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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, 2022

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 2, 2022, there were 71,338,279 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;
- 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;
- 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 maintain our marketing authorization of Translarna™ (ataluren) 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 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;
- our ability to utilize results from Study 041 to support a marketing approval for Translarna for the treatment of nmDMD in the United States;
- the anticipated period of market exclusivity for Emflaza® (deflazacort) for the treatment of DMD in the United States under the Orphan Drug Act of 1983, or Orphan Drug Act;
- our expectations with respect to the 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;
- our expectations and the potential financial impact and benefits related to our Collaboration and License Agreement with a subsidiary of Ionis Pharmaceuticals, Inc. 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 royalty payments by us based on our potential achievement of certain net sales thresholds;
- 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 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 emvododstat 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 products and product candidates through alternative means if pricing and reimbursement negotiations in the applicable territory do not have a positive outcome;
- the timing of, and our ability to obtain additional marketing authorizations for 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 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 complete any post-marketing requirements imposed by regulatory agencies with respect to our products;
- our ability to operate 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, 2021, 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, 2021 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, 2022	December 31, 2021
Assets		
Current assets:		
Cash and cash equivalents	$ 144,178	$ 189,718
Marketable securities	443,615	583,658
Trade and royalty receivables, net	141,288	110,455
Inventory, net	15,281	15,856
Prepaid expenses and other current assets	29,647	54,681
Total current assets	774,009	954,368
Fixed assets, net	59,088	52,585
Intangible assets, net	763,665	724,841
Goodwill	82,341	82,341
Operating lease ROU assets	76,093	77,421
Deposits and other assets	44,395	46,500
Total assets	$ 1,799,591	$ 1,938,056
Liabilities and stockholders' (deficit) equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 246,521	$ 288,784
Current portion of long-term debt	149,721	149,540
Operating lease liabilities- current	7,369	7,273
Finance lease liabilities- current	1,772	3,000
Liability for sale of future royalties- current	69,943	59,291
Other current liabilities	1,450	1,460
Total current liabilities	476,776	509,348
Long-term debt	282,176	281,894
Contingent consideration payable	228,200	239,900
Deferred tax liability	137,110	137,110
Operating lease liabilities- noncurrent	72,432	73,619
Finance lease liabilities- noncurrent	18,675	20,053
Liability for sale of future royalties- noncurrent	674,803	674,694
Total liabilities	1,890,172	1,936,618
Stockholders' (deficit) equity:		
Common stock, $0.001 par value. Authorized 250,000,000 shares; issued and outstanding 71,337,041 shares at March 31, 2022. Authorized 250,000,000 shares; issued and outstanding 70,828,226 shares at December 31, 2021.	71	71
Additional paid-in capital	2,152,639	2,123,606
Accumulated other comprehensive loss	(18,608)	(24,282)
Accumulated deficit	(2,224,683)	(2,097,957)
Total stockholders' (deficit) equity	(90,581)	1,438
Total liabilities and stockholders' equity	$ 1,799,591	$ 1,938,056

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,	
	2022	2021
Revenues:		
Net product revenue	$ 129,832	$ 91,280
Collaboration revenue	7	20,007
Royalty revenue	18,896	6,655
Total revenues	148,735	117,942
Operating expenses:		
Cost of product sales, excluding amortization of acquired intangible assets	10,135	9,104
Amortization of acquired intangible assets	23,473	11,278
Research and development	140,078	134,513
Selling, general and administrative	73,271	61,095
Change in the fair value of deferred and contingent consideration	(11,700)	100
Total operating expenses	235,257	216,090
Loss from operations	(86,522)	(98,148)
Interest expense, net	(23,514)	(19,159)
Other expense, net	(11,855)	(10,884)
Loss before income tax expense	(121,891)	(128,191)
Income tax expense	(4,835)	(451)
Net loss attributable to common stockholders	$ (126,726)	$ (128,642)
Weighted-average shares outstanding:		
Basic and diluted (in shares)	71,215,105	70,188,602
Net loss per share—basic and diluted (in dollars per share)	$ (1.78)	$ (1.83)

See accompanying unaudited notes.

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

	Three Months Ended March 31,	
	2022	2021
Net loss	$ (126,726)	$ (128,642)
Other comprehensive (loss) income:		
Unrealized loss on marketable securities, net of tax of $0	(2,913)	(1,294)
Foreign currency translation gain, net of tax of $0	8,587	23,508
Comprehensive loss	$ (121,052)	$ (106,428)

See accompanying unaudited notes.

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

Three months ended March 31, 2022	Common stock		Additional paid-in capital	Accumulated other comprehensive (loss) income	Accumulated deficit	Total stockholders' (deficit) equity
	Shares	Amount				
Balance, December 31, 2021	70,828,226	$ 71	$ 2,123,606	$ (24,282)	$ (2,097,957)	$ 1,438
Exercise of options	97,188	—	2,444	—	—	2,444
Restricted stock vesting and issuance, net	411,627	—	—	—	—	—
Share-based compensation expense	—	—	26,589	—	—	26,589
Net loss	—	—	—	—	(126,726)	(126,726)
Comprehensive income	—	—	—	5,674	—	5,674
Balance, March 31, 2022	71,337,041	$ 71	$ 2,152,639	$ (18,608)	$ (2,224,683)	$ (90,581)

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

See accompanying unaudited notes.

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

	Three Months Ended March 31,	
	2022	2021
Cash flows from operating activities		
Net loss	$ (126,726)	(128,642)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	26,314	13,587
Non-cash operating lease expense	1,908	1,819
Non-cash royalty revenue related to sale of future royalties	(8,113)	(2,858)
Non-cash interest expense on liability related to sale of future royalties	18,874	18,080
Change in valuation of deferred and contingent consideration	(11,700)	100
Unrealized loss (gain) on ClearPoint Equity Investments	1,049	(6,764)
Unrealized loss (gain) on ClearPoint convertible debt security	1,542	(7,858)
Unrealized loss on marketable securities- equity investments	6,477	302
Amortization of premiums on investments, net	887	1,359
Amortization of debt issuance costs	464	452
Share-based compensation expense	26,589	25,707
Disposal of asset	79	—
Unrealized foreign currency transaction losses, net	2,135	24,691
Changes in operating assets and liabilities:		
Inventory, net	350	1,963
Prepaid expenses and other current assets	25,118	15,605
Trade and royalty receivables, net	(28,372)	(25,010)
Deposits and other assets	(510)	136
Accounts payable and accrued expenses	(30,680)	(28,756)
Other liabilities	(3,089)	(1,916)
Deferred revenue	—	(2,154)
Net cash used in operating activities	$ (97,404)	$ (100,157)
Cash flows from investing activities		
Purchases of fixed assets	$ (9,312)	(5,669)
Purchases of marketable securities- available for sale	(39,035)	(141,985)
Purchases of marketable securities- equity investments	—	(200,000)
Sale and redemption of marketable securities- available for sale	167,101	392,093
Sale and redemption of marketable securities- equity investments	2,423	—
Acquisition of product rights and licenses	(72,134)	(14,192)
Purchase of equity investment in ClearPoint	—	(100)
Net cash provided by investing activities	$ 49,043	$ 30,147
Cash flows from financing activities		
Proceeds from exercise of options	2,444	11,755
Payment of finance lease principal	(1,276)	(2,224)
Net cash provided by financing activities	$ 1,168	$ 9,531
Effect of exchange rate changes on cash	1,653	(1,701)
Net decrease in cash and cash equivalents	(45,540)	(62,180)
Cash and cash equivalents, and restricted cash beginning of period	197,218	216,312
Cash and cash equivalents, and restricted cash end of period	$ 151,678	$ 154,132
Supplemental disclosure of cash information		
Cash paid for interest	$ 6,130	5,182
Cash paid for income taxes	1,987	687
Supplemental disclosure of non-cash investing and financing activity		
Unrealized loss on marketable securities, net of tax	$ (2,913)	(1,294)
Right-of-use assets obtained in exchange for operating lease obligations	587	13
Acquisition of product rights and licenses	12,589	8,870

See accompanying unaudited notes.

PTC Therapeutics, Inc.

Notes to Consolidated Financial Statements (unaudited)
March 31, 2022
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. PTC's ability to innovate to identify new therapies and to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. PTC's mission is to provide access to best-in-class treatments for patients who have few or no treatment options. PTC's strategy is to leverage its strong scientific and clinical expertise and global commercial infrastructure to bring therapies to patients. PTC believes that this allows it to maximize value for all of its stakeholders.

PTC has 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 for rare diseases.

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 Russia for the treatment of nmDMD in patients aged two 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. Translarna also has marketing authorization in Brazil for the treatment of nmDMD in ambulatory patients two years and older and for continued treatment of patients that become non-ambulatory. 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 "Tegsedi-Waylivra Agreement"), dated August 1, 2018, by and between the Company and Akcea Therapeutics, Inc. ("Akcea"), a subsidiary of Ionis Pharmaceuticals, Inc. 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"). The Company began to make commercial sales of Tegsedi for the treatment of hATTR amyloidosis in Brazil in the second quarter of 2022 and it continues to make Tegsedi available in certain other countries within Latin America and the Caribbean through early access programs ("EAP Programs"). In August 2021, ANVISA, the Brazilian health regulatory authority, approved Waylivra as the first treatment for familial chylomicronemia syndrome ("FCS") in Brazil, and the Company has initiated its commercial launch in Brazil while continuing to make Waylivra available in certain other countries within Latin America and the Caribbean through EAP Programs. Waylivra has also received marketing authorization in the EU for the treatment of FCS. Additionally, the Company submitted an application to ANVISA in December 2021 for the approval of Waylivra for the treatment of familial partial lipodystrophy, and it expects a regulatory decision on approval in the second half of 2022.

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 and Japan in June 2021. In January 2022, the FDA granted priority review of a supplemental new drug application for Evrysdi to expand the indication to include pre-symptomatic infants under two months old with SMA and a regulatory decision on approval is expected in May 2022. 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 the results from its Phase 1 study of PTC518 in healthy volunteers in September 2021 demonstrating dose-dependent lowering of huntingtin messenger ribonucleic acid and protein levels, that PTC518 efficiently crosses the blood brain barrier at significant levels and that PTC518 was well tolerated. The Company initiated a Phase 2 study of PTC518 for the treatment of HD in the first quarter of 2022, which consists of an initial 12-week placebo-controlled phase focused on safety, pharmacology and pharmacodynamic effects followed by a nine-month placebo-controlled phase focused on PTC518 biomarker effect. The Company expects results from the initial 12-week phase of the Phase 2 study by the end of 2022.

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 a marketing authorization application ("MAA") for PTC-AADC for the treatment of AADC deficiency in the EEA to the European Medicines Agency ("EMA"). In April 2022, the Company completed the Scientific Advisory Group and Oral Explanation meetings for PTC-AADC with the EMA's Committee for Advanced Therapies. We expect an opinion from the Committee for Medicinal Products for Human Use ("CHMP") in May 2022. The Company is also preparing a biologics license application ("BLA") for PTC-AADC for the treatment of AADC deficiency in the United States. In response to discussions with the FDA, the Company intends to provide additional information concerning the use of the commercial cannula for PTC-AADC in young patients. The Company expects to submit a BLA to the FDA in the third quarter of 2022.

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 associated seizures in the third quarter of 2020. The Company previously experienced delays in enrolling this trial due to the COVID-19 pandemic and anticipates results from this trial to be available in the fourth 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 results from this trial to be available in the second quarter of 2023. In the third quarter of 2021, the Company completed a Phase 1 trial in healthy volunteers to evaluate the safety and pharmacology of PTC857. PTC857 was found to be well-tolerated with no reported serious adverse events while demonstrating predictable pharmacology. The Company initiated a Phase 2 trial of PTC857 for amyotrophic lateral sclerosis in the first quarter of 2022.

The most advanced molecule in the Company's metabolic platform is 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. The Company initiated a registration-directed Phase 3 trial for PTC923 for phenylketonuria ("PKU") in the third quarter of 2021 and expects results from this trial to be available by the end of 2022.

The Company also has two oncology agents that are in clinical development, unesbulin and emvododstat. The Company completed its Phase 1 trials evaluating unesbulin in leiomyosarcoma ("LMS") and diffuse intrinsic pontine glioma ("DIPG") in the fourth quarter of 2021. The Company initiated a registration-directed Phase 2/3 trial of unesbulin for the treatment of LMS in the first quarter of 2022, and it expects to initiate a registration-directed Phase 2 trial of unesbulin for the treatment of DIPG in the third quarter of 2022. The Company completed its Phase 1 trial evaluating emvododstat in acute myelogenous leukemia ("AML"), in the fourth quarter of 2021. The Company expects to provide further updates regarding its emvododstat program at a later date.

In June 2020, the Company initiated a Phase 2/3 clinical trial evaluating the efficacy and safety of emvododstat in patients hospitalized with COVID-19. In February 2021, the Company announced the completion of the first stage of the Phase 2/3 trial. The Company expects results from this trial to be available in the second quarter of 2022.

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 for rare diseases.

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. The marketing authorization in the EEA was last renewed in June 2021 and is effective, unless extended, through August 5, 2022. In February 2022, the Company submitted a marketing authorization renewal request to the EMA and, in April 2022, the CHMP issued an opinion recommending the renewal. 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 Company refers to the trial and open-label extension together as Study 041. The Company anticipates reporting results from the placebo-controlled trial by the end of the second quarter of 2022 after data analysis is completed. The Company then expects to submit a report on the placebo-controlled trial and the open-label extension data that has been collected to date to the EMA by the end of the third quarter of 2022, as required.

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 PTC's appeal of the Complete Response Letter. In its response, the Office of New Drugs recommended a possible path forward for the ataluren NDA submission based on the accelerated approval pathway. This would involve a re-submission of an NDA containing the current data on effectiveness of ataluren with new data to be generated on dystrophin production in nmDMD patients' muscles. The Company 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. Study 045 did not meet its pre-specified primary endpoint. The Company anticipates reporting results from the placebo-controlled trial of Study 041 by the end of the second quarter of 2022 after data analysis is completed, and subject to a positive outcome in that study, the Company expects to re-submit the NDA.

As of March 31, 2022, the Company had an accumulated deficit of approximately $2,224.7 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 (see Note 9), public offerings of common stock in February 2014, October 2014, April 2018, January 2019, and September 2019, "at the market offering" of its common stock, its initial public offering of common stock in June 2013, proceeds from the 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, 2021 included in the Company's Annual Report on Form 10-K filed with the Securities and Exchange Commission (the "SEC") on February 22, 2022 (the "2021 Form 10-K"). Selected significant accounting policies are discussed in further detail below.

Basis of presentation

The accompanying financial information as of March 31, 2022 and for the three months ended March 31, 2022 and 2021 has been prepared by the Company, without audit, pursuant to the rules and regulations of the SEC. Certain information and footnote disclosures normally included in financial statements prepared in accordance with generally accepted accounting principles in the United States ("GAAP") have been condensed or omitted pursuant to such rules and regulations. These interim financial statements should be read in conjunction with the Company's audited financial statements as of December 31, 2021 and notes thereto included in the 2021 Form 10-K.

In the opinion of management, the unaudited financial information as of March 31, 2022 and for the three months ended March 31, 2022 and 2021 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, 2022 are not necessarily indicative of the results to be expected for the year ended December 31, 2022 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, 2022	Beginning of period- December 31, 2021
Cash and cash equivalents	$ 144,178	$ 189,718
Restricted cash included in deposits and other assets	7,500	7,500
Total Cash, cash equivalents and restricted cash per statement of cash flows	$ 151,678	$ 197,218

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 periods ended March 31, 2022 and 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) income, 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. Amounts related to clinical development programs and marketing efforts are immaterial.

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

	March 31, 2022	December 31, 2021
Raw materials	$ 1,501	$ 1,418
Work in progress	8,070	7,721
Finished goods	5,710	6,717
Total inventory	$ 15,281	$ 15,856

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, 2022 and 2021, the Company recorded inventory write-downs of $0.6 million and $1.4 million, 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, 2022 and 2021, 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, 2022 and 2021, net product sales outside of the United States were $81.2 million and $47.8 million, respectively, and net product sales in the United States were $48.6 million and $43.5 million, respectively, consisting solely of Emflaza. Translarna net revenues made up $79.2 million and $46.5 million of the net product sales outside of the United States for the three months ended March 30, 2022 and 2021, 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 Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("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, 2022 and 2021, the Company recognized $0.0 million and $20.0 million of collaboration revenue, respectively, related to the SMA License Agreement with Roche. The first commercial sale of Evrysdi in the EU was made in March 2021. This event triggered a $20.0 million milestone payment to the Company from Roche.

For the three months ended March 31, 2022 and 2021, the Company has recognized $18.9 million and $6.7 million of royalty revenue, respectively, related to Evrysdi.

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, 2022 and 2021, no allowance was recorded for credit losses. The allowance for doubtful accounts was $0.1 million as of March 31, 2022 and $0.1 million as of December 31, 2021. Bad debt expense was immaterial for the three month periods ended March 31, 2022 and 2021.

Liability for sale of future royalties

On July 17, 2020, the Company, RPI, and, for the limited purposes set forth in the agreement, Royalty Pharma PLC, 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 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 the SMA program. 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 $300.0 million in the aggregate as of March 31, 2022. 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 is being amortized using the effective interest method over the life of the arrangement, in accordance with the respective guidance. The Company utilizes 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 a strain of novel coronavirus, COVID-19. The CARES Act includes modifications for net operating loss carryovers and carrybacks, limitations of business interest expense for tax, immediate refund of alternative minimum tax (“AMT”) credit carryovers as well as a technical correction to the 2017 Tax Cuts and Jobs Act ("the 2017 Tax Act") for qualified improvement property. On December 27, 2020, the Coronavirus Response and Relief Supplemental Appropriations Act of 2021 – a $900 billion relief package to deliver the second round of economic stimulus for individuals, families, and businesses was signed into law. The bill provides relief through multiple measures and expands many of the provisions already put into place under the CARES Act. As of March 31, 2022, the Company expects that these provisions will not have a material impact. 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 $1.3 million as of March 31, 2022, which is accrued in other current liabilities on the consolidated balance sheet.

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, 2022.

Starting in 2022, TCJA amendments to IRC Section 174 will no longer permit an immediate deduction for research and development (R&D) expenditures in the tax year that such costs are incurred. Instead, these IRC Section 174 development costs must now be capitalized and amortized over either a five- or 15-year period, depending on the location of the activities performed. The new amortization period begins with the midpoint of any taxable year that IRC Section 174 costs are first incurred, regardless of whether the expenditures were made prior to or after July 1, and runs until the midpoint of year five for activities conducted in the United States or year 15 in the case of development conducted on foreign soil. As a result of this tax law change, the Company recorded a federal and state tax provision for the three month period ended March 31, 2022, in the amount of $0.6 million and $2.6 million, respectively.

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.

On August 23, 2018, the Company completed 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., a Delaware corporation and the Company's wholly owned, indirect subsidiary, Agilis and, solely in its capacity as the representative, agent and attorney-in-fact of the equityholders of Agilis, Shareholder Representative Services LLC, (the "Agilis Merger"). The Company recorded a deferred tax liability in conjunction with the Agilis Merger of $122.0 million in 2018, related to the tax basis difference in the IPRD 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.

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 and laboratory space in Bridgewater, New Jersey and 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 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 with 24 months prior written notice to MassBio. Pursuant to the terms of the agreement, MassBio agreed to provide the Company with certain dedicated space 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. The agreement included guaranteed lease payments of $15.0 million at the onset of the agreement and $3.0 million annually thereafter. The present value of the guaranteed lease payments was determined to be $41.4 million, which exceeded the assessed fair value of the Company's share of the building. Therefore, the Company determined that the agreement was a finance lease, for which the Company recorded a finance lease ROU asset and corresponding finance lease liability at the onset of the lease agreement. Given that the leased asset 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. Accordingly, the full $41.4 million relating to the finance lease ROU asset was written off and expensed to research and development during the twelve month period ending December 31, 2020. The remaining balance for the finance lease ROU asset related to this arrangement is $0 as of March 31, 2022 and as of December 31, 2021. As of March 31, 2022, the balance of the finance lease liabilities-current and finance lease liabilities-non-current are $1.8 million and $18.7 million, respectively, and are directly related to the Company's MassBio agreement. As of December 31, 2021, the balance of the finance lease liabilities-current and finance lease liabilities-non current were $3.0 million and $20.1 million, respectively. Additionally, the Company recorded finance lease costs of $0.4 million related to interest on the lease liability during both the three month period ending March 31, 2022, and the three month period ending March 31, 2021.

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 0.1 years to 13.3 years and certain of the leases include renewal options to extend the lease for up to 10 years. Rent expense was $5.3 million and $5.4 million for the three month periods ended March 31, 2022 and 2021, respectively.

The components of operating lease expense were as follows:

	Three Months Ended March 31, 2022	Three Months Ended March 31, 2021
Operating Lease Cost		
Fixed lease cost	$ 4,126	$ 4,104
Variable lease cost	1,076	1,093
Short-term lease cost	74	164
Total operating lease cost	$ 5,276	$ 5,361

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

The Company entered into one new lease, an office in Tokyo, Japan, during the three months ended March 31, 2022; this new Japan lease did not have a material impact on the consolidated financial statements.

Supplemental lease term and discount rate information related to leases was as follows as of March 31, 2022 and December 31, 2021:

	March 31, 2022	December 31, 2021
Weighted-average remaining lease terms - operating leases (years)	10.67	10.87
Weighted-average discount rate - operating leases	8.91 %	8.91 %
Weighted-average remaining lease terms - finance lease (years)	10.75	11.00
Weighted-average discount rate - finance lease	7.80 %	7.80 %

Supplemental cash flow information related to leases was as follows as of March 31, 2022 and 2021:

	Three Months Ended March 31, 2022	2021
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows from operating leases	$ 3,411	$ 3,406
Financing cash flows from finance lease	1,276	2,224
Operating cash flows from finance leases	1,724	776
Right-of-use assets obtained in exchange for lease obligations:		
Operating leases	$ 587	$ 13

Future minimum lease payments under non-cancelable leases as of March 31, 2022 were as follows:

	Operating Leases	Finance Lease
2022 (excludes the three months ended March 31, 2022)	$ 10,154	$ —
2023	13,298	3,000
2024	12,619	3,000
2025	11,231	3,000
2026 and thereafter	80,769	21,000
Total lease payments	128,071	30,000
Less: Imputed Interest expense	48,270	9,553
Total	$ 79,801	$ 20,447

In conjunction with the Asset Purchase Agreement by and between the Company and BioElectron Technology Corporation, dated October 1, 2019 (the "BioElectron 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, 2022 are $0.9 million for the remainder of 2022 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, 2022, the Company recorded an unrealized loss of $1.0 million. During the three month period ended March 31, 2021, the Company recorded an unrealized gain of $6.8 million. These unrealized gains and losses are components of other (expense) income, net within the consolidated statement of operations. The fair value of the ClearPoint Equity Investments was $13.5 million and $14.5 million as of March 31, 2022 and December 31, 2021, 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, 2022, the Company recorded an unrealized loss of $1.5 million. During the three month period ended March 31, 2021, the Company recorded an unrealized gain of $7.9 million. These unrealized gains and losses are components of other (expense) income, net within the consolidated statement of operations. The fair value of the convertible debt security was $19.4 million and $21.0 million as of March 31, 2022 and December 31, 2021, 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. In August 2021, the Company made a $5.4 million and $4.6 million investment into a third mutual fund that is denominated in a foreign currency. All of these are equity investments and 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) income, net within the consolidated statement of operations. For the three month periods ended March 31, 2022 and 2021, the Company had $6.5 million and $0.3 million of unrealized net losses relating to the equity investments still held at the reporting date, respectively. For the three month periods ended March 31, 2022 and 2021 the Company had redemptions of $2.4 million and $0.0 million, respectively. For the three month periods ended March 31, 2022 and 2021, the Company had foreign currency unrealized gains relating to these equity investments of $0.7 million and $0.0 million, respectively.

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, 2022 and December 31, 2021:

	March 31, 2022			
	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	$ 244,820	$ —	$ 244,820	$ —
Marketable securities - equity investments	$ 198,795	$ 198,795	$ —	$ —
ClearPoint Equity Investments	$ 13,477	$ 13,477	$ —	$ —
ClearPoint convertible debt security	$ 19,429	$ —	$ 19,429	$ —
Contingent consideration payable- development and regulatory milestones	$ 134,700	$ —	$ —	$ 134,700
Contingent consideration payable- net sales milestones and royalties	$ 93,500	$ —	$ —	$ 93,500

	December 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	$ 376,685	$ —	$ 376,685	$ —
Marketable securities - equity investments	$ 206,973	$ 206,973	$ —	$ —
ClearPoint Equity Investments	$ 14,525	$ 14,525	$ —	$ —
ClearPoint convertible debt security	$ 20,971	$ —	$ 20,971	$ —
Contingent consideration payable- development and regulatory milestones	$ 139,300	$ —	$ —	$ 139,300
Contingent consideration payable- net sales milestones and royalties	$ 100,600	$ —	$ —	$ 100,600

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, 2022 and December 31, 2021.

The following is a summary of marketable securities accounted for as available for sale debt securities at March 31, 2022 and December 31, 2021:

	March 31, 2022			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 27,292	—	(11)	27,281
Corporate debt securities	187,121	18	(3,139)	184,000
Asset-backed securities	10,202	7	(37)	10,172
Government obligations	23,720	5	(358)	23,367
Total	$ 248,335	$ 30	$ (3,545)	$ 244,820

	December 31, 2021			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 75,275	5	(1)	$ 75,279
Corporate debt securities	268,246	81	(644)	267,683
Asset-backed securities	15,287	16	(5)	15,298
Government obligations	18,479	5	(59)	18,425
Total	$ 377,287	$ 107	$ (709)	$ 376,685

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, 2022, 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 basis, 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, 2022 and 2021, 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, 2022, the Company had $0.1 million realized losses from the sale of available for sale debt securities. 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. 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, 2022 are as follows:

	March 31, 2022					
	Securities in an unrealized loss position less than 12 months		Securities in an unrealized loss position greater than or equal to 12 months		Total	
	Unrealized losses	Fair Value	Unrealized losses	Fair Value	Unrealized losses	Fair Value
Commercial paper	$ (11)	27,281	—	—	(11)	27,281
Corporate debt securities	(2,996)	174,146	(143)	4,876	(3,139)	179,022
Asset-backed securities	(37)	8,435	—	—	(37)	8,435
Government obligations	(358)	18,056	—	—	(358)	18,056
Total	$ (3,402)	$ 227,918	$ (143)	$ 4,876	$ (3,545)	$ 232,794

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, 2021 are as follows:

	December 31, 2021					
	Securities in an unrealized loss position less than 12 months		Securities in an unrealized loss position greater than or equal to 12 months		Total	
	Unrealized losses	Fair Value	Unrealized losses	Fair Value	Unrealized losses	Fair Value
Commercial paper	$ (1)	12,992	—	—	(1)	12,992
Corporate debt securities	(608)	217,540	(36)	4,985	(644)	222,525
Asset-backed securities	(5)	10,786	—	—	(5)	10,786
Government obligations	(59)	15,483	—	—	(59)	15,483
Total	$ (673)	$ 256,801	$ (36)	$ 4,985	$ (709)	$ 261,786

Available for sale debt securities at March 31, 2022 and December 31, 2021 mature as follows:

	March 31, 2022	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 27,281	$ —
Corporate debt securities	72,783	111,217
Asset-backed securities	4,100	6,072
Government obligations	7,278	16,089
Total	$ 111,442	$ 133,378

	December 31, 2021	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 75,279	$ —
Corporate debt securities	131,606	136,077
Asset-backed securities	8,724	6,574
Government obligations	6,002	12,423
Total	$ 221,611	$ 155,074

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 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, 2022 and December 31, 2021 was $155.7 million and $158.3 million, respectively. The estimated fair value of the 2026 Convertible notes at March 31, 2022 and December 31, 2021 was $287.5 million and $305.3 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, 2022, the weighted average discount rate for the development and regulatory milestones was 5.1% 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, 2022, the weighted average discount rate for the net sales milestones and royalties was 12.0% 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, 2022 and March 31, 2021:

	Level 3 liabilities	
	Contingent consideration payable- development and regulatory milestones	Contingent consideration payable- net sales milestones and royalties
Beginning balance as of December 31, 2021	$ 139,300	$ 100,600
Additions	—	—
Change in fair value	(4,600)	(7,100)
Payments	—	—
Ending balance as of March 31, 2022	$ 134,700	$ 93,500

	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

The following significant unobservable inputs were used in the valuation of the contingent consideration payable for the periods ended March 31, 2022 and December 31, 2021:

	March 31, 2022			
	Fair Value	Valuation Technique	Unobservable Input	Range
Contingent consideration payable- development and regulatory milestones	$134,700	Probability-adjusted discounted cash flow	Potential development and regulatory milestones Probabilities of success Discount rates Projected years of payments	$0 - $381 million 25% - 94% 3.8% - 6.1% 2022 - 2028
Contingent considerable payable- net sales milestones and royalties	$93,500	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% 12.0% 2023 - 2040

	December 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% 1.7% - 4.7% 2022 - 2028
Contingent considerable payable- net sales milestones and royalties	$100,600	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% 11.0% 2023 - 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, 2022 and December 31, 2021 consist of the following:

	March 31, 2022	December 31, 2021
Employee compensation, benefits, and related accruals	$ 29,485	$ 55,733
Income tax payable	4,549	1,287
Consulting and contracted research	28,391	26,434
Professional fees	5,013	3,547
Sales allowance	63,109	61,662
Sales rebates	57,680	68,770
Royalties	30,670	35,679
Accounts payable	18,365	23,033
Other	9,259	12,639
Total	$ 246,521	$ 288,784

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. No shares were sold during the three month periods ended March 31, 2022 and 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, 2022.

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,	
	2022	2021
Numerator		
Net loss	$ (126,726)	$ (128,642)
Denominator		
Denominator for basic and diluted net loss per share	71,215,105	70,188,602
Net loss per share:		
Basic and diluted	$ (1.78)*	$ (1.83)*

* In the three month periods ended March 31, 2022 and 2021, 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,	
	2022	2021
Stock Options	11,751,713	10,989,202
Unvested restricted stock awards and units	2,514,981	1,494,638
Total	14,266,694	12,483,840

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, 2022, awards for 922,076 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, 2022, awards for 823,211 shares of common stock were available for issuance under the 2020 Inducement Stock Incentive Plan. In April 2022, the Company's Board of Directors approved a reduction in the total number of shares of common stock that may be issued under the 2020 Inducement Stock Incentive Plan to 1,300,000 shares.

The Board of Directors has the authority to select the individuals to whom options are granted and determine the terms of each option, including (i) the number of shares of common stock subject to the option; (ii) the date on which the option becomes exercisable; (iii) the option exercise price, which, in the case of incentive stock options, must be at least 100% (110% in the case of incentive stock options granted to a stockholder owning in excess of 10% of the Company's stock) of the fair market value of the common stock as of the date of grant; and (iv) the duration of the option (which, in the case of incentive stock options, may not exceed ten years). Options typically vest over a four-year period.

From January 1, 2022 through March 31, 2022, the Company issued a total of 1,275,830 stock options to various employees. Of those, 18,255 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, 2021	10,772,582	$ 43.66		
Granted	1,275,830	38.10		
Exercised	(97,188)	25.15		
Forfeited/Cancelled	(199,511)	49.92		
Outstanding at March 31, 2022	11,751,713	$ 43.11	6.97 years	$ 38,075
Vested or Expected to vest at March 31, 2022	4,497,327	$ 48.04	8.54 years	$ 2,127
Exercisable at March 31, 2022	6,763,376	$ 39.44	5.79 years	$ 35,859

The fair value of grants made in the three months ended March 31, 2022 was contemporaneously estimated on the date of grant using the following assumptions:

	Three months ended March 31, 2022
Risk-free interest rate	1.55%
Expected volatility	73.56%
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 months ended March 31, 2022 was $23.92 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. For the three month period ended March 31, 2022, the Company issued a total of 1,490,190 restricted stock units to various employees. Of those, 10,675 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, 2021	1,519,831	$ 55.43
Granted	1,490,190	38.10
Vested	(426,470)	51.26
Forfeited	(68,570)	48.45
Unvested at March 31, 2022	2,514,981	$ 46.06

Employee Stock Purchase Plan—In June 2016, the Company established an Employee Stock Purchase Plan (as amended, "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. In June 2021, the Plan was amended to increase the total number of shares available for purchase under the Plan from one million shares to two 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 months ended March 31, 2022, 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,	
	2022	2021
Research and development	$ 13,034	$ 13,725
Selling, general and administrative	13,555	11,982
Total	$ 26,589	$ 25,707

As of March 31, 2022, there was approximately $251.8 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 2.62 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 is being 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 utilizes the prospective method to account for subsequent changes in the estimated future payments to be made to RPI and updates 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, 2022:

Liability for sale of future royalties- (current and noncurrent)	Three Months Ended March 31, 2022
Beginning balance as of December 31, 2021	$ 733,985
Less: Non-cash royalty revenue payable to RPI	(8,113)
Plus: Non-cash interest expense recognized	18,874
Ending balance	$ 744,746
Effective interest rate as of March 31, 2022	10.1 %

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 of the 2026 Convertible Notes 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, 2022	December 31, 2021
Principal	$ 287,500	$ 287,500
Less: Debt issuance costs	(5,324)	(5,606)
Net carrying amount	$ 282,176	$ 281,894

As of March 31, 2022, the remaining contractual life of the 2026 Convertible Notes is approximately 4.5 years.

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

	Three Months Ended March 31,	
	2022	2021
Contractual interest expense	$ 1,069	$ 1,069
Amortization of debt issuance costs	283	277
Total	$ 1,352	$ 1,346
Effective interest rate of the liability component	1.9 %	1.9 %

In April 2022, under the terms of the 2026 Convertible Notes Indenture, the Company paid additional interest on the 2026 Convertible Notes at a rate equal to 0.5% per annum, for a total interest payment of approximately $2.1 million, for the period beginning September 25, 2020 and ending March 14, 2022. This amount is not included in the table above, but was recorded as interest expense, net within the statement of operations for the three month period ended March 31, 2022.

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 of the 2022 Convertible Notes 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.

As of 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 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 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, 2022	December 31, 2021
Principal	$ 150,000	$ 150,000
Less: Debt issuance costs	(279)	(460)
Net carrying amount	$ 149,721	$ 149,540

As of March 31, 2022, the remaining contractual life of the 2022 Convertible Notes is approximately 0.4 years.

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

	Three Months Ended March 31,	
	2022	2021
Contractual interest expense	$ 1,110	$ 1,110
Amortization of debt issuance costs	181	175
Total	$ 1,291	$ 1,285
Effective interest rate of the liability component	3.5 %	3.5 %

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 made the first development milestone payment of $0.8 million to Wellcome Trust under the oncology platform funding agreement during 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 is 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. As of the three month period ended March 31, 2022, $14.0 million was owed and is classified as accounts payable and accrued expenses on the Company's consolidated balance sheets. 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 received a $50.0 million sales-based milestone during the three month period ended March 31, 2022.

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 BioElectron 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 BioElectron 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, former 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 Tegsedi-Waylivra Agreement for the commercialization of Tegsedi and Waylivra, and products containing those compounds in countries in Latin America and the Caribbean. Pursuant to the Tegsedi-Waylivra 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. In addition, a $4.0 million milestone was paid upon receipt of regulatory approval for Waylivra from ANVISA in August 2021. Akcea is also entitled to receive royalty payments subject to certain terms set forth in the Tegsedi-Waylivra 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. 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, 2022 and 2021, net product sales in the United States were $48.6 million and $43.5 million, respectively, consisting solely of Emflaza, and net product sales outside of the United States were $81.2 million and $47.8 million, respectively, consisting of Translarna, Tegsedi, and Waylivra. Translarna net revenues made up $79.2 million and $46.5 million of the net product sales outside of the United States for the three months ended March 31, 2022 and 2021, respectively. For the three months ended March 31, 2022 and 2021, the Company had a total of two and two distributors, respectively, that each accounted for over 10% of the Company's net product sales.

As of March 31, 2022 and December 31, 2021, the Company did not have any contract liabilities or assets. For the three month period ended March 31, 2022, the Company did not recognize any revenue related to the amounts included in the contract liability balance at the beginning of the period. For the three month period ended March 31, 2021, the Company recognized $2.1 million of revenue, 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, 2022 and 2021.

Remaining performance obligations

Remaining performance obligations represent the transaction price for goods the Company has yet to provide. As of March 31, 2022 and December 31, 2021 the Company does not have any remaining performance obligations relating to Translarna net product revenue.

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.

Under the SMA License Agreement, the Company is eligible to receive additional payments from Roche if specified events are achieved with respect to each licensed product, including up to $135.0 million in research and development event milestones, up to $325.0 million in sales milestones upon achievement of 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, 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, 2022, the Company does not have any remaining research and development event milestones that can be received. The remaining potential sales milestones that can be received is $300.0 million.

For the three months ended March 31, 2022 and 2021, the Company recognized $0.0 million and $20.0 million, respectively, related to the SMA License Agreement with Roche. The first commercial sale of Evrysdi in the EU was made in March 2021. This event triggered a $20.0 million milestone payment to the Company from Roche for the three months ended March 31, 2021.

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, 2022, the Company has recognized $18.9 million of royalty revenue, related to Evrysdi. For the three months ended March 31, 2021, the Company has recognized $6.7 million of royalty revenue related to Evrysdi.

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. Marathon received a $50.0 million sales-based milestone during the three month period ended March 31, 2022. For the three months ended March 31, 2022 and 2021, total milestone payments of $62.1 million and $8.9 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, 2022, a milestone payable to Marathon of $12.1 million was recorded on the balance sheet within accounts payable and accrued expenses.

Pursuant to the Tegsedi-Waylivra Agreement, in May 2019 the Company made a $6.0 million milestone payment to Akcea upon regulatory approval of Waylivra from the EMA. In December 2019, the Company made a $4.0 million milestone payment to Akcea upon regulatory approval of Tegsedi from ANVISA. Both payments were recorded as intangible assets and are being amortized to cost of product sales over their expected useful life of approximately ten years on a straight line basis. Additionally, in August 2021, the Company made a $4.0 million milestone payment to Akcea upon regulatory approval of Waylivra from ANVISA. In accordance with the guidance for an asset acquisition, the Company recorded the milestone payment when it became payable to Akcea, and it increased the cost basis for the Waylivra intangible asset. This payment is being amortized to cost of product sales over the expected remaining useful life of the Waylivra asset on a straight line basis.

Akcea is also entitled to receive royalty payments subject to certain terms set forth in the Tegsedi-Waylivra Agreement related to sales of Waylivra and Tegsedi. 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 and Tegsedi intangible assets, respectively. For the three months ended March 31, 2022, a royalty payment of $0.4 million was recorded for Tegsedi. No royalty payment was recorded for the three months ended March 31, 2021. As of March 31, 2022, a royalty payable of $0.5 million was recorded on the balance sheet within accounts payable and accrued expenses.

For the three months ended March 31, 2022 and 2021, the Company recognized amortization expense of $23.5 million and $11.3 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, 2022
2022	$ 70,475
2023	93,966
2024	16,041
2025	1,542
2026 and thereafter	5,140
Total	$ 187,164

The weighted average remaining amortization period of the definite-lived intangibles as of March 31, 2022 is 2.2 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, which included a measurement period adjustment of $18.0 million recorded during the three month period ended December 31, 2018. This adjustment was related to the finalization of the fair values assigned to the intangible assets and corresponding deferred

tax liability, the contingent consideration, and the deferred consideration. There have been no changes to the balance of goodwill since the date of the Agilis Merger. Accordingly, the goodwill balance as of March 31, 2022 is $82.3 million.

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 and analysis is meant to provide material information relevant to an assessment of the financial condition and results of operations of our company, including an evaluation of the amounts and certainty of cash flows from operations and from outside resources, so as to allow investors to better view our company from management's perspective. 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, 2021 included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 22, 2022, or our 2021 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 2021 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 innovate to identify new therapies and to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. Our mission is to provide access to best-in-class treatments for patients who have few or no treatment options. Our strategy is to leverage our strong scientific and clinical expertise and global commercial infrastructure to bring therapies to patients. We believe that this allows us to maximize value for all of our 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 for rare diseases.

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, 2021, the following expectations have been revised as a result of the impact or expected impact of the COVID-19 pandemic:

- As of the date of this Report on Form 10-Q, except as otherwise previously 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 support our existing patient base and remotely connect with them, as necessary. We 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, 2021.

Global Commercial Footprint

Global DMD Franchise

We have two products, Translarna™ (ataluren) and Emflaza® (deflazacort), for the treatment of Duchenne muscular dystrophy, or DMD, a rare, life threatening disorder. Translarna has marketing authorization in the European Economic Area, or EEA, for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, in ambulatory patients aged two years and older and in Russia for the treatment of nmDMD in patients aged two 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. Translarna also has marketing authorization in Brazil for the treatment of nmDMD in ambulatory patients two years and older and for continued treatment of patients that become non-ambulatory. During the quarter ended March 31, 2022, we recognized $79.2 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, 2022, we recognized $48.6 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 2021, the European Commission renewed our marketing authorization, making it effective, unless extended, through August 5, 2022. In February 2022, we submitted a marketing authorization renewal request to the EMA and, in April 2022, the Committee for Medicinal Products for Human Use, or CHMP, issued an opinion recommending the renewal. 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. We anticipate reporting results from the placebo-controlled trial by the end of the second quarter of 2022 after data analysis is completed. We then expect to submit a report on the placebo-controlled trial and the open-label extension data that has been collected to date to the EMA by the end of the third quarter of 2022, as required.

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 United States Food and Drug Administration, or 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. Study 045 did not meet its pre-specified primary endpoint. We anticipate reporting results from the placebo-controlled trial of Study 041 by the end of the second quarter of 2022 after data analysis is completed, and subject to a positive outcome in that study, we expect to re-submit the NDA.

Tegsedi® (inotersen) and Waylivra™ (volanesorsen)

We hold the rights for the commercialization of Tegsedi and Waylivra for the treatment of rare diseases in countries in Latin America and the Caribbean pursuant to a Collaboration and License Agreement, or the Tegsedi-Waylivra Agreement, dated August 1, 2018, by and between us and Akcea Therapeutics, Inc., or Akcea, a subsidiary of Ionis Pharmaceuticals, Inc. 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. We began to make commercial sales of Tegsedi for the treatment of hATTR amyloidosis in Brazil in the second quarter of 2022 and we continue to make Tegsedi available in certain other countries within Latin America and the Caribbean through early access programs, or EAP Programs. In August 2021, ANVISA, the Brazilian health regulatory authority, approved Waylivra as the first treatment for familial chylomicronemia syndrome, or FCS, in Brazil and we have initiated our commercial launch in Brazil while continuing to make Waylivra available in certain other countries within Latin America and the Caribbean through EAP programs. Waylivra has also received marketing authorization in the European Union, or EU, for the treatment of FCS. Additionally, we submitted an application to ANVISA in December 2021 for the approval of Waylivra for the treatment of familial partial lipodystrophy, or FPL, and we expect a regulatory decision on approval in the second half of 2022.

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 and Japan in June 2021. In January 2022, the FDA granted priority review of a supplemental new drug application for Evrysdi to expand the indication to include pre-symptomatic infants under two months old with SMA and a regulatory decision on approval is expected in May 2022.

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 the results from our Phase 1 study of PTC518 in healthy volunteers in September 2021 demonstrating dose-dependent lowering of huntingtin messenger ribonucleic acid and protein levels, that PTC518 efficiently crosses blood brain barrier at significant levels and that PTC518 was well tolerated. We initiated a Phase 2 study of PTC518 for the treatment of HD in the first quarter of 2022, which consists of an initial 12-week placebo-controlled phase focused on safety, pharmacology and pharmacodynamic effects followed by a nine-month placebo-controlled phase focused on PTC518 biomarker effect. We expect results from the initial 12-week phase of the Phase 2 study by the end of 2022.

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 a marketing authorization application, or MAA, for PTC-AADC for the treatment of AADC deficiency in the EEA to the EMA. In April 2022, we completed the Scientific Advisory Group and Oral Explanation meetings for PTC-AADC with the EMA's Committee for Advanced Therapies. We expect an opinion from the CHMP in May 2022. We are also preparing a biologics license application, or BLA, for PTC-AADC for the treatment of AADC deficiency in the United States. In response to discussions with the FDA, we intend to provide additional information concerning the use of the commercial cannula for PTC-AADC in young patients. We expect to submit a BLA to the FDA in the third quarter of 2022.

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 associated seizures in the third quarter of 2020. We previously experienced delays in enrolling this trial due to the COVID-19 pandemic and anticipate results from this trial to be available in the fourth 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 results from this trial to be available in the second quarter of 2023. In the third quarter of 2021, we completed a Phase 1 trial in healthy volunteers to evaluate the safety and pharmacology of PTC857. PTC857 was found to be well-tolerated with no reported serious adverse events while demonstrating predictable pharmacology. We initiated a Phase 2 trial of PTC857 for amyotrophic lateral sclerosis in the first quarter of 2022.

Metabolic Platform

The most advanced molecule in our metabolic platform is 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 initiated a registration-directed Phase 3 trial for PTC923 for phenylketonuria, or PKU, in the third quarter of 2021 and expect results from this trial to be available by the end of 2022.

Oncology Platform

We also two oncology agents in that are in clinical development, unesbulin and emvododstat. We completed our Phase 1 trials evaluating unesbulin in leiomyosarcoma, or LMS, and diffuse intrinsic pontine glioma, or DIPG, in the fourth quarter of 2021. We initiated a registration-directed Phase 2/3 trial of unesbulin for the treatment of LMS in the first quarter of 2022 and we expect to initiate a registration-directed Phase 2 trial of unesbulin for the treatment of DIPG in the third quarter of 2022. We completed our Phase 1 trial evaluating emvododstat in acute myelogenous leukemia, or AML, in the fourth quarter of 2021. We expect to provide further updates regarding our emvododstat program at a later date.

Emvododstat for COVID-19

In June 2020, we initiated a Phase 2/3 clinical trial evaluating the efficacy and safety of emvododstat in patients hospitalized with COVID-19. In February 2021, we announced the completion of the first stage of the Phase 2/3 trial. We expect results from this trial to be available in the second quarter of 2022.

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 a License and Collaboration Agreement, or the SMA License Agreement, by and among us, Roche and, for the limited purposes set forth therein, the SMA Foundation, under our SMA program.

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. As of 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. Upon conversion, we will pay or deliver, as the case may be, cash, shares of our common stock or any combination thereof at our election. We received net proceeds from the offering of approximately $145.4 million, after deducting the initial purchasers' discounts and commissions and the 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. The remaining shares of our common stock available to be issued and sold, under the Sales Agreement, have an aggregate offering price of up to $93.0 million as of December 31, 2021. During the three months ended March 31, 2022, 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, 2022, we had an accumulated deficit of $2,224.7 million. We had a net loss of $126.7 million and $128.6 million for the three month periods ended March 31, 2022 and 2021, 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 emvododstat 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 May 2022. We are also preparing a BLA for PTC-AADC for the treatment of AADC deficiency in the United States and we anticipate submitting a BLA to the FDA in the third quarter of 2022. We filed for marketing authorization for Waylivra with ANVISA for the treatment of FPL and we expect a regulatory decision on approval from ANVISA in the second half of 2022. 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. The 2022 Convertible Notes will mature on August 15, 2022 and we will be required to pay any outstanding principal amount of the 2022 Convertible Notes at that time, unless earlier converted, redeemed or repurchased in accordance with their terms prior to such date. As of 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. Upon conversion, we will pay or deliver, as the case may be, cash, shares of our common stock or any combination thereof at our election. 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.

In addition, in the first quarter of 2022, we paid Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC), or Marathon, a $50.0 million sales-based milestone in connection with Emflaza. We expect to pay the former equityholders of Agilis an aggregate of $70.0 million upon the achievement of certain development and regulatory milestones in 2022 relating to PTC-AADC. We also expect to pay the former securityholders of Censa Pharmaceuticals, Inc., or Censa, a $30.0 million development milestone for the completion of enrollment of a Phase 3 clinical trial for PTC923 for PKU in 2022. If achieved, we have the option to pay such milestone payment in cash or shares of our common stock.

We also have certain significant contractual obligations and commercial commitments that require funding and we have disclosed these items under the heading “Management’s Discussion and Analysis of Financial Condition and Results of Operations-Funding Obligations” in our 2021 Annual Report on Form 10-K. There were no material changes to these obligations and commitments during the period ended March 31, 2022. 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, 2022 and 2021, net product sales outside of the United States were $81.2 million and $47.8 million, respectively. For the three months ended March 31, 2022 and 2021, net product sales in the United States were $48.6 million and $43.5 million, respectively, consisting solely of Emflaza. Translarna net revenues made up $79.2 million and $46.5 million of the net product sales outside of the United States for the three months ended March 31, 2022 and 2021, 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. As of March 31, 2022, we had recognized a total of $160.0 million in milestone payments and $78.3 million royalties on net sales pursuant to the SMA License Agreement. As of March 31, 2022, there are no remaining research and development event milestones that we can receive. The remaining potential sales milestones as of March 31, 2022 are $300.0 million upon achievement of certain sales events.

For the three months ended March 31, 2022 and 2021, we recognized $0.0 million and $20.0 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 us from Roche for the three months ended March 31, 2021.

For the three months ended March 31, 2022 and 2021, we have recognized $18.9 million and $6.7 million of royalty revenue, respectively, related to Evrysdi.

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 emvododstat 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, 2022 and 2021. Certain prior period expenses have been reclassified to conform to the current period presentation.

	Three Months Ended March 31,	
	2022	2021
	(in thousands)	
Global DMD Franchise	$ 17,694	$ 15,457
Metabolic	15,756	11,579
Gene Therapy	42,570	54,633
Bio-e	14,864	13,601
Oncology	6,255	3,151
Splicing	14,397	9,624
Emvododstat for COVID-19	2,348	10,874
Discovery	26,194	15,594
Total research and development	$ 140,078	$ 134,513

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, and from the Convertible Notes outstanding.

Critical accounting policies and significant judgments and estimates

Our management’s discussion and analysis of our financial condition and results of operations is based on our financial statements, which we have prepared in accordance with generally accepted accounting principles in the United States. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts

of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported revenues and expenses during the reporting periods. Actual results may differ from these estimates under different assumptions or conditions.

During the three months ended March 31, 2022, there were no material changes to our critical accounting policies as reported in our 2021 Annual Report on Form 10-K.

Results of operations

Three months ended March 31, 2022 compared to three months ended March 31, 2021

The following table summarizes revenues and selected expense and other income data for the three months ended March 31, 2022 and 2021.

(in thousands)	Three Months Ended March 31, 2022	2021	Change 2022 vs. 2021
Net product revenue	$ 129,832	$ 91,280	$ 38,552
Collaboration revenue	7	20,007	(20,000)
Royalty revenue	18,896	6,655	12,241
Cost of product sales, excluding amortization of acquired intangible asset	10,135	9,104	1,031
Amortization of acquired intangible asset	23,473	11,278	12,195
Research and development expense	140,078	134,513	5,565
Selling, general and administrative expense	73,271	61,095	12,176
Change in the fair value of deferred and contingent consideration	(11,700)	100	(11,800)
Interest expense, net	(23,514)	(19,159)	(4,355)
Other expense, net	(11,855)	(10,884)	(971)
Income tax expense	(4,835)	(451)	(4,384)

Net product revenues. Net product revenues were $129.8 million for the three months ended March 31, 2022, an increase of $38.6 million, or 42%, from $91.3 million for the three months ended March 31, 2021. The increase in net product revenue was primarily due to an increase in net product sales of Translarna and Emflaza. Translarna net product revenues were $79.2 million for three months ended March 31, 2022, an increase of $32.7 million, or 70%, compared to $46.5 million for the three months ended March 31, 2021. These results reflect an increase in net product sales in existing markets as well as continued geographic expansion. Emflaza net product revenues were $48.6 million for the three months ended March 31, 2022, an increase of $5.1 million, or 12%, compared to $43.5 million for the three months ended March 31, 2021. These results reflect continued addition of new patients, continued high compliance, and appropriate weight-based dosing.

Collaboration revenues. Collaboration revenues were $0.0 million for the three months ended March 31, 2022, a decrease of $20.0 million, or over 100%, from $20.0 million for the three months ended March 31, 2021. The decrease is related to no milestones triggered from Roche in the three months ended March 31, 2022, as compared to a $20.0 million milestone payment from Roche that was triggered in the three months ended March 31, 2021, relating to the first commercial sale of Evrysdi in the EU, which was made in March 2021.

Royalty revenue. Royalty revenue was $18.9 million for the three months ended March 31, 2022, an increase of $12.2 million, or over 100%, from $6.7 million for the three months ended March 31, 2021. The increase in royalty revenue was due to higher Evrysdi sales in the three months ended March 31, 2022 as compared to the three months ended March 31, 2021. 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 $10.1 million for the three months ended March 31, 2022, an increase of $1.0 million,

or 11%, from $9.1 million for the three months ended March 31, 2021. 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 and royalty revenue.

Amortization of acquired intangible asset. Amortization of our intangible assets was $23.5 million for the three months ended March 31, 2022, an increase of $12.2 million, or over 100%, from $11.3 million for the three months ended March 31, 2021. 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, including a $50 million contingent milestone payment made in the three months ended March 31, 2022, 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. Additionally, in August 2021, we made a $4.0 million milestone payment to Akcea upon regulatory approval of Waylivra from ANVISA. In accordance with the guidance for an asset acquisition, we recorded the milestone payment when it became payable to Akcea, and it increased the cost basis for the Waylivra intangible asset. This payment is being amortized to cost of product sales over the expected remaining useful life of the Waylivra asset on a straight line basis.

Research and development expense. Research and development expense was $140.1 million for the three months ended March 31, 2022, an increase of $5.6 million, or 4%, from $134.5 million for the three months ended March 31, 2021. The increase in research and development expenses is primarily related to increased investment in research programs and advancement of the clinical pipeline.

Selling, general and administrative expense. Selling, general and administrative expense was $73.3 million for the three months ended March 31, 2022, an increase of $12.2 million, or 20%, from $61.1 million for the three months ended March 31, 2021. The increase reflects our continued investment to support our commercial activities including our expanding commercial portfolio.

Change in the fair value of deferred and contingent consideration. The change in the fair value of deferred and contingent consideration was a gain of $11.7 million for the three months ended March 31, 2022, a change of $11.8 million, or over 100%, from a loss of $0.1 million for the three months ended March 31, 2021. 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 $23.5 million for the three months ended March 31, 2022, an increase of $4.4 million, or 23%, from $19.2 million for the three months ended March 31, 2021. 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.

Other expense, net. Other expense, net was $11.9 million for the three months ended March 31, 2022, an increase of $1.0 million, or 9%, from other expense, net of $10.9 million for the three months ended March 31, 2021. The change in other expense, net resulted primarily from an unrealized foreign exchange loss from the remeasurement of our intercompany loan and unrealized losses on our equity investments and convertible debt security in ClearPoint Neuro, Inc. of $1.0 million and $1.5 million, respectively.

Income tax expense. Income tax expense was $4.8 million for the three months ended March 31, 2022, an increase of $4.4 million, or over 100%, compared to income tax expense of $0.5 million for the three months ended March 31, 2021. The increase in income tax expense is primarily attributable to the capitalization and amortization of Section 174 expenditures which took effect in 2022 pursuant to TCJA amendments to IRC Section 174. We incur 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 authorizations in Brazil, Russia 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, 2022, we had cash, cash equivalents and marketable securities of $587.8 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)	**2022**	**2021**
Cash (used in) provided by:		
Operating activities	(97,404)	(100,157)
Investing activities	49,043	30,147
Financing activities	1,168	9,531

Net cash used in operating activities was $97.4 million for the three months ended March 31, 2022 and $100.2 million for the three months ended March 31, 2021. The net cash used in operating activities primarily relates to supporting clinical development and commercial activities.

Net cash provided by investing activities was $49.0 million for the three months ended March 31, 2022 and $30.1 million for the three months ended March 31, 2021. Cash provided by investing activities for the three months ended March 31, 2022 and 2021 were primarily due to net sales and redemption of marketable securities, offset by acquisition of product rights.

Net cash provided by financing activities was $1.2 million for the three months ended March 31, 2022 and $9.5 million for the three months ended March 31, 2021. Cash provided by financing activities for the three months ended March 31, 2022 and 2021 were primarily attributable to cash received from the exercise of options, partially offset by payments on our finance lease principal.

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 emvododstat 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 May 2022. We are preparing a BLA for PTC-AADC for the treatment of AADC deficiency in the United States and we expect to submit a BLA to the FDA in the third quarter of 2022. We filed for marketing authorization for Waylivra with ANVISA for the treatment of FPL and we expect a regulatory decision on approval from ANVISA in the second half of 2022. 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 Tegsedi-Waylivra Agreement;

- satisfy contractual and regulatory obligations that we assumed through our other acquisitions and collaborations;
- execute our commercialization strategy for our products and product candidates that may receive marketing authorization;
- are required to complete any additional clinical trials, non-clinical studies or Chemistry, Manufacturing and Controls, or CMC, assessments or analyses in order to advance Translarna for the treatment of nmDMD in the United States or elsewhere;
- utilize the Hopewell Facility to manufacture 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 emvododstat 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 increases 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 emvododstat 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 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 emvododstat for COVID-19 and Translarna in other territories;

- our ability to utilize the Hopewell Facility to manufacture 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. The 2022 Convertible Notes will mature on August 15, 2022 and we will be required to pay any outstanding principal amount of the 2022 Convertible Notes at that time, unless earlier converted, redeemed or repurchased in accordance with their terms prior to such date. As of 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. Upon conversion, we will pay or deliver, as the case may be, cash, shares of our common stock or any combination thereof at our election. 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.

In addition, in the first quarter of 2022, we paid Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC), or Marathon, a single $50.0 million sales-based milestone in connection with Emflaza. We expect to pay the former equityholders of Agilis an aggregate of $70.0 million upon the achievement of certain development and regulatory milestones in 2022 relating to PTC-AADC. We also expect to pay the former securityholders of Censa a $30.0 million development milestone for the completion of enrollment of a Phase 3 clinical trial for PTC923 for PKU in 2022. If achieved, we have the option to pay such milestone payment in cash or shares of our common stock.

We also have certain significant contractual obligations and commercial commitments that require funding and we have disclosed these items under the heading “Management’s Discussion and Analysis of Financial Condition and Results of Operations-Funding Obligations” in our 2021 Annual Report on Form 10-K. There were no material changes to these obligations and commitments during the period ended March 31, 2022 . 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.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

During the period ended March 31, 2022, 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 2021 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, 2022. 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, 2022, 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, 2022 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, 2021, 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.

Item 6. Exhibits.

Exhibit Number	Description of Exhibit
10.1†	Collaborative Research Agreement Amendment 11, dated as of January 31, 2022 by and between National Taiwan University and PTC Therapeutics GT, Inc.
10.2†	Commercial Manufacturing Agreement, dated as of September 18, 2015, as amended, by and between Alcami Corporation (f/k/a/ AAI Pharma Services Corp.) and Complete Pharma Holdings, LLC (f/k/a Marathon Pharmaceuticals, LLC), as assigned by Complete Pharma Holdings, LLC to the Registrant on April 20, 2017
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 3, 2022

By: /s/ Emily Hill
Emily Hill
Chief Financial Officer
(Principal Financial Officer and Duly Authorized Signatory)

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) is the type of information that the registrant treats as private or confidential. Double asterisks denote omissions.

Collaborative Research Agreement Amendment No. 11

This Amendment No. 11 is made and entered into as of the last date of signature (the "Amendment 11 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 11 supersedes the prior amendments.

WHEREAS, the COMPANY and NTU previously amended the agreement via Amendments 1 through 10. 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. **Modified Research Budget (Amendment No. 11).** In addition to the estimated budget for NTU's fees, based upon the fee schedule for the Services outlined in Attachment A, the budget will be updated to include an additional table 1(b) as follows:

   [**]

   COMPANY shall pay NTU within [**] of its receipt of the invoice. The invoice must be sent to [**] with reference to the relevant purchase order number included on the 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 11, 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: 31-Jan-2022	**Date:**

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) is the type of information that the registrant treats as private or confidential. Double asterisks denote omissions.

COMMERCIAL MANUFACTURING AGREEMENT

THIS MANUFACTURING AGREEMENT (the "Agreement") is made and entered into this 18th day of September, 2015 (the "Effective Date"), by and between **AAIPharma Services Corp.,** having a place of business at 2320 Scientific Park Drive, Wilmington, NC 28405 ("AAIPharma") and **Marathon Pharmaceuticals, LLC** having a place of business at 1033 Skokie Blvd., Suite 600, Northbrook, IL 60062 USA ("Company"). AAIPharma and Company, as used herein, may be referred to, collectively, as "Parties" and individually as a "Party".

Recitals

WHEREAS, subject to the terms and conditions contained in this Agreement, Company desires to engage the services of AAIPharma to Manufacture the Products (each as defined below) for subsequent commercial distribution by Company.

WHEREAS, AAIPharma is willing to undertake such Manufacture for Company according to the terms and conditions provided for in this Agreement.

NOW, THEREFORE, for and in consideration of the foregoing premises and of the mutual covenants of the Parties hereinafter set forth, the Parties hereto agree as follows:

ARTICLE 1
DEFINITIONS

The following words, terms and phrases, when used herein, shall have the following respective meanings:

1.1 "AAIPharma" shall have the meaning set forth in the preamble.

1.2 "AAIPharma Indemnified Parties" shall have the meaning set forth in Section 8.2.

1.3 "Act" shall mean the United States Food, Drug, and Cosmetic Act (21 U.S.C. 301 et seq.), as amended from time to time, and the regulations promulgated thereunder.

1.4 "API" shall mean the active pharmaceutical ingredient with respect to each Product.

1.5 "Applicable Law(s)" shall have the meaning set forth in Section 3.3.

1.6 "Batch" shall mean a specific quantity of material produced in a contiguous process or series of processes that is expected to be homogeneous within specified limits. The Batch size for each Product is set forth in Exhibit A attached hereto and incorporated herein by reference.

1.7 "cGMP" or **"GMP"** shall mean the recognized pharmaceutical regulations and requirements of regulatory authorities such as those defined by the U.S. FDA's regulations at 21CFR Parts 210 and 211, those defined by Eudralex, "The Rules Governing Medicinal Products in the European Union," and specifically Volume 4, "Guidelines for Good Manufacturing Practices for Medicinal Products for Human and Veterinary Use" and applicable Annexes (Directives 2001/83/EC and amendments including Directives 2003/94/EC dated October 2003 and 2004/27/EC dated March 2004 and/or others that may be appropriate for the particular project) and as may be amended from time to time.

1.8 "Commercialize" or **"Commercialization"** shall mean, with respect to a Product, the marketing, promotion, sale and distribution of such Product.

1.9 "Company" shall have the meaning set forth in the preamble.

1.10 "Company Indemnified Parties" shall have the meaning set forth in Section 8.1**.**

1.11 "Firm Commitment" shall have the meaning set forth in Section 4.1**.**

1.12 "Firm Forecast" shall have the meaning set forth in Section 4.1.

1.13 "Firm Order" shall have the meaning set forth in Section 4.2(b).

1.14 "Indemnification Claim" shall have the meaning set forth in Section 8.3(a).

1.15 "Initial Term" shall have the meaning set forth in Section 9.1**.**

1.16 "Launch" means, with respect to a Product, the first commercial product shipped from AAIPharma's site.

1.17 "Long-Term Forecast" shall have the meaning set forth in Section 4.1**.**

1.18 "Losses" shall have the meaning set forth in Section 8.1.

1.19 "Manufacture"/"Manufacturing" shall mean the manufacture, processing, packaging, labeling (subject to Section 3.7), quality control and testing of the Products performed prior to their delivery by AA1 Pharma in accordance with the terms of this Agreement.

1.20 "Marketing Authorizations" shall mean the United States new drug application or abbreviated new drug application, as applicable, for the Product(s).

1.21 "Master Batch Record" shall mean the batch record as mutually agreed upon by the Parties.

1.22 "Material Change" shall have the meaning set forth in Section 3.3.

1.23 "Minimum Order Requirement" shall have the meaning set forth in Section 4.2(a).

1.24 "Product(s)" shall mean those products described in Exhibit A, as the same may be amended from time to time upon mutual agreement of the Parties; provided, however, that no product shall become a Product until such time as AAIPharma has successfully completed the registration batches for such product to Company's reasonable satisfaction.

1.25 "Purchase Prices" shall have the meaning set forth in Section 5.1.

1.26 "Quality Agreement" shall have the meaning set forth in Section 6.5.

1.27 "Raw Materials" shall mean any excipient and component materials used to Manufacture the Products, but excluding the API.

1.28 "Raw Material Costs" shall have the meaning set forth in Section 5.2.

1.29 "Recall" shall have the meaning set forth in Section 6.4(b).

1.30 "Release To The Client" shall mean AAIPharma has: i) manufactured and/or packaged and/or labeled the Product according to the Master Batch Record; ii) fulfilled its testing/analytical obligations as further set forth herein; and iii) all manufacturing and testing services performed by AAIPharma have been reviewed and approved by AAIPharma's Quality department.

1.31 "Renewal Period" shall have the meaning set forth in Section 9.1.

1.32 "Services" shall mean certain pharmaceutical development services in addition to manufacturing services including, for example, analytical method development and analysis, stability services, clinical packaging, validation services, quality assurance and regulatory consulting provided by AAIPharma.

1.33 "Specifications" shall mean the specifications for the Products agreed upon by the Parties and included in the Master Batch Record, an example of which is set forth in Exhibit B attached hereto and incorporated herein by reference.

1.34 "Term" shall have the meaning set forth in Section 9.1.

1.35 "Territory" shall mean the United States, its territories and possessions.

ARTICLE 2
LICENSE GRANT TO AAIPHARMA TO MANUFACTURE PRODUCT

2.1 Grant. Company hereby grants to AAIPharma during the Term of this Agreement, on a Product-by-Product basis, a nonexclusive, royalty-free right to Manufacture the Products in the Territory and to use any and all of Company's licenses, trademarks, regulatory data and/or technical information, know how and Confidential Information of Company related to the Products for the purpose of AAIPharma carrying out its obligations hereunder, subject to the conditions of this Agreement.

2.2 Marketing Authorizations. Company shall maintain the Marketing Authorizations in full force and effect at all times. Upon request by Company, AAIPharma shall use commercially reasonable efforts to assist Company in connection therewith; provided that, in exchange, Company will pay AAIPharma its standard fees and expenses therefor.

ARTICLE 3
MANUFACTURING

3.1 Engagement.

(a) During the Term of this Agreement and subject to the terms and conditions set forth herein, Company agrees to exclusively purchase from AAIPharma, and AAIPharma agrees to exclusively manufacture and supply, one hundred percent (100%) of Company's requirements for each Product for Commercialization in the Territory. Notwithstanding the foregoing, Company shall be entitled, at its sole cost and expense, to qualify other manufacturer(s) to manufacture Products solely for the purpose of such manufacturer(s) supplying Company with quantities of Product that AAIPharma is unable to supply (i) in breach of this Agreement, or (ii) during events of force majeure. Following the Initial Term, AAIPharma may notify Company if such exclusivity commitment would prevent AAIPharma from providing services to a third party. The Parties shall negotiate in good faith mutually acceptable revised terms including but not limited to termination of exclusivity or compensation to AAIPharma for the lost opportunity to provide services to such third party. If the Parties fail to agree on terms within [**] days of such notification, either Party shall have the right to terminate this Agreement effective eighteen (18) months following the [**] day negotiation period.

(b) Notwithstanding the foregoing, to the extent Company intends to Commercialize a Product in a jurisdiction outside the Territory, for purposes of such Product only, the term "Territory" shall be expanded to include such jurisdiction provided that AAIPharma agrees in writing and AAIPharma is or becomes compliant with all laws, regulations and other legal and industry requirements applicable to the Manufacture of such Product for subsequent Commercialization of such Product in such jurisdiction. In the event AAIPharma is unwilling or unable to supply Product to a jurisdiction outside the Territory that Company intends to Commercialize, then Company may qualify other manufacturer(s) to manufacture Product solely for that jurisdiction outside the Territory and Company's obligation to purchase Product exclusively from AAIPharma shall be waived for that jurisdiction.

3.2 Manufacture of Commercial Drug Product. Subject to the terms and conditions contained herein, AAIPharma shall Manufacture, hold, handle and prepare for shipment all Product Manufactured pursuant to this Agreement (a) in accordance with this Agreement and the Quality Agreement, and (b) in material compliance with cGMP applicable to the Manufacturing of the Product to be Commercialized in the Territory.

3.3 AAIPharma Changes to Manufacturing Process. Except as required by applicable federal, state, provincial or local law and/or respective regulations as established by the FDA and/or other regulatory authority (collectively, "Applicable Law(s)"), or cGMP, AAIPharma shall not Materially Change the Manufacturing process of a Product or change the facility where a Product is Manufactured that requires a change to a Marketing Authorization without the prior

written consent of Company, which consent shall not be unreasonably withheld or delayed. AAIPharma shall notify Company of all material changes, including Material Changes required by Applicable Law, as soon as practicable after AAIPharma learns of such change. A "Material Change" is one that requires a submission to the FDA, EU, or other applicable regulatory authority.

3.4 Company Requested Changes. Company shall inform AAIPharma in writing of any proposed modifications to the Specifications or the Manufacturing process. Any proposed change shall require AAIPharma's prior written consent, which consent shall not be unreasonably withheld or delayed. AAIPharma shall make changes it agrees to as promptly as practicable; provided, however, that such changes comply with Applicable Law, cGMP and the Marketing Authorizations.

3.5 Costs of Changes. Unless otherwise agreed by the Parties, any and all direct costs associated with changes requested by AAIPharma and changes required by Applicable Law that apply generally to AAIPharma's facility where the applicable Manufacturing occurs shall be borne by AAIPharma; provided however, in the event Applicable Law imposes a registration fee (such as GDUFA) or similar fee on AAIPharma's cGMP facilities, and the fee relates to AAIPharma's services hereunder, the Parties shall determine in good faith an equitable portion of such fee to be paid by Company. Unless otherwise agreed by the Parties, any and all direct costs associated with all other changes, including, without limitation, changes requested by Company, changes required by Applicable Laws that apply specifically to a Product, and changes required by a change to a Marketing Authorization, shall be borne by Company (collectively, the "Other Changes"). If the change is an Other Change, (i) the Purchase Prices shall be adjusted by the change in AAIPharma's cost of Manufacture of the Product caused by such Other Change, plus an amount necessary to maintain AAIPharma's profit margin on such, and (ii) Company shall reimburse AAIPharma for costs, expenses or losses associated with write-offs, obsolescence and/or destruction of any work in process or finished inventory resulting from any such Other Change.

3.6 Notification and Approval of Changes. Company shall have sole responsibility for obtaining any and all necessary regulatory approvals from the relevant regulatory agencies in the Territory for changes to the Specifications and the Marketing Authorizations and for reporting any changes to such Specifications and the Marketing Authorizations to the relevant regulatory agencies in the Territory as appropriate. Upon request by Company, AAIPharma shall use commercially reasonable efforts to assist Company in obtaining any such approvals; provided that Company will pay AAIPharma its standard fees and expenses therefor.

3.7 Labeling. Company shall be responsible for the labeling to be used on each Product and the packaging thereof, including any changes to such labels; provided that Company shall ensure that all such labeling complies with Applicable Laws. AAIPharma shall use the specified labeling (and only such labeling) on the Products, and shall not use such labeling on any other product. Any Company-directed change to a Product label shall be implemented by AAIPharma as soon as reasonably practicable following AAIPharma's receipt of written notification of such label changes. Company shall reimburse AAIPharma for costs incurred in connection with any such label changes, including without limitation, the costs of obsolescence of goods-in-process, packaging materials and supplies and finished goods not suitable for Commercializing in the Territory due to such label changes.

3.8 Finished Product Release. AAIPharma will provide Company with manufacturing documents as are necessary for Company to release each lot of Product for human use. Company shall be responsible for the final release of Product for human use.

3.9 Raw Materials and API. AAIPharma shall purchase at its own expense and for its own account all Raw Materials, packaging components and other items of any nature whatsoever that AAIPharma may use to Manufacture the Products. Except as otherwise agreed to between the Parties, all right, title and interest in and to these items, and in and to all work-in-process incorporating these items, shall remain the sole property of AAIPharma until Products incorporating such items are delivered for shipment to Company. However, the total cost of changing the source and/or type of Raw Materials shall be at the sole cost of Company. Company shall supply to AAIPharma at its own expense and for its own account all API to be used in the Manufacture of Products hereunder, and such API shall remain the sole property of Company. AAIPharma shall have no liability for lost or damaged API unless caused by its negligence or intentional misconduct. If such losses, in an annual reconciliation, lead to actual yields below [**] percent ([**]%) of theoretical, AAIPharma shall issue a credit to Company for the lesser of (a) an amount equal to Company's then current replacement cost of the API, or (b) the amount AAIPharma would have charged Company for the amount of Product the lost quantity of API would have yielded. As such, if Company desires to insure its API, Company should do so under its own appropriate insurance policy. Company shall provide AAIPharma with documentation of its API cost at least annually on or before the anniversary of the Effective Date.

ARTICLE 4
FORECASTS, ORDERS, DELIVERY AND ACCEPTANCE

4.1 Forecasting. Company shall provide to AAIPharma a written good faith forecast estimating Company's quarterly requirements (broken out on a month-to-month basis) of each Product for each of the first [**] calendar quarters during the Term at least [**] months prior to Launch of each Product. In addition, within [**] weeks after the start of each quarter (i.e. [**]) during the Term, Company shall provide AAI Pharma with an updated rolling [**] month forecast estimating Company's requirements (broken out on a month-to-month basis) of the Product that shall cover the succeeding [**] calendar month period (or the period until the expiration of the Term, if shorter) (each such forecast, a "Long-Term Forecast"). Except as set forth in Sections 4.2(a) and 4.2(b), the Long-Term Forecast shall not be binding on either Party, but for the first [**] calendar months of a Long-Term Forecast, which shall be a "Firm Commitment" with respect to the Product, and but for the [**] calendar months of a Long-Term Forecast, which shall be a "Firm Forecast" with respect to the Product.

4.2 Firm Commitments.

(a) Each Firm Commitment shall be a binding commitment for the quantities of each Product forecast for the first [**] calendar months of the Long-Term Forecast. The quantity of each Product specified in any Firm Commitment for delivery to, and purchase by, Company in any calendar quarter shall not be less than [**] percent ([**]%) of the quantities forecasted for such quantities when it was the applicable Firm Forecast (the "Minimum Order Requirement").

(b) With respect to each Firm Commitment, Company shall submit to AAIPharma binding written purchase orders (a "Firm Order") no later than [**] days prior to the requested delivery dates confirming the quantity of each Product ordered (which shall be in full Batch quantities), the requested delivery dates, and such other information as AAIPharma may find reasonably necessary to Manufacture the ordered Products. AAIPharma will confirm the requested delivery dates within [**] business days of receipt of a Firm Order.

(c) If Company fails to order and purchase the Minimum Order Requirement, then within [**] days following the end of the quarter in which the Minimum Order Requirement was not met, Company shall pay to AAIPharma the difference between: (i) the Purchase Price for the applicable Minimum Order Requirement, and (ii) the Purchase Price that was paid by Company for the quantity ordered.

Furthermore, Company agrees that purchases may be made by AAIPharma of the Raw Materials, packaging components and other items to satisfy the production requirements for the Long-Term Forecast. In such circumstances, if such Raw Materials, packaging components and other items are not included in finished Products purchased by Company within [**] months after such purchases have been made (or such longer period as the Parties may have agreed to), Company will pay to AAIPharma its costs thereof and, in the event such Materials are incorporated into Products subsequently purchased by Company, Company will receive credit for any of such costs previously paid to AAIPharma by Company.

(d) AAIPharma shall Manufacture and prepare for shipment the quantity of a Product specified in the Firm Commitment and related purchase orders. Notwithstanding the foregoing, with respect to a Product, in no event shall AAIPharma be required in any calendar quarter to deliver more than [**] percent ([**]%) of the quantities in the applicable Firm Forecast, but AAIPharma shall use its commercially reasonable and good faith efforts to deliver quantities in excess of [**]% of the applicable Firm Forecast. The Firm Commitments shall be made available for shipment in accordance with Section 4.4.

4.3 Changes in Orders. AAIPharma shall exercise its commercially reasonable efforts to comply with any proposed amendments to accepted Firm Orders that Company may request, but AAIPharma shall not be liable in any way for its inability to do so. Firm Orders may be amended only by mutual agreement of the Parties and such amendments shall not affect the Minimum Order Requirement.

4.4 Delivery. AAIPharma shall use commercially reasonable efforts to make Product available for shipment within [**] business days of the delivery date requested in the applicable Firm Order. Company shall pay all crating, skidding, rigging, customs, freight, shipping, insurance and common carrier charges on all shipments in connection with Company's chosen method of shipment of the Product. All Product(s) shall be shipped EX WORKS (Incoterms 2010) AAIPharma's manufacturing facility. Title and risk of loss of Product shall pass to Company at the time the Products are placed on AAIPharma's loading dock at Company's disposal, not cleared for export and not loaded on any collecting vehicle. Company shall be responsible for arranging the shipment of the Product(s) from AAIPharma's manufacturing facility to its final destination (and storage charges shall be imposed [**] days after notice to Company that Product is available for shipment); provided, however, that Company must provide AAIPharma with reasonable

evidence (e.g. a copy of the current DEA registration for the destination, when applicable) that such destination is authorized to handle the Product. Notwithstanding anything to the contrary in this Agreement, Company acknowledges and agrees that AAIPharma shall have no obligation to release Product for shipment to any destination for which Company has not provided adequate evidence of authorization as required in this Section 4.4. AAIPharma shall not be liable to Company for Product which is damaged or lost while in possession of a common carrier, and it shall be Company's responsibility to recover any and all damage directly from such common carrier.

4.5 Inspection, Acceptance and Rejection of Delivered Products.

(a) Company will have [**] days from receipt by Company to inspect and test Products for noncompliance with the applicable Specifications (the "Inspection Period").

(b) Except as provided in Section 4.5(c), Company shall give written notice if it intends to reject a Batch(es) of Product(s) - for not complying with the Specifications - within [**] days after the Inspection Period expires; otherwise such Batch(es) shall be deemed accepted.

(c) If, after the Inspection Period, Company first discovers that a Batch(es) of Product(s) do not comply with the applicable Specifications, then Company shall so notify AAIPharma if it intends to reject such Batch(es) within [**] days after such discovery; otherwise such Batch(es) shall be deemed accepted. AAIPharma will only be responsible for Batch(es) of Product(s) rejected after the Inspection Period solely to the extent that AAIPharma is responsible for said non-conformity.

(d) Notwithstanding anything to the contrary herein, AAIPharma shall not be responsible for damages to Product during shipment, and in no event shall AAIPharma be responsible for noncompliance with Specifications for Product that met Specifications at time of Release To The Client or from non-conformities that result from a deficiency or change in the API utilized in such Batch(es) of Product(s) or a defect in the Specifications for the Products.

(e) In the event that Company rejects Product(s) as provided in this Agreement, AAIPharma shall use commercially reasonable efforts (but within [**] days after AAIPharma's receipt of Company's notice of noncompliance) to replace the defective Product(s) or give notice that it disagrees with the rejection. If Company and AAIPharma do not agree whether the Product(s) failed to meet applicable Specifications at the time of Release To The Client, such Products shall be submitted for testing to an independent laboratory or other authority of national reputation acceptable to both Parties for the purpose of determining the results. Any determination by such authority shall be final and binding upon the Parties hereto. If Company's rejection is substantiated by the authority, AAIPharma shall pay the expenses associated with such analyses; otherwise Company shall pay such expenses and purchase the Product.

4.6 Non-Conforming Product(s). Notwithstanding any other provisions of this Agreement, Company agrees, if so requested by AAIPharma, to return to AAIPharma any Product(s) that fail to meet Specifications or otherwise to dispose of such Product(s) as AAIPharma may direct, each at AAIPharma's expense.

ARTICLE 5
PRICE, TERMS OF PAYMENT

5.1 Purchase of Product(s). The initial prices to be paid for the Products by Company to AAIPharma shall be set forth in Exhibit A attached hereto and incorporated herein by reference (the "Purchase Prices"). The Purchase Prices are in United States dollars, and are exclusive of applicable taxes. Company shall be responsible for the payment of any and all taxes applicable to the Products and Services described herein.

5.2 Price Change; Notice. AAIPharma may increase the Purchase Prices during the Term by [**]. Upon request by Company, AAIPharma shall provide reasonable documentation that reflects the increase in cost of Raw Material Costs. AAIPharma shall provide written notification of any annual increase in the Purchase Prices prior to the January 1st effective date of the increase in Purchase Prices, or as increases in the cost of Raw Materials occur, as applicable.

5.3 Invoices. AAIPharma shall provide invoices to Company for the Product(s) upon each Release To The Client (e.g. finished bulk, finished packaged, or finished packaged and labeled), and Company shall pay each such invoice, in United States dollars, within [**] days after the date of each invoice regardless of when or whether Company has arranged for shipment of the Product(s) to its final destination. Company shall make no setoff or deduction of any kind from any payments due to AAIPharma unless Company receives written authorization from AAIPharma authorizing such setoff or deduction. Undisputed invoice balances not remitted within [**] days of the date of each invoice shall be subject to a [**] percent ([**]%) per month interest charge. Should any part of the invoice be in dispute, Company shall pay the balance of the undisputed amount according to the terms and conditions described herein while said dispute is being resolved. Should payment of undisputed amounts not be received within [**] days of invoice date, and after [**] days notice to Company, the payment shall be deemed in default and AAIPharma reserves the right to cease all work and pursue collection activities. In the event of default in payment, Company shall be responsible for all collection fees and expenses incurred by AAIPharma, including reasonable attorney's fees.

ARTICLE 6
REGULATORY MATTERS; RECORDS

6.1 Annual Review and Stability Testing. If listed in Exhibit A, AAIPharma will conduct an annual product review for the Products and upon completion of such review will forward a copy to Company. The Parties agree that AAIPharma's Manufacturing process and the Purchase Prices do not include stability testing or any other work not specifically set forth herein or in an Exhibit hereto. Stability testing services and other services shall be provided at the then current AAIPharma rates for such services.

6.2 Access to AAIPharma's Facilities by Company Representatives for Quality Audit. During regular business hours and mutually agreed upon times, Company may review the records of AAIPharma and observe the manufacturing processes relating to the Services performed and expenses incurred to assure compliance with all provisions of this Agreement. Such review must be completed in not more than [**] business days and shall be offered to Company by AAIPharma [**] and may be more pursuant to cause. Subsequent reviews during the same calendar year or such reviews that cannot be completed in [**] business days will be at Company's

sole cost and expense, at AAIPharma's then current rates. Company shall also be provided an invoice for any incidental expenses AAIPharma incurs resulting from such review. Company's rights in this Section 6.2 shall be subject to compliance with AAIPharma's reasonable measures for purposes of confidentiality, safety, and security, and will be further subject to Company's compliance with AAIPharma's premises rules that are generally applicable to all persons at AAIPharma's facilities. Should Company utilize one or more third party(ies) in exercising its rights in this paragraph, Company certifies that such party(ies) shall be subject to an obligation of confidentiality consistent with the obligations of confidentiality required of Company hereunder and such third party(ies) shall be subject to any and all conditions upon Company's rights that are set forth in this Section.

6.3 Inspections by Governmental or Regulatory Authority. AAIPharma shall be responsible for handling and responding to any FDA or other governmental body inspections or inquiries received by Company or AAIPharma regarding the Manufacturing of any Product during the Term. In cases where AAIPharma is required to provide significant Company or Product specific support to such inspections or inquiries, Company agrees to pay AAIPharma for the time required at the then current AAIPharma regulatory support rate. Each Party shall promptly notify the other regarding any such inquiries and provide the other Party copies of any pertinent correspondence from such authorities related to the Product or Services covered in this Agreement. AAIPharma shall provide to Company and any governmental body any information reasonably requested by Company and/or such governmental body concerning any governmental inspection related to any Product (with all information provided to Company being subject to the confidentiality provisions in Section 10.1 herein and with AAIPharma being able to redact any information provided to Company to remove third party confidential information that does not relate to the Products). AAIPharma agrees to notify Company of any regulatory inspection specific to one or more of the Products and shall allow Company to send a representative to the site being audited, however participation in the audit will be at the sole discretion of AAIPharma. Company agrees to fully cooperate with and assist as requested by AAIPharma in fulfilling the obligations pursuant to this Section 6.3.

6.4 Complaints, Recalls, and Insurance

(a) Complaints. Product complaints received by Company with respect to Product Manufactured by AAIPharma hereunder shall be faxed to AAIPharma within [**] business days after receipt to:

AAIPharma Services Corp.
Attention: Corporate Quality
2320 Scientific Park Drive
Wilmington, NC 28405
Facsimile No.: [**]

As more fully described in the Quality Agreement, AAIPharma shall investigate all complaints directly associated with the Manufacture of Product(s) and shall provide an update every [**] days and a report to Company regarding its investigation and any conclusions. Company shall investigate all other complaints associated with the Product(s).

(b) Recall Procedures. In the event that a recall, withdrawal or field correction of any Product (a "Recall") is initiated, whether by a statutory or regulatory authority in any jurisdiction or by Company, AAIPharma shall reimburse Company for all costs and expenses incurred in procuring or complying with the requirements of such Recall to the extent that such Recall is initiated as a result of AAIPharma's breach of this Agreement (which shall include but not be limited to AAIPharma's noncompliance or nonconformity with the Specifications, GMP, or any Applicable Laws), intentional misconduct, negligence, or defective manufacturing, processing, testing, packing, or storage of Product prior to delivery to Company, and, in addition, AAIPharma shall refund to Company an amount equal to the cost of all API supplied to AAIPharma and incorporated into the recalled Product; but not more than the cost of the Batch(es) or portion of a Batch, prorated. Company shall be responsible for all other costs and expenses associated with a Recall. AAIPharma shall reasonably cooperate with Company in connection with any Recall.

(c) Insurance. At all times while this Agreement is in effect and for [**] years thereafter, AAIPharma and Company shall each:

i maintain general liability insurance (including, without limitation, product liability insurance, liability for property damage, personal injury and contractual liability) with Products/Professional at limits not less than $[**] per occurrence/$[**] aggregate;

ii maintain Workers' Compensation as required by all applicable laws and Employer's Liability coverage with a limit of not less than $[**]; and

iii provide, within [**] days of the other Party's request, Certificates of Insurance verifying insurance limits agreed upon as well as a [**] day Notice of Cancellation or Non-Renewal.

AAIPharma and Company shall each obtain all the insurance policies described in clauses 6.4(c)(i) and (ii) from insurers having A.M. Best ratings of A-VII or higher.

Company shall, at its own cost and expense, obtain and maintain in full force and effect during the Term of this Agreement All Risk Property Insurance, including transit coverage, in an amount equal to full replacement value covering Company's property while it is at AAIPharma's facilities or in transit to or from AAIPharma's facilities. Company shall obtain a waiver from any insurance carrier with whom Company carries All Risk Property Insurance releasing its subrogation rights against AAIPharma. Company shall not seek reimbursement for any property claim, or portion thereof, that is not fully recovered from Company's All Risk Property Insurance policy.

6.5 Quality Agreement. The Parties intend to enter into a quality agreement acceptable to both Parties (the "Quality Agreement") as soon as practicable after the Effective Date. The Quality Agreement will detail the quality and regulatory obligations and responsibilities of the Parties with respect to the Products to the extent these obligations and responsibilities are not fully covered in this Agreement; provided, however, that in the event of conflict between the terms of this Agreement and the Quality Agreement, (i) the provisions of the Quality Agreement

will prevail with respect to all matters pertaining to, or governed by, GMP and (ii) in all other respects, the provisions of this Agreement will prevail.

ARTICLE 7
REPRESENTATIONS AND WARRANTIES

7.1 Representations and Warranties of AAIPharma. AAIPharma hereby represents and warrants as follows:

(a) As of Release To The Client, all Product(s) delivered to Company during the Term of this Agreement: (i) shall have been Manufactured by AAIPharma in material compliance with this Agreement, the Quality Agreement, the Marketing Authorizations and cGMP, in each case, as in effect at the time of Manufacture, (ii) assuming compliance by Company with Section 3.7, shall not be adulterated or misbranded within the meaning of the Act, and (iii) shall not have been Manufactured by AAIPharma in violation of any Applicable Law in any material respect.

(b) Upon delivery, AAIPharma shall convey good title to all Product(s) so delivered to Company.

(c) The execution, delivery and performance of this Agreement and the consummation of the transactions contemplated hereby are within AAIPharma's powers and have been duly authorized by all necessary action on the part of AAIPharma. This Agreement has been duly executed and delivered by AAIPharma and constitutes legal, valid and binding obligations of AAIPharma, enforceable against AAIPharma in accordance with its terms.

(d) The execution, delivery and performance by AAIPharma of this Agreement does not and will not (i) contravene or conflict with the organizational documents of AAIPharma Services Corp., (ii) contravene or conflict with or constitute a violation of any Applicable Laws, or (iii) breach or constitute a default under the provisions of any material contract, agreement or instrument to which it is a party or by which it is bound.

(e) AAIPharma is not debarred and has not and shall not knowingly and intentionally use in any capacity the services of any third person debarred under subsections 306(a) or (b) of the Generic Drug Enforcement Act of 1992.

EXCEPT AS SET FORTH IN THIS SECTION 7.1, AAIPHARMA MAKES NO REPRESENTATIONS OR WARRANTIES, EXPRESS OR IMPLIED, AND SPECIFICALLY DISCLAIMS ALL SUCH REPRESENTATIONS AND WARRANTIES, INCLUDING, WITHOUT LIMITATION, ANY IMPLIED WARRANTIES OF MERCHANTABILITY, INFRINGEMENT, TITLE OR FITNESS FOR A PARTICULAR PURPOSE OR USE.

7.2 Representations and Warranties of Company. Company hereby represents and warrants as follows:

(a) The execution, delivery and performance of this Agreement and the consummation of the transactions contemplated hereby are within Company's powers and have been duly authorized by all necessary action on the part of Company. This Agreement has been duly

executed and delivered by Company and constitutes legal, valid and binding obligations of Company, enforceable against Company in accordance with its terms.

(b) The execution, delivery and performance by Company of this Agreement does not and will not (i) contravene or conflict with the organizational documents of Company, (ii) contravene or conflict with or constitute a violation of any Applicable Laws, or (iii) breach or constitute a default under the provisions of any material contract, agreement or instrument to which it is a party or by which it is bound.

(c) Company shall comply in all material respects with all Applicable Laws relating to its Commercialization of the Product(s).

(d) To the extent that Company supplies any Raw Materials, or API, or other information to AAIPharma (including packaging and labeling requirements) or engages in manufacturing with respect to any of the Products (either directly or indirectly through a third party), all such Raw Materials, API or other information and formulas will comply with the Specifications and applicable laws, including GMP.

(e) Company represents that to the best of its knowledge, the manufacture or the sale of the Products does not and will not infringe any third party intellectual property rights or other rights and that it is not aware of any patents existing in the Territory in which Company markets or distributes such Products relating in any manner to the Products or any use, method, activity or application relating thereto which could adversely impact upon or prevent AAIPharma from Manufacturing the Products as contemplated by the terms hereof.

ARTICLE 8
INDEMNIFICATION

8.1 By AAIPharma. AAIPharma hereby indemnifies Company and its directors, officers, employees, Affiliates, stockholders, agents, attorneys, representatives, successors and Permitted Assigns (collectively, the "Company Indemnified Parties") against and agrees to hold each of them harmless from any and all product liability claims associated with the Products, losses, liabilities, obligations, damages, costs and expenses ("Losses") incurred by any Company Indemnified Party as a result of third party claims, actions or proceedings (collectively, "Third Party Claims") to the extent based upon, attributable to or resulting from: (a) any material misrepresentation or material breach of warranty made by AAIPharma in this Agreement, (b) any material breach of any covenant or agreement made or to be performed by AAIPharma pursuant to this Agreement, and (c) the negligence or willful misconduct by an AAIPharma Indemnified Party in connection with this Agreement; except in each case, to the extent such Losses are attributable to Company's material breach of this Agreement or arising from the negligence or willful misconduct of Company.

8.2 By Company. Company hereby indemnifies AAIPharma and its directors, officers, employees, Affiliates, stockholders, agents, attorneys, representatives, successors and assigns (collectively, the "AAIPharma Indemnified Parties") against and agrees to hold each of them harmless from any and all Third Party Claims, including Losses incurred by any AAIPharma Indemnified Party to the extent based upon, attributable to or resulting from the performance of this Agreement and Services hereunder by AAIPharma (including, without limitation, any

products liability claims related to Company products) other than for Losses for which AAIPharma is obligated to indemnify the Company Indemnified Parties under Section 8.1 above.

8.3 Indemnification Procedures.

(a) The indemnified Party shall give the indemnifying Party prompt notice of any such claim or lawsuit ("Indemnification Claim") (including a copy thereof) served upon it and shall fully cooperate with the indemnifying Party and its legal representatives in the investigation of any matter the subject of indemnification. The indemnifying Party may enter into a settlement agreement with a claimant but shall not admit liability to a claimant without the prior written permission of the party or parties seeking indemnification, which permission shall not be unreasonably withheld.

(b) The failure of the indemnified Party to give reasonably prompt notice of any Indemnification Claim shall not release, waive or otherwise affect the indemnifying Party's obligations with respect thereto except to the extent that the indemnifying Party can demonstrate actual loss and prejudice as a result of such failure.

8.4 Limitation on Liability. Except as set forth in Section 8.6 (Exceptions), neither Party shall be liable, whether in contract, tort (including negligence) or otherwise, for any punitive, special, indirect, incidental, consequential or exemplary damages (including lost profit or business interruption even if notified in advance of such possibility) arising out of or pertaining to the subject matter of this Agreement.

8.5 Aggregate Cap. Except as set forth in Section 8.6 (Exceptions), the total aggregate liability of either Party to the other Party arising out of this Agreement shall be limited to the total amounts paid and payable by Company to AAIPharma under this Agreement during the twelve (12) months preceding the event in question. Such liability cap amount does not alter each Party's insurance obligations under Section 6.4(c) (Insurance).

8.6 Exceptions. Sections 8.4 (Limitation on Liability) and 8.5 (Aggregate Cap) shall not apply to the following: (a) a Party's obligations to indemnify the other for Claims under Sections 8.1 and 8.2 (Indemnification); or (b) damages due to a Party's breach of its confidentiality obligations or claims for infringement of proprietary rights.

ARTICLE 9
TERM AND TERMINATION

9.1 Term of the Agreement. Unless earlier terminated in accordance with this Article 9, this Agreement shall take effect and commence on the Effective Date and continue in effect, on a Product-by-Product basis, for five (5) years following Launch of a particular Product (the "Initial Term"). In addition, after the expiration of the Initial Term with respect to a particular Product, this Agreement will automatically renew with respect to such Product for consecutive two (2) year terms (each, a "Renewal Period") unless either of the Parties terminates this Agreement with respect to such Product at the end of the applicable Initial Term or any applicable Renewal Period by providing the other Party with written notice, in the case of Company, at least twelve (12) months, and in the case of AAIPharma, at least eighteen (18) months, prior to the end of the

applicable Initial Term or applicable Renewal Period. The Initial Term and all Renewal Periods for each Product shall be collectively referred to herein as the "Term" for such Product.

9.2 Termination. Notwithstanding Section 9.1 herein, this Agreement may be terminated as follows:

(a) immediately upon the delivery of written notice by one Party, if the other Party materially breaches any of the provisions of this Agreement and such breach is not cured within [**] calendar days after receipt of written notice identifying such breach (or if cure has been commenced during such period, if it is not diligently prosecuted to completion); or

(b) immediately upon the delivery of written notice by one Party, if the other Party has been unable to perform its obligations hereunder for one hundred twenty (120) calendar days by reason of force majeure (as defined in Section 12.11).

(c) either Party at its sole option may immediately terminate this Agreement upon written notice, but without prior advance notice, to the other Party in the event that (i) the other Party is declared insolvent or bankrupt by a court of competent jurisdiction; (ii) a voluntary petition of bankruptcy is filed in any court of competent jurisdiction by such other Party; (iii) ceases or threatens to cease to carry on business, or (iv) this Agreement is assigned by such other Party for the benefit of creditors.

(d) Company may terminate this Agreement as to any Product upon forty-five (45) days' written notice in the event that any governmental agency takes any action, or raises any objection, that prevents Company from importing, exporting, purchasing or selling such Product.

(e) Company may at any time unilaterally terminate this Agreement only with respect to an individual Product if: (i) such individual Product is withdrawn from the market; (ii) Company divests, out-licenses or otherwise disposes of such individual Product to a party other than an Affiliate of Company; provided, however, for greater certainty, that this Subsection 9.2(e) shall not entitle Company to terminate this Agreement in whole or in part in connection with a sale or other disposition of all or substantially of its interest in the Products as a whole or any significant portion thereof; or (iii) such individual Product is found to infringe a third party's Intellectual Property.

Company shall provide to AAIPharma not less than twelve (12) months' advance written notice of such partial termination of this Agreement except where it results from either a market withdrawal at the mandate of a competent authority having jurisdiction or an infringement as in Subsection 9.2(e)(iii) above, in which cases the termination can be effective immediately; provided, however, in respect of Subsection 9.2(e)(ii), Company may provide less than the twelve months advance notice if the acquiring party agrees in writing to purchase the particular individual Product from AAIPharma for the balance of the notice period on the same terms and conditions as contained herein.

Any termination pursuant to this Section 9.2 may be effected with respect to this entire Agreement or with respect to any individual Product or Products, at the discretion of the terminating Party, and shall be effected by delivering written notice of such termination to the

other Party and shall be effective upon the date of such written notice unless a later date is specified in such written notice.

9.3 Effect of Termination. Upon termination or expiration of this Agreement, in its entirety or with respect to any particular Product(s):

(a) Cessation of Activities. Except as provided in Section 9.3(c), AAIPharma shall stop the Manufacturing of Products; each Party shall return to the other any Confidential Information of such other Party concerning the Product(s) subject to such termination or expiration.

(b) Payment of Minimum Order Requirement; Company to Take Product. In the event of termination by AAIPharma pursuant to Section 9.2(a), (b), or (c) above, Company shall pay AAIPharma any balance remaining of the Minimum Order Requirement in the same manner as set forth in Section 4.2(c) in the case of a failure to order and purchase the Minimum Order Requirement in any calendar quarter. Company shall, at its option and with respect to any Products that are subject to termination, be permitted to take delivery for any Raw Materials, work-in-process (at AAIPharma's material costs) or finished Product (at prices then in effect under this Agreement).

(c) Firm Orders. If this Agreement is terminated by Company pursuant to Section 9.2(a), at Company's option, Firm Orders with respect to the Product(s) not yet started shall be cancelled, or, if requested by Company in writing, AAIPharma will, with respect to the Product(s) subject to such termination, complete or cause the completion of the Manufacturing of any work-in-process that is subject to a valid and effective Firm Order on the date on which the termination is effective. Once such work-in-process is completed, the resulting Product(s) shall be shipped in accordance with Company's Firm Orders and paid for by Company in accordance with Section 5.3.

9.4 Survival. The Parties agree that the following provisions shall survive the termination of this Agreement; the definitions of Article 1 to the extent such Definitions pertain to terms in surviving provisions, Sections 4.5, 4.6, 6.4, and Articles 5, 7, 8, 9, 10, 11 and 12.

ARTICLE 10
CONFIDENTIALITY AND PUBLIC DISCLOSURE

10.1 AAIPharma will hold in strict confidence, and shall not disclose to any third party without Company's prior written consent, all proprietary or confidential information concerning Product, API and all materials and information provided by Company (collectively, "Company Information"). AAIPharma further agrees that it shall not use Company Information for any purpose other than the Manufacturing of Products for Company under this Agreement.

10.2 Company will hold in strict confidence, and shall not disclose to any third party without AAIPharma's prior written consent, all proprietary or confidential information and materials belonging to AAIPharma ("AAIPharma Information").

10.3 "Confidential Information" shall mean Company Information and AAIPharma Information. Each Party may disclose Confidential Information only to its directors, officers and

employees who have need to know Confidential Information for the purposes of this Agreement, and each Party will be responsible for ensuring that all its directors, officers, and employees to whom Confidential Information is disclosed will also observe such obligations of confidentiality and non-use as provided herein.

10.4 The above confidentiality obligation shall not apply or shall cease to apply to any information which the receiving party can demonstrate by documentary proof:

(a) is already in the possession of the receiving party at the time it is disclosed by the disclosing party;

(b) is in the public domain at the time it is disclosed by the disclosing party;

(c) enters the public domain through sources independent of the receiving party and through no fault of the receiving party;

(d) is lawfully obtained by the receiving party without any confidentiality restrictions from a third party who has a right to disclose such information to the receiving party;

(e) has been at any time developed by the receiving party independently of disclosure from the disclosing party.

10.5 Neither Party (nor any of their respective Affiliates) shall issue any press release or make any public announcement with respect to this Agreement and the transactions contemplated hereby without obtaining the prior written consent of the other Party (such consent not to be unreasonably withheld or delayed), except as may be required by Applicable Law upon the advice of counsel and only if the disclosing Party provides the non-disclosing Party with a reasonable opportunity to first review the release or other public announcement, to the extent practicable.

10.6 These confidentiality obligations shall survive termination or expiration of this Agreement for a period of [**] years.

ARTICLE 11
INTELLECTUAL PROPERTY

11.1 AAIPharma further agrees that all Company Information, know-how, data, discoveries and inventions relating to Product and API which result from the Manufacture of Products shall constitute the sole and exclusive property of Company. AAIPharma hereby assigns to Company all right, title and interest throughout the world in and to all inventions (whether or not patentable), processes, techniques, improvements, discoveries and developments discovered and reduced to practice by AAIPharma (collectively, “Project IP”) in the course of providing Services which are directly and solely related to the Manufacture of Product hereunder. AAIPharma will, at the expense and the written request of Company, do all reasonable acts and measures and execute all documents as Company may reasonably request to transfer to and vest in Company the ownership and registration of all intellectual property rights that may exist in such Project IP.

11.2 Company acknowledges that AAIPharma possesses certain inventions, processes, techniques, improvements, know-how, trade secrets, discoveries and other intellectual property and other proprietary assets, including drug delivery technologies (hereinafter, "AAIPharma Proprietary Technology") which have been independently developed by AAIPharma. In the event Company chooses to further develop and/or commercialize a technology comprising, in whole or in part, AAIPharma Proprietary Technology, Company will obtain a license from AAIPharma to use such AAIPharma Proprietary Technology. Such license agreement shall be memorialized in a separate agreement to be negotiated in good faith by the Parties.

11.3 Company acknowledges that AAIPharma is in the business of providing services for a variety of organizations other than Company. Accordingly, nothing in this Agreement, with the exception of the exclusivity obligations set forth in Article 3.1 herein, shall preclude or limit AAIPharma from providing services or developing materials for itself or other clients, or from utilizing the general knowledge gained during the course of its performance hereunder to perform similar services for other clients, provided that such provision of services or development of materials do not constitute a breach of confidentiality under Article 10 or the exclusivity obligations set forth in Article 3.1 herein.

ARTICLE 12
MISCELLANEOUS

12.1 Successors and Assigns. Neither Party may assign its rights or obligations under this Agreement without the prior written consent of the other Party; provided, however, that either Party may assign this Agreement, in whole or in part, without such consent, to an Affiliate of such Party or to a Third Party that acquires substantially all of the assets of a Party to which this Agreement relates, upon written notice to the other Party of any such assignment and such Party hereby guarantees the performance of any such Affiliate, and, in the case of a Third Party assignment, such Third Party shall assume the obligations of the assigning Party under this Agreement. No assignment shall relieve any Party of responsibility for the performance of any obligation, which such Party may have or incur hereunder. This Agreement shall be binding upon and inure to the benefit of each of the Parties and each such Party's successors and permitted assigns.

12.2 Notices. Any notice required or permitted under this Agreement shall be in writing, shall specifically refer to this Agreement, and shall be sent by hand, recognized overnight courier, confirmed facsimile transmission, or registered or certified mail service, postage prepaid, return receipt requested, to the following addresses or facsimile numbers of the Parties:

Company:

Marathon Pharmaceuticals, LLC
1033 Skokie Blvd, Suite 600
Skokie, IL 60062 USA
Attn: General Counsel
Fax: [**]

AAIPharma:

AAIPharma Services Corp.
2320 Scientific Park Drive
Wilmington, NC 28405
Attn: Legal Department
Fax: [**]

All notices under this Agreement shall be deemed received (i) upon receipt when sent by hand, (ii) two (2) business days after deposit with a recognized overnight courier, (iii) upon confirmation of delivery when sent by facsimile, and (iv) five (5) business days after deposit in registered or certified mail service. A Party may change its contact information immediately upon written notice to the other Party in the manner provided in this Section.

12.3 Waiver. No delay on the part of AAIPharma or Company in exercising any right, power or privilege hereunder shall operate as a waiver thereof, nor shall any waiver on the part of either Party of any right, power or privilege hereunder operate as a waiver of any other right, power or privilege hereunder, nor shall any single or partial exercise of any right, power or privilege hereunder preclude any other or further exercise thereof or the exercise of any other right, power or privilege hereunder. Any provision of this Agreement may be waived if, and only if, such waiver is in writing and signed by the Party against whom the waiver is to be effective.

12.4 Entire Agreement. This Agreement and the Quality Agreement constitute the entire agreement between the Parties with respect to the subject matter hereof and supersede all prior agreements, understanding and negotiations, both written and oral, between the Parties with respect to the subject matter of this Agreement.

12.5 Amendment. This Agreement may be modified or amended only by written agreement of the Parties hereto.

12.6 Counterparts. This Agreement may be executed by facsimile and in any number of counterparts, each of which shall be deemed an original but all of which together shall constitute a single instrument. This Agreement may be executed on signature pages exchanged by facsimile, in which event each Party shall promptly deliver to the others such number of original executed copies as the others may reasonably request.

12.7 Governing Law; Jurisdiction. This Agreement shall be governed and construed in accordance with the laws of the State of Delaware excluding any choice of law rules which may direct the application of the law of another state.

12.8 Severability. If any provision of this Agreement is held to be illegal, invalid or unenforceable under any present or future law, and if the rights or obligations of any Party hereto under this Agreement will not be materially and adversely affected thereby, (a) such provision will be fully severable, (b) this Agreement will be construed and enforced as if such illegal, invalid or unenforceable provision had never comprised a part hereof, (c) the remaining provisions of this Agreement will remain in full force and effect and will not be affected by the illegal, invalid or unenforceable provision or by its severance herefrom, and (d) in lieu of such illegal, invalid or unenforceable provision, there will be added automatically as a part of this Agreement a legal,

valid and enforceable provision as similar to the terms to such illegal, invalid or unenforceable provision as may be possible and reasonably acceptable to the Parties herein.

12.9 No Third Party Rights. Except as otherwise expressly set forth herein, no provision of this Agreement shall be deemed or construed in any way to result in the creation of any rights or obligations in any person not a Party to this Agreement.

12.10 Exhibits. The Exhibits referenced in this Agreement are an integral part of this Agreement and are incorporated herein by reference.

12.11 Force Majeure. If either Party is prevented from complying, either totally or in part, with any of the terms or provisions set forth herein by reason of force majeure, including, by way of example and not of limitation, fire, flood, explosion, storm, hurricane, strike, lockout or other labor dispute, riot, war, rebellion, accidents, acts of God, or acts of governmental agencies or instrumentalities, in each case to the extent beyond its control despite its commercially reasonable efforts to avoid, minimize, and resolve such cause as promptly as possible, said Party shall (a) provide written notice of same to the other Party, and (b) subject to the obligations set forth above with respect to said Party's efforts to remove the disability, its obligations that are prevented from compliance by such force majeure are suspended, without liability, during such period of force majeure. Said notice shall be provided within ten (10) business days of the occurrence of such event and shall identify the requirements of this Agreement or such of its obligations as may be affected. The Party so affected shall give to the other Party a good faith estimate of the continuing effect of the force majeure condition and the duration of the affected Party's nonperformance.

12.12 No Other Relationship. It is expressly agreed that AAIPharma, on the one hand, and Company, on the other hand, shall be independent contractors and that nothing contained herein shall be deemed to create any joint venture or partnership between the Parties hereto, and, except as is expressly set forth herein, neither Party shall have any right by virtue of this Agreement to bind the other Party in any manner whatsoever.

12.13 Additional Product. The Parties covenant and agree that additional products may be added to this Agreement and such additional products shall be governed by the general conditions hereof with any special terms (including, without limitation, price) governed by an addendum hereto.

12.14 Dispute Resolution.

(a) Negotiated Settlement. In the event of a dispute regarding payment or the performance of Services pursuant to this Agreement (each, a "Dispute"), the Parties shall endeavor to negotiate in good faith an agreeable solution. If after [**] business days following receipt of a Party's written notification of a Dispute such Dispute has not been resolved, the Dispute shall be brought to the attention of the senior management of each Party and such senior manager or his/her designee will negotiate in good faith to define and implement a final resolution. The intent of this Section 12.14 is to encourage the Parties to work together to resolve any Dispute without having to rely on arbitration or any other legal proceeding. However, nothing in this Section 12.14 shall prevent or inhibit either Party to institute any other action to resolve such Dispute(s).

(b) Binding Arbitration. If not resolved in accordance with the preceding paragraph (a) then any controversy or claim arising out of or relating to this Agreement, or the breach thereof, shall be settled by arbitration administered by the American Arbitration Association in accordance with its Commercial Arbitration Rules, and judgment on the award rendered by the arbitrator(s) may be entered in any court having jurisdiction thereof.

[SIGNATURE PAGE FOLLOWS]

IN WITNESS WHEREOF, the Parties hereto have executed this Agreement as of the date first above written.

Marathon Pharmaceuticals, LLC

By: /s/ Patrick J. Morris

Printed Name: Patrick J. Morris

Title: EVP of Legal Affairs and General Counsel

Date: 10/9/15

AAIPharma Services Corp.

By: /s/ Syed T. Husain

Printed Name: Syed T. Husain

Title: Chief Commercial Officer

Date: 10/7/15

Exhibit A

Product(s), Batch Sizes, and Cost

Deflazacort tablets in the following strengths to be marketed in the Territory.

Dosage Strength	Dosage Form	Lot Size	Bottle Count	Price/ Bottle
6-mg	**Tablet**	**[**]**	**100**	**$[**]**
18-mg	**Tablet**	**[**]**	**30**	**$[**]**
30-mg	**Tablet**	**[**]**	**30**	**$[**]**
36-mg	**Tablet**	**[**]**	**30**	**$[**]**

Amendment #1

THIS AMENDMENT #1 ("Amendment") is entered into as of September 18, 2016 (the "Amendment Effective Date") by and between Alcami Corporation, formerly known as AAIPharma Services Corp. ("Alcami") and Marathon Pharmaceuticals, LLC ("Company").

WHEREAS, Company and Alcami entered into a Commercial Manufacturing Agreement with an effective date of September 18, 2015 (the "Agreement");

WHEREAS, the Parties have requested modifications to Exhibits A and B of the Agreement; and

WHEREAS, the Parties wishes to implement the requested modifications upon the terms and conditions set forth herein.

NOW, THEREFORE, for good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged by each party hereto to the other, both parties mutually agree as follows:

1. Capitalized terms not otherwise defined herein will have the meaning given to them in the Agreement.

2. Where found in the Agreement, references to AAIPharma Services Corp. and AAIPharma shall be deleted and replaced with Alcami Corporation and Alcami, respectively.

3. Exhibit A, Product(s), Batch Sizes, and Cost, shall be deleted in its entirety and replaced with the attached Exhibit A1.

4. The specifications in Exhibit B shall be deleted in their entirety and replaced with the attached Exhibit B1.

5. [**].

6. Notwithstanding, the Parties expressly agree that purchase orders #2348, #2349, #2350, #2351, #2365, and #2366 submitted by Company to Alcami prior to the Amendment Effective Date, shall be prepared and shipped according to the terms outlined in the previously agreed to Exhibit A and Exhibit B respectively.

7. [**].

Except as otherwise modified herein, the Agreement will remain in full force and effect.

ACKNOWLEDGED, ACCEPTED, AND AGREED TO:

Alcami Corporation	**Marathon Pharmaceuticals, LLC**
By: /s/ Syed T. Husain	By: /s/ Patrick J. Morris
Name: Syed T. Husain	Name: Patrick J. Morris
Title: Chief Commercial Officer	Title: EVP of Legal Affairs and General Counsel
Date: 11/30/2016	Date: 11/11/16

Exhibit A1

Product(s), Batch Sizes, and Cost

Deflazacort tablets in the following strengths to be marketed in the Territory:

Dosage Strength	**Dosage Form**	**Lot Size (Bottles)**	**Bottle Count**	**Price/Bottle (USD)**
3 mg	Tablet	[**]	100	$[**]
6 mg	Tablet	[**]	100	$[**]
18 mg	Tablet	[**]	30	$[**]
30 mg	Tablet	[**]	30	$[**]
36 mg	Tablet	[**]	30	$[**]

Secondary Packaging of Pre-Filled Deflazacort Oral Suspension Bottles
(13ml fill, 22.75 mg/ml)

Dosage Strength	**Dosage Form**	**Lot Size (Bottles)**	**Fill Volume**	**Price/Bottle (USD)**
22.75 mg/mL	Oral Suspension	[**]	13mL	$[**]

Amendment #2

THIS AMENDMENT #2 ("Amendment") is entered into as of January 6, 2017 (the "Amendment Effective Date") by and between Alcami Corporation, formerly known as AAIPharma Services Corp. ("Alcami") and Marathon Pharmaceuticals, LLC ("Company").

WHEREAS, Company and Alcami entered into a Commercial Manufacturing Agreement with an effective date of September 18, 2015 and as amended September 18, 2016 (the "Agreement");

WHEREAS, the Parties have requested modifications to the Agreement; and

WHEREAS, the Parties wish to implement the requested modifications upon the terms and conditions set forth herein.

NOW, THEREFORE, for good and valuable consideration, the receipt and sufficiency of which is herby acknowledged by each party hereto to the other, both parties mutually agree as follows:

1. Capitalized terms not otherwise defined herein will have the meaning given to them in the Agreement.

2. Section 1.35, Territory, shall be deleted and replaced with the following:

   "**1.35 "Territory"** shall mean the United States, its territories and possessions[**]."

Except as otherwise modified herein, the Agreement will remain in full force and effect.

IN WITNESS WHEREOF, the Parties hereto have executed this Amendment as of the Amendment Effective Date.

Alcami Corporation	**Marathon Pharmaceuticals, LLC**
By: /s/ Syed T. Husain	By: /s/ Patrick J. Morris
Name: Syed T. Husain	Name: Patrick J. Morris
Title: Chief Commercial Officer	Title: EVP of Legal Affairs and General Counsel
Date: 1/11/2017	Date: 1/6/17

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 3, 2022

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 3, 2022

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, 2022 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 3, 2022

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, 2022 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 her 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 3, 2022

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