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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, 2021

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 May 3, 2021, there were 70,455,908 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:

- 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;
- 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 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 utilize the dystrophin results from Study 045 and the totality of existing clinical and real-world data or, alternatively, data from Study 041 to support a marketing approval for Translarna for the treatment of nmDMD in the United States;
- our expectations with respect to the development, regulatory and commercial status of Evrysdi™ (risdiplam) and our 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 sales-based royalty payments or the achievement of milestones in that program;
- the timing and scope of our commercialization of our products and product candidates;
- 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 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 splicing, gene therapy, Bio-e, metabolic and oncology programs and studies of PTC299 for COVID-19 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 any post-marketing requirements imposed by regulatory agencies with respect to 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 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 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, metabolic 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, 2020, 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, 2020 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, 2021	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 146,632	$ 208,812
Marketable securities	841,774	894,838
Trade receivables, net	94,124	69,929
Inventory, net	16,234	18,697
Prepaid expenses and other current assets	23,601	39,469
Total current assets	1,122,365	1,231,745
Fixed assets, net	37,123	33,831
Intangible assets, net	712,574	715,328
Goodwill	82,341	82,341
Operating lease ROU assets	82,382	84,410
Deposits and other assets	75,156	60,623
Total assets	$ 2,111,941	$ 2,208,278
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 205,636	$ 242,168
Deferred revenue	1,887	4,151
Operating lease liabilities- current	7,333	7,465
Finance lease liabilities- current	1,694	1,276
Liability for sale of future royalties- current	33,149	21,023
Other current liabilities	1,250	1,250
Total current liabilities	250,949	277,333
Long-term debt	430,038	309,145
Contingent consideration payable	240,500	240,400
Deferred tax liability	136,735	136,735
Operating lease liabilities- noncurrent	77,863	79,499
Finance lease liabilities- noncurrent	20,053	23,053
Liability for sale of future royalties- noncurrent	661,835	658,739
Other long-term liabilities	1,392	1,392
Total liabilities	1,819,365	1,726,296
Stockholders' equity:		
Common stock, $0.001 par value. Authorized 125,000,000 shares; issued and outstanding 70,405,905 shares at March 31, 2021. Authorized 125,000,000 shares; issued and outstanding 69,718,096 shares at December 31, 2020.	70	70
Additional paid-in capital	2,033,972	2,171,746
Accumulated other comprehensive income	(38,743)	(60,957)
Accumulated deficit	(1,702,723)	(1,628,877)
Total stockholders' equity	292,576	481,982
Total liabilities and stockholders' equity	$ 2,111,941	$ 2,208,278

See accompanying unaudited notes.

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

	Three Months Ended March 31,	
	2021	2020
Revenues:		
Net product revenue	$ 91,280	68,196
Collaboration revenue	20,007	63
Royalty revenue	6,655	—
Total revenues	117,942	68,259
Operating expenses:		
Cost of product sales, excluding amortization of acquired intangible assets	9,104	4,085
Amortization of acquired intangible assets	11,278	7,949
Research and development	134,513	90,107
Selling, general and administrative	61,095	58,209
Change in the fair value of deferred and contingent consideration	100	900
Total operating expenses	216,090	161,250
Loss from operations	(98,148)	(92,991)
Interest expense, net	(19,159)	(5,642)
Other expense, net	(10,884)	(13,832)
Loss before income tax expense	(128,191)	(112,465)
Income tax expense	(451)	(222)
Net loss attributable to common stockholders	$ (128,642)	$ (112,687)
Weighted-average shares outstanding:		
Basic and diluted (in shares)	70,188,602	62,389,158
Net loss per share—basic and diluted (in dollars per share)	$ (1.83)	$ (1.81)

See accompanying unaudited notes.

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

	Three Months Ended March 31,	
	2021	2020
Net loss	$ (128,642)	$ (112,687)
Other comprehensive (loss) income:		
Unrealized loss on marketable securities, net of tax of $0	(1,294)	(63)
Foreign currency translation gain, net of tax of $0	23,508	8,662
Comprehensive loss	$ (106,428)	$ (104,088)

See accompanying unaudited notes.

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

Three months ended March 31, 2021	Common stock		Additional paid-in capital	Accumulated other comprehensive (loss) income	Accumulated deficit	Total stockholders' equity
	Shares	Amount				
Balance, December 31, 2020	69,718,096	$ 70	$ 2,171,746	$ (60,957)	$ (1,628,877)	$ 481,982
Adjustment for adoption of ASU 2020-06			(175,236)	—	54,796	(120,440)
Exercise of options	415,783	—	11,755	—	—	11,755
Restricted stock vesting and issuance, net	272,026	—	—	—	—	—
Share-based compensation expense	—	—	25,707	—	—	25,707
Net loss	—	—	—	—	(128,642)	(128,642)
Comprehensive income	—	—	—	22,214	—	22,214
Balance, March 31, 2021	70,405,905	$ 70	$ 2,033,972	$ (38,743)	$ (1,702,723)	$ 292,576

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 offering	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

See accompanying unaudited notes.

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

	Three Months Ended March 31,	
	2021	2020
Cash flows from operating activities		
Net loss	$ (128,642)	(112,687)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	13,587	9,235
Non-cash operating lease expense	1,819	1,330
Non-cash royalty revenue related to sale of future royalties	(2,858)	—
Non-cash interest expense on liability related to sale of future royalties	18,080	—
Change in valuation of deferred and contingent consideration	100	900
Unrealized (gain) loss on ClearPoint Equity Investments	(6,764)	1,574
Unrealized (gain) loss on ClearPoint convertible debt security	(7,858)	2,799
Unrealized loss on marketable securities- equity investments	302	—
Amortization of premiums (discounts) on investments, net	1,359	(333)
Amortization of debt issuance costs	452	256
Share-based compensation expense	25,707	15,220
Non-cash interest expense	—	5,459
Unrealized foreign currency transaction gains, net	24,691	8,675
Changes in operating assets and liabilities:		
Inventory, net	1,963	102
Prepaid expenses and other current assets	15,605	(6,675)
Trade receivables, net	(25,010)	2,346
Deposits and other assets	136	(192)
Accounts payable and accrued expenses	(28,756)	(4,000)
Other liabilities	(1,916)	(4,363)
Deferred revenue	(2,154)	(1,200)
Net cash used in operating activities	$ (100,157)	$ (81,554)
Cash flows from investing activities		
Purchases of fixed assets	$ (5,669)	(6,023)
Purchase of convertible debt security	—	(10,000)
Purchases of marketable securities- available for sale	(141,985)	(298,814)
Purchases of marketable securities- equity investments	(200,000)	—
Sale and redemption of marketable securities- available for sale	392,093	224,997
Acquisition of product rights and licenses	(14,192)	(11,434)
Purchase of equity investment in ClearPoint	(100)	—
Net cash provided by (used in) investing activities	$ 30,147	$ (101,274)
Cash flows from financing activities		
Proceeds from exercise of options	11,755	9,987
Net proceeds from public offerings	—	13,503
Repayment of senior secured term loan	—	(5,000)
Payment of finance lease principal	(2,224)	—
Net cash provided by financing activities	$ 9,531	$ 18,490
Effect of exchange rate changes on cash	(1,701)	(403)
Net decrease in cash and cash equivalents	(62,180)	(164,741)
Cash and cash equivalents, and restricted cash beginning of period	216,312	295,528
Cash and cash equivalents, and restricted cash end of period	$ 154,132	$ 130,787
Supplemental disclosure of cash information		
Cash paid for interest	$ 5,182	4,878
Cash paid for income taxes	687	507
Supplemental disclosure of non-cash investing and financing activity		
Unrealized loss on marketable securities, net of tax	$ (1,294)	(63)
Right-of-use assets obtained in exchange for operating lease obligations	13	22,642
Acquisition of product rights and licenses	8,870	2,775

See accompanying unaudited notes.

PTC Therapeutics, Inc.

Notes to Consolidated Financial Statements (unaudited)
March 31, 2021
In thousands (except share and per share amounts 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 leverage its strong scientific expertise and 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 ("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 ("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. In July 2020, the European Commission approved the removal of the statement "efficacy has not been demonstrated in non-ambulatory patients" from the indication statement for Translarna. Emflaza is approved in the United States for the treatment of DMD in patients two years and older.

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 Collaboration and License Agreement (the "Akcea Collaboration and License Agreement"), dated August 1, 2018, by and between the Company and 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 ("hATTR amyloidosis"). While the Company is in the process of initiating its commercial launch for Tegsedi for the treatment of hATTR amyloidosis in Latin America, it continues to make Tegsedi available in certain countries within Latin America and the Caribbean through early access programs. Waylivra has received marketing authorization in the EU for the treatment of familial chylomicronemia syndrome ("FCS"). The Company filed for marketing authorization for Waylivra for the treatment of FCS with ANVISA, the Brazilian health regulatory authority, in June 2020 and, subject to potential delays in the review process related to the COVID-19 pandemic, expects a regulatory decision on approval from ANVISA in the third quarter of 2021.

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 ("SMA Foundation"). The SMA program has one approved product, Evrysdi™ (risdiplam), which was approved by the United States Food and Drug Administration ("FDA") in August 2020 for the treatment of SMA in adults and children two months and older and by the European Commission in March 2021 for the treatment of 5q SMA in patients two months and older with a clinical diagnosis of SMA Type 1, Type 2 or Type 3 or with one to four SMN2 copies. Evrysdi also received marketing authorization for the treatment of SMA in Brazil in October 2020. Additionally, in October 2020, Chugai Pharmaceutical Co., Ltd. ("Chugai"), a subsidiary of Roche, filed a New Drug Application ("NDA") in Japan for Evrysdi for the treatment of SMA and a regulatory decision on approval is expected in 2021. In addition to the Company's SMA program, the Company's splicing platform also includes PTC518, which is being developed for the treatment of Huntington's disease ("HD"). The Company announced preliminary results from its ongoing Phase 1 study of PTC518 in healthy volunteers in April 2021 demonstrating dose-dependent lowering of huntingtin messenger ribonucleic acid levels and that PTC518 was well tolerated with no safety-related findings.

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 ("AADC") deficiency ("AADC deficiency"), a rare CNS disorder arising from reductions in the enzyme AADC that results from mutations in the dopa decarboxylase gene. In January 2020, the Company submitted an 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 ("CHMP") in the third quarter of 2021. The Company is also preparing a biologics license application ("BLA") for PTC-AADC for the treatment of AADC deficiency in the United States. The Company anticipates the BLA submission to the FDA to be delayed by at least one quarter.

The Company's Bio-e platform consists of small molecule compounds that target oxidoreductase enzymes that regulate oxidative stress and inflammatory pathways central to the pathology of a number of CNS diseases. The two most advanced molecules in the Company's Bio-e platform are vatiquinone and PTC857. The Company initiated a registration-directed Phase 2/3 placebo-controlled trial of vatiquinone in children with mitochondrial disease and associated refractory epilepsy in the third quarter of 2020 and anticipates data from this trial to be available in the third quarter of 2022. The Company also initiated a registration-directed Phase 3 trial of vatiquinone in children and young adults with Friedreich ataxia in the fourth quarter of 2020 and anticipates data from this trial to be available in 2023. In the second quarter of 2020, the Company initiated a Phase 1 trial in healthy volunteers to evaluate the safety and pharmacology of PTC857. This Phase 1 trial was recently completed and the Company expects data to be available in the second quarter of 2021.

On May 29, 2020, the Company completed its acquisition of Censa Pharmaceuticals, Inc. ("Censa") pursuant to an Agreement and Plan of Merger, dated as of May 5, 2020 (the "Censa Merger Agreement"), by and among the Company, Hydro Merger Sub, Inc., the Company's wholly owned, indirect subsidiary, and, solely in its capacity as the representative, agent and attorney-in-fact of the securityholders of Censa, Shareholder Representative Services LLC (the "Censa Merger"). The transaction was accounted for as an asset acquisition. In connection with the Censa Merger, the Company acquired PTC923, an oral formulation of synthetic sepiapterin, a precursor to intracellular tetrahydrobiopterin, which is a critical enzymatic cofactor involved in metabolism and synthesis of numerous metabolic products. The Company expects to initiate a registration-directed Phase 3 trial for PTC923 for phenylketonuria ("PKU") in mid-2021.

In June 2020, the Company initiated a Phase 2/3 clinical trial evaluating the efficacy and safety of PTC299, a dihydroorotate dehydrogenase inhibitor that the Company has also been developing in oncological indications, in patients hospitalized with COVID-19. The Company expects data from this trial to be available in the second half of 2021.

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 June 2020 and is effective, unless extended, through August 5, 2021. In February 2021, the Company submitted a marketing authorization renewal request to the EMA. 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.

Translarna is an investigational new drug in the United States. During the first quarter of 2017, the Company filed a New Drug Application ("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 followed the FDA's recommendation and collected, using newer technologies via procedures and methods that the Company designed, such dystrophin data in a new study, Study 045, and announced the results of Study 045 in February 2021. Although Study 045 did not meet its pre-specified primary endpoint, the Company plans to discuss the Study 045 dystrophin results and the totality of existing clinical and real-world data with the FDA to determine if there is a potential path to approval based on these results and data. There is substantial risk that the FDA will determine that the results from the Company's clinical trials and existing real-world data are not sufficient to support a marketing approval for Translarna for the treatment of nmDMD in the United States. In that case, as the Company expects to have data for Study 041 in the third quarter of 2022, and subject to a positive outcome in that study, the Company would plan to re-submit the NDA at that time.

As of March 31, 2021, the Company had an accumulated deficit of approximately $1,702.7 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 (collectively, the "Convertible Notes") (see Note 9), 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, proceeds from a Royalty Purchase Agreement dated as of July 17, 2020, by and among the Company, RPI 2019 Intermediate Finance Trust ("RPI"), and, solely for the limited purposes set forth therein, Royalty Pharma PLC (the "Royalty Purchase Agreement") (see Note 2), private placements of its convertible preferred stock, collaborations, bank and institutional lender 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 has also relied on revenue associated with milestone and royalty payments from Roche pursuant to the License and Collaboration Agreement (the "SMA License Agreement") dated as of November 23, 2011, by and among the Company, Roche and, for the limited purposes set forth therein, the SMA Foundation, under its SMA program. 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, 2020 included in the Company's Annual Report on Form 10-K filed with the Securities and Exchange Commission (the "SEC") on February 25, 2021 (the "2020 Form 10-K"). Selected significant accounting policies are discussed in further detail below.

Basis of presentation

The accompanying financial information as of March 31, 2021 and for the three months ended March 31, 2021 and 2020 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. Certain prior period balances have been reclassified to conform to the current period presentation. These reclassifications did not have a material impact on the consolidated statements of operations, consolidated balance sheets, consolidated statements of cash flows, or notes to the consolidated financial statements. These interim financial statements should be read in conjunction with the Company's audited financial statements as of December 31, 2020 and notes thereto included in the 2020 Form 10-K.

In the opinion of management, the unaudited financial information as of March 31, 2021 and for the three months ended March 31, 2021 and 2020 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, 2021 are not necessarily indicative of the results to be expected for the year ended December 31, 2021 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, royalty revenue, certain accruals related to the Company's research and development expenses, valuation procedures for liability for sale of future royalties, 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 the Company's 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:

	End of period- March 31, 2021	Beginning of period- December 31, 2020
Cash and cash equivalents	$ 146,632	$ 208,812
Restricted cash included in deposits and other assets	7,500	7,500
Total Cash, cash equivalents and restricted cash per statement of cash flows	$ 154,132	$ 216,312

Marketable securities

The Company's marketable securities consists of both debt securities and equity investments. The Company considers its investments in debt 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, 2021, no allowance was recorded for credit losses.

Marketable securities that are equity investments are measured at fair value, as it is readily available, and as such are classified as Level 1 assets. Unrealized holding gains and losses for these equity investments are components of other expense, net within the consolidated statement of operations.

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. For the three month period ended March 31, 2021, no amounts related to clinical development programs and marketing efforts were recorded.

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

	March 31, 2021	December 31, 2020
Raw materials	$ 678	$ 824
Work in progress	8,401	8,745
Finished goods	7,155	9,128
Total inventory	$ 16,234	$ 18,697

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 periods ended March 31, 2021 and 2020, the Company recorded a $1.4 million and a $0.2 million inventory write down, respectively, primarily related to product approaching expiration. 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, 2021 and 2020, 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, royalty payments associated with net product sales, and royalty payments to collaborative partners associated with royalty revenues and collaboration revenue related to milestones. Production costs are expensed as cost of product sales when the related products are sold or royalty revenues and collaboration revenue milestones are earned.

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, 2021 and 2020, net product sales outside of the United States were $47.8 million and $40.7 million, respectively, and net product sales in the United States were $43.5 million and $27.5 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 and royalty 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 royalties from product sales at the later of when the related sales occur or when the performance obligation to which the royalty has been allocated has been satisfied. If it is probable that a significant revenue reversal will not occur, the Company will estimate the royalty payments using the most likely amount method.

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.

For the three months ended March 31, 2021 and 2020, the Company recognized $20.0 million and $0.1 million of collaboration revenue, respectively, related to the SMA License Agreement with Roche. The first commercial sale of Evrysdi in the European Union was made in March 2021. This event triggered a $20.0 million milestone payment to the Company from Roche. No milestones were triggered in the three months ended March 31, 2020.

For the three months ended March 31, 2021, the Company has recognized $6.7 million of royalty revenue related to Evrysdi. No royalty revenue related to Evrysdi was recognized in the three months ended March 31, 2020.

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 Company also assesses whether an allowance for expected credit losses may be required which includes a review of the Company's receivables portfolio, which are pooled on a customer basis or country basis. In making its assessment of whether an allowance for credit losses is required, the Company considers its historical experience with customers, current balances, levels of delinquency, regulatory and legal environments, and other relevant current and future forecasted economic conditions. For the three month periods ended March 31, 2021 and 2020, no allowance was recorded for credit losses. The allowance for doubtful accounts was $0.2 million as of March 31, 2021 and $0.1 million as of December 31, 2020. Bad debt expense was immaterial for the three month periods ended March 31, 2021 and 2020.

Liability for sale of future royalties

On July 17, 2020, the Company entered into the Royalty Purchase Agreement. Pursuant to the Royalty Purchase Agreement, the Company sold to RPI 42.933% (the "Assigned Royalty Payment") of the Company's right to receive sales-based royalty payments (the "Royalty") on worldwide net sales of Evrysdi and any other product developed pursuant to the SMA License Agreement. In consideration for the sale of the Assigned Royalty Payments, RPI paid the Company $650.0 million in cash consideration. The Company has retained a 57.067% interest in the Royalty and all economic rights to receive the remaining potential regulatory and sales milestone payments under the SMA License Agreement, which milestone payments equal $335.0 million in the aggregate as of March 31, 2021. The Royalty Purchase Agreement will terminate 60 days following the earlier of the date on which Roche is no longer obligated to make any payments of the Royalty pursuant to the SMA License Agreement and the date on which RPI has received $1.3 billion in respect of the Assigned Royalty Payments.

The cash consideration obtained pursuant to the Royalty Purchase Agreement is classified as debt and is recorded as "liability for sale of future royalties-current" and "liability for sale of future royalties-noncurrent" on the Company's consolidated balance sheet based on the timing of the expected payments to be made to RPI. The fair value for the liability for sale of future royalties at the time of the transaction was based on the Company's estimates of future royalties expected to be paid to RPI over the life of the arrangement, which was determined using forecasts from market data sources, which are considered Level 3 inputs. The liability will be amortized using the effective interest method over the life of the arrangement, in accordance with the respective guidance. The Company will utilize the prospective method to account for subsequent changes in the estimated future payments to be made to RPI. Refer to Note 9 for further details.

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 the pandemic caused by a strain of novel coronavirus, COVID-19. The CARES Act includes modifications for net operating loss carryovers and carrybacks, limitations of tax deductibility for net interest expense, 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") for qualified improvement property. On December 27, 2020, additional COVID-19 relief provisions included in the Consolidated Appropriations Act, 2021, were signed into law. The new relief provisions include multiple measures and expands many of the provisions already put into place under the CARES Act. As of March 31, 2021, the Company expects that these provisions will not have a material impact on the Company's financial statements. Tax provisions of the CARES Act also include the deferral of certain payroll taxes, relief for retaining employees, and other provisions. The relief for retaining employees was not material to the financial statements and the deferral of certain payroll taxes amounted to $2.5 million as of March 31, 2021, which is accrued in other current liabilities and other long-term liabilities on the consolidated balance sheets.

Additionally, the Organization for Economic Co-operation and Development ("OECD"), the European Community ("the EC"), and individual taxing jurisdictions where the Company and its affiliates do business have recently focused on issues related to the taxation of multinational corporations. The OECD has released its comprehensive plan to create an agreed set of international rules for fighting base erosion and profit shifting. In addition, the OECD, the EC and individual taxing jurisdictions are examining changes to how taxing rights should be allocated among countries considering the digital economy. As a result, the tax laws in the U.S. and other countries in which the Company and its affiliates do business could change on a prospective or retroactive basis and any such changes could materially adversely affect the Company's business.

On December 22, 2017, the U.S. government enacted the 2017 Tax Act, which significantly revised U.S. tax law by, among other provisions, lowering the U.S. federal statutory corporate 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-Taxed 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, 2021.

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 its acquisition of Agilis Biotherapeutics, Inc. (“Agilis”), pursuant to an Agreement and Plan of Merger, dated as of July 19, 2018 (the “Agilis Merger Agreement”), by and among the Company, 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, (the “Agilis 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 and finance leases are classified as right of use ("ROU") assets, short term lease liabilities, and long term lease liabilities. Operating and finance 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 August 2020, the Financial Accounting Standards Board (“FASB”) issued Accounting Standards Update (“ASU”) 2020-06, “Debt—Debt with Conversion and Other Options (Subtopic 470-20) and Derivatives and Hedging—Contracts in Entity’s Own Equity (Subtopic 815-40): Accounting for Convertible Instruments and Contracts in an Entity’s Own

Equity." ASU 2020-06 simplifies the accounting for convertible instruments by removing certain separation models in Subtopic 470- 20, Debt—Debt with Conversion and Other Options, for convertible instruments. Under ASU 2020-06, the embedded conversion features no longer are separated from the host contract for convertible instruments with conversion features that are not required to be accounted for as derivatives under Topic 815, Derivatives and Hedging, or that do not result in substantial premiums accounted for as paid-in capital. Consequently, a convertible debt instrument will be accounted for as a single liability measured at its amortized cost and a convertible preferred stock will be accounted for as a single equity instrument measured at its historical cost, as long as no other features require bifurcation and recognition as derivatives. By removing those separation models, the interest rate of convertible debt instruments typically will be closer to the coupon interest rate when applying the guidance in Topic 835, Interest. The amendments under ASU 2020-06 also include revisions related to the derivatives scope exception for contracts in an entity's own equity and earnings per share. The amendments under ASU 2020-06 are effective for public business entities that meet the definition of a SEC filer, excluding entities eligible to be smaller reporting companies as defined by the SEC, for fiscal years beginning after December 15, 2021, including interim periods within those fiscal years. For all other entities, the amendments are effective for fiscal years beginning after December 15, 2023, including interim periods within those fiscal years. Early adoption is permitted, but no earlier than fiscal years beginning after December 15, 2020, including interim periods within those fiscal years. The FASB specified that an entity should adopt the guidance as of the beginning of its annual fiscal year. The Company early adopted this guidance on January 1, 2021, utilizing the modified retrospective method. The Company now accounts for its Convertible Notes as single liabilities measured at amortized cost. As a result, the adoption of the guidance had a material impact on the consolidated financial statements and accompanying notes, resulting in adjustments of $175.2 million, $54.8 million, and $120.4 million to the opening balances of additional paid-in capital, retained earnings, and long term debt, respectively, as of January 1, 2021. Additionally, due to the adoption, the Company reversed the remaining balance of the deferred tax liability of $29.6 million which was initially recorded in connection with the Convertible Notes. Additionally, the Company increased the existing valuation allowance by $29.6 million as part of the adoption adjustment. The Company concluded that the adoption of the ASU did not change its prior valuation allowance conclusions. The Company has updated its debt note (Note 9) with additional and modified disclosures as required by the standard upon adoption.

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 approximately 220,500 square feet of office, manufacturing and laboratory space at a facility located in Hopewell Township, New Jersey (the "Campus") pursuant to a Lease Agreement (the "Lease") with Hopewell Campus Owner LLC (the "Landlord"). The rental term of the Lease commenced on July 1, 2020 and has an initial term of fifteen years (the "Initial Term"), with two consecutive ten 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.

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 replaced the Company's existing lease on the property beginning on May 1, 2020 and includes additional rental property of approximately 59,000 square feet.

On June 19, 2020, the Company entered into a commercial manufacturing service agreement for a term of 12.5 years with MassBiologics of the University of Massachusetts Medical School ("MassBio"). The agreement will expire on December 31, 2032 unless the Company terminates it on 24 months prior written notice to MassBio. Pursuant to the terms of the agreement, MassBio agreed to provide the Company with four dedicated rooms for its gene therapy AADC program. The Company concluded that the agreement contains an embedded lease as the Company controls the use of the four dedicated rooms and the equipment therein. As the present value of the facilities exceeds the assessed fair value, the Company determined that it is a finance lease. Given that the embedded finance lease is designed for the production of PTC's AADC program and would not have an alternate use outside the PTC gene therapy platform without incurring significant costs, the Company determined that the lease should be treated as research and development expense under ASC 730 and accordingly, expensed the present value of all guaranteed future cash payments of $41.4 million during the twelve month period ending December 31, 2020. Additionally, during the three month period ending March 31, 2021, the Company recorded finance lease costs of $0.4 million related to interest on the lease liability.

The Company also leases certain vehicles, lab equipment, and office equipment under operating leases. The Company's leases have remaining operating lease terms ranging from 0.3 years to 14.3 years and certain of the leases include renewal options to extend the lease for up to 10 years. Rent expense was $5.4 million and $2.4 million for the three month periods ended March 31, 2021 and 2020, respectively.

The components of operating lease expense were as follows:

	Three Months Ended March 31, 2021	Three Months Ended March 31, 2020
Operating Lease Cost		
Fixed lease cost	$ 4,104	$ 2,118
Variable lease cost	1,093	228
Short-term lease cost	164	77
Total operating lease cost	$ 5,361	$ 2,423

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, 2021	December 31, 2020
Operating lease ROU asset	$ 82,382	$ 84,410
Operating lease liabilities- current	$ 7,333	$ 7,465
Operating lease liabilities- noncurrent	77,863	79,499
Total operating lease liability	$ 85,196	$ 86,964

	March 31, 2021	December 31, 2020
Finance lease liabilities- current	$ 1,694	$ 1,276
Finance lease liabilities- noncurrent	20,053	23,053
Total finance lease liability	$ 21,747	$ 24,329

The Company did not enter into any new leases during the three month period ended March 31, 2021. The decrease in the operating lease ROU asset primarily resulted from amortization of existing leases, along with the termination of several car and furniture leases during the period ended March 31, 2021.

Supplemental lease term and discount rate information related to leases was as follows as of March 31, 2021 and December 31, 2020:

	March 31, 2021	December 31, 2020
Weighted-average remaining lease terms - operating leases (years)	11.34	11.49
Weighted-average discount rate - operating leases	8.88 %	8.86 %
Weighted-average remaining lease terms - finance lease (years)	11.75	12.00
Weighted-average discount rate - finance lease	7.80 %	7.80 %

Supplemental cash flow information related to leases was as follows as of March 31, 2021 and 2020:

	Three Months Ended March 31, 2021	2020
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows from operating leases	$ 3,406	$ 1,971
Financing cash flows from finance lease	2,224	—
Operating cash flows from finance leases	776	—
Right-of-use assets obtained in exchange for lease obligations:		
Operating leases	$ 13	$ 22,642
Finance lease	—	—

Future minimum lease payments under non-cancelable leases as of March 31, 2021 were as follows:

	Operating Leases	Finance Lease
2021 (excludes the three months ended March 31, 2021)	$ 10,242	$ —
2022	13,240	3,000
2023	12,843	3,000
2024	12,150	3,000
2025 and thereafter	91,994	24,000
Total lease payments	140,469	33,000
Less: Imputed Interest expense	55,273	11,253
Total	$ 85,196	$ 21,747

In conjunction with the Asset Purchase Agreement by and between the Company and BioElectron Technology Corporation, dated October 1, 2019 (the “Asset Acquisition Agreement”), the Company acquired BioElectron’s lease in Mountainview, California. As substantially all of the fair value of the gross assets acquired was related to vatiquinone, 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, 2021 are $1.4 million, and $1.4 million for the remainder of 2021 and for 2022, respectively, and $0 thereafter.

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.

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. In February 2021, the Company purchased $0.1 million of shares of ClearPoint's common stock, at a purchase price of $23.50 per share, in connection with ClearPoint's underwritten public offering of common stock. The Company determined that the May 2019 and February 2021 ClearPoint equity investments (collectively, the "ClearPoint Equity Investments") represent financial instruments, and therefore, are recorded at fair value, which is readily determinable. The ClearPoint Equity Investments are components of deposits and other assets on the consolidated balance sheet. During the three month period ended March 31, 2021, the Company recorded an unrealized gain of $6.8 million on the ClearPoint Equity Investments. During the three month period ended March 31, 2020, the Company recorded an unrealized loss of $1.6 million. These unrealized gains and losses are components of other expense, net within the consolidated statement of operations. The fair value of the ClearPoint Equity Investments was $27.4 million and $20.5 million as of March 31, 2021 and December 31, 2020, respectively. The Company classifies the ClearPoint Equity Investments as Level 1 assets 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, 2021, the Company recorded an unrealized gain of $7.9 million. During the three month period ended March 31, 2020, the Company recorded an unrealized loss of $2.8 million. These unrealized gains and losses are components of other expense, net within the consolidated statement of operations. The fair value of the convertible debt security was $37.1 million and $29.3 million as of March 31, 2021 and December 31, 2020, respectively. 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 ClearPoint Equity Investments and the convertible debt security, no other items included in deposits and other assets on the consolidated balance sheets are fair valued.

In February 2021, the Company invested $200.0 million in two mutual funds which are classified as marketable securities on the Company's consolidated balance sheets. These equity investments are reported at fair value, as it is readily available, and as such are classified as Level 1 assets. Unrealized holding gains and losses for these equity investments are included as components of other expense, net within the consolidated statement of operations. For the three month period ended March 31, 2021, the Company had $0.3 million unrealized net losses relating to the equity investments still held at the reporting date.

Fair value of marketable securities that are classified as available for sale debt 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 available for sale debt securities, 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, 2021 and December 31, 2020:

	March 31, 2021			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities - available for sale	$ 642,077	$ —	$ 642,077	$ —
Marketable securities - equity investments	$ 199,697	$ 199,697	$ —	$ —
ClearPoint Equity Investments	$ 27,367	$ 27,367	$ —	$ —
ClearPoint convertible debt security	$ 37,109	$ —	$ 37,109	$ —
Contingent consideration payable- development and regulatory milestones	$ 139,300	$ —	$ —	$ 139,300
Contingent consideration payable- net sales milestones and royalties	$ 101,200	$ —	$ —	$ 101,200

	December 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 - available for sale	$ 894,838	$ —	$ 894,838	$ —
ClearPoint Equity Investments	$ 20,503	$ 20,503	$ —	$ —
ClearPoint convertible debt security	$ 29,252	$ —	$ 29,252	$ —
Contingent consideration payable- development and regulatory milestones	$ 139,200	$ —	$ —	$ 139,200
Contingent consideration payable- net sales milestones and royalties	$ 101,200	$ —	$ —	$ 101,200

No transfers of assets between Level 1, Level 2, or Level 3 of the fair value measurement hierarchy occurred during the periods ended March 31, 2021 and December 31, 2020.

The following is a summary of marketable securities accounted for as available for sale debt securities at March 31, 2021 and December 31, 2020:

	March 31, 2021			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 252,778	$ 25	$ (1)	$ 252,802
Corporate debt securities	330,781	591	(139)	331,233
Asset-backed securities	19,216	104	—	19,320
Government obligations	38,696	30	(4)	38,722
Total	$ 641,471	$ 750	$ (144)	$ 642,077

	December 31, 2020			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 276,855	$ 19	$ (37)	$ 276,837
Corporate debt securities	474,030	1,658	(29)	475,659
Asset-backed securities	28,681	210	(3)	28,888
Government obligations	113,372	88	(6)	113,454

Total	$ 892,938	$ 1,975	$ (75)	$ 894,838

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, 2021, 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 periods ended March 31, 2021 and 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.

For the three month period ended March 31, 2021, the Company had $0.7 million realized gains from the sale of available for sale debt securities. No realized gains or losses from the sale of available for sale debt securities were recorded for the three month period ended March 31, 2020. Realized gains and losses are reported as a component of interest expense, net in the consolidated statement of operations.

The unrealized losses and fair values of available for sale debt securities that have been in an unrealized loss position for a period of less than and greater than 12 months as of March 31, 2021 are as follows:

	March 31, 2021					
	Securities in an unrealized loss position less than 12 months		Securities in an unrealized loss position greater than 12 months		Total	
	Unrealized losses	Fair Value	Unrealized losses	Fair Value	Unrealized losses	Fair Value
Commercial paper	$ (1)	69,096	—	—	(1)	69,096
Corporate debt securities	(139)	177,817	—	—	(139)	177,817
Asset-backed securities	—	—	—	—	—	—
Government obligations	(4)	19,137	—	—	(4)	19,137
Total	$ (144)	$ 266,050	$ —	$ —	$ (144)	$ 266,050

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

	December 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	$ (37)	129,630	—	—	(37)	129,630
Corporate debt securities	(29)	102,426	—	—	(29)	102,426
Asset-backed securities	(3)	1,830	—	—	(3)	1,830
Government obligations	(6)	27,084	—	—	(6)	27,084
Total	$ (75)	$ 260,970	$ —	$ —	$ (75)	$ 260,970

Available for sale debt securities at March 31, 2021 and December 31, 2020 mature as follows:

	March 31, 2021	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 252,802	$ —
Corporate debt securities	164,640	166,593
Asset-backed securities	5,321	13,999
Government obligations	28,160	10,562
Total	$ 450,923	$ 191,154

	December 31, 2020	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 276,837	$ —
Corporate debt securities	240,139	235,520
Asset-backed securities	6,363	22,525
Government obligations	65,524	47,930
Total	$ 588,863	$ 305,975

The Company classifies all of its marketable securities as current as they are all either available for sale debt securities or equity investments 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 9. 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, 2021 and December 31, 2020 was $173.4 million and $193.2 million, respectively. The estimated fair value of the 2026 Convertible Notes at March 31, 2021 and December 31, 2020 was $333.8 million and $394.9 million, respectively.

Level 3 valuation

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, 2021, the weighted average discount rate for the development and regulatory milestones was 3.2% and the weighted average probability of success was 42%. 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, 2021, the weighted average discount rate for the net sales milestones and royalties was 12.5% and the weighted average probability of success for the net sales milestones was 48%.

The table presented below is a summary of changes in the fair value of the Company's Level 3 valuations for the contingent consideration payable for the periods ended March 31, 2021 and March 31, 2020:

	Level 3 liabilities	
	Contingent consideration payable-development and regulatory milestones	Contingent consideration payable-net sales milestones and royalties
Beginning balance as of December 31, 2020	$ 139,200	$ 101,200
Additions	—	—
Change in fair value	100	—
Payments	—	—
Ending balance as of March 31, 2021	$ 139,300	$ 101,200

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

The following significant unobservable inputs were used in the valuation of the contingent consideration payable for the periods ended March 31, 2021 and December 31, 2020:

	March 31, 2021			
	Fair Value	Valuation Technique	Unobservable Input	Range
Contingent consideration payable- development and regulatory milestones	$139,300	Probability-adjusted discounted cash flow	Potential development and regulatory milestones Probabilities of success Discount rates Projected years of payments	$0 - $381 million 25% - 94% 1.5% - 4.9% 2021 - 2028
Contingent considerable payable- net sales milestones and royalties	$101,200	Option-pricing model with Monte Carlo simulation	Potential net sales milestones Probabilities of success Potential percentage of net sales for royalties Discount rate Projected years of payments	$0 - $150 million 25% - 94% 2% - 6% 12.5% 2022 - 2040

	December 31, 2020			
	Fair Value	Valuation Technique	Unobservable Input	Range
Contingent consideration payable- development and regulatory milestones	$139,200	Probability-adjusted discounted cash flow	Potential development and regulatory milestones Probabilities of success Discount rates Projected years of payments	$0 - $381 million 25% - 94% 2.2% - 4.5% 2021 - 2028
Contingent considerable payable- net sales milestones and royalties	$101,200	Option-pricing model with Monte Carlo simulation	Potential net sales milestones Probabilities of success Potential percentage of net sales for royalties Discount rate Projected years of payments	$0 - $150 million 25% - 94% 2% - 6% 11.5% 2022 - 2040

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. Accounts payable and accrued expenses

Accounts payable and accrued expenses at March 31, 2021 and December 31, 2020 consist of the following:

	March 31, 2021	December 31, 2020
Employee compensation, benefits, and related accruals	$ 24,859	$ 53,291
Income tax payable	3,909	4,315
Consulting and contracted research	23,927	18,250
Professional fees	4,685	3,614
Sales allowance	55,638	54,327

Sales rebates	49,665	63,774
Royalties	16,181	16,575
Accounts payable	19,114	18,665
Other	7,658	9,357
Total	$ 205,636	$ 242,168

6. 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. During the three month period ending March 31, 2020, the Company received net proceeds of $13.5 million after deducting sales agents fees and commissions and other offering expenses payable by the Company. No shares were sold during the three month period ending March 31, 2021. The remaining shares of the Company's common stock available to be issued and sold, under the At the Market Offering, have an aggregate offering price of up to $93.0 million as of March 31, 2021.

7. Net loss per share

Basic and diluted net loss per share is computed by dividing net loss by the weighted-average number of common shares outstanding. Potentially dilutive securities were excluded from the diluted calculation because their effect would be anti-dilutive.

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

	Three Months Ended March 31,	
	2021	2020
Numerator		
Net loss	$ (128,642)	$ (112,687)
Denominator		
Denominator for basic and diluted net loss per share	70,188,602	62,389,158
Net loss per share:		
Basic and diluted	$ (1.83)*	$ (1.81)*

* In the three months ended March 31, 2021 and 2020, 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,	
	2021	2020
Stock Options	10,989,202	12,670,068
Unvested restricted stock awards and units	1,494,638	927,151
Total	12,483,840	13,597,219

8. Stock award 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, 2021, awards for 826,484 shares of common stock are available for issuance under the 2013 Long Term Incentive Plan.

There are no additional shares of common stock available for issuance under the Company's 1998 Employee, Director and Consultant Stock Option Plan, 2009 Equity and Long Term Incentive Plan or 2013 Stock 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. In December 2020, the Company's Board of Directors approved an additional 1,000,000 shares of common stock that may be issued under the 2020 Inducement Stock Incentive Plan. As of March 31, 2021, awards for 1,205,375 shares of common stock are available for issuance under the 2020 Inducement Stock Incentive Plan.

From January 1, 2021 through March 31, 2021, the Company issued a total of 1,954,690 stock options to various employees. Of those, 13,465 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, 2020	9,663,677	$ 38.72		
Granted	1,954,690	66.49		
Exercised	(415,783)	28.32		
Forfeited/Cancelled	(213,382)	48.58		
Outstanding at March 31, 2021	10,989,202	$ 43.86	7.59 years	$ 92,033
Vested or Expected to vest at March 31, 2021	5,591,504	$ 48.46	8.69 years	$ 31,047
Exercisable at March 31, 2021	4,750,569	$ 37.14	6.07 years	$ 58,970

The fair value of grants made in the three months ended March 31, 2021 was contemporaneously estimated on the date of grant using the following assumptions:

	Three months ended March 31, 2021
Risk-free interest rate	0.51%
Expected volatility	88.61%
Expected term	5.5 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, 2021 was $46.90 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. From January 1, 2021 through March 31, 2021, the Company issued a total of 850,365 restricted stock units to various employees. Of those, 4,665 were inducement grants for restricted stock units, all of which were made pursuant to the 2020 Inducement Stock Incentive Plan.

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
Unvested at December 31, 2020	982,058	$ 41.78
Granted	850,365	66.49
Vested	(298,982)	34.13
Forfeited	(38,803)	56.13
Unvested at March 31, 2021	1,494,638	$ 57.00

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, 2021, the Company recorded $0.5 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,	
	2021	2020
Research and development	$ 13,725	$ 8,179
Selling, general and administrative	11,982	7,041
Total	$ 25,707	$ 15,220

As of March 31, 2021, there was approximately $276.7 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 3.02 years.

9. Debt

Liability for sale of future royalties

In July 2020, the Company entered into the Royalty Purchase Agreement. As RPI's interest is explicitly limited, the $650.0 million cash consideration was classified as debt and is recorded as "liability for sale of future royalties-current" and "liability for sale of future royalties-noncurrent" on the Company's consolidated balance sheet based on the timing of the expected payments to be made to RPI. The fair value for the liability for sale of future royalties at the time of the transaction was based on the Company's estimates of future royalties expected to be paid to RPI over the life of the arrangement, which was determined using forecasts from market data sources, which are considered Level 3 inputs. The liability will be amortized using the effective interest method over the life of the arrangement, in accordance with ASC 470 and ASC 835. The initial annual effective interest rate was determined to be 11.0%. The Company will utilize the prospective method to account for subsequent changes in the estimated future payments to be made to RPI and will update the effective interest rate on a quarterly basis. Issuance costs related to the transaction were determined to be immaterial.

The following table shows the activity within the "liability for sale of future royalties- current" and "liability for sale of future royalties- noncurrent" accounts for the three month period ended March 31, 2021:

	Three Months Ended March 31,	
Liability for sale of future royalties- (current and noncurrent)	**2021**	
Beginning balance as of December 31, 2020	$	679,762
Less: Non-cash royalty revenue payable to RPI		(2,858)
Plus: Non-cash interest expense recognized		18,080
Ending balance	$	694,984
Effective interest rate as of March 31, 2021		10.6 %

Non-cash interest expense is recorded in the statement of operations within "Interest expense, net".

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, which was exercised in full by the initial purchasers. 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.

Prior to the adoption of ASU 2020-06, the Company accounted for the 2026 Convertible Notes as a liability and equity component where the carrying value of the liability component was 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 did 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, was amortized to interest expense over the seven-year term of the 2026 Convertible Notes. The equity component was not re-measured as long as it continued 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 were amortized to interest expense over the seven-year term of the 2026 Convertible Notes, and transaction costs attributable to the equity component were 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.

Effective January 1, 2021 the Company adopted ASU 2020-06. After adoption, the Company now accounts for the 2026 Convertible Notes as a single liability measured at amortized cost. As the equity component is no longer required to be split into a separate component, the Company recorded an adjustment for the initial $123.0 million that was allocated to additional paid in capital and $16.1 million of life to date interest expense recorded as amortization of debt discount. Additionally, the net deferred tax liability recorded for the 2026 Convertible Notes was reversed. The principal amount of the liability over its carrying amount is amortized to interest expense over the seven-year term of the 2026 Convertible Notes. Since the 2026 Convertible Notes are classified as a single liability, there is no debt discount required to be amortized.

The 2026 Convertible Notes consist of the following:

Liability component	**March 31, 2021**	**December 31, 2020**
Principal	$ 287,500	$ 287,500
Less: Debt issuance costs	(6,457)	(4,058)
Less: Debt discount, net(1)	—	(106,065)
Net carrying amount	$ 281,043	$ 177,377

(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, 2021, the remaining contractual life of the 2026 Convertible Notes is approximately 5.5 years.

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

	Three Months Ended March 31,	
	2021	**2020**
Contractual interest expense	$ 1,069	$ 1,076
Amortization of debt issuance costs	277	124
Amortization of debt discount	—	3,234
Total	$ 1,346	$ 4,434
Effective interest rate of the liability component	1.9 %	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.

Prior to the adoption of ASU 2020-06, the Company accounted for the 2022 Convertible Notes as a liability and equity component where the carrying value of the liability component was 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 did 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, was amortized to interest expense over the seven-year term of the 2022 Convertible Notes. The equity component was not re-measured as long as it continued 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 were 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.

Effective January 1, 2021 the Company adopted ASU 2020-06. After adoption, the Company now accounts for the 2022 Convertible Notes as a single liability measured at amortized cost. As the equity component is no longer required to be split into a separate component, the Company recorded an adjustment for the initial $57.5 million that was allocated to additional paid in capital and $38.7 million of life to date interest expense recorded as amortization of debt discount. Additionally, the net deferred tax liability recorded for the 2022 Convertible Notes was reversed. The principal amount of the liability over its carrying amount is amortized to interest expense over the seven-year term of the 2022 Convertible Notes. Since the 2022 Convertible Notes are classified as a single liability, there is no debt discount required to be amortized.

The 2022 Convertible Notes consist of the following:

Liability component	**March 31, 2021**	**December 31, 2020**
Principal	$ 150,000	$ 150,000
Less: Debt issuance costs	(1,005)	(865)
Less: Debt discount, net (1)	—	(17,372)
Net carrying amount	$ 148,995	$ 131,763

(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, 2021, the remaining contractual life of the 2022 Convertible Notes is approximately 1.4 years.

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

	Three Months Ended March 31,	
	2021	2020
Contractual interest expense	$ 1,110	$ 1,119
Amortization of debt issuance costs	175	111
Amortization of debt discount	—	2,225
Total	$ 1,285	$ 3,455
Effective interest rate of the liability component	3.5 %	11.0 %

10. 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, with respect to collaboration products the Company outlicenses, including Evrysdi, 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 an aggregate of $52.5 million.

Pursuant to the asset purchase agreement ("Asset Purchase Agreement") between the Company and Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC) ("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 Agilis Merger Agreement, Agilis equityholders were previously 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 was required to pay $40.0 million of the development milestone payments upon the passing of the second anniversary of the closing of the Agilis Merger, regardless of whether the applicable milestones have been achieved.

Pursuant to the terms of a Rights Exchange Agreement, by and among the Company, the Rightholders set forth therein, and, for the limited purposes set forth therein, Shareholder Representatives Services LLC, dated as of April 29, 2020 (the "Rights Exchange Agreement"), the former equityholders of Agilis (the "Participating Rightholders") canceled and forfeited their rights under the Agilis 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 that would have been due upon the passing of the second anniversary of the closing of the Agilis Merger, regardless of whether the milestones are achieved.

The Rights Exchange Agreement has no effect on the Agilis 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 Agilis Merger Agreement remain in effect pursuant to their terms, including the Company's obligation to pay up to an aggregate maximum amount of $20.0 million upon the achievement of certain development milestones (representing the remaining portion of potential development milestone payments for which rights were not canceled and forfeited pursuant to the Rights Exchange Agreement while excluding the remaining $2.4 million milestone payment that was due and paid upon the passing of the second anniversary of the closing of the Agilis Merger), 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 Agilis Merger Agreement.

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.

Subject to the terms and conditions of the Censa Merger Agreement, Censa securityholders may become entitled to receive contingent payments from the Company based on (i) the achievement of certain development and regulatory milestones up to an aggregate maximum amount of $217.5 million for PTC923's two most advanced programs and receipt of a priority review voucher from the FDA as set forth in the Censa Merger Agreement, (ii) $109.0 million in development and regulatory milestones for each additional indication of PTC923, (iii) the achievement of certain net sales milestones up to an aggregate maximum amount of $160.0 million, (iv) a percentage of annual net sales during specified terms, ranging from single to low double digits of the applicable net sales threshold amount, and (v) any sublicense fees paid to the Company in consideration of any sublicense of Censa's intellectual property to commercialize PTC923, on a country-by-country basis, which contingent payment shall equal to a mid-double digit percentage of any such sublicense fees. Pursuant to the Censa Merger Agreement, the Company has the option to pay the initial $30.0 million development milestone, for the completion of enrollment of a Phase 3 clinical trial for PTC923 for PKU, if achieved, in cash or shares of the Company's common stock.

The Company also has the Akcea Collaboration and License Agreement for the commercialization of Tegsedi and Waylivra, and products containing those compounds in countries in Latin America and the Caribbean. Pursuant to the Akcea Collaboration and License 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.

11. 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, 2021 and 2020, net product sales in the United States were $43.5 million and $27.5 million, respectively, consisting solely of Emflaza, and net product sales not in the United States were $47.8 million and $40.7 million, respectively, consisting of Translarna, Tegsedi, and Waylivra. For the three months ended March 31, 2021 and 2020, the Company had a total of two and three distributors, respectively, that each accounted for over 10% of the Company's net product sales.

The Company's contract liabilities balances as of March 31, 2021 and as of December 31, 2020 were $1.9 million and $4.2 million, respectively. The Company did not have any contract assets as of March 31, 2021 and as of December 31, 2020. During the three month periods ended March 31, 2021 and 2020, the Company recognized $2.1 million and $2.0 million of revenue, respectively, related to the amounts included in 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 month periods ending March 31, 2021 and 2020.

Remaining performance obligations

Remaining performance obligations represent the transaction price for goods the Company has yet to provide. As of March 31, 2021 and December 31, 2020, the aggregate amount of the transaction price allocated to the remaining performance obligations relating to Translarna net product revenue was $1.9 million and $4.2 million, respectively. The Company expects to recognize revenue within the next one year, as the specific timing for satisfying the performance obligations is contingent upon a number of factors, including customers' needs and schedules.

Collaboration and Royalty revenue

In November 2011, the Company and the SMA Foundation entered into the SMA License Agreement with Roche. Under the terms of the SMA License Agreement, Roche acquired an exclusive worldwide license to the Company's SMA 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.

The SMA program currently has one approved product, Evrysdi™ (risdiplam), which was approved in August 2020 by the FDA for the treatment of SMA in adults and children two months and older. As of March 31, 2021, the remaining potential research and development event milestones that can be received is $10.0 million, and the remaining potential sales milestones that can be received is $325.0 million.

For the three months ended March 31, 2021 and 2020, the Company recognized $20.0 million and $0.1 million of collaboration revenue, respectively, related to the licensing and collaboration agreement with Roche. The first commercial sale of Evrysdi in the European Union was made in March 2021. This event triggered a $20.0 million milestone payment

to the Company from Roche, which was recognized in the three months ended March 31, 2021. No milestones were triggered in the three months ended March 31, 2020.

In addition to research and development and sales milestones, the Company is eligible to receive up to double-digit royalties on worldwide annual net sales of a commercial product under the SMA License Agreement. For the three months ended March 31, 2021, the Company has recognized $6.7 million of royalty revenue related to Evrysdi. No royalty revenue related to Evrysdi was recognized in the three months ended March 31, 2020.

12. 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 records the milestone payment when it becomes payable to Marathon and increases the cost basis for the Emflaza rights intangible asset. For the three month periods ended March 31, 2021 and 2020, milestone payments of $8.9 million and $2.8 million were recorded, respectively. These payments are being amortized over the remaining useful life of the Emflaza rights asset on a straight line basis. As of March 31, 2021, a milestone payable to Marathon of $8.9 million was recorded on the balance sheet within accounts payable and accrued expenses.

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 months ended March 31, 2021 and 2020, the Company recognized amortization expense of $11.3 million and $7.9 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, 2021
2021	$ 33,826
2022	45,100
2023	45,100
2024	7,489
2025 and thereafter	4,559
Total	$ 136,074

The weighted average remaining amortization period of the definite-lived intangibles as of March 31, 2021 is 3.2 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 Agilis 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 Agilis Merger.

Goodwill

As a result of the Agilis Merger on August 23, 2018, the Company recorded $82.3 million of goodwill. There were no changes to the recorded value of goodwill for the three month period ended March 31, 2021.

13. Subsequent events

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

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

The following discussion of our financial condition and results of operations should be read in conjunction with our financial statements and the notes to those financial statements appearing elsewhere in this Quarterly Report on Form 10-Q and the audited consolidated financial statements and notes thereto and management's discussion and analysis of financial condition and results of operations for the year ended December 31, 2020 included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 25, 2021, or our 2020 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 2020 Annual Report, 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 globally 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 leverage our strong scientific expertise and global commercial infrastructure to maximize value for our patients and other stakeholders. We have a portfolio pipeline that includes several commercial products and 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 and is continuing to impact the timing of certain of our clinical trials and regulatory submissions as well as other aspects of our business operations. In addition to our previous disclosures regarding the impact of the COVID-19 pandemic, such as those set forth in our Annual Report on Form 10-K for the year ended December 31, 2020, the following expectations have been revised as a result of the impact or expected impact of the COVID-19 pandemic:

- Due to delays related to responsive measures to the COVID-19 pandemic taken in Europe, including travel bans and quarantines, the Committee for Medicinal Products for Human Use, or CHMP requires additional time to complete its pre-approval inspections and has imposed a clock stop extension with respect to our marketing authorization application, or MAA, for PTC-AADC for the treatment of Aromatic L-Amino Acid Decarboxylase, or AADC, deficiency in the European Economic Area, or EEA. We currently expect an opinion from the CHMP in the third quarter of 2021.

- As previously disclosed, in response to discussions with the United States Food and Drug Administration, or FDA, we intend to provide additional information concerning the use of the commercial cannula for PTC-AADC in young patients. However, due to hospitals generally canceling elective surgeries in response to the COVID-19 pandemic and other ongoing administrative delays resulting from the COVID-19 pandemic, we have been delayed in our ability to gather such information. We anticipate a BLA submission for PTC-AADC for the treatment of AADC deficiency in the United States to be delayed by at least one quarter.

- As a result of the COVID-19 pandemic, the Brazilian Ministry of Health is continuing to experience significant delays processing centralized group purchase orders. Almost all of our product revenue for Translarna in Brazil is attributable to such purchase orders. These centralized group purchase order delays have caused, and may continue to cause, fluctuations in our ability to generate revenue in Brazil.

- As of the date of this Report on Form 10-Q, except as otherwise disclosed with respect to Translarna product revenue in Brazil, 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.

- As previously disclosed, in response to the global uncertainty caused by the COVID-19 pandemic, we are continuing to prioritize our expenses where we deem appropriate and strategically positioning our capital allocation.

The COVID-19 pandemic and responsive measures thereto may result in further negative impacts, including additional delays in our clinical and regulatory activities and further fluctuations in our revenue. 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 has disrupted, and may continue to disrupt, our operations and may significantly impact our operating results. In addition, the COVID-19 pandemic has caused substantial disruption in the financial markets and economies, which could result in adverse effects on our business and operations.*" in our Annual Report on Form 10-K for the year ended December 31, 2020.

European Approval of Evrysdi™ (risdiplam)

In March 2021, the European Commission approved Evrysdi for the treatment of 5q spinal muscular atrophy, or SMA, in patients two months and older with a clinical diagnosis of SMA Type 1, Type 2 or Type 3 or with one to four SMN2 copies in the European Economic Area, or the EEA. The first commercial sale of Evrysdi in the European Union was made in March 2021, triggering a $20.0 million milestone payment to us from F. Hoffman-La Roche Ltd. and Hoffman-La Roche Inc., or, together with F. Hoffman-La Roche Ltd, Roche, pursuant to the License and Collaboration Agreement, or the SMA License Agreement, dated as of November 23, 2011, by and among us, Roche and, for the limited purposes set forth therein, the Spinal Muscular Atrophy Foundation, or the SMA Foundation, under our SMA program.

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 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. In July 2020, the European Commission approved the removal of the statement "efficacy has not been demonstrated in non-ambulatory patients" from the indication statement for Translarna. During the quarter ended March 31, 2021, we recognized $46.5 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, 2021, we recognized $43.5 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 June 2020, the European Commission renewed our marketing authorization, making it effective, unless extended, through August 5, 2021. In February 2021, 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 challenge in any particular country and can also be affected by political, economic and regulatory developments in such country.

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

Translarna is an investigational new drug in the United States. During the first quarter of 2017, we filed a New Drug Application, or NDA, for Translarna for the treatment of nmDMD over protest with the 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 followed the FDA's recommendation and collected, using newer technologies via procedures and methods that we designed, such dystrophin data in a new study, Study 045, and announced the results of Study 045 in February 2021. Although Study 045 did not meet its pre-specified primary endpoint, we plan to discuss the Study 045 dystrophin results and the totality of existing clinical and real-world data with the FDA to determine if there is a potential path to approval based on these results and data. There is substantial risk that the FDA will determine that the results from our clinical trials and existing real-world data are not sufficient to support a marketing approval for Translarna for the treatment of nmDMD in the United States. In that case, as we expect to have data for Study 041 in the third quarter of 2022, and subject to a positive outcome in that study, we would plan to re-submit the NDA at that time.

Tegsedi™ (inotersen) and Waylivra™ (volanesorsen)

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, European Union, or EU, and Brazil for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis, or hATTR amyloidosis. While we are in the process of initiating our commercial launch for Tegsedi for the treatment of hATTR amyloidosis in Latin America, we continue to make Tegsedi available in certain countries within Latin America and the Caribbean through early access programs. Waylivra has received marketing authorization in the EU for the treatment of familial chylomicronemia syndrome, or FCS. We filed for marketing authorization for Waylivra for the treatment of FCS with ANVISA, the Brazilian health regulatory authority, in June 2020 and, subject to potential delays in the review process related to the COVID-19 pandemic, expect a regulatory decision on approval from ANVISA the third quarter of 2021.

Evrysdi

We also have an SMA collaboration with Roche and the SMA Foundation. The SMA program has one approved product, Evrysdi, which was approved by the FDA in August 2020 for the treatment of SMA in adults and children two months and older and by the European Commission in March 2021 for the treatment of 5q SMA in patients two months and older with a clinical diagnosis of SMA Type 1, Type 2 or Type 3 or with one to four SMN2 copies. Evrysdi also received marketing authorization for the treatment of SMA in Brazil in October 2020. Additionally, in October 2020, Chugai Pharmaceutical Co., Ltd., or Chugai, a subsidiary of Roche, filed an NDA in Japan for Evrysdi for the treatment of SMA and a regulatory decision on approval is expected in 2021.

Diversified Development Pipeline

Splicing Platform

In addition to our SMA program, our splicing platform also includes PTC518, which is being developed for the treatment of Huntington's disease, or HD. We announced preliminary results from our ongoing Phase 1 study of PTC518 in healthy volunteers in April 2021 demonstrating dose-dependent lowering of huntingtin messenger ribonucleic acid levels and that PTC518 was well tolerated with no safety-related findings.

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 Aromatic L-Amino Acid Decarboxylase, or AADC, deficiency, a rare CNS disorder arising from reductions in the enzyme AADC that result from mutations in the dopa decarboxylase gene. In January 2020, we submitted an MAA for PTC-AADC for the treatment of AADC deficiency in the EEA to the EMA and we expect an opinion from the CHMP in the third quarter of 2021. We are also preparing a biologics license application, or BLA, for PTC-AADC for the treatment of AADC deficiency in the United States. We anticipate the BLA submission to the FDA to be delayed by at least one quarter.

Bio-e Platform

Our Bio-e platform consists of small molecule compounds that target oxidoreductase enzymes that regulate oxidative stress and inflammatory pathways central to the pathology of a number of CNS diseases. The two most advanced molecules in our Bio-e platform are vatiquinone and PTC857. We initiated a registration-directed Phase 2/3 placebo-controlled trial of vatiquinone in children with mitochondrial disease and associated refractory epilepsy in the third quarter of 2020 and anticipate data from this trial to be available in the third quarter of 2022. We also initiated a registration-directed Phase 3 trial of vatiquinone in children and young adults with Friedreich ataxia in the fourth quarter of 2020 and anticipate data from this trial to be available in 2023. In the second quarter of 2020, we initiated a Phase 1 trial in healthy volunteers to evaluate the safety and pharmacology of PTC857. This Phase 1 trial was recently completed and we expect to data to be available in the second quarter of 2021.

Metabolic Platform

On May 29, 2020, we acquired Censa Pharmaceuticals, Inc., or Censa, a biopharmaceutical company focused on the development of PTC923, an oral formulation of synthetic sepiapterin, a precursor to intracellular tetrahydrobiopterin, which is a critical enzymatic cofactor involved in metabolism and synthesis of numerous metabolic products, for orphan diseases. We expect to initiate a registration-directed Phase 3 trial for PTC923 for phenylketonuria, or PKU, in mid-2021.

PTC299 for COVID-19

In June 2020, we initiated a Phase 2/3 clinical trial evaluating the efficacy and safety of PTC299, a dihydroorotate dehydrogenase inhibitor that we have also been developing in oncological indications, in patients hospitalized with COVID-19. We expect data to be available from this trial in the second half of 2021.

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.

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. We have also recognized revenue associated with milestone and royalty payments from Roche pursuant to the SMA License Agreement.

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, proceeds from a Royalty Purchase Agreement dated as of July 17, 2020, by and among us, RPI 2019 Intermediate Finance Trust, or RPI, and, solely for the limited purposes set forth therein, Royalty Pharma PLC, or the Royalty Purchase Agreement, private placements of our preferred stock, collaborations, bank and institutional lender debt and convertible debt financings, 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. We have also relied on revenue associated with milestone and royalty payments from Roche pursuant to the SMA License Agreement.

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.

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 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 twelve month period ending December 31, 2020, we issued and sold an aggregate of 542,470 shares of common stock pursuant to the Sales Agreement at a weighted average public offering price of $53.37 per share. We received net proceeds of $28.1 million after deducting sales agent fees and other offering expenses payable by us. During the three month period ending March 31, 2021, we did not issue or sell any shares of common stock pursuant to the Sales Agreement.

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, 2021, we had an accumulated deficit of $1,702.7 million. We had a net loss of $128.6 million and $112.7 million for the three month periods ended March 30, 2021 and 2020, 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 leased 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 splicing, gene therapy, Bio-e, metabolic and oncology programs, our studies of PTC299 for

COVID-19 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 expect an opinion from the CHMP in the third quarter of 2021. We are also preparing a BLA for PTC-AADC for the treatment of AADC deficiency in the United States. We filed for marketing authorization for Waylivra with ANVISA in June 2020 and, subject to potential delays in the review process related to the COVID-19 pandemic, expect a regulatory decision on approval from ANVISA in the third quarter of 2021. 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 require total funding of $4.3 million annually. 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, the determination for which we expect to potentially occur, subject to potential delays in the review process related to the COVID-19 pandemic, in the third quarter of 2021. 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, 2021 and 2020, net product sales outside of the United States were $47.8 million and $40.7 million, respectively, and net product sales in the United States were $43.5 million and $27.5 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 the SMA License Agreement 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. We are 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. The first commercial sale of Evrysdi in the European Union was made in March 2021, triggering a $20.0 million milestone payment to us from Roche. As of March 31, 2021, we had recognized a total of $125.0 million in milestone payments and $11.4 million royalties on net sales pursuant to the SMA License Agreement. As of March 31, 2021, the remaining potential research and development event milestones that can be received is $10.0 million. The remaining potential sales milestones as of March 31, 2021 are $325.0 million upon achievement of certain sales events.

Pursuant to the Royalty Purchase Agreement, we sold to RPI 42.933%, or the Assigned Royalty Payment, of our right to receive sales-based royalty payments, or the Royalty, on worldwide net sales of Evrysdi and any other product developed pursuant to the SMA License Agreement in consideration for $650.0 million. We have retained a 57.067% interest in the Royalty and all economic rights to receive the remaining potential regulatory and sales milestone payments under the SMA License Agreement. The Royalty Purchase Agreement will terminate 60 days following the earlier of the date on which Roche is no longer obligated to make any payments of the Royalty pursuant to the SMA License Agreement and the date on which RPI has received $1.3 billion in respect of the Assigned Royalty Payment.

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 splicing, gene therapy, Bio-e, metabolic and oncology programs and our studies of PTC299 for COVID-19 and performance of our post-marketing requirements imposed by regulatory agencies with respect to our products. 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, 2021 and 2020.

	Three Months Ended March 31,	
	2021	2020
	(in thousands)	
Global DMD Franchise	$ 20,569	$ 36,484
PTC923	11,579	—
Gene Therapy	54,633	30,899
Bio-e	13,601	3,800
Oncology	3,151	4,755
Splicing	9,624	4,279
PTC299 for COVID-19	10,874	—
Discovery	10,482	9,890
Total research and development	$ 134,513	$ 90,107

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;
- 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, has caused 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 expense from the liability for the sale of future royalties related to the Royalty Purchase Agreement, the Convertible Notes outstanding, and from our credit and security agreement with MidCap Financial Trust that was terminated in July 2020 offset by interest income earned on investments.

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.

Effective January 1, 2021, we early adopted ASU 2020-06, "Debt—Debt with Conversion and Other Options (Subtopic 470-20) and Derivatives and Hedging—Contracts in Entity's Own Equity (Subtopic 815-40): Accounting for Convertible Instruments and Contracts in an Entity's Own Equity" using the modified retrospective method of adoption. ASU 2020-06 simplifies the accounting for convertible instruments by removing certain separation models in Subtopic 470- 20, Debt—Debt with Conversion and Other Options, for convertible instruments. Under ASU 2020-06, the embedded conversion features no longer are separated from the host contract for convertible instruments with conversion features that are not required to be accounted for as derivatives under Topic 815, Derivatives and Hedging, or that do not result in substantial premiums accounted for as paid-in capital. Consequently, a convertible debt instrument will be accounted for as a single liability measured at its amortized cost as long as no other features require bifurcation and recognition as derivatives. By removing those separation models, the interest rate of convertible debt instruments typically will be closer to the coupon interest rate when applying the guidance in Topic 835, Interest. We now account for our Convertible Notes as single liabilities measured at amortized cost. As a result, the adoption of the guidance had a material impact on the consolidated financial statements and accompanying notes, resulting in adjustments of $175.2 million, $54.8 million, and $120.4 million to the opening balances of additional paid-in capital, retained earnings, and long term debt, respectively, as of January 1, 2021. Additionally, due to the adoption, we reversed the remaining balance of the deferred tax liability of $29.6 million, which was initially recorded in connection with the Convertible Notes. Additionally, we increased the existing valuation allowance by $29.6 million as part of the adoption adjustment. We concluded that the adoption of the ASU did not change our prior valuation allowance conclusions. We have updated our debt note (Note 9) with additional and modified disclosures as required by the standard upon adoption.

Other than the adoption of ASU 2020-06, during the three months ended March 31, 2021, there were no material changes to our critical accounting policies as reported in our 2020 Annual Report on Form 10-K.

Results of operations

Three months ended March 31, 2021 compared to three months ended March 31, 2020

The following table summarizes revenues and selected expense and other income data for the three months ended March 31, 2021 and 2020.

(in thousands)	Three Months Ended March 31, 2021	2020	Change 2021 vs. 2020
Net product revenue	$ 91,280	$ 68,196	$ 23,084
Collaboration and grant revenue	20,007	63	19,944
Royalty revenue	6,655	—	6,655
Cost of product sales, excluding amortization of acquired intangible asset	9,104	4,085	5,019
Amortization of acquired intangible asset	11,278	7,949	3,329
Research and development expense	134,513	90,107	44,406
Selling, general and administrative expense	61,095	58,209	2,886
Change in the fair value of deferred and contingent consideration	100	900	(800)
Interest expense, net	(19,159)	(5,642)	(13,517)
Other expense, net	(10,884)	(13,832)	2,948
Income tax expense	(451)	(222)	(229)

Net product revenues. Net product revenues were $91.3 million for the three months ended March 31, 2021, an increase of $23.1 million, or 34%, from $68.2 million for the three months ended March 31, 2020. The increase in net product revenue was primarily due to an increase in net product sales of Emflaza due to new patient prescriptions, high compliance, and fewer discontinuations. Additionally, there was an increase in net product sales in existing markets where Translarna is available as well as continued geographic expansion into new territories.

Collaboration and grant revenues. Collaboration and grant revenues were $20.0 million for the three months ended March 31, 2021, an increase of $20.0 million, or over 100%, from $0.1 million for the three months ended March 31, 2020. The increase is due to a $20.0 million milestone that was triggered from Roche in the three months ended March 31, 2021 relating to the first commercial sale of Evrysdi in the European Union, which was made in March 2021. No milestones were triggered in the three months ended March 31, 2020. Revenues in the three months ended March 31, 2020 were related to our ongoing collaboration agreements.

Royalty revenue. Royalty revenue was $6.7 million for the three months ended March 31, 2021, an increase of $6.7 million, or 100%, from $0.0 million for the three months ended March 31, 2020. The increase in royalty revenue was due to the FDA approval of Evrysdi in August 2020. In accordance with the SMA License Agreement, we are entitled to royalties on worldwide annual net sales of the product.

Cost of product sales, excluding amortization of acquired intangible asset. Cost of product sales, excluding amortization of acquired intangible asset, were $9.1 million for the three months ended March 31, 2021, an increase of $5.0 million, or over 100%, from $4.1 million for the three months ended March 31, 2020. Cost of product sales consist primarily of royalty payments associated with Emflaza and Translarna net product sales, excluding contingent payments to Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC), or Marathon, costs associated with Emflaza and Translarna product sold during the period, and royalty expense related to royalty revenues and collaboration milestone revenues. The increase in cost of product sales, excluding amortization of acquired intangible asset, is primarily due to the increase in net product revenue, royalty revenue, and collaboration milestone revenue.

Amortization of acquired intangible asset. Amortization of our intangible assets was $11.3 million for the three months ended March 31, 2021, an increase of $3.3 million, or 42%, from $7.9 million for the three months ended March 31, 2020.

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 $134.5 million for the three months ended March 31, 2021, an increase of $44.4 million, or 49%, from $90.1 million for the three months ended March 31, 2020. The increase reflects increased investment in research programs and advancement of the clinical pipeline.

Selling, general and administrative expense. Selling, general and administrative expense was $61.1 million for the three months ended March 31, 2021, an increase of $2.9 million, or 5%, from $58.2 million for the three months ended March 31, 2020. The increase was primarily due to rent and related expenses associated with entering into a long term lease for our facility located in Hopewell Township, New Jersey, or the Hopewell Facility, that commenced on July 1, 2020.

Change in the fair value of deferred and contingent consideration. The change in the fair value of deferred and contingent consideration was $0.1 million for the three months ended March 31, 2021, a decrease of $0.8 million, or 89%, from $0.9 million for the three months ended March 31, 2020. The change is related to the fair valuation of the potential future consideration to be paid to former equityholders 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 $19.2 million for the three months ended March 31, 2021, an increase of $13.5 million, or over 100%, from $5.6 million for the three months ended March 31, 2020. The increase in interest expense, net was primarily due to interest expense recorded from the liability for the sale of future royalties related to the Royalty Purchase Agreement, partially offset by a decrease in interest expense recorded from the 2022 and 2026 Convertible Notes as a result of the adoption of ASU 2020-06 and interest income from our investments.

Other expense, net. Other expense, net was $10.9 million for the three months ended March 31, 2021, a decrease of $2.9 million, or 21%, from other expense, net of $13.8 million for the three months ended March 31, 2020. The decrease in other expense, net resulted primarily from an unrealized foreign exchange loss from the remeasurement of our intercompany loan, partially offset by unrealized gains on our equity investments and convertible debt security in ClearPoint Neuro, Inc. of $6.8 million and $7.9 million, respectively.

Income tax expense. Income tax expense was $0.5 million for the three months ended March 31, 2021, an increase of $0.2 million, or over 100%, compared to income tax benefit of $0.2 million for the three months ended March 31, 2020. 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.

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, proceeds from the Royalty Purchase Agreement, the private placements of our preferred stock, collaborations, bank and institutional lender debt, convertible debt financings 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 fiscal year. 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.

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. See "Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations—Corporate Updates—Funding" for additional information.

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 full 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.

In July 2020, we entered into the Royalty Purchase Agreement. Pursuant to the Royalty Purchase Agreement, we sold to RPI the Assigned Royalty Payment in consideration for $650.0 million.

Cash flows

As of March 31, 2021, we had cash, cash equivalents and marketable securities of $988.4 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)	**2021**	**2020**
Cash (used in) provided by:		
Operating activities	(100,157)	(81,554)
Investing activities	30,147	(101,274)
Financing activities	9,531	18,490

Net cash used in operating activities was $100.2 million for the three months ended March 31, 2021 and $81.6 million for the three months ended March 31, 2020. The net cash used in operating activities primarily relates to supporting clinical development and commercial activities.

Net cash provided by investing activities was $30.1 million for the three months ended March 31, 2021. Net cash used in investing activities was $101.3 million for the three months ended March 31, 2020, respectively. Cash provided by investing activities for the three months ended March 31, 2021 was primarily due to net sales and redemption of marketable securities. Cash used in investing activities for the three months ended March 31, 2020 was primarily related to net purchases of marketable securities.

Net cash provided by financing activities was $9.5 million for the three months ended March 31, 2021. Cash provided by financing activities for the three months ended March 31, 2021 was primarily attributable to cash received from the exercise of options, partially offset by payments on our finance lease principal. Cash provided by financing activities was $18.5 million for the three months ended March 31, 2020. Cash provided by financing activities for the three months ended March 31, 2020 was primarily attributable to our at the market equity offering and the exercise of options, partially offset by the repayment of our senior secured term loan.

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 splicing, gene therapy, Bio-e, metabolic and oncology programs and our studies of PTC299 for COVID-19 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. We submitted an MAA to the EMA for the treatment of AADC deficiency with PTC-AADC in the EEA and we expect an opinion from the CHMP in the third quarter of 2021. We are preparing a BLA for PTC-AADC for the treatment of AADC deficiency in the United States. We filed for marketing authorization for Waylivra with ANVISA in June 2020 and, subject to potential delays in the review process related to the COVID-19 pandemic, expect a regulatory decision on approval from ANVISA in the third quarter of 2021. 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 Merger;
- seek to satisfy contractual and regulatory obligations in conjunction with the Collaboration and License Agreement, dated August 1, 2018, by and between the Company and Akcea Therapeutics, Inc., including an additional milestone payment of $4.0 million that Akcea is eligible to receive upon receipt of regulatory approval for Waylivra from ANVISA;
- 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 splicing, gene therapy, Bio-e, metabolic and oncology programs and our studies of PTC299 for COVID-19 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 our offerings of the Convertible Notes, public offerings of common stock, our "at the market offering" of our common stock, proceeds from the Royalty Purchase Agreement 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 re-submission by us and, ultimately, may support approval of Translarna for nmDMD in the United States;
- unexpected decreases in revenue or increase in expenses resulting from the COVID-19 pandemic;
- our ability to maintain orphan exclusivity in the United States for Emflaza;
- our ability to successfully complete all post-marketing requirements imposed by regulatory agencies with respect to our products;
- the progress and results of activities under our splicing, gene therapy, Bio-e, metabolic and oncology programs and our studies of PTC299 for COVID-19 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 splicing, gene therapy, Bio-e, metabolic and oncology programs and our studies of PTC299 for COVID-19 and Translarna in other territories;

- 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 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 splicing, gene therapy, Bio-e, metabolic and oncology programs;

- 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 acquisitions of Emflaza, Agilis, our Bio-E platform and Censa and our licensing of Tegsedi and Waylivra; and

- our ability to establish and maintain collaborations, including our collaborations with Roche and the SMA Foundation, and our ability to obtain research funding and achieve milestones under these agreements.

With respect to our outstanding 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. 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, debt or other 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, 2021, 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 2020 Annual Report on Form 10-K.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

During the period ended March 31, 2021, 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 2020 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, 2021. 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, 2021, 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, 2021 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, 2020, risk factors relating to our business, our industry, our structure and our common stock. Readers of this Quarterly Report on Form 10-Q are referred to such Item 1A for a more complete understanding of risks concerning us. There have been no material changes in our risk factors since those published in such Form 10-K for the year ended December 31, 2020.

Item 6. Exhibits.

Exhibit Number	Description of Exhibit
10.1†	Collaborative Research Agreement Amendment 9, dated as of March 21, 2021 by and between National Taiwan University and PTC Therapeutics GT, 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: May 4, 2021

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.

Collaborative Research Agreement Amendment No. 9

This Amendment No. 9 is made and entered into as of March 21, 2021 (the "Amendment 9 Effective Date") to that certain Collaborative Research Agreement dated September 30, 2015 (as amended, the "Agreement") by and between National Taiwan University at No. 1, Sec. 4, Roosevelt Road, Taipei, 10617 Taiwan (R.O.C) (hereinafter "NTU") and PTC Therapeutics GT, Inc. (formerly Agilis Biotherapeutics), a Delaware corporation duly organized under law and having an address at 6 Kimball Lane, Suite 320, Lynnfield, Massachusetts, 01940 USA (hereinafter "COMPANY"). Capitalized terms herein shall have the meaning ascribed to them in the Agreement. To the extent of any conflict with the prior amendments to the Agreement, this Amendment 9 supersedes the prior amendments.

WHEREAS, the COMPANY and NTU previously amended the agreement via Amendments 1 through 8. In the previous amendments, the COMPANY and NTU agreed to a continue the active studies, including active Phase IIb Protocol studies and related research and appointing a new principal investigator; and an additional research project and corresponding increases in the budget for [**].

NOW, THEREFORE, the COMPANY and NTU wish to further amend the Agreement as follows:

1. **Modifications**

   a. **Research Budget (Amendment No. 9).** For the quarter beginning April 1, 2021 through the end of 2021, the estimated budget shall be [**] U.S. Dollars (USD$ [**]) per quarter, which amount is inclusive of NTU's fees, based upon the fee schedule for the Services outlined in Attachment A. The actual amounts invoiced may vary depending upon [**]. Invoices shall reflect the fees due in U.S. Dollars and shall be sent to [**], with a copy to COMPANY's project manager. Each invoice will include the Purchase Order numbers specific for each service fee as provided in Attachment A.

   COMPANY shall pay NTU within [**] of its receipt of each invoice.

2. **Effect of Amendment.** As of the Amendment Effective Date, this Amendment shall amend, modify and supersede, to the extent of any inconsistencies, the provisions of the Agreement. Except as expressly modified by this Amendment, the Agreement shall remain in full force and effect. Capitalized terms used in this Amendment and not otherwise defined shall have the meaning ascribed to such terms in the Agreement. As of the Amendment Effective Date, any reference to the Agreement shall be deemed a reference to the Agreement as amended by this Amendment.

3. **General Provisions.**

   a. Counterparts. This Amendment may be executed in any one or more counterparts, each of which shall be deemed to be an original but all of which together shall constitute one and the same instrument.

   b. Miscellaneous. The Agreement, as amended by this Amendment, sets forth the entire understanding between and among the parties and there are no other understandings or promises, written or verbal, not set forth herein, relating specifically to the subject matter hereof. The Agreement, as amended by this Amendment, supersedes any prior or contemporaneous agreements with respect to the subject matter hereof.

   c. Headings. The headings and subheadings of the sections of this Amendment have been included solely for the ease of reference and do not form part of this Amendment.

IN WITNESS WHEREOF, both NTU and COMPANY have executed this Amendment 9, in duplicate

originals, electronic mail of PDFs or electronic signatures, by their respective and duly authorized officers on the day and year written.

PTC THERAPEUTICS GT, INC.	**NATIONAL TAIWAN UNIVERSITY**
By: /s/ Matthew Klein **Authorized Signature**	By: /s/ Chung-Ming Kuan **Authorized Signature**
Matthew Klein, MD, MS, FACS **Chief Development Officer**	**Printed Name & Title**
Date: 12-Apr-2021	**Date:**

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: May 4, 2021

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: May 4, 2021

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, 2021 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: May 4, 2021

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, 2021 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: May 4, 2021

By: /s/ EMILY HILL
Emily Hill
Chief Financial Officer
(Principal Financial Officer)