;
- our ability to satisfy our obligations under the terms of the Credit Agreement with MidCap Financial;
- our ability to satisfy our obligations under the indentures governing the Convertible Notes;
- the timing and scope of growth in our employee base;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our other product candidates, including those in our gene therapy, splicing, Bio-e and oncology programs and Translarna for additional indications;
- revenue received from commercial sales of our products or any of our product candidates;
- our ability to obtain additional and maintain existing reimbursed named patient and cohort EAP programs for Translarna for the treatment of nmDMD on adequate terms, or at all;
- the ability and willingness of patients and healthcare professionals to access Translarna through alternative means if pricing and reimbursement negotiations in the applicable territory do not have a positive outcome;
- the costs of preparing, filing and prosecuting patent applications, maintaining, and protecting our intellectual property rights and defending against intellectual property-related claims;
- the extent to which we acquire or invest in other businesses, products, product candidates, and technologies, including the success of any acquisition, in-licensing or other strategic transaction we may pursue, and the costs of subsequent development requirements and commercialization efforts, including with respect to our acquisition of Emflaza, our acquisition of Agilis, our licensing of Tegsedi and Waylivra and our acquisition of our Bio-e platform; and
- our ability to establish and maintain collaborations, including our collaborations with Roche and the SMA Foundation, and our ability to obtain research funding and achieve milestones under these agreements.

With respect to our outstanding 2022 Convertible Notes, cash interest payments are payable on a semi-annual basis in arrears, which require total funding of $4.5 million annually. With respect to our outstanding 2026 Convertible Notes, cash interest payments are payable on a semi-annual basis in arrears, which will require total funding of $4.3 million annually. Additionally, under the terms of our Credit Agreement cash interest payments are payable monthly in arrears. Furthermore, since we are a public company, we have incurred and expect to continue to incur additional costs associated with operating as such, including significant legal, accounting, investor relations and other expenses.

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

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

Off-Balance Sheet Arrangements

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

Contractual obligations

During the period ended March 31, 2020, there were no material changes to our contractual obligations and commitments outside the ordinary course of business from those disclosed under the heading "Management's Discussion and Analysis of Financial Condition and Results of Operations-Contractual Obligations" in our 2019 Annual Report on Form 10-K, other than as disclosed below.

(in thousands)	Total	Less than 1 year	1 - 3 years	3 - 5 years	More than 5 years
Operating lease obligations, not yet commenced (1)	$ 111,528	3,758	13,354	13,893	80,523
Operating lease obligations, not yet commenced (2)	$ 14,721	1,730	4,000	4,202	4,789
Total contractual obligations	$ 126,249	$ 5,488	$ 17,354	$ 18,095	$ 85,312

(1) Obligations stem from our lease agreement entered into with Bristol-Myers Squibb Company in August 2019 relating to the lease of approximately 185,000 square feet of office, manufacturing and laboratory space at a facility located in Hopewell Township, New Jersey. On March 25, 2020, the Company entered into an amendment increasing the rented space to approximately 220,500 square feet. The term of occupancy has not yet commenced as of March 31, 2020.

(2) We entered into a lease agreement with COE Bridgewater LLC on March 20, 2020 relating to the lease of office and laboratory space located in Bridgewater, New Jersey. This lease will replace our existing lease on the property beginning on May 1, 2020 and includes additional rental property of approximately 59,000 square feet. Obligations stem from the occupancy of the additional space, which has not yet commenced as of March 31, 2020.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

During the period ended March 31, 2020, there were no material changes in our market risk or how our market risk is managed, compared to those disclosed under the heading "Quantitative and Qualitative Disclosures about Market Risk" in our 2019 Annual Report on Form 10-K.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and our Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of March 31, 2020. The term "disclosure controls and procedures", as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the Securities and Exchange Commission's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of March 31, 2020, our Chief Executive

Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

No change in our internal control over financial reporting occurred during the quarter ended March 31, 2020 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

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

Item 1A. Risk Factors

We have set forth in Item 1A to our Annual Report on Form 10-K for the year ended December 31, 2019, risk factors relating to our business, our industry, our structure and our common stock. Readers of this Quarterly Report on Form 10-Q are referred to such Item 1A for a more complete understanding of risks concerning us. The COVID-19 pandemic has heightened, and in some cases manifested, certain of the risks we normally face in operating our business, including those disclosed in our Annual Report on Form 10-K for the year ended December 31, 2019, and the risk factor disclosure in our Annual Report on Form 10-K for the year ended December 31, 2019 is qualified by the information related to COVID-19 that is described in this Quarterly Report on Form 10-Q, including the new risk factor set forth below.

Other than as set forth below, there have been no material changes in our risk factors since those published in such Form 10-K for the year ended December 31, 2019.

We face risks related to health epidemics and other widespread outbreaks of contagious disease, which are, and may continue to, delay our ability to complete our ongoing clinical trials and initiate future clinical trials, disrupt regulatory activities and have other adverse effects on our business and operations, including the novel coronavirus (COVID-19) pandemic, which could significantly disrupt our operations and impact our operating results. In addition, this pandemic has caused substantial disruption in the financial markets and economies, which could result in adverse effects on our business and operations.

Significant outbreaks of contagious diseases, and other adverse public health developments, could have a material impact on our business operations and operating results. In December 2019, a strain of novel coronavirus, COVID-19, causing respiratory illness emerged in the city of Wuhan in the Hubei province of China. Since that time, multiple other countries throughout the world, including the United States, have been affected by the spread of the virus. To date, responsive measures such as social distancing, travel bans and quarantines have been put into place in many countries throughout the world, including the United States. These responsive measures have had a significant impact, both direct and indirect, on business and commerce worldwide, as worker shortages have occurred, supply chains have been disrupted, facilities and production have been suspended.

The spread of COVID-19 and the responsive measures taken to date have limited our access to our facilities and caused the majority of our employees to work from home. We continue to monitor the global spread and response of international, national and local authorities of COVID-19 and have put in place and will continue to put in place measures as appropriate and necessary for our business and the safety of our employees and our business. While we expect the pandemic to have an adverse effect on our business, and the pandemic may have an adverse effect on our financial conditions and results of operations, we are unable to predict the extent or nature of the future progression of the outbreak or its effects on our business, operations, financial condition and results of operations at this time.

Furthermore, we have clinical trial sites located in countries that have been affected by COVID-19 that have been and may continue to be disrupted. The disruption of our clinical trial sites is having an adverse impact on our clinical trial plans and timelines. For example, we initiated Study 045 in the fourth quarter of 2018 to evaluate the ability of ataluren to increase dystrophin protein levels in boys with nmDMD. We intend to use the data from Study 045 in support of our NDA resubmission for Translarna for the treatment of nmDMD. As a result of the COVID-19 pandemic, our clinical trial site for Study 045 at the University of California, Los Angeles is temporarily closed to elective procedures, resulting in the delay of Study 045's expected completion as certain patients still require final study muscle biopsies. During the delay, patients are remaining on drug until the biopsies can be performed. We currently expect data for Study 045 will be available in the third quarter of 2020 followed by a potential re-submission of the NDA thereafter, however, the current pandemic may cause additional unforeseen delays. Other clinical trial sites as well as significant suppliers and manufacturing located in countries that have been affect by COVID-19 may also be disrupted, which may affect our ability to procure items that are essential for our research and development activities and may cause disruptions. The response to the COVID-19 pandemic may redirect resources with respect to regulatory matters in a way that would adversely impact our ability to progress regulatory approval. We may also choose to redirect our own resources in a way that may adversely impact or delay certain of our programs.
In addition, we may face impediments to regulatory meetings and approvals due to measures intended to limit in-person interactions.

We cannot foresee if and when the outbreak of COVID-19 will be effectively contained, nor can we predict the severity and duration of its impact. If the COVID-19 pandemic is not effectively and timely controlled, we may experience prolonged disruption

of our clinical trials, suppliers or contract manufacturers, extended closures of facilities, such as clinical trial sites, suppliers, manufacturers and distributors, including single source suppliers, and further delays with respect to regulatory approvals or the commercialization of any current or future products. Such events may materially and adversely affect our business operations and financial condition. Additionally, the pandemic has already caused significant disruptions in the financial markets, and may continue to cause such disruptions, which could impact our ability to raise additional funds and has also impacted, and may continue to impact, the volatility of our stock price and trading in our stock. Moreover, it is possible the pandemic will significantly impact economies worldwide, which could result in adverse effects on our business and operations. We cannot be certain what the overall impact of the COVID-19 pandemic will be on our business and it has the potential to materially adversely affect our business, financial condition, results of operations, and prospects.

Item 5. Other Information.

On April 28, 2020, our Compensation Committee adopted resolutions, or the Resolutions, ratifying the issuance of certain shares of our common stock to employees and a consultant in connection with existing stock options and restricted stock units pursuant to Section 204 of the General Corporation Law of the State of Delaware, or the Ratification, after it determined that the issuances may not have been duly authorized in accordance with Sections 152 and 157 of the General Corporation Law of the State of Delaware. The following share issuances have been ratified pursuant to the Resolutions: (i) 1,250 shares issued on March 19, 2019, (ii) 156 shares issued on June 3, 2019, (iii) 312 shares issued on June 27, 2019, (iv) 156 shares issued on September 3, 2019, (v) 156 shares issued on December 2, 2019, (vi) 93 shares issued on December 10, 2019, (vi) 1,164 shares issued on January 3, 2020, (vii) 766 shares issued on January 6, 2020, (viii) 10,346 shares issued on January 8, 2020 (ix) 896 shares issued on January 22, 2020 and (x) 313 shares issued on February 3, 2020. Any claim that the defective corporate acts (including all putative stock) ratified in the Resolutions are void or voidable due to the failure of authorization, or any claim that the Court of Chancery of the State of Delaware should declare in its discretion that the ratifications not be effective or be effective only on certain conditions, must be brought within 120 days from the date of the filing of this Quarterly Report on Form 10-Q.

Item 6. Exhibits.

Exhibit Number	Description of Exhibit
10.1†	Second Amendment to Lease Agreement dated as of March 25, 2020 by and between Bristol-Myers Squibb Company and PTC Therapeutics, Inc.
31.1	Certification of Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2	Certification of Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1	Certification of Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
32.2	Certification of Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.INS	Inline XBRL Instance Document*
101.SCH	Inline XBRL Taxonomy Extension Schema Document*
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document*
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Database*
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document*
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document*
104	The cover page from this Quarterly Report on Form 10-Q, formatted in Inline XBRL

* Submitted electronically herewith.

† Portions of this exhibit have been omitted pursuant to Item 601(b)(10)(iv) of Regulation S-K.

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

SIGNATURES

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

PTC THERAPEUTICS, INC.

Date: April 30, 2020

By: /s/ Emily Hill

Emily Hill
Chief Financial Officer
(Principal Financial Officer and Duly Authorized Signatory)

EXHIBIT 10.1

Certain identified information has been marked in the exhibit because it is both (i) not material and (ii) would likely cause competitive harm to the Company, if publicly disclosed.
Double asterisks denote omissions.

SECOND AMENDMENT TO LEASE AGREEMENT

This Second Amendment ("Second Amendment") to the Lease Agreement ("Lease") is made on this 25th day of March 2020 by and between **BRISTOL-MYERS SQUIBB COMPANY**, a Delaware corporation, having an office at 3551 Lawrenceville Princeton Road, Princeton, New Jersey 08540 ("Landlord"), and **PTC THERAPEUTICS, INC.**, a Delaware Corporation, having an office at 100 Corporate Court, South Plainfield, NJ 07080-2449 ("Tenant").

WHEREAS, Landlord and Tenant entered into a certain Lease dated August 3, 2019 pursuant to which Landlord agreed to lease to Tenant and Tenant agreed to lease from Landlord a portion of 311 Pennington Rocky Hill Road, Hopewell Township, Mercer County, New Jersey, designated in the Lease as the Premises and further described in Exhibit A to the Lease; and

WHEREAS, Landlord and Tenant entered into a certain First Amendment to Lease Agreement dated October 7, 2019 ("First Amendment") whereby the terms of the Lease were amended to change the definition of Controllable CAM Expenses set forth in Section 4.2(a)(iii) and to incorporate the Letter of Credit issued by HSBC Bank USA, N.A. as an exhibit to the Lease in connection with Section 39(a) of the Lease (hereinafter the term "Lease" will refer to the original Lease as modified by the First Amendment); and

WHEREAS, Tenant has not yet taken occupancy of the Premise; and

WHEREAS, Landlord and Tenant have agreed to further amend the Lease to change the size and location of a portion of the Premises by providing that Tenant will no longer lease Building 13, consisting of [**] Rentable Square Fee ("RSF") and but will instead lease Buildings 3A, 3B and 5, consisting of [**] RSF; and

WHEREAS, this Second Amendment is executed by Landlord and Tenant to memorialize the change in the Premises by the substitution of Buildings 3A, 3B and 5 for Building 13 and to address the other modifications to the Lease necessitated by this change.

NOW, THEREFORE, for good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged, Landlord and Tenant agree to amend the Lease as follows:

1. The WHEREAS clauses set forth above are incorporated into this Second Amendment by reference.

2. In Section 1.2, the description of the Premises is amended to remove the reference to "Building 13" in Section 1.2(c) and replace it with "Buildings 3A, 3B and 5".

3. In Section 1.3, the total rentable area of the Premises is changed from 183,667 RSF to 220,517 RSF. Section 1.3.c. is removed and replaced as follows:

c. Building 3A, 3B and 5 [**] RSF

4. Section 1.7.c is removed and replaced as follows:

c. For Buildings 3A, 3B and 5, the Base Rent Commencement Date shall be six (6) months after the Commencement Date.

5. Section 1.9.c is removed and replaced as follows:

c. Buildings 3A, 3B and 5: $[**] per RSF on a triple net basis (NNN).

6. In Section 1.15, all reference to Landlord's Base Building Work in connection with Building 13 is deleted. The only Landlord's Base Building Work that will be completed by Landlord in connection with Buildings 3A, 3B and 5 is the installation of a submeter for Utilities as required pursuant to Section 12.5(a) of the Lease.

7. In Section 1.17, the first sentence is removed and replaced as follows:

On the Commencement Date of this Lease, the "Tenant's Share" is Nineteen and Eighty-Eight One Hundredths Percent (19.88%) which represents the RSF of the Premises (220,517 square feet, subject to the terms of Section 2.2), calculated as a percentage of the aggregate rentable area of the buildings located within the CAM Area of the Campus (see Section 2.3 below and **Exhibit D**).

8. Section 1.18 is amended to state that the total number of parking spaces allocated to Tenant is increased from 360 to 434. Section 1.18(a) through (e) are removed and replaced as set forth below to provide the allocation of the parking spaces, which spaces are shown on the replacement **Exhibit E** attached to this Second Amendment:

(a) [**] spaces, including [**] handicapped spaces, shall be allocated exclusively to Tenant, comprising all of Parking Area 1 adjacent to Buildings 9 and 10.

(b) [**] spaces shall be allocated exclusively to Tenant in Parking Area 2.

(c) [**] spaces shall be allocated exclusively to Tenant in Parking Area 6.

(d) [**] spaces shall be allocated exclusively to Tenant in Parking Area 7.

(e) [**] spaces shall be allocated exclusively to Tenant in Parking Area 8.

(f) The remaining [**] spaces shall be unassigned and unreserved spaces allocated to Tenant in Parking Areas 3, 4, 5, and 9.

9. In Section 2.2(i), the RSF for the Premises Buildings is changed from 183,667 RSF to 220,517 RSF.

10. In Section 2.4, the total number of parking spaces comprising "Tenant's Parking" is increased from [**] to [**]. The following sentences are added to the end of the last paragraph of Section 2.4:

> As set forth in Section 1.18, Tenant shall also have exclusive rights to certain parking areas in Parking Area 6, Parking Area 7 and Parking Area 8 as shown on Exhibit E. Tenant may not install gates in these parking areas but may install such signage as it deems reasonable necessary to ensure that this parking is retained for the exclusive use of Tenant.

11. In Section 2.5(a), add the following sentence after the first sentence: "All fixed hoods and chambers, and furniture, laboratory benches, freezers and related equipment located in Buildings 3A, 3B and 5 shall be delivered to Tenant with the Premises and shall be considered "FF&E under this Lease, but not "Exception Equipment".

12. In Section 4.2(a)(iii), all references to "calendar year" shall be changed to "Lease Year."

13. In Section 11.3(a), the first sentence is replaced as follows:

> "Tenant's Share is Nineteen and Eighty-Eight One Hundredths Percent (19.88%), which figure is calculated based upon Tenant's proportionate share of the Rentable Area of the Premises (approximately 220,517 square feet, subject to the terms of Section 2.2) as a percentage of the aggregate rentable area of the buildings located in both the CAM Area and the CAM Exclusion Area of the Campus."

14. The first sentence of Section 12.7 is replaced as follows: "Buildings 3A/3B/5 and 18 have existing diesel back-up generators (the "Existing Generators") that provide back-up power solely to those buildings." It is the intent of this provision that Tenant will be responsible for the existing diesel back-up generator serving Building 3A/3B/5 instead of the existing diesel back-up generator serving Building 13. A second paragraph is added to Section 12.7 as follows:

> Landlord will conduct testing of the generators for Building 9/10 in accordance with good industry practices and is currently conducting testing on a monthly basis. Landlord agrees to provide Tenant the results of Landlord's testing of the generators that service Building 9/10 within thirty (30) days of the completion of said testing. Landlord and Tenant agree and acknowledge that Tenant must submit the generator testing results for these buildings Building 9/10 to the Food and Drug Administration ("FDA") on a periodic basis and that if additional testing is required to satisfy FDA requirements and Landlord, after reasonable notice from Tenant, does not agree to undertake said testing, Tenant may undertake said testing at its own cost. Copies of the results of the testing, if undertaken by Tenant, will be submitted to Landlord within thirty (30) days of the completion of said testing.

15. The phrase "modify Tenant's exclusive parking in Parking Area 1 and Parking Area 3 or reduce Tenant's parking in Parking Area 2 and Parking Area 3 more than to a de minimis extent" in clause (i) of Section 26(d) is replaced with "modify Tenant's exclusive parking in Parking Area 1 and Parking Area 2 or reduce Tenant's parking in Parking Area 3, Parking Area 4, Parking Area

5, Parking Area 6, Parking Area 7, Parking Area 8 and Parking Area 9 more than to a de minimis extent".

16. The phrase "one (1) sign on the parapet wall of each of Buildings 9, 10, 13 and 18 (the "Building Exterior Signage")" in clause (i) of Section 40(a) is replaced with "one (1) sign on the parapet wall of each of Buildings 3A, 3B, 5, 9, 10 and 18 (the "Building Exterior Signage")".

17. The Schedules and Exhibits to the Lease are amended as follows:

a. **Schedule I** (Rent Schedule) is replaced with the revised **Schedule I** attached hereto.

b. **Exhibit A** (Campus and Premises Site Map) is replaced with the revised **Exhibit A** attached hereto.

c. **Exhibit D** (CAM Inclusion and Exclusion Area) is revised to remove and replace the chart showing the calculation of Tenant's Share attached hereto. The CAM Inclusion and Exclusion Area remains unchanged.

d. **Exhibit E** (Parking Plan) is replaced with the revised **Exhibit E** attached hereto.

e. **Exhibit J** (Initial Tenant Improvements) is amended to delete all reference to Initial Tenant Improvements to Building 13. No revised **Exhibit J** is attached hereto.

f. **Exhibit K** (Utility Services Terms) is amended to replace Schedule A to that exhibit with the revised Schedule A attached hereto.

g. **Exhibit M** (Existing Telecommunication Systems Wiring and Conduits) is replaced with the revised **Exhibit M** attached hereto.

h. **Exhibit N** (Pass-Through Utilities) is amended delete all reference to Building 13 as set forth in the revised **Exhibit N** attached hereto.

i. **Exhibit T** (Building 13, 17 & 21 Elevation) is deleted from the lease because none of the referenced buildings (13, 17 & 21) will be leased to Tenant.

18. Tenant shall have access to the loading dock and lavatory located in Building 18 as shown on **Schedule A** attached to this Amendment on a twenty four (24) hour per day, seven (7) day per week basis. Landlord shall be responsible for the maintenance and repair of such loading dock and lavatory.

19. This Second Amendment may be executed in one or more counterparts, which shall be deemed an original, and all of which together shall be deemed one and the same instrument. A facsimile or pdf transmission of an original signature shall be deemed an original signature.

20. Except as modified hereby, all terms of the Lease remain in full force and effect. In the event of a conflict between the terms of the Lease and this Second Amendment, the terms of this Second Amendment shall prevail.

[SIGNATURE PAGE TO FOLLOW]

IN WITNESS WHEREOF, Landlord and Tenant, intending to be legally bound, execute this First Amendment as of the dates set forth below.

LANDLORD:

BRISTOL-MYERS SQUIBB COMPANY, a Delaware corporation

By: /s/ Bruce K. Mayer
Name: Bruce K. Mayer
Title: Head Global Real Estate

TENANT:

PTC THERAPEUTICS, INC., a Delaware corporation

By: /s/ Mark Boulding
Name: Mark Boulding
Title: Chief Legal Officer and Executive Vice President

Exhibit 31.1

CERTIFICATIONS

I, Stuart W. Peltz, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: April 30, 2020

By: /s/ STUART W. PELTZ

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

Exhibit 31.2

CERTIFICATIONS

I, Emily Hill, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: April 30, 2020

By: /s/ EMILY HILL

Emily Hill
Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

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

In connection with the Quarterly Report on Form 10-Q of PTC Therapeutics, Inc. (the "Company") for the period ended March 31, 2020 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Stuart W. Peltz, Chief Executive Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his knowledge:

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

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

Date: April 30, 2020

By: /s/ STUART W. PELTZ

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

Exhibit 32.2

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

In connection with the Quarterly Report on Form 10-Q of PTC Therapeutics, Inc. (the "Company") for the period ended March 31, 2020 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Emily Hill, Chief Financial Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his knowledge:

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

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

Date: April 30, 2020

By: /s/ EMILY HILL

Emily Hill
Chief Financial Officer
(Principal Financial Officer)