Table of Contents

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, 2024

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.)
500 Warren Corporate Center Drive **Warren, NJ**	**07059**
(Address of principal executive offices)	(Zip Code)

(908) 222-7000

(Registrant's telephone number, including area code)

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

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

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

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☑ No ☐

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

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

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

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

As of April 23, 2024, there were 76,696,785 shares of Common Stock, $0.001 par value per share, outstanding.

TABLE OF CONTENTS
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 ability to negotiate, secure and maintain adequate pricing, coverage and reimbursement terms and processes on a timely basis, or at all, with third-party payors for our products or product candidates that we commercialize or may commercialize in the future;
- our expectations with respect to the European Commission's potential adoption of the Committee for Medicinal Products for Human Use's negative opinion for the renewal of the conditional marketing authorization for Translarna™ (ataluren) for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, in the European Economic Area, or EEA, following a re-examination procedure and our ability to maintain such conditional marketing authorization or identify other potential mechanisms in which we may provide Translarna to nmDMD patients in the EEA;
- Our ability to maintain our marketing authorizations in other jurisdictions in which Translarna has been approved;
- our ability to utilize results from Study 041 and from our international drug registry study to support a marketing approval for Translarna for the treatment of nmDMD in the United States;
- expectations with respect to our ability to commercialize Upstaza™ (eladocagene exuparvovec) for the treatment of Aromatic L-Amino Acid Decarboxylase, or AADC deficiency, in the EEA, any potential regulatory submissions and potential approvals for our product candidates, 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 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. and the Spinal Muscular Atrophy 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 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 ability to obtain additional and maintain existing reimbursed named patient and cohort early access programs for our products on adequate terms, or at all;

- 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 for sepiapterin and our splicing and ferroptosis and inflammation programs as well as studies in our products for maintaining authorizations, label extensions and additional indications, including the timing of initiation, enrollment and completion of the trials and the period during which the results of the trials will become available;
- our ability to realize the anticipated benefits of our acquisitions or other strategic transactions, including the possibility that the expected impact of benefits from the acquisitions or strategic transactions will not be realized or will not be realized within the expected time period, significant transaction costs, the integration of operations and employees into our business, our ability to obtain marketing approval of our product candidates we acquired from the acquisitions or other strategic transactions and unknown liabilities;
- the rate and degree of market acceptance and clinical utility of any of our products or product candidates;
- the ability and willingness of patients and healthcare professionals to access our 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;
- the potential receipt of revenues from future sales of our products or product candidates;
- the expected impact of our loss of market exclusivity for Emflaza® (deflazacort) for the treatment of Duchenne muscular dystrophy in the United States under the Orphan Drug Act of 1983;
- 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;
- the extent, timing and financial aspects of our strategic pipeline prioritization and reductions in workforce;
- our ability to complete any post-marketing requirements imposed by regulatory agencies with respect to our products;
- 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 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 and ferroptosis and inflammation 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, 2023, 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, 2023 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, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 548,355	$ 594,001
Marketable securities	336,458	282,738
Trade and royalty receivables, net	197,660	160,822
Inventory, net	30,058	30,577
Prepaid expenses and other current assets	56,207	150,491
Total current assets	1,168,738	1,218,629
Fixed assets, net	92,779	87,089
Intangible assets, net	330,040	379,497
Goodwill	82,341	82,341
Operating lease ROU assets	91,186	91,896
Deposits and other assets	24,545	36,246
Total assets	$ 1,789,629	$ 1,895,698
Liabilities and stockholders' deficit		
Current liabilities:		
Accounts payable and accrued expenses	$ 330,924	$ 391,983
Deferred revenue	—	801
Operating lease liabilities- current	14,572	13,002
Finance lease liabilities- current	1,948	3,000
Liability for sale of future royalties- current	227,102	194,314
Total current liabilities	574,546	603,100
Long-term debt	284,512	284,213
Contingent consideration payable	36,200	36,300
Deferred tax liability	55,904	55,905
Operating lease liabilities- noncurrent	104,837	97,627
Finance lease liabilities- noncurrent	15,574	17,184
Liability for sale of future royalties- noncurrent	1,611,831	1,619,783
Other long-term liabilities	141	141
Total liabilities	2,683,545	2,714,253
Stockholders' deficit:		
Common stock, $0.001 par value. Authorized 250,000,000 shares; issued and outstanding 76,653,960 shares at March 31, 2024. Authorized 250,000,000 shares; issued and outstanding 75,708,889 shares at December 31, 2023.	76	75
Additional paid-in capital	2,486,722	2,466,233
Accumulated other comprehensive loss	(5,560)	(1,285)
Accumulated deficit	(3,375,154)	(3,283,578)
Total stockholders' deficit	(893,916)	(818,555)
Total liabilities and stockholders' deficit	$ 1,789,629	$ 1,895,698

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,	
	2024	2023
Revenues:		
Net product revenue	$ 177,604	$ 187,557
Collaboration revenue	—	6
Royalty revenue	31,154	30,831
Manufacturing revenue	1,360	1,988
Total revenues	210,118	220,382
Operating expenses:		
Cost of product sales, excluding amortization of acquired intangible assets	14,740	14,144
Amortization of acquired intangible assets	51,530	39,415
Research and development	116,129	195,124
Selling, general and administrative	73,272	86,914
Change in the fair value of contingent consideration	(100)	2,400
Total operating expenses	255,571	337,997
Loss from operations	(45,453)	(117,615)
Interest expense, net	(40,834)	(27,331)
Other income, net	1,591	9,956
Loss before income tax expense	(84,696)	(134,990)
Income tax expense	(6,880)	(3,969)
Net loss attributable to common stockholders	$ (91,576)	$ (138,959)
Weighted-average shares outstanding:		
Basic and diluted (in shares)	76,496,127	73,729,284
Net loss per share—basic and diluted (in dollars per share)	$ (1.20)	$ (1.88)

See accompanying unaudited notes.

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

	Three Months Ended March 31,	
	2024	2023
Net loss	$ (91,576)	$ (138,959)
Other comprehensive (loss) income:		
Unrealized (loss) gain on marketable securities, net of tax	(444)	54
Foreign currency translation loss, net of tax	(3,831)	(6,437)
Comprehensive loss	$ (95,851)	$ (145,342)

See accompanying unaudited notes.

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

Three months ended March 31, 2024	Common stock		Additional paid-in capital	Accumulated other comprehensive loss	Accumulated deficit	Total stockholders' deficit
	Shares	Amount				
Balance, December 31, 2023	75,708,889	$ 75	$ 2,466,233	$ (1,285)	$ (3,283,578)	$ (818,555)
Exercise of options	109,892	—	2,030	—	—	2,030
Restricted stock vesting and issuance, net	835,179	1	—	—	—	1
Share-based compensation expense	—	—	18,378	—	—	18,378
Receivable from investor	—	—	81	—	—	81
Net loss	—	—	—	—	(91,576)	(91,576)
Comprehensive loss	—	—	—	(4,275)	—	(4,275)
Balance, March 31, 2024	76,653,960	$ 76	$ 2,486,722	$ (5,560)	$ (3,375,154)	$ (893,916)

Three months ended March 31, 2023	Common stock		Additional paid-in capital	Accumulated other comprehensive income (loss)	Accumulated deficit	Total stockholders' deficit
	Shares	Amount				
Balance, December 31, 2022	73,104,692	$ 72	$ 2,305,020	$ 4,796	$ (2,656,974)	$ (347,086)
Exercise of options	211,561	—	5,655	—	—	5,655
Restricted stock vesting and issuance, net	695,781	1	—	—	—	1
Share-based compensation expense	—	—	28,815	—	—	28,815
Receivable from investor	—	—	396	—	—	396
Net loss	—	—	—	—	(138,959)	(138,959)
Comprehensive loss	—	—	—	(6,383)	—	(6,383)
Balance, March 31, 2023	74,012,034	$ 73	$ 2,339,886	$ (1,587)	$ (2,795,933)	$ (457,561)

See accompanying unaudited notes.

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

	Three Months Ended March 31,	
	2024	2023
Cash flows from operating activities		
Net loss	$ (91,576)	$ (138,959)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	55,395	42,825
Non-cash operating lease expense	1,792	2,942
Non-cash royalty revenue related to sale of future royalties	(25,228)	(13,237)
Non-cash interest expense on liability related to sale of future royalties	50,064	18,902
Change in valuation of contingent consideration	(100)	2,400
Unrealized (gain) loss on ClearPoint Equity Investments	(9)	39
Unrealized gain on ClearPoint convertible debt security	(166)	(59)
Unrealized gain on marketable securities- equity investments	(710)	(2,166)
Disposal of asset	544	—
Deferred income taxes	(1)	(4)
Amortization of discounts on investments, net	(3,697)	(85)
Amortization of debt issuance costs	296	438
Share-based compensation expense	18,378	28,815
Unrealized foreign currency transaction losses (gains), net	269	(10,057)
Changes in operating assets and liabilities:		
Inventory, net	120	(4,612)
Prepaid expenses and other current assets	93,904	57,611
Trade and royalty receivables, net	(39,500)	(43,426)
Deposits and other assets	11,192	743
Accounts payable and accrued expenses	(5,389)	33,524
Other liabilities	5,984	(3,988)
Deferred revenue	(801)	(1,137)
Net cash provided by (used in) operating activities	$ 70,761	$ (29,491)
Cash flows from investing activities		
Purchases of fixed assets	$ (9,588)	$ (10,270)
Purchases of marketable securities- available for sale	(104,373)	—
Purchases of marketable securities- equity investments	(9,065)	—
Sale and redemption of marketable securities- available for sale	61,650	12,500
Sale and redemption of marketable securities- equity investments	1,207	2,196
Acquisition of product rights and licenses	(54,763)	(33,397)
Net cash used in investing activities	$ (114,932)	$ (28,971)
Cash flows from financing activities		
Proceeds from exercise of options	$ 2,030	$ 5,655
Debt issuance costs related to secured loan	—	(182)
Payment of finance lease principal	(1,490)	(1,379)
Net cash provided by financing activities	$ 540	$ 4,094
Effect of exchange rate changes on cash	(2,030)	(7,963)
Net decrease in cash and cash equivalents	(45,661)	(62,331)
Cash and cash equivalents, and restricted cash beginning of period	610,284	295,925
Cash and cash equivalents, and restricted cash end of period	$ 564,623	$ 233,594
Supplemental disclosure of cash information		
Cash paid for interest	$ 3,666	$ 12,956
Cash paid for income taxes	2,042	2,215
Supplemental disclosure of non-cash investing and financing activity		
Unrealized (loss) gain on marketable securities, net of tax	$ (444)	$ 54
Right-of-use assets obtained in exchange for operating lease obligations	1,723	—
Acquisition of product rights and licenses	2,296	19,406
Milestone payable	2,500	32,500
Debt issuance costs related to senior secured term loan	—	45
Capital expenditures unpaid at the end of the period	—	28

See accompanying unaudited notes.

PTC Therapeutics, Inc.

Notes to Consolidated Financial Statements (unaudited)
March 31, 2024
In thousands (except share and per share amounts unless otherwise noted)

1. The Company

PTC Therapeutics, Inc. (the "Company" or "PTC") is a 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 little to 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 diversified therapeutic 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 relating to neurology and metabolism.

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. Translarna also has marketing authorization in Russia for the treatment of nmDMD in patients aged two years and older and in Brazil for the treatment of nmDMD in ambulatory patients two years and older and for continued treatment of patients that become non-ambulatory, as well as in various other countries. Emflaza is approved in the United States for the treatment of DMD in patients two years and older.

The Company's marketing authorization for Translarna in the EEA is subject to annual review and renewal by the European Commission ("EC") following reassessment by the European Medicines Agency ("EMA") of the benefit-risk balance of the authorization, which the Company refers to as the annual EMA reassessment. In September 2022, the Company submitted a Type II variation to the EMA to support conversion of the conditional marketing authorization for Translarna to a standard marketing authorization, which included a report on the placebo-controlled trial of Study 041 and data from the open-label extension. In February 2023, the Company also submitted an annual marketing authorization renewal request to the EMA. In September 2023, the Committee for Medicinal Products for Human Use ("CHMP"), gave a negative opinion on the conversion of the conditional marketing authorization to full marketing authorization of Translarna for the treatment of nmDMD and a negative opinion on the renewal of the existing conditional marketing authorization of Translarna for the treatment of nmDMD. On January 25, 2024, the CHMP issued a negative opinion for the renewal of the conditional marketing authorization following a re-examination procedure. In accordance with EMA regulations, the EC has approximately 67 days to adopt the opinion. At this time, the EC has not yet adopted the negative opinion. If the EC adopts the negative opinion, Translarna would no longer have marketing authorization in the member states of the EEA.

Translarna is an investigational new drug in the United States. Following the Company's announcement of top-line results from the placebo-controlled trial of Study 041 in June 2022, the Company submitted a meeting request to the U.S. Food and Drug Administration ("FDA") to gain clarity on the regulatory pathway for a potential re-submission of a New Drug Application ("NDA") for Translarna. The FDA provided initial written feedback that Study 041 does not provide substantial evidence of effectiveness to support an NDA re-submission. The Company held a Type C meeting with the FDA in the fourth quarter of 2023 to discuss the totality of Translarna data. Based on feedback from the FDA, the Company plans to re-submit the NDA based on results from Study 041 and from the Company's international drug registry study for nmDMD patients receiving Translarna by mid-year 2024.

The Company has developed Upstaza (eladocagene exuparvovec), a gene therapy used for the treatment of Aromatic L-Amino Acid Decarboxylase ("AADC") deficiency ("AADC deficiency"), a rare central nervous system ("CNS") disorder arising from reductions in the enzyme AADC that results from mutations in the dopa decarboxylase gene. In July 2022,

the EC approved Upstaza for the treatment of AADC deficiency for patients 18 months and older within the EEA. In November 2022, the Medicines and Healthcare Products Regulatory Agency approved Upstaza for the treatment of AADC deficiency for patients 18 months and older within the United Kingdom. In March 2024, the Company submitted a biologics license application ("BLA") for Upstaza for the treatment of AADC deficiency in the United States.

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"). In August 2021, ANVISA, the Brazilian health regulatory authority, approved Waylivra as the first treatment for familial chylomicronemia syndrome ("FCS") in Brazil. In December 2022, ANVISA approved Waylivra for the treatment of familial partial lipodystrophy ("FPL"). Waylivra has also received marketing authorization in the EU for the treatment of FCS.

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 FDA in August 2020 for the treatment of SMA in adults and children two months and older and by the EC 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 has also received marketing authorization for the treatment of SMA in over 100 countries. In May 2022, the FDA approved a label expansion for Evrysdi to include infants under two months old with SMA. In August 2023, the EC approved an extension of the Evrysdi marketing authorization to include infants under two months old in the EU.

One of the Company's most advanced clinical stage molecules is sepiapterin. Sepiapterin is a precursor to intracellular tetrahydrobiopterin, which is a critical enzymatic cofactor involved in metabolism and synthesis of numerous metabolic products. In May 2023, the Company announced that the primary endpoint was achieved in its registration-directed Phase 3 trial for sepiapterin for phenylketonuria ("PKU"). The primary endpoint of the study was the achievement of statistically-significant reduction in blood Phe level. In March 2024, the Company submitted a marketing authorization application ("MAA") to the EMA for sepiapterin for the treatment of PKU in the EEA. Additionally, the Company participated in a pre-NDA meeting with the FDA in the third quarter of 2023. At that meeting, the FDA stated that the sepiapterin clinical safety and efficacy data supported NDA submission for the treatment of pediatric and adult PKU patients. However, the FDA has requested that PTC completes a 26-week nonclinical mouse study to assess sepiapterin carcinogenicity potential prior to NDA submission. Based on the timing to complete this study, PTC expects to submit an NDA to the FDA for sepiapterin for the treatment of PKU no later than the third quarter of 2024 and PTC intends to discuss with the FDA the potential for an earlier submission if PTC is permitted to submit the 26-week mouse study report during the NDA review. PTC also expects to make regulatory submissions for sepiapterin for the treatment of PKU in Japan and Brazil in 2024.

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 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. In June 2023, the Company announced interim data from the 12-week placebo-controlled phase. The Phase 2 study is actively ongoing outside the United States, while it has been paused within the United States as the FDA requested additional data to allow the Phase 2 study to proceed. The Company expects results from the 12-month interim data from the Phase 2 study of PTC518 for the treatment of HD in the second quarter of 2024. The Company expects to submit a safety data update to the FDA in the second quarter of 2024 to support lifting of the partial clinical hold on the program.

The Company's ferroptosis and inflammation 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 ferroptosis and inflammation platform are vatiquinone and utreloxastat. The Company announced topline results from a registration-directed Phase 3 trial of vatiquinone in children and young adults with Friedreich ataxia, called MOVE-FA, in May 2023. While the study did not meet its primary

endpoint, vatiquinone treatment did demonstrate significant benefit on key disease subscales, including the upright stability subscale, as well as on other disease relevant endpoints. In the first quarter of 2024, the Company met with the FDA, who expressed willingness to review an NDA for vatiquinone for the treatment of Friedreich ataxia based on the MOVE-FA trial as well as data from the ongoing open label extension study following the MOVE-FA trial, potentially allowing for the submission of an NDA in late 2024. In the first quarter of 2024, the Company also received scientific advice from the EMA on the MOVE-FA trial results, in which the EMA stated that the MOVE-FA data would likely not be sufficient for conditional authorization. The Company initiated a Phase 2 registration directed trial of utreloxastat for amyotrophic lateral sclerosis, or ALS, in the first quarter of 2022. The Company expects topline results from this trial in the fourth quarter of 2024.

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.

As of March 31, 2024, the Company had an accumulated deficit of approximately $3,375.2 million. The Company has financed its operations to date primarily through the private offerings of convertible senior notes (see Note 9), public and “at the market offerings” of common stock, proceeds from royalty purchase agreements (see Note 2), net proceeds from the Company’s’ borrowings under its credit agreement with Blackstone (see Note 9), private placements of its convertible preferred stock and common stock, collaborations, bank and institutional lender debt, other convertible 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. 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 since 2014, Emflaza for the treatment of DMD in the United States since 2017, and Upstaza for the treatment of AADC deficiency in the EEA since May 2022. 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, milestone and royalty payments from Roche, 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, 2023 included in the Company’s Annual Report on Form 10-K filed with the Securities and Exchange Commission (the "SEC") on February 29, 2024 (the "2023 Form 10-K"). Selected significant accounting policies are discussed in further detail below.

Basis of presentation

The accompanying financial information as of March 31, 2024 and for the three months ended March 31, 2024 and 2023 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, 2023 and notes thereto included in the 2023 Form 10-K.

In the opinion of management, the unaudited financial information as of March 31, 2024 and for the three months ended March 31, 2024 and 2023 reflects all adjustments, which are normal recurring adjustments, necessary to present a fair statement of financial position, results of operations, stockholders’ (deficit) equity, and cash flows. The results of operations for the three months ended March 31, 2024 are not necessarily indicative of the results to be expected for the year ended December 31, 2024 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, 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 contains 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. Restricted cash also contains an unconditional, irrevocable and transferable letter of credit that was entered into during June 2022 in connection with obligations for the Company's new facility lease in Warren, New Jersey. The amount of the letter of credit is $8.1 million and has the potential to be reduced to $4.1 million if after five years the Company is not in default of its lease. Both amounts are classified within deposits and other assets on the consolidated balance sheet due to the long-term nature of the respective letters of credit. Restricted cash also includes a bank guarantee of $0.6 million denominated in a foreign currency.

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, 2024	Beginning of period- December 31, 2023
Cash and cash equivalents	$ 548,355	$ 594,001
Restricted cash included in deposits and other assets	16,268	16,283
Total Cash, cash equivalents and restricted cash per statement of cash flows	$ 564,623	$ 610,284

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 months ended March 31, 2024 and 2023, 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 income (expense), net within the consolidated statement of operations.

Inventory and cost of product sales

Inventory

Inventories are stated at the lower of cost and net realizable value with cost determined on a first-in, first-out basis by product. The Company capitalizes inventory costs associated with products following regulatory approval when future commercialization is considered probable and the future economic benefit is expected to be realized. Products which may be used in clinical development programs are included in inventory and charged to research and development expense when the product enters the research and development process and no longer can be used for commercial purposes. Inventory used for marketing efforts are charged to selling, general and administrative expense. 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, 2024	December 31, 2023
Raw materials	$ 1,121	$ 952
Work in progress	22,178	17,991
Finished goods	6,759	11,634
Total inventory	$ 30,058	$ 30,577

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 months ended March 31, 2024, the Company recorded inventory write-downs of $3.7 million primarily related to adjustments to inventory reserves and product approaching expiration. For the three months ended March 31, 2023, the Company recorded inventory write-downs of $0.1 million 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 months ended March 31, 2024 and 2023, 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, 2024 and 2023, net product sales outside of the United States were $120.1 million and $133.0 million, respectively, consisting of sales of Translarna, Tegsedi, Waylivra, and Upstaza. Translarna net revenues made up $103.6 million and $115.1 million of the net product sales outside of the United States for the three months ended March 31, 2024 and 2023, respectively. For the three months ended March 31, 2024 and 2023, net product sales in the United States were $57.5 million and $54.6 million, respectively, consisting solely of sales of Emflaza. During the three months ended March 31, 2024, two countries, the United States and Russia, accounted for at least 10% of the Company's net product sales, representing $57.5 million and $52.6 million of net product sales, respectively. During the three months ended March 31, 2023, three countries, the United States, Russia, and Brazil, accounted for at least 10% of the Company's net product sales, representing $54.6 million, $44.6 million, and $25.9 million of net product sales, 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, 2024, the Company did not recognize collaboration revenue related to the SMA License Agreement with Roche. For the three months ended March 31, 2023, the amounts recognized for the collaboration revenue related to the SMA License Agreement with Roche were immaterial.

For the three months ended March 31, 2024 and 2023, the Company has recognized $31.2 million and $30.8 million of royalty revenue, respectively, related to Evrysdi.

Manufacturing Revenue

The Company has manufacturing services related to the production of plasmid deoxyribonucleic acid ("DNA") and adeno-associated virus ("AAV") vectors for gene therapy applications for external customers. Performance obligations vary but may include manufacturing plasmid DNA and/or AAV vectors, material testing, stability studies, and other services related to material development. The transaction prices for these arrangements are fixed and include amounts stated in the contracts for each promised service. Typically, the performance obligations within a manufacturing contract are highly interdependent, in which case, the Company will combine them into a single performance obligation. The Company has determined that the assets created have no alternative use to the Company, and the Company has an enforceable right to payment for the performance completed to date, therefore revenue related to these services are recognized over time and is measured using an output method based on performance of manufacturing milestones completed to date.

Manufacturing service contracts may also include performance obligations related to project management services or obtaining materials from third parties. The Company has determined that these are separate performance obligations for which revenue is recognized at the point in time the services are performed. For performance obligations related to obtaining third party materials, the Company has determined that it is the principal as the Company has control of the materials and has discretion in setting the price. Therefore, the Company recognizes revenue on a gross basis related to obtaining third party materials.

Certain arrangements require a portion of the contract consideration to be received in advance at the commencement of the contract, and such advance payment is initially recorded as a contract liability. A contract asset may be recognized in the event the Company's satisfaction of performance obligations outpaces customer billings.

For the three months ended March 31, 2024 and 2023, the Company recognized $1.4 million and $2.0 million of manufacturing revenue, respectively, related to plasmid DNA and AAV vector production for external customers. As of March 31, 2024, the Company has contract assets of $0.8 million and no remaining performance obligations related to the production of plasmid DNA and AAV vectors for gene therapy applications for external customers. For the period ended December 31, 2023, the Company had contract assets of $0.2 million and remaining performance obligations of $0.8 million related to the production of plasmid DNA and AAV vectors for gene therapy applications for external customers.

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 months ended March 31, 2024 and 2023, no allowance was recorded for credit losses. The allowance for doubtful accounts was $1.1 million as of March 31, 2024, and $1.2 million as of December 31, 2023. For the three months ended March 31, 2024 and 2023, bad debt expense was immaterial.

Liability for sale of future royalties

On July 17, 2020, the Company, RPI Intermediate Finance Trust ("RPI"), and, for the limited purposes set forth in the agreement, Royalty Pharma PLC, entered into a royalty purchase agreement (the "Original Royalty Purchase Agreement"). Pursuant to the Original Royalty Purchase Agreement, the Company sold to RPI 42.933% (the " Original Assigned Royalty Rights") 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 "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 Original Assigned Royalty Rights, 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 License Agreement, which remaining milestone payments equal $150.0 million in the aggregate as of March 31, 2024. The Original Royalty Purchase Agreement was set to 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 License Agreement and the date on which RPI has received $1.3 billion in respect of the Original Assigned Royalty Rights.

Pursuant to the guidance in ASC 470-10-25-2, the Company determined that the $650.0 million cash consideration obtained pursuant to the Original Royalty Purchase Agreement should be classified as debt and recorded it 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 at the time of the transaction. The liability was subsequently amortized using the effective interest method over the life of the arrangement, in accordance with the respective guidance, utilizing the prospective method to account for subsequent changes in the estimated future payments to be made to RPI.

On October 18, 2023, the Company, Royalty Pharma Investments 2019 ICAV ("Royalty Pharma"), and, for the limited purposes set forth in the agreement, Royalty Pharma plc, entered into an Amended and Restated Royalty Purchase Agreement (the "A&R Royalty Purchase Agreement"), which amends and restates in its entirety the Original Royalty Purchase Agreement. Pursuant to the A&R Royalty Purchase Agreement, the Company has sold or agreed to sell to Royalty Pharma certain portions of the Company's remaining Royalty on worldwide net sales of Evrysdi and any other product (the "Products") developed pursuant to the SMA License Agreement (all such retained Royalty rights of the Company, the "Retained Royalty Rights," and all such Royalty rights that are sold to Royalty Pharma pursuant to the A&R Royalty Purchase Agreement, the ("A&R Assigned Royalty Rights"). At closing, Royalty Pharma paid the Company $1.0 billion in cash consideration for 38.0447% of the Company's Retained Royalty Rights (which is in addition to the 42.9330% assigned to Royalty Pharma in connection with the Original Royalty Purchase Agreement, for a total of 80.9777% of the total Royalty) until such time as Royalty Pharma has received payments in respect of the Original Assigned Royalty Rights equal to $1.3 billion in the aggregate, and thereafter 66.6667% of the total Royalty. In addition, the Company may sell to Royalty Pharma the remainder of the Company's Retained Royalty Rights in exchange for an aggregate of $500.0 million in additional cash consideration after the closing of the A&R Royalty Purchase Agreement, less royalties received in respect of the Retained Royalty Rights put to Royalty Pharma, which will be payable by Royalty Pharma pursuant to five put options held by the Company that are exercisable at the Company's option between January 1, 2024 and December 31, 2025. If the Company exercises two or fewer of the put options, Royalty Pharma may exercise

a call option during the period from and after January 1, 2026 until and including March 31, 2026 for up to 50% of the remainder of the Company's Retained Royalty Rights less amounts exercised by the Company via its put options at a purchase price that is proportional to the purchase price of the Company's unexercised put options. Royalty Pharma's exercise of the call option would result in Royalty Pharma owning 90.4888% of the total Royalty until such time as Royalty Pharma has received payments in respect of the Original Assigned Royalty Rights equal to $1.3 billion in the aggregate, and thereafter 83.3333% of the total Royalty. The A&R Royalty Purchase Agreement will terminate 60 days following the date on which Roche is no longer obligated to make any payments of the Royalty pursuant to the License Agreement.

The change in rights and obligations from the A&R Royalty Purchase Agreement resulted in a change in the terms of the liability for sale of future royalties, which was evaluated by the Company in accordance with ASC 470-50, Debt — Modifications and Extinguishments. The Company determined that the present value of the cash flows under the A&R Royalty Purchase Agreement were substantially different from the present value of the cash flows under the Original Royalty Purchase Agreement. This resulted in the derecognition of the old liability for sale of future royalties and the new liability for sale of future royalties being recorded at fair value, which was determined to be $1,809.9 million as of the date of the A&R Royalty Purchase Agreement. This resulted in an extinguishment loss of $44.9 million, which was recorded within loss on extinguishment of debt, within the Company's statement of operations for the year ended December 31, 2023.

The fair value for the new liability for sale of future royalties on the date of the A&R Royalty Purchase Agreement was based on the Company's estimates of future royalties expected to be paid to Royalty Pharma 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 10.8%. The Company utilizes the prospective method to account for subsequent changes in the estimated future payments to be made to Royalty Pharma and updates the effective interest rate on a quarterly basis. Issuance costs related to the transaction were determined to be immaterial. 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 reassesses 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 December 15, 2022, the EU Member States formally adopted the EU's Pillar Two Directive, which generally provides for a minimum effective tax rate of 15%, as established by the Organization for Economic Co-operation and Development ("OECD") Pillar Two Framework that was supported by over 130 countries worldwide. The EU effective dates are January 1, 2024, and January 1, 2025, for different aspects of the directive. A significant number of other countries are also implementing similar legislation. As a result, the tax laws in the U.S. and other countries in which PTC 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. The Company is continuing to evaluate the potential impact on future periods of the Pillar Two Framework, pending legislative adoption by additional individual countries, including those within the European Union.

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, 2024.

Starting in 2022, TCJA amendments to IRC Section 174 no longer permits an immediate deduction for research and development 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. This tax law change is anticipated to result in an increased current taxable income of the Company by $42.6 million for the year ending December 31, 2024.

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. In July 2022, the Company received EMEA approval for a portion of the IPR&D assets, and thus, began the amortization of the intangible.

In May 2023, the Company announced the discontinuation of its preclinical and early research programs in gene therapy as part of a strategic portfolio prioritization. In conjunction with the announcement, the Company recorded an impairment to its indefinite-lived intangible for IP research and development relating to the Friedreich ataxia and Angelman syndrome gene therapy assets. As a result of the impairment, the Company recorded a deferred tax benefit of $46.9 million during the 2023 tax year.

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.

Recently issued accounting standards

In November 2023, the FASB issued ASU 2023-07, Segment Reporting (Topic 280) – Improvements to Reportable Segment Disclosures. This ASU requires that a public entity provide additional segment disclosures on an interim and annual basis. The amendments in this ASU should be applied retrospectively to all prior periods presented in the financial statements, unless impracticable. Upon transition, the segment expense categories and amounts disclosed in the prior periods should be based on the significant segment expense categories identified and disclosed in the period of adoption. The ASU is effective for fiscal years beginning after December 15, 2023 and interim periods within fiscal years beginning after December 15, 2024. Early adoption is permitted. The Company is currently planning to adopt this guidance when effective. The Company is assessing the impact of the adoption on the Company's consolidated financial statements and accompanying footnotes.

In December 2023, the FASB issued ASU 2023-09, Improvements to Income Tax Disclosures. ASU 2023-09 enhances the transparency about income tax information through improvements to income tax disclosures primarily related to the rate reconciliation and income taxes paid information. The guidance is effective for public business entities for annual

periods beginning after December 15, 2024. For entities other than public business entities, the amendments are effective for annual periods beginning after December 15, 2025. Early adoption is permitted. The Company is currently planning to adopt this guidance when effective. The Company is assessing the impact of the adoption on the Company's consolidated financial statements and accompanying footnotes.

3. Leases

The Company leases office space in South Plainfield, New Jersey for its principal office under two noncancelable operating leases through August 2024, in addition to office and laboratory space in Bridgewater, New Jersey and other locations throughout the United States and office space in various countries for international employees primarily through workspace providers. Effective April 2024, the Company will utilize the Warren Premises, as described below, as its principal office space.

The Company also leases approximately 220,500 square feet of office, manufacturing and laboratory space at a facility located in Hopewell Township, New Jersey pursuant to a Lease Agreement (the "Hopewell Lease") with Hopewell Campus Owner LLC. The rental term of the Hopewell Lease commenced on July 1, 2020 and has an initial term of fifteen years (the "Hopewell Initial Term"), with two consecutive ten year renewal periods, each at the Company's option. The aggregate rent for the Hopewell 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 Hopewell 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 Hopewell Lease contains customary events of default, representations, warranties and covenants.

In May 2022, the Company entered into a Lease Agreement (the "Warren Lease") with Warren CC Acquisitions, LLC (the "Warren Landlord") relating to the lease of two entire buildings comprised of approximately 360,000 square feet of shell condition, modifiable space (the "Warren Premises") at a facility located in Warren, New Jersey. The rental term of the Warren Lease commenced on June 1, 2022, with an initial term of seventeen years (the "Warren Initial Term"), followed by three consecutive five-year renewal periods at the Company's option. The aggregate base rent for the Warren Initial Term will be approximately $163.0 million; provided, however, that if the Company is not subject to an Event of Default (as defined in the Warren Lease), the Company will be entitled to a base rent abatement over the first three years of the Warren Initial Term of approximately $18.6 million, reducing the Company's total base rent obligation to $144.4 million. The rental rate for the renewal periods will be at the Fair Market Rental Value (as defined in the Warren Lease) and determined at the time of the exercise of the renewal. Beginning in the second lease year, the Company is also responsible for the payment of all taxes and operating expenses for the Warren Premises. As a result, the Company recorded an operating lease ROU asset of $28.9 million and an operating lease ROU liability of $28.9 million as of the commencement date.

The Company is entitled to an allowance of approximately $36.2 million to be provided by the Warren Landlord to be used towards such improvements. The Landlord is providing the allowance to cover those assets that are real property improvements, such as structural components, roofs, flooring, etc., whose useful lives are typically longer in nature. The Company evaluated the leasehold improvements under ASC 842 and determined that the Company will be the owner of the improvements, and therefore the $36.2 million allowance and $5.0 million due from the Landlord were treated as lease incentives at the commencement of the lease and included in the calculation of the lease ROU asset and lease ROU liability, effectively reducing both at Commencement Date. In connection with the execution of the Warren Lease, the Company also committed to fund a construction account with $3.6 million to go towards the Company's improvements of the Warren Premises. Subject to the terms of the Warren Lease, the Company has a right of first offer to purchase the Warren Premises if the Warren Landlord receives a bona fide third party offer to purchase the Warren Premises or the Warren Landlord decides to sell the Warren Premises.

The Company also has a finance lease related to its commercial manufacturing agreement with MassBiologics of the University of Massachusetts Medical School ("MassBio"). As of March 31, 2024, the balance of the finance lease liabilities-current and finance lease liabilities-noncurrent are $1.9 million and $15.6 million, respectively, and are directly related to the Company's MassBio agreement. As of December 31, 2023, the balance of the finance lease liabilities-current

and finance lease liabilities-noncurrent were $3.0 million and $17.2 million, respectively. Additionally, the Company recorded finance lease costs of $0.3 million and $0.4 million related to interest on the lease liability during the three months ended March 31, 2024 and 2023, respectively.

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 0.2 years to 15.2 years and certain of the leases include renewal options to extend the lease for up to 15 years. Rent expense was $6.9 million and $7.1 million for the three months ended March 31, 2024 and 2023, respectively.

The components of operating lease expense were as follows:

	Three Months Ended March 31, 2024	Three Months Ended March 31, 2023
Operating Lease Cost		
Fixed lease cost	$ 5,575	$ 5,473
Variable lease cost	1,069	1,353
Short-term lease cost	214	303
Total operating lease cost	$ 6,858	$ 7,129

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

Supplemental lease term and discount rate information related to leases was as follows as March 31, 2024 and December 31, 2023:

	March 31, 2024	December 31, 2023
Weighted-average remaining lease terms - operating leases (years)	11.66	11.55
Weighted-average discount rate - operating leases	8.75 %	8.69 %
Weighted-average remaining lease terms - finance lease (years)	8.76	9.01
Weighted-average discount rate - finance lease	7.80 %	7.80 %

Supplemental cash flow information related to leases was as follows as of March 31, 2024 and 2023:

	Three Months Ended March 31, 2024	2023
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows from operating leases	$ 3,948	$ 3,811
Financing cash flows from finance lease	1,490	1,379
Operating cash flows from finance lease	1,510	1,621
Right-of-use assets obtained in exchange for lease obligations:		
Operating leases	$ 1,723	$ —

Future minimum lease payments under non-cancelable leases as of March 31, 2024 were as follows:

	Operating Leases	Finance Lease
2024 (excludes the three months ended March 31, 2024)	$ 14,981	$ —
2025	20,995	3,000
2026	20,539	3,000
2027	18,400	3,000
2028 and thereafter	175,853	15,000
Total lease payments	250,768	24,000
Less: Imputed Interest expense	131,359	6,478
Total	$ 119,409	$ 17,522

4. Fair value of financial instruments and marketable securities

The Company follows the fair value measurement rules, which provide guidance on the use of fair value in accounting and disclosure for assets and liabilities when such accounting and disclosure is called for by other accounting literature. These rules establish a fair value hierarchy for inputs to be used to measure fair value of financial assets and liabilities. This hierarchy prioritizes the inputs to valuation techniques used to measure fair value into three levels: Level 1 (highest priority), Level 2, and Level 3 (lowest priority).

- Level 1—Unadjusted quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the balance sheet date.

- Level 2—Inputs other than quoted prices included within Level 1 that are observable for the asset or liability, either directly or indirectly. Level 2 inputs include quoted prices for similar assets and liabilities in active markets, quoted prices for identical or similar assets or liabilities in markets that are not active, inputs other than quoted prices that are observable for the asset or liability (i.e., interest rates, yield curves, etc.), and inputs that are derived principally from or corroborated by observable market data by correlation or other means (market corroborated inputs).

- Level 3—Inputs are unobservable and reflect the Company's assumptions as to what market participants would use in pricing the asset or liability. The Company develops these inputs based on the best information available.

Cash equivalents and marketable securities are reflected in the accompanying financial statements at fair value. The carrying amount of receivables and accounts payable and accrued expenses approximates fair value due to the short-term nature of those instruments.

The Company owns common stock in ClearPoint Neuro, Inc. ("ClearPoint") (formerly MRI Interventions, Inc.), a publicly traded medical device company. The 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 prepaids and other current assets on the consolidated balance sheet as of March 31, 2024 and December 31, 2023. 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. The convertible debt security is included as a component of prepaids and other current assets on the consolidated balance sheet as of March 31,

2024 and as a component of deposits and other assets as of December 31, 2023. Other than the ClearPoint Equity Investments and the ClearPoint convertible debt security, no other items included in prepaids and other current assets on the consolidated balance sheets are fair valued.

The Company has investments in mutual funds, including one 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 they are readily available, and as such are classified as Level 1 assets. Unrealized holding gains and losses for these equity investments are included as components of other expense, net within the consolidated statement of operations.

The table presented below is a summary of changes in the fair value for the Company's marketable securities – equity investments, ClearPoint Equity Investments, and ClearPoint convertible debt security for the three months ended March 31, 2024 and March 31, 2023:

	Ending Balance at December 31, 2023	Unrealized Gain	Foreign Currency Unrealized Loss	Investments Purchased	Redemptions/ Sale	Ending Balance at March 31, 2024
Marketable securities - equity investments	$ 22,634	710	(823)	9,065	(1,207)	$ 30,379
ClearPoint Equity Investments	6,074	9	—	—	—	6,083
ClearPoint convertible debt security	12,553	166	—	—	—	12,719
Total Fair Value	$ 41,261	$ 885	$ (823)	$ 9,065	$ (1,207)	$ 49,181

	Ending Balance at December 31, 2022	Unrealized Gain/(Loss)	Foreign Currency Unrealized Gain	Investments Purchased	Redemptions/ Sale	Ending Balance at March 31, 2023
Marketable securities - equity investments	$ 108,261	2,166	328	—	(2,196)	$ 108,559
ClearPoint Equity Investments	10,965	(39)	—	—	—	10,926
ClearPoint convertible debt security	15,231	59	—	—	—	15,290
Total Fair Value	$ 134,457	$ 2,186	$ 328	$ —	$ (2,196)	$ 134,775

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, 2024 and December 31, 2023:

	March 31, 2024			
	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	$ 306,079	$ —	$ 306,079	$ —
Marketable securities - equity investments	$ 30,379	$ 30,379	$ —	$ —
ClearPoint Equity Investments	$ 6,083	$ 6,083	$ —	$ —
ClearPoint convertible debt security	$ 12,719	$ —	$ 12,719	$ —
Contingent consideration payable- development and regulatory milestones	$ 26,900	$ —	$ —	$ 26,900
Contingent consideration payable- net sales milestones	$ 9,300	$ —	$ —	$ 9,300

	December 31, 2023			
	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	$ 260,104	$ —	$ 260,104	$ —
Marketable securities - equity investments	$ 22,634	$ 22,634	$ —	$ —
ClearPoint Equity Investments	$ 6,074	$ 6,074	$ —	$ —
ClearPoint convertible debt security	$ 12,553	$ —	$ 12,553	$ —
Contingent consideration payable- development and regulatory milestones	$ 26,600	$ —	$ —	$ 26,600
Contingent consideration payable- net sales milestones and royalties	$ 9,700	$ —	$ —	$ 9,700

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, 2024 and December 31, 2023.

The following is a summary of marketable securities accounted for as available for sale debt securities at March 31, 2024 and December 31, 2023:

	March 31, 2024			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 132,080	$ 26	$ (121)	$ 131,985
Government Obligations	174,118	41	(65)	174,094
Total	$ 306,198	$ 67	$ (186)	$ 306,079

	December 31, 2023			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 117,044	$ 128	$ (12)	$ 117,160
Corporate debt securities	1,650	—	(2)	1,648
Government Obligations	141,084	212	—	141,296
Total	$ 259,778	$ 340	$ (14)	$ 260,104

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 months ended March 31, 2024 and 2023, 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 months ended March 31, 2024 and 2023, no allowance was recorded for credit losses. Unrealized gains and losses are reported as a component of accumulated other comprehensive (loss) income in stockholders' deficit.

For the three months ended March 31, 2024 and 2023, the Company did not have any realized gains or losses 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. Reclassified amounts from other comprehensive items were determined using the actual realized gains and losses from the sales of marketable securities.

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 or equal to 12 months as of March 31, 2024 are as follows:

	March 31, 2024					
	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	$ (121)	73,250	—	—	(121)	73,250
Government bonds	$ (65)	40,302	—	—	(65)	$ 40,302
Total	$ (186)	$ 113,552	$ —	$ —	$ (186)	$ 113,552

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 or equal to 12 months as of December 31, 2023 are as follows:

	December 31, 2023					
	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	$ (12)	44,446	—	—	(12)	44,446
Corporate debt securities	$ —	—	(2)	1,648	(2)	$ 1,648
Total	$ (12)	$ 44,446	$ (2)	$ 1,648	$ (14)	$ 46,094

Available for sale debt securities at March 31, 2024 and December 31, 2023 mature as follows:

	March 31, 2024	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 131,985	$ —
Government obligations	174,094	—
Total	$ 306,079	$ —

	December 31, 2023	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 117,160	$ —
Corporate debt securities	1,648	—
Government obligations	141,296	—
Total	$ 260,104	$ —

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 September 2019, the Company issued $287.5 million of 1.50% convertible senior notes due September 15, 2026 (the "2026 Convertible Notes,"). The fair value of the 2026 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 2026 Convertible Notes observed in market trading which are Level 2 inputs. The estimated fair value of the 2026 Convertible Notes at March 31, 2024 and December 31, 2023 was $267.7 million and $265.3 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 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. The fair value of the net sales milestones is determined utilizing a valuation framework that estimates net sales volatility to simulate a range of possible payment scenarios. The average of the payments in these scenarios is then discounted to calculate present fair value. As of March 31, 2024, the contingent consideration balance, consisting solely of the Upstaza intangible asset, is $36.2 million.

As of March 31, 2024, the weighted average discount rate for the Upstaza development and regulatory milestones was 6.0% and the weighted average probability of success was 90%. As of March 31, 2024, the weighted average discount rate for the Upstaza net sales milestones was 13.5% and the weighted average probability of success for the net sales milestones was 93%.

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, 2024 and March 31, 2023:

	Level 3 liabilities	
	Contingent consideration payable- development and regulatory milestones	Contingent consideration payable- net sales milestones and royalties
Beginning balance as of December 31, 2023	$ 26,600	$ 9,700
Additions	—	—
Change in fair value	300	(400)
Payments	—	—
Ending balance as of March 31, 2024	$ 26,900	$ 9,300

	Level 3 liabilities	
	Contingent consideration payable- development and regulatory milestones	Contingent consideration payable- net sales milestones and royalties
Beginning balance as of December 31, 2022	$ 82,500	$ 81,500
Additions	—	—
Change in fair value	3,000	(600)
Payments	—	—
Ending balance as of March 31, 2023	$ 85,500	$ 80,900

The following significant unobservable inputs were used in the valuation of the contingent consideration payable for the periods ended March 31, 2024 and December 31, 2023:

	March 31, 2024			
	Fair Value	Valuation Technique	Unobservable Input	Range
Contingent consideration payable- development and regulatory milestones	$26,900	Probability-adjusted discounted cash flow	Potential development and regulatory milestones Probabilities of success Discount rates Projected years of payments	$0 - $31 million 85% - 92% 5.9% - 6.2% 2024 - 2026
Contingent considerable payable- net sales milestones and royalties	$9,300	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 - $50 million 85% - 100% 0% 13.5% 2026 - 2034

	December 31, 2023			
	Fair Value	Valuation Technique	Unobservable Input	Range
Contingent consideration payable- development and regulatory milestones	$26,600	Probability-adjusted discounted cash flow	Potential development and regulatory milestones Probabilities of success Discount rates Projected years of payments	$0 - $31 million 85% - 92% 5.8% - 6.1% 2024 - 2026
Contingent considerable payable- net sales milestones and royalties	$9,700	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 - $50 million 85% - 100% 0% 11% 2026 - 2034

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, 2024 and December 31, 2023 consist of the following:

	March 31, 2024	December 31, 2023
Employee compensation, benefits, and related accruals	$ 35,060	$ 62,643
Income tax payable	1,833	—
Consulting and contracted research	22,745	27,500
Professional fees	2,380	2,246
Sales allowance	67,075	77,176
Sales rebates	132,047	131,334
Royalties	28,952	74,111
Accounts payable	27,459	6,045
Other	13,373	10,928
Total	$ 330,924	$ 391,983

As of March 31, 2024 and December 31, 2023, there were $1.7 million and $9.0 million, respectively, of accrued restructuring costs included above within employee compensation, benefits, and related accruals from a reduction in workforce in the year ended December 31, 2023 in connection with the Company's strategic pipeline prioritization and discontinuation of its preclinical and early research programs in its gene therapy platform.

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 months ended March 31, 2024 and 2023. 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, 2024.

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,	
	2024	2023
Numerator		
Net loss	$ (91,576)	$ (138,959)
Denominator		
Denominator for basic and diluted net loss per share	76,496,127	73,729,284
Net loss per share:		
Basic and diluted	$ (1.20)*	$ (1.88)*

* In the three months ended March 31, 2024 and 2023, 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,	
	2024	2023
Stock Options	9,530,185	12,100,550
Unvested restricted stock awards and units	3,730,166	3,649,742
Total	13,260,351	15,750,292

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. On June 8, 2022 (the "Restatement Effective Date"), the Company's stockholders approved the Amended and Restated 2013 Long-Term Incentive Plan (the "Amended 2013 LTIP"). The Amended 2013 LTIP provides for the grant of incentive stock options, nonstatutory stock options, restricted stock units and other stock-based awards. The number of shares of common stock reserved for issuance under the Amended 2013 LTIP is the sum of (A) the number of shares of the Company's common stock (up to 16,724,212 shares) that is equal to the sum of (1) the number of shares issued under the 2013 Long-Term Incentive Plan prior to the Restatement Effective Date, (2) the number of shares that remain available for issuance under the 2013 Long-Term Incentive Plan immediately prior to the Restatement Effective Date and (3) the number of shares subject to awards granted under the 2013 Long-Term Incentive Plan prior to the Restatement Effective Date that are outstanding as of the Restatement Effective Date, plus (B) from and after the Restatement Effective Date, an additional 8,475,000 shares of Common Stock. As of March 31, 2024, awards for 6,618,698 shares of common stock are available for issuance under the Amended 2013 LTIP.

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, initially, up to at the time, 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. 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. In December 2022, the Company's Board of Directors approved an additional 1,700,000 shares of common stock that may be issued under the 2020 Inducement Stock Incentive Plan. As of March 31, 2024, awards for 1,989,827 shares of common stock were available for issuance under the 2020 Inducement Stock Incentive Plan.

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.

Inducement stock option awards

From January 1, 2024 through March 31, 2024, the Company issued a total of 855,385 stock options to various employees. Of those, 1,840 were inducement grants for non-statutory stock options, all of which were made pursuant to the 2020 Inducement Stock Incentive Plan.

Stock option activity—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, 2023	9,600,399	$ 43.59		
Granted	855,385	$ 25.69		
Exercised	(109,892)	$ 19.22		
Forfeited/Cancelled	(815,707)	$ 45.32		
Outstanding at March 31, 2024	9,530,185	$ 42.12	5.90 years	$ 12,025
Vested or Expected to vest at March 31, 2024	2,112,348	$ 38.57	8.67 years	$ 2,949
Exercisable at March 31, 2024	7,173,552	$ 43.44	4.98 years	$ 8,598

The fair value of grants made in the three months ended March 31, 2024 was contemporaneously estimated on the date of grant using the following assumptions:

	Three months ended March 31, 2024
Risk-free interest rate	4.23%
Expected volatility	53%
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, 2024 was $13.49 per share.

The expected term of options was estimated based on the Company's historical exercise data and the expected volatility of options was estimated based on the Company's historical stock volatility. The risk-free rate of the options was based on U.S. Government Securities Treasury Constant Maturities yields at the date of grant for a term similar to the expected term of the option.

Restricted Stock Awards and Restricted Stock Units—Restricted stock awards and restricted stock units are granted subject to certain restrictions, including in some cases service or time conditions (restricted stock). The grant-date fair value of restricted stock awards and restricted stock units, which have been determined based upon the market value of the Company's shares on the grant date, are expensed over the vesting period. From January 1, 2024, through March 31, 2024,

the Company issued a total of 1,785,660 restricted stock units to various employees. Of those, 4,275 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, 2023	2,866,270	$ 41.82
Granted	1,785,660	25.69
Vested	(835,179)	44.56
Forfeited	(86,585)	40.98
Unvested at March 31, 2024	3,730,166	$ 33.51

Performance-based Restricted Stock Units—In December 2023, the Company granted 150,000 performance-based restricted stock units ("PSUs") to its Chief Executive Officer, Dr. Matthew Klein, which will vest only if certain regulatory milestones are achieved over an approximately two year performance period. As of March 31, 2024, the achievements of the performance goals have not yet been deemed probable and therefore no expense has been recognized to date.

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, 2024, the Company recorded $0.6 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,	
	2024	2023
Research and development	$ 8,967	$ 15,314
Selling, general and administrative	9,411	13,501
Total	$ 18,378	$ 28,815

As of March 31, 2024, there was approximately $156.3 million of total unrecognized compensation cost related to unvested share-based compensation arrangements granted under the Company's equity award plans. This cost is expected to be recognized as share-based compensation expense over the weighted average remaining service period of approximately 2.4 years.

9. Debt

Liability for sale of future royalties

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 months ended March 31, 2024:

Liability for sale of future royalties- (current and noncurrent)	Three Months Ended March 31, 2024
Beginning balance as of December 31, 2023	$ 1,814,097
Less: Non-cash royalty revenue payable to RPI	(25,228)
Plus: Non-cash interest expense recognized	50,064
Ending balance	$ 1,838,933
Effective interest rate as of March 31, 2024	10.8%

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

Senior Secured Term Loan

On October 27, 2022 (the "Closing Date"), the Company entered into a credit agreement (the "Blackstone Credit Agreement") for fundings of up to $950.0 million consisting of a committed loan facility of $450.0 million and further contemplating the potential for up to $500.0 million of additional financing, to the extent that the Company requested such additional financing and subject to the Lenders' agreement to provide such additional financing and to mutual agreement on terms, among the Company, certain subsidiaries of the Company (together with the Company, the "Loan Parties") and funds and other affiliated entities advised or managed by Blackstone Life Sciences and Blackstone Credit (collectively, "Blackstone", and such lenders, together with their permitted assignees, the "Lenders" and each a "Lender") and Wilmington Trust, National Association, as the administrative agent for the Lenders.

The Blackstone Credit Agreement provided for a senior secured term loan facility funded on the Closing Date in the aggregate principal amount of $300.0 million (the "Initial Loans") and a committed delayed draw term loan facility of up to $150.0 million (the "Delayed Draw Loans" and, together with the Initial Loans, the "Loans") to be funded at the Company's request within 18 months of the Closing Date subject to specified conditions. In addition, the Blackstone Credit Agreement contemplated the potential for further financings by Blackstone, by providing for incremental discretionary uncommitted further financings of up to $500.0 million. The Company capitalized approximately $11.6 million of debt issuance costs which are presented on the balance sheet as a direct deduction from the debt liability and are being amortized over the term of the senior secured term loan facility using the effective interest rate method.

The Loans were to mature on the date that is seven years from the Closing Date. Borrowings under the Blackstone Credit Agreement bore interest at a variable rate equal to, at the Company's option, either an adjusted Term SOFR rate plus seven and a quarter percent (7.25%) or the Base Rate plus six and a quarter percent (6.25%), subject to a floor of one percent (1%) and two percent (2%) with respect to Term SOFR rate and Base Rate (each as defined in the Blackstone Credit Agreement), respectively. Payment of the Loans were subject to certain premiums specified in the Blackstone Credit Agreement, in each case, from the date of the applicable Loan funded.

On October 19, 2023, the Company terminated the Blackstone Credit Agreement. In connection with the termination of the Credit Agreement, the Company repaid outstanding principal of $300.0 million, accrued interest of $2.1 million, an additional $82.0 million in prepayment premiums, exit fees, and creditor expenses, and $0.2 million in legal fees. The Company recorded a loss on the extinguishment of debt of $92.7 million which was included on the statement of operations for the period ended December 31, 2023. The loss on extinguishment of debt consisted of $82.0 million in prepayment premiums, exit fees, and creditor expenses and debt issuance costs of $10.7 million. All liens and security interests securing the loans made pursuant to the Blackstone Credit Agreement were released upon termination.

The Blackstone Credit Agreement consisted of the following:

	March 31, 2024	December 31, 2023
Principal	$ —	$ 300,000
Less: Debt issuance costs	—	—
Repayment of senior secured term loan	—	(300,000)
Net carrying amount	$ —	$ —

The following table sets forth total interest expense recognized related to the Blackstone Credit Agreement:

	Three Months Ended March 31, 2024	Three Months Ended March 31, 2023
Contractual interest expense	$ —	$ 9,178
Amortization of debt issuance costs	—	150
Total	$ —	$ 9,328
Effective interest rate	— %	13.2 %

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

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

The 2026 Convertible Notes represent senior unsecured obligations and will rank senior in right of payment to the Company's future indebtedness that is expressly subordinated in right of payment to the notes, equal in right of payment to the Company's existing and future unsecured indebtedness that is not so subordinated, effectively junior in right of payment to any of the Company's secured indebtedness to the extent of the value of the assets securing such indebtedness, and structurally subordinated to all existing and future indebtedness and other liabilities (including trade payables) incurred by the Company's subsidiaries. The 2026 Convertible Notes Indenture contains customary events of default with respect to the 2026 Convertible Notes, including that upon certain events of default (including the Company's failure to make any payment of principal or interest on the 2026 Convertible Notes when due and payable) occurring and continuing, the 2026 Convertible Notes Trustee by notice to the Company, or the holders of at least 25% in principal amount of the outstanding 2026 Convertible Notes by notice to the Company and the Convertible Notes Trustee, may, and the 2026 Convertible Notes Trustee at the request of such holders (subject to the provisions of the 2026 Convertible Notes Indenture) shall, declare 100% of the principal of and accrued and unpaid interest, if any, on all the 2026 Convertible Notes to be due and payable. In case of certain events of bankruptcy, insolvency or reorganization, involving the Company or a significant subsidiary, 100% of the principal of and accrued and unpaid interest on the 2026 Convertible Notes will automatically become due and payable. Upon such a declaration of acceleration, such principal and accrued and unpaid interest, if any, will be due and payable immediately.

The 2026 Convertible Notes consist of the following:

	March 31, 2024	December 31, 2023
Principal	$ 287,500	$ 287,500
Less: Debt issuance costs	(2,988)	(3,287)
Net carrying amount	$ 284,512	$ 284,213

As of March 31, 2024, the remaining contractual life of the 2026 Convertible Notes is approximately 2.5 years.

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

	Three Months Ended March 31, 2024	Three Months Ended March 31, 2023
Contractual interest expense	$ 1,076	$ 1,069
Amortization of debt issuance costs	296	288
Total	$ 1,372	$ 1,357
Effective interest rate	1.9 %	1.9 %

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. During the year ended December 31, 2022, the Company incurred $2.5 million of development milestones in connection with the enrollment of patients in the registration-directed Phase 2/3 trial of unesbulin for the treatment of LMS, which is recorded in accounts payable and accrued expenses on the balance sheet and will be payable upon the earlier to occur of the first dose administered to the last patient enrolled in the study or the termination of dosing of all patients in the study. However, as part the Company's continuous platform review, the Company has decided to deprioritize its programs for unesbulin for the treatment of diffuse intrinsic pontine glioma and leiomyosarcoma. Accordingly, the Company no longer expects to pay additional milestones to Wellcome Trust under this agreement.

The Company has also entered into a collaboration agreement with the SMA Foundation. The Company was obligated to pay the SMA Foundation single-digit royalties on worldwide net product sales of any collaboration product that is successfully developed and subsequently commercialized or, with respect to collaboration products the Company outlicenses, including Evrysdi, a specified percentage of certain payments the Company receives from its licensee. The Company was not obligated to make such payments unless and until annual sales of a collaboration product exceed a designated threshold. Since inception, the SMA Foundation has earned $52.5 million in royalty payments, $35.3 million which was paid and $17.2 million which was accrued as of March 31, 2024. During the year ended December 31, 2023, the Company had reached its obligations to make such payments to the SMA Foundation of an aggregate of $52.5 million, and therefore, there are no further payment obligations due.

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.

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.

On April 29, 2020, the Company, certain of the former equity holders of Agilis ("the Participating Rightholders"), and, for the limited purposes set forth in the agreement, Shareholder Representative Services LLC, entered into a Rights Exchange Agreement (the "Rights Exchange Agreement"). Pursuant to the terms of the Rights Exchange Agreement, 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.

In July 2022, the European Commission approved Upstaza for the treatment of AADC deficiency for patients 18 months and older within the EEA. As a result of such approval, the Company paid the former equityholders of Agilis $50.0 million in accordance with the terms of the Agilis Merger Agreement in the year ended December 31, 2022. In May 2023, as part of the Company's strategic portfolio prioritization, the Company decided to discontinue its preclinical and early research programs in its gene therapy platform, which included Friedreich ataxia and Angelman syndrome. As a result, the Company does not expect the milestones related to Friedreich ataxia and Angelman syndrome to be achieved. In addition, the Company does not expect to pay the 2% to 6% royalties on annual net sales related to Friedreich ataxia and Angelman syndrome. As of March 31, 2024, the remaining potential development and regulatory milestones the Company expects to achieve is $31.1 million, and the remaining potential sales milestones the Company expects to achieve is $50.0 million, both of which relate solely to Upstaza.

On October 25, 2019, the Company completed the acquisition of substantially all of the assets of BioElectron Technology Corporation ("BioElectron"), a Delaware corporation, including certain compounds that the Company has begun to develop as part of its Bio-e platform, pursuant to an asset purchase agreement by and between the Company and BioElectron, dated October 1, 2019 (the "BioElectron Asset Purchase Agreement"). BioElectron was a private company with a pipeline focused on inflammatory and central nervous system (CNS) disorders. The lead program, vatiquinone, is in late stage development for Friedreich ataxia with substantial unmet need and significant commercial opportunity that are complementary to PTC's existing pipeline.

Subject to the terms and conditions of the BioElectron Asset Purchase 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 Purchase 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 Agreement and Plan of Merger, dated as of May 5, 2020 (the "Censa Merger Agreement") by and among the Company, Hydro Merger Sub, Inc., the Company's wholly owned, indirect subsidiary,

and, solely in its capacity as the representative, agent and attorney-in-fact of the securityholders of Censa, Shareholder Representative Services LLC (such merger pursuant thereto, the "Censa Merger"), 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 sepiapterin'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 sepiapterin, (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 sepiapterin, on a country-by-country basis, which contingent payment shall equal to a mid-double digit percentage of any such sublicense fees.

In February 2023, the Company completed enrollment of its Phase 3 placebo-controlled clinical trial for sepiapterin for PKU. In connection with this event and pursuant to the Censa Merger Agreement, the Company paid a $30.0 million development milestone to the former Censa securityholders during the three months ended March 31, 2023. The Company elected to pay this milestone in the form of shares of its common stock, less certain cash payments in accordance with the Censa Merger Agreement. Pursuant to such election, the Company issued 657,462 shares of its common stock and paid $0.4 million to the former Censa securityholders. The Company expects to make payments to the former Censa securityholders of $65.0 million in the aggregate in cash upon the potential achievement in 2024 of regulatory milestones relating to sepiapterin pursuant to the Censa Merger Agreement.

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. Akcea is 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, Emflaza, and Upstaza net product revenue, 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, 2024 and 2023, net product sales outside of the United States were $120.1 million and $133.0 million, respectively, consisting of sales of Translarna, Tegsedi, Waylivra, and Upstaza. Translarna net revenues made up $103.6 million and $115.1 million of the net product sales outside of the United States for the three months ended March 31, 2024 and 2023, respectively. During the three months ended March 31, 2024 and 2023, net product sales in the United States were $57.5 million and $54.6 million, respectively, consisting solely of sales of Emflaza. During the three months ended March 31, 2024, two countries, the United States and Russia, accounted for at least 10% of the Company's net product sales, representing $57.5 million and $52.6 million of the net product sales, respectively. During the three months ended March 31, 2023, three countries, the United States, Russia, and Brazil, accounted for at least 10% of the Company's net product sales, representing $54.6 million, $44.6 million, and $25.9 million of the net product sales, respectively. For the three months ended March 31, 2024 and 2023, two of the Company's distributors each accounted for over 10% of the Company's net product sales.

As of March 31, 2024 and December 31, 2023, the Company does not have a contract liabilities balance related to net product sales, and has not made significant changes to the judgments made in applying ASC Topic 606.

Collaboration and Royalty revenue

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

Under the agreement, the Company is eligible to receive additional payments from Roche if specified events are achieved with respect to each licensed product, including up to $135.0 million in research and development event milestones, up to $325.0 million in sales milestones upon achievement of 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, 2024, 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 $150.0 million.

For the three months ended March 31, 2024, the Company did not recognize collaboration revenue related to the licensing and collaboration agreement with Roche. For the three months ended March 31, 2023, the amounts recognized for the collaboration revenue related to the licensing and collaboration agreement with Roche were immaterial.

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, 2024 and 2023, the Company has recognized $31.2 million and $30.8 million of royalty revenue, respectively, related to Evrysdi.

Manufacturing Revenue

For the three months ended March 31, 2024 and 2023, the Company recognized $1.4 million and $2.0 million of manufacturing revenue, respectively, related to the production of plasmid DNA and AAV vectors for gene therapy applications for external customers. The Company has not made significant changes to the judgments made in applying ASC Topic 606 for the three months ended March 31, 2024 and 2023.

As of March 31, 2024, the Company does not have a contract liabilities balance related to the production of plasmid DNA and AAV vectors for gene therapy applications for external customers. As of December 31, 2023, the Company had a contract liabilities balance of $0.8 million related to the production of plasmid DNA and AAV vectors for external customers, which is recorded within deferred revenue on the consolidated balance sheet. For the three-month period ended March 31, 2024, the Company recognized $0.8 million related to the amounts included in the contract liability balance at the beginning of the period.

As of March 31, 2024, the Company has contract assets of $0.8 million related to plasmid DNA and AAV production for external customers, which is recorded within prepaid expenses and other current assets on the consolidated balance sheet. As of December 31, 2023, the Company had contract assets of $0.2 million related to plasmid DNA and AAV production for external customers, which is recorded within prepaid expenses and other current assets on the consolidated balance sheet.

Remaining performance obligations

There are no remaining performance obligations as of March 31, 2024. The Company's remaining performance obligations of $0.8 million as of December 31, 2023 were fully recognized during the three months ended March 31, 2024.

12. Intangible assets and goodwill

Definite-lived intangibles

Definite-lived intangible assets consisted of the following at March 31, 2024 and December 31, 2023:

Definite-lived intangibles assets, gross	Ending Balance at December 31, 2023	Additions	Foreign currency translation	Ending Balance at March 31, 2024
Emflaza	$ 527,417	$ —	$ —	$ 527,417
Waylivra	10,218	1,658	(246)	11,630
Tegsedi	13,322	792	(320)	13,794
Upstaza	89,550	—	—	89,550
Total definite-lived intangibles, gross	$ 640,507	$ 2,450	$ (566)	$ 642,391

Definite-lived intangibles assets, accumulated amortization	Ending Balance at December 31, 2023	Amortization	Foreign currency translation	Ending Balance at March 31, 2024
Emflaza	$ (478,618)	$ (48,799)	$ —	$ (527,417)
Waylivra	(3,965)	(366)	106	(4,225)
Tegsedi	(3,311)	(499)	83	(3,727)
Upstaza	(10,882)	(1,866)	—	(12,748)
Total definite-lived intangibles, accumulated amortization	$ (496,776)	$ (51,530)	$ 189	$ (548,117)

Total definite-lived intangibles, net	$ 94,274

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, which expired February 2024. In accordance with the guidance for an asset acquisition, the Company recorded the milestone payments when they became payable to Marathon and increased the cost basis for the Emflaza rights intangible asset. As of March 31, 2024, the Emflaza rights intangible asset was fully amortized, therefore for the three months ended March 31, 2024, the milestone payment was recorded on the consolidated statement of operations within cost of product sales, excluding amortization of acquired intangible assets.

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 records royalty payments when they become payable to Akcea and increase the cost basis for the Waylivra and Tegsedi intangible assets. For the three months ended March 31, 2024, royalty payments of $0.8 million and $1.7 million were recorded for Tegsedi and Waylivra, respectively. As of March 31, 2024, a royalty payable of $0.8 million and $1.6 million for Tegsedi and Waylivra, respectively, was recorded on the consolidated balance sheet within accounts payable and accrued expenses.

For the three months ended March 31, 2024 and 2023, the Company recognized amortization expense of $51.5 million and $39.4 million, respectively, related to the Emflaza rights, Upstaza, Waylivra, and Tegsedi intangible assets. The estimated future amortization of the Upstaza, Waylivra, and Tegsedi intangible assets is expected to be as follows:

	As of March 31, 2024
2024	$ 8,182
2025	10,898
2026	10,898
2027	10,898
2028 and thereafter	53,398
Total	$ 94,274

The weighted average remaining amortization period of the definite-lived intangibles as of March 31, 2024 is 9.4 years.

Indefinite-lived intangibles

Indefinite-lived intangible assets consisted of the following at March 31, 2024 and December 31, 2023:

Indefinite-lived intangibles assets	Ending Balance at December 31, 2023	Additions	Impairment	Ending Balance at March 31, 2024
Upstaza	$ 235,766	$ —	$ —	$ 235,766
Total indefinite-lived intangibles	$ 235,766	$ —	$ —	$ 235,766
Total intangible assets, net				$ 330,040

In connection with the acquisition of the Company's gene therapy platform from Agilis, the Company acquired rights to Upstaza, 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. 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. There have been no changes to the indefinite lived intangible assets balance since the year ended December 31, 2023. Accordingly, the indefinite lived intangible asset balance as of March 31, 2024 is $235.8 million.

Goodwill

As a result of the Agilis Merger on August 23, 2018, the Company recorded $82.3 million of goodwill. As of March 31, 2024, 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, 2024 is $82.3 million.

13. Subsequent events

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

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

The following discussion 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, 2023 included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 29, 2024, as amended, or our 2023 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 2023 Annual Report, our actual results may differ materially from those anticipated in these forward-looking statements.

Our Company

We are a 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 little to 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 diversified therapeutic 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 relating to neurology and metabolism.

Corporate Updates

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 currently has conditional 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. Translarna also has marketing authorization in Russia for the treatment of nmDMD in patients aged two years and older, and in Brazil for the treatment of nmDMD in ambulatory patients two years and older and for continued treatment of patients that become non-ambulatory, as well as in various other countries. During the quarter ended March 31, 2024, we recognized $103.6 million in net sales of 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, 2024, we recognized $57.5 million in net sales of Emflaza.

Our marketing authorization for Translarna in the EEA is subject to annual review and renewal by the European Commission, or EC, 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 September 2022, we submitted a Type II variation to the EMA to support conversion of the conditional marketing authorization for Translarna to a standard marketing authorization, which included a report on the placebo-controlled trial of Study 041 and data from the open-label extension as further described below. Study 041 was an 18-month, placebo-controlled trial, followed by an 18-month open-label extension of Translarna in the treatment of ambulatory patients with nmDMD aged five years or older. In February 2023, we also submitted an annual marketing authorization renewal request to the EMA. In September 2023, the Committee for Medicinal Products for Human Use, or CHMP, gave a negative opinion on the conversion of the conditional marketing authorization to full marketing authorization of Translarna for the treatment of nmDMD and a negative opinion on the

renewal of the existing conditional marketing authorization of Translarna for the treatment of nmDMD. On January 25, 2024, the CHMP issued a negative opinion for the renewal of the conditional marketing authorization following a re-examination procedure. In accordance with EMA regulations, the EC has approximately 67 days to adopt the opinion. At this time, the EC has not yet adopted the negative opinion. If the EC adopts the negative opinion, Translarna would no longer have marketing authorization in the member states of the EEA. We are exploring other potential mechanisms in which we may provide Translarna to nmDMD patients in the EEA if the negative opinion is adopted by the EC.

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, or similar styled 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 the EC ratifies the CHMP's negative opinion, or we are otherwise unable to renew our EEA marketing authorization during any annual renewal cycle, or we are unable to identify other potential mechanisms in which we may provide Translarna to nmDMD patients in the EEA should the EC adopt the CHMP's negative opinion or our product label is materially restricted, 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. In June 2022, we announced top-line results from the placebo-controlled trial of Study 041. Following this announcement, we submitted a meeting request to the FDA to gain clarity on the regulatory pathway for a potential re-submission of an NDA for Translarna. The FDA provided initial written feedback that Study 041 does not provide substantial evidence of effectiveness to support NDA re-submission. We held a Type C meeting with the FDA in the fourth quarter of 2023 to discuss the totality of Translarna data. Based on feedback from the FDA, we plan to re-submit the NDA based on results from Study 041 and from our international drug registry study for nmDMD patients receiving Translarna by mid-year 2024.

We have previously relied on Emflaza's seven-year marketing exclusivity period in the United States for its approved indications under the provisions of the Orphan Drug Act of 1983, or the Orphan Drug Act, when commercializing Emflaza. Emflaza's seven-year period of orphan drug exclusivity related to the treatment of DMD in patients five years and older expired in February 2024. We expect the expiration of this orphan drug exclusivity to have a significant negative impact on Emflaza net product revenue. Emflaza's orphan drug exclusivity related to the treatment of DMD in patients two years of age to less than five expires in June 2026.

Upstaza™ (eladocagene exuparvovec)

Our product Upstaza is a gene therapy used for the treatment of Aromatic L-Amino Decarboxylase, or AADC, deficiency, a rare central nervous system, or CNS, disorder arising from reductions in the enzyme AADC that results from mutations in the dopa decarboxylase gene. In July 2022, the EC approved Upstaza for the treatment of AADC deficiency for patients 18 months and older within the EEA. In November 2022, the Medicines and Healthcare Products Regulatory Agency

approved Upstaza for the treatment of AADC deficiency for patients 18 months and older within the United Kingdom. In March 2024, we submitted a biologics license application, or BLA, to the FDA for Upstaza for the treatment of AADC deficiency in the United States.

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. In August 2021, ANVISA, the Brazilian health regulatory authority, approved Waylivra as the first treatment for familial chylomicronemia syndrome, or FCS, in Brazil. In December 2022, ANVISA approved Waylivra for the treatment of familial partial lipodystrophy, or FPL. Waylivra has also received marketing authorization in the EU for the treatment of FCS.

Evrysdi® (risdiplam)

We also have a spinal muscular atrophy, or SMA, collaboration with F. Hoffman La Roche Ltd. and Hoffman La Roche inc., which we refer to collectively as Roche, and the Spinal Muscular Atrophy Foundation, or SMA Foundation. The SMA program has one approved product, Evrysdi® (risdiplam), 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 EC 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 over 100 countries. In May 2022, the FDA approved a label expansion for Evrysdi to include infants under two months old with SMA. In August 2023, the EC approved an extension of the Evrysdi marketing authorization to include infants under two months old in the EU.

Diversified Development Pipeline

Sepiapterin

One of our most advanced clinical stage molecules is sepiapterin. Sepiapterin is a precursor to intracellular tetrahydrobiopterin, which is a critical enzymatic cofactor involved in metabolism and synthesis of numerous metabolic products. In May 2023, we announced that the primary endpoint was achieved in our registration-directed Phase 3 trial for sepiapterin for phenylketonuria, or PKU. The primary endpoint of the study was the achievement of statistically-significant reduction in blood Phe level. The primary analysis population included those patients who have a greater than 30% reduction in blood Phe levels during the Part 1 run-in phase of the trial. Sepiapterin demonstrated Phe level reduction of approximately 63% in the overall primary analysis population and Phe level reduction of approximately 69% in the subset for classical PKU patients. Additionally, sepiapterin was well tolerated with no serious adverse events. Following the placebo-controlled study, patients were eligible to enroll in a long-term open-label study, which is still ongoing and will evaluate long-term safety, durability and Phe tolerance. In March 2024, we submitted a marketing authorization application, or MAA, to the EMA for sepiapterin for the treatment of PKU in the EEA. Additionally, we participated in a pre-NDA meeting with the FDA in the third quarter of 2023. At that meeting, the FDA stated that the sepiapterin clinical safety and efficacy data supported NDA submission for the treatment of pediatric and adult PKU patients. However, the FDA has requested that we complete a 26-week nonclinical mouse study to assess sepiapterin carcinogenicity potential prior to NDA submission. This nonclinical study was not initially required when we acquired sepiapterin, as the NDA submission was planned under the Section 505(b)(2) pathway. Given that sepiapterin is a novel therapy with distinct pharmacology, biodistribution, mechanism of action and differentiated efficacy, we subsequently decided to make the NDA submission under the Section 505(b)(1) pathway, which requires the 26-week study, which is considered a required NDA component needed to inform labeling and is typically completed prior to submission. Based on the timing to complete this study, we expect to submit an NDA to the FDA for sepiapterin for the treatment of PKU no later than the third quarter of 2024 and we intend to discuss with the FDA the potential for an earlier submission if we are permitted to submit the 26-week mouse study report during the NDA review process. We also expect to make regulatory submissions for sepiapterin for the treatment of PKU in Japan and Brazil in 2024.

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. In June 2023, we announced interim data from the 12-week placebo-controlled phase. The study demonstrated dose-dependent lowering of Huntingtin, or HTT, protein levels in peripheral blood cells, reaching an approximate mean 30% reduction in mutant HTT levels at the 10mg dose level. In addition, PTC518 exposure in the cerebrospinal fluid was consistent with or higher than plasma unbound drug levels. Furthermore, PTC518 was well tolerated with no treatment-related serious adverse events. The Phase 2 study is actively ongoing outside the United States, while it has been paused within the United States as the FDA requested additional data to allow the Phase 2 study to proceed. We expect to provide 12-month interim data from the Phase 2 study of PTC518 for the treatment of HD in the second quarter of 2024. We expect to submit a safety data update to the FDA in the second quarter of 2024 to support lifting of the partial clinical hold on the program.

Ferroptosis and Inflammation Platform

Our ferroptosis and inflammation 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 ferroptosis and inflammation platform are vatiquinone and utreloxastat. We announced topline results from a registration-directed Phase 3 trial of vatiquinone in children and young adults with Friedreich ataxia, called MOVE-FA, in May 2023. While the study did not meet its primary endpoint of statistically significant change in modified Friedreich Ataxia Rating Scale, or mFARS, score at 72 weeks in the primary analysis population, vatiquinone treatment did demonstrate significant benefit on key disease subscales and secondary endpoints. In addition, in the population of subjects that completed the study protocol, significance was reached in the mFARS endpoint and several secondary endpoints, including the upright stability subscale. Furthermore, vatiquinone was well tolerated. In the first quarter of 2024, we met with the FDA, who expressed willingness to review an NDA for vatiquinone for the treatment of Friedreich ataxia based on the MOVE-FA trial as well as data from the ongoing open label extension study following the MOVE-FA trial, potentially allowing for the submission of an NDA in late 2024. In the first quarter of 2024, we also received scientific advice from the EMA on the MOVE-FA trial results, in which the EMA stated that the MOVE-FA data would likely not be sufficient for conditional authorization. In the third quarter of 2021, we completed a Phase 1 trial in healthy volunteers to evaluate the safety and pharmacology of utreloxastat. Utreloxastat was found to be well-tolerated with no reported serious adverse events while demonstrating predictable pharmacology. We initiated a Phase 2 registration directed trial of utreloxastat for amyotrophic lateral sclerosis, or ALS, in the first quarter of 2022. We expect topline results from this trial in the fourth quarter of 2024.

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.

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 or through similar styled programs, and from sales of Emflaza for the treatment of DMD in the United States. We also

generate revenue from sales of Upstaza for the treatment of AADC deficiency in the EEA and have 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 the private offerings of convertible senior notes, public and "at the market offerings" of common stock, proceeds from royalty purchase agreements, net proceeds from our borrowings under our credit agreement with Blackstone, private placements of our convertible preferred stock and common stock, collaborations, bank and institutional lender debt, other convertible debt, grant funding and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease area addressed by our product candidates.. We have relied on revenue generated from net sales of Translarna for the treatment of nmDMD in territories outside of the United States since 2014, Emflaza for the treatment of DMD in the United States since 2017 and Upstaza for the treatment of AADC deficiency in the EEA since 2022. We have also relied on revenue associated with milestone and royalty payments from Roche pursuant to the SMA License Agreement, under our SMA program.

In October 2023, we entered into an Amended and Restated Royalty Purchase Agreement, or the A&R Royalty Purchase Agreement, with Royalty Pharma Investments 2019 ICAV, or Royalty Pharma, and, for the limited purposes set forth in the agreement, Royalty Pharma plc which amends and restates in its entirety the Original Royalty Purchase Agreement. Pursuant to the A&R Royalty Purchase Agreement, we have sold or agreed to sell to Royalty Pharma certain portions of our remaining retained right, title and interest in and to 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 under the SMA program (all such Royalty rights retained by us, are referred to as the Retained Royalty Rights, and all such Royalty rights that are sold to Royalty Pharma pursuant to the A&R Royalty Purchase Agreement, are referred to as the A&R Assigned Royalty Rights). At closing, Royalty Pharma paid us $1.0 billion in cash consideration for 38.0447% of our Retained Royalty Rights (which is in addition to the 42.9330% assigned to Royalty Pharma in connection with the Original Royalty Purchase Agreement and referred to as the Original Assigned Royalty Rights, for a total of 80.9777% of the total Royalty) until such time as Royalty Pharma has received payments in respect of the Original Assigned Royalty Rights equal to $1.3 billion in the aggregate, and thereafter 66.6667% of the total Royalty.

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.

In August 2019, we entered into an At the Market Offering Sales Agreement, or the Sales Agreement, with Cantor Fitzgerald and RBC Capital Markets, LLC, or together, the Sales Agents, pursuant to which, we may offer and sell shares of our common stock, having an aggregate offering price of up to $125.0 million from time to time through the Sales Agents by any method that is deemed to be an "at the market offering" as defined in Rule 415(a)(4) promulgated under the Securities Act of 1933, as amended, or the Securities Act. During the three months ended March 31, 2024, we did not issue or sell any shares of common stock pursuant to the Sales Agreement. 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 March 31, 2024.

As of March 31, 2024, we had an accumulated deficit of $3,375.2 million. We had a net loss of $91.6 million and $139.0 million for the three months ended March 31, 2024 and 2023, respectively.

We anticipate that we will continue to incur significant expenses in connection with our commercialization efforts in the United States, the EEA, Latin America and other territories, including expenses related to our commercial infrastructure and corresponding sales and marketing, legal and regulatory, and distribution and manufacturing undertakings as well as administrative and employee-based expenses. In addition to the foregoing, we expect to continue to incur significant costs in connection with ongoing, planned and potential future clinical trials and studies for sepiapterin and our splicing and ferroptosis and inflammation programs as well as studies in our products for maintaining authorizations, label extensions and additional indications. 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 are exploring other potential mechanisms in which we may provide Translarna to nmDMD patients in the EEA if the EC adopts the CHMP's negative opinion for Translarna following a re-examination procedure. In March 2024, we submitted a BLA to the FDA for Upstaza for the treatment of AADC deficiency in the United States. Also in March 2024, we submitted an MAA to the EMA for sepiapterin for the treatment of PKU and we expect to submit an NDA to the FDA for sepiapterin for the treatment of PKU no later than the third quarter of 2024. These efforts may significantly impact the timing and extent of our commercialization and manufacturing 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.

We expect to make payments to the former Censa Pharmaceuticals, Inc., or Censa, securityholders of $65.0 million in the aggregate in cash upon the potential achievement in 2024 of regulatory milestones relating to sepiapterin pursuant to the Agreement and Plan of Merger, dated as of May 5, 2020, or the Censa Merger Agreement, by and among us, Hydro Merger Sub, Inc., our wholly owned, indirect subsidiary, Censa and, solely in its capacity as the representative, agent and attorney-in-fact of the securityholders of Censa, Shareholder Representative Services LLC. We also expect to pay the former equityholders of Agilis Biotherapeutics, Inc., or Agilis, $20.0 million in regulatory milestone payments upon the acceptance for filing by the FDA of a BLA for Upstaza for the treatment of AADC deficiency and $4.5 million in regulatory milestone payments upon the approval of the BLA from the FDA pursuant to the Agreement and Plan of Merger, dated as of July 19, 2018, or the Agilis Merger Agreement, by and among us, Agility Merger Sub, Inc., a Delaware corporation and our wholly owned, indirect subsidiary, Agilis and, solely in its capacity as the representative, agent and attorney-in-fact of the equityholders of Agilis, Shareholder Representative Services LLC. The BLA for Upstaza to the FDA was submitted in March 2024.

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 2023 Annual Report. There were no material changes to these obligations and commitments during the period ended March 31, 2024. 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. During the three months ended March 31, 2024 and 2023, net product sales outside of the United States were $120.1 million and $133.0 million, respectively, consisting of sales of Translarna, Tegsedi, Waylivra, and Upstaza. Translarna net revenues made up $103.6 million and $115.1 million of the net product sales outside of the United States for the three months ended March 31, 2024 and 2023, respectively. During the three months ended March 31, 2024 and 2023, net product sales in the United States were $57.5 million and $54.6 million, respectively, consisting solely of sales of Emflaza. During the three months ended March 31, 2024, two countries, the United States and Russia, accounted for at least 10% of the Company's net product sales, representing $57.5 million and $52.6 million of the net product sales, respectively. During the three months ended March 31, 2023, three countries, the United States, Russia, and Brazil, accounted for at least 10% of the Company's net product sales, representing $54.6 million, $44.6 million, and $25.9 million of the net product sales, respectively. For the three months ended March 31, 2024 and 2023, two of the Company's distributors each accounted for over 10% of the Company's net product sales.

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, 2024, we had recognized a total of $310.0 million in milestone payments and $372.9 million royalties on net sales pursuant to the SMA License Agreement. As of March 31, 2024, there are no remaining research and development event milestones that we can receive. The remaining potential sales milestones as of March 31, 2024 are $150.0 million upon achievement of certain sales events.

For the three months ended March 31, 2024, we did not recognize collaboration revenue related to the SMA License Agreement with Roche. For the three months ended March 31, 2023, the amounts recognized for the collaboration revenue related to the SMA License Agreement with Roche were immaterial.

For the three months ended March 31, 2024 and 2023, we recognized $31.2 million and $30.8 million of royalty revenue, respectively, related to Evrysdi.

In July 2020, we entered into the Original Royalty Purchase Agreement. Pursuant to the Original Royalty Purchase Agreement, we sold to Royalty Pharma 42.933%, or the Original Assigned Royalty Rights, of the Royalty for $650.0 million. At that time, we 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.

In October 2023, we entered into the A&R Royalty Purchase Agreement. At closing, Royalty Pharma paid us $1.0 billion in cash consideration for 38.0447% of our Retained Royalty Rights (which is in addition to the Original Assigned Royalty Rights, for a total of 80.9777% of the total Royalty) until such time as Royalty Pharma has received payments in respect of the Original Assigned Royalty Rights equal to $1.3 billion in the aggregate, and thereafter 66.6667% of the total Royalty.

See “Item 2. Management’s Discussion and Analysis of Financial Condition and Results of Operations—Liquidity and capital resources—Sources of Liquidity” for additional information.

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 and ferroptosis and inflammation programs 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 table provides research and development expense for our most advanced principal product development programs, for the three months ended March 31, 2024 and 2023.

	Three Months Ended March 31,	
	2024	2023
	(in thousands)	
Development	$ 53,424	$ 66,808
Research	13,499	21,760
Milestones	—	30,000
Payroll, benefits, and share-based stock compensation	39,352	63,712
Facilities and other	9,854	12,844
Total research and development	$ 116,129	$ 195,124

Development. Consists of costs incurred for product candidates following initiation of a clinical trial.

For 2024 compared to 2023, the decrease in development expenses primarily reflected the decrease in program spend related to our strategic pipeline prioritization as we continue to focus on our resources on our differentiated, high potential research and development programs.

Research. Consists of costs incurred for product candidates before initiation of a clinical trial.

For 2024 compared to 2023, the decrease in research expenses primarily reflected the decrease in program spend related to our strategic pipeline prioritization as we continue to focus on our resources on our differentiated, high potential research and development programs.

Milestones. Consists of development and regulatory milestone expenses incurred in connection with our collaborative arrangements.

For 2024 compared to 2023, the decrease in milestone expenses primarily related to the achievement of a $30.0 million success-based development milestone for the completion of enrollment of a Phase 3 clinical trial for sepiapterin for PKU in 2023.

Payroll, benefits, and share-based stock compensation. Consists of costs incurred for salaries and wages, bonus, payroll taxes, benefits and stock-based compensation associated with employees involved in research and development activities. Stock-based compensation may fluctuate from period to period based on factors that are not within our control, such as our stock price on the dates stock-based grants are issued.

For 2024 compared to 2023, the decrease in payroll, benefits, and share-based stock compensation expenses primarily relates to our reduction in workforce in connection with our strategic pipeline prioritization and discontinuation of our preclinical and early research programs in our gene therapy platform.

Facilities and other. Consists of indirect costs incurred for the benefit of multiple programs, including information technology, and other facility-based expenses, such as rent expense.

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 those products or product candidates. 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.

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 Original Royalty Purchase Agreement, the A&R Royalty Purchase Agreement, the 2026 Convertible Notes outstanding, the Blackstone Credit Agreement that we repaid and terminated in October 2023, offset by interest income earned on investments.

Critical accounting policies and significant judgments and estimates

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

During the three months ended March 31, 2024, there were no material changes to our critical accounting policies as reported in our 2023 Annual Report.

Results of operations

Three months ended March 31, 2024 compared to three months ended March 31, 2023

The following table summarizes revenues and selected expense and other income data for the three months ended March 31, 2024 and 2023.

	Three Months Ended March 31,		Change
(in thousands)	2024	2023	2024 vs. 2023
Net product revenue	$ 177,604	$ 187,557	$ (9,953)
Collaboration revenue	—	6	(6)
Royalty revenue	31,154	30,831	323
Manufacturing revenue	1,360	1,988	(628)
Cost of product sales, excluding amortization of acquired intangible asset	14,740	14,144	596
Amortization of acquired intangible asset	51,530	39,415	12,115
Research and development expense	116,129	195,124	(78,995)
Selling, general and administrative expense	73,272	86,914	(13,642)
Change in the fair value of contingent consideration	(100)	2,400	(2,500)
Interest expense, net	(40,834)	(27,331)	(13,503)
Other income, net	1,591	9,956	(8,365)
Income tax expense	(6,880)	(3,969)	(2,911)

Net product revenues. Net product revenues were $177.6 million for the three months ended March 31, 2024, a decrease of $10.0 million, or 5%, from $187.6 million for the three months ended March 31, 2023. The decrease in net product revenue was primarily due to a decrease in net product sales of Translarna, offset by an increase in net product sales of Emflaza. Emflaza net product revenues were $57.5 million for the three months ended March 31, 2024, an increase of $2.9

million, or 5%, compared to $54.6 million for the three months ended March 31, 2023. These results were driven by new patient starts and high compliance. Translarna net product revenues were $103.6 million for the three months ended March 31, 2024, a decrease of $11.5 million, or 10%, compared to $115.1 million for the three months ended March 31, 2023, driven by new patients in existing geographies and continued geographic expansion. The decrease was due to the timing of bulk patient orders.

Collaboration revenues. Collaboration revenues were $0.0 million for the three months ended March 31, 2024, a decrease of $6.0 thousand, or 100%, from $6.0 thousand for the three months ended March 31, 2023. The activity for collaboration revenue was immaterial for the three months ended March 31, 2024 and the three months ended March 31, 2023.

Royalty revenue. Royalty revenue was $31.2 million for the three months ended March 31, 2024, an increase of $0.3 million, or 1%, from $30.8 million for the three months ended March 31, 2023. The increase in royalty revenue was due to higher Evrysdi sales in the three months ended March 31, 2024 as compared to the three months ended March 31, 2023. In accordance with the SMA License Agreement, we are entitled to royalties on worldwide annual net sales of the product.

Manufacturing revenue. Manufacturing revenues were $1.4 million for the three months ended March 31, 2024, a decrease of $0.6 million, or 32%, from $2.0 million for the three months ended March 31, 2023. The decrease is due to less manufacturing services related to the production of plasmid DNA and AAV vectors for gene therapy applications for external customers being performed in the three months ended March 31, 2024 as compared to the three months ended March 31, 2023.

Cost of product sales, excluding amortization of acquired intangible asset. Cost of product sales, excluding amortization of acquired intangible asset, were $14.7 million for the three months ended March 31, 2024, an increase of $0.6 million, or 4%, from $14.1 million for the three months ended March 31, 2023. Cost of product sales consist primarily of royalty payments associated with Emflaza, Translarna, and Upstaza net product sales, excluding contingent payments to Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC), or Marathon, costs associated with Emflaza, Translarna, and Upstaza products 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 increases in Translarna and Upstaza inventory reserves, and increases in royalty costs driven by the full amortization of the Emflaza intangible asset during the three months ended March 31, 2024. All Emflaza milestone payments are now recorded on the consolidated statement of operations within cost of product sales, excluding amortization of acquired intangible assets.

Amortization of acquired intangible asset. Amortization of our intangible assets was $51.5 million for the three months ended March 31, 2024, an increase of $12.1 million, or 31%, from $39.4 million for the three months ended March 31, 2023. These amounts are related to the Emflaza rights acquisition, as well as the Waylivra, Tegsedi, and Upstaza intangible assets, which are all being amortized on a straight-line basis over their estimated useful lives. The amortization increase is primarily related to additional Marathon contingent payments for Emflaza. The Emflaza intangible asset was fully amortized during the three months ended March 31, 2024.

Research and development expense. Research and development expense was $116.1 million for the three months ended March 31, 2024, a decrease of $79.0 million, or 40%, from $195.1 million for the three months ended March 31, 2023. The decrease in research and development expenses reflects strategic portfolio prioritization as we continue to focus our resources on our differentiated, high potential research and development programs.

Selling, general and administrative expense. Selling, general and administrative expense was $73.3 million for the three months ended March 31, 2024, a decrease of $13.6 million, or 16%, from $86.9 million for the three months ended March 31, 2023. The decrease reflects lower employee costs as a result of the reduction in workforce in 2023.

Change in the fair value of contingent consideration. The change in the fair value of contingent consideration was a gain of $0.1 million for the three months ended March 31, 2024, a change of $2.5 million, or over 100%, from a loss of $2.4 million for the three months ended March 31, 2023. 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 $40.8 million for the three months ended March 31, 2024, an increase of $13.5 million, or 49%, from $27.3 million for the three months ended March 31, 2023. 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 A&R Royalty Purchase Agreement, offset by a decrease in interest expense due to the termination of our Blackstone Credit Agreement.

Other income, net. Other income, net was $1.6 million for the three months ended March 31, 2024, a decrease of $8.4 million, or 84%, from other income, net of $10.0 million for the three months ended March 31, 2023. For the three months ended March 31, 2024, unrealized and realized foreign exchange gains were $1.2 million, a decrease of $7.1 million, compared to gains of $8.3 million for the three months ended March 31, 2023. For the three months ended March 31, 2024, unrealized gains from our ClearPoint debt and equity investments and marketable securities - equity investments were $0.4 million, a decrease of $1.3 million, compared to unrealized gains of $1.7 million for the three months ended March 31, 2023.

Income tax expense. Income tax expense was $6.9 million for the three months ended March 31, 2024, an increase of $2.9 million, or 73%, compared to income tax expense of $4.0 million for the three months ended March 31, 2023. The increase in income tax expense is attributable to the recognition of the revenue associated with the Royalty Pharma agreement of 2023. Additionally, we incur income tax expenses 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, our product revenue has primarily consisted of 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 or similar styled programs in the EEA and other territories. On January 25, 2024, the CHMP issued a negative opinion for the renewal of the conditional marketing authorization following a re-examination procedure. In accordance with EMA regulations, the EC has approximately 67 days to adopt the opinion from the date of its issuance. At this time, the EC has not yet adopted the negative opinion. If the EC adopts the negative opinion, Translarna would no longer have marketing authorization in the EEA. Emflaza is approved in the United States for the treatment of DMD in patients two years and older. 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. Additionally, Emflaza's seven-year period of orphan drug exclusivity related to the treatment of DMD in patients five years and older expired in February 2024. We have previously relied on this exclusivity period to commercialize Emflaza in the United States. We expect the expiration of this orphan drug exclusivity to have a significant negative impact on Emflaza net product revenue. Emflaza's orphan drug exclusivity related to the treatment of DMD in patients two years of age to less than five expires in June 2026.

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 A&R Royalty Purchase Agreement, the private placements of our preferred stock, collaborations, bank and institutional lender debt, private offerings of convertible senior notes 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. 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 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 Original Royalty Purchase Agreement. Pursuant to the Original Royalty Purchase Agreement, we sold to Royalty Pharma the Original Assigned Royalty Rights in consideration for $650.0 million.

In October 2022, we entered into the Blackstone Credit Agreement for fundings of up to $950.0 million consisting of a committed loan facility funded on the Closing Date, in the aggregate principal amount of $300.0 million, and a delayed draw term loan facility of up to $150.0 million to be funded at our request within 18 months of the Closing Date subject to specified conditions, and further contemplating the potential for up to $500.0 million of additional financing, to the extent that we request such additional financing and subject to the Lenders' agreement to provide such additional financing and to mutual agreement on terms. In October 2023, we terminated the Blackstone Credit Agreement. All liens and security interests securing the loans made pursuant to the Blackstone Credit Agreement were released upon termination.

In October 2023, we entered into the A&R Royalty Purchase Agreement. At closing, Royalty Pharma paid us $1.0 billion in cash consideration for 38.0447% of our Retained Royalty Rights (which is in addition to the Original Assigned Royalty Rights, for a total of 80.9777% of the total Royalty) until such time as Royalty Pharma has received payments in respect of the Original Assigned Royalty Rights equal to $1.3 billion in the aggregate, and thereafter 66.6667% of the total Royalty. In addition, we may sell to Royalty Pharma the remainder of our Retained Royalty Rights in exchange for an aggregate of $500.0 million in additional cash consideration after the closing of the A&R Royalty Purchase Agreement, less royalties received in respect of the Retained Royalty Rights put to Royalty Pharma, which will be payable by Royalty Pharma pursuant to five put options held by us that are exercisable at our option between January 1, 2024 and December 31, 2025. If we exercise two or fewer of the put options, Royalty Pharma may exercise a call option during the period from and after January 1, 2026 until and including March 31, 2026 for up to 50% of the remainder of our Retained Royalty Rights less amounts exercised by us via our put options at a purchase price that is proportional to the purchase price of our unexercised put options. Royalty Pharma's exercise of the call option would result in Royalty Pharma owning 90.4888% of the total Royalty until such time as Royalty Pharma has received payments in respect of the Original Assigned Royalty Rights equal to $1.3 billion in the aggregate, and thereafter 83.3333% of the total Royalty.

The A&R Royalty Purchase Agreement includes specified negative and affirmative covenants with respect to our rights under the SMA License Agreement as well as other customary representations and warranties, covenants and other provisions. The A&R Royalty Purchase Agreement will terminate 60 days following the date on which Roche is no longer obligated to make any payments of the Royalty pursuant to the SMA License Agreement.

Cash flows

As of March 31, 2024, we had cash, cash equivalents and marketable securities of $884.8 million.

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

(in thousands)	Three Months Ended March 31, 2024	2023
Cash provided by (used in):		
Operating activities	70,761	(29,491)
Investing activities	(114,932)	(28,971)
Financing activities	540	4,094

Net cash provided by operating activities was $70.8 million for the three months ended March 31, 2024. Net cash used in operating activities was $29.5 million for the three months ended March 31, 2023. The net cash provided by operating activities for the three months ended March 31, 2024 was primarily related to the cash received from the sales milestone of $100.0 million for the achievement of $1.5 billion in worldwide net sales from Evrysdi, offset by spend supporting clinical development and commercial activities. The net cash used in operating activities for the three months ended March 31, 2023 primarily relates to supporting clinical development and commercial activities.

Net cash used in investing activities was $114.9 million for the three months ended March 31, 2024 and net cash used in investing activities was $29.0 million for the three months ended March 31, 2023. Cash used in investing activities for the three months ended March 31, 2024, was primarily related to the acquisition of product rights, purchases of marketable securities-equity investments, purchases of marketable securities available-for-sale, and purchases of fixed assets, partially offset by net sales and redemption of marketable securities. Cash used in investing activities for the three months ended March 31, 2023 was primarily related to the acquisition of product rights and purchases of fixed assets, partially offset by net sales and redemption of marketable securities.

Net cash provided by financing activities was $0.5 million for the three months ended March 31, 2024 and $4.1 million for the three months ended March 31, 2023. Cash provided by financing activities for the three months ended March 31, 2024 and March 31, 2023 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 we will continue to incur significant expenses in connection with our commercialization efforts in the United States, the EEA, Latin America and other territories, including expenses related to our commercial infrastructure and corresponding sales and marketing, legal and regulatory, and distribution and manufacturing undertakings as well as administrative and employee-based expenses. In addition to the foregoing, we expect to continue to incur significant costs in connection with ongoing, planned and potential future clinical trials and studies for sepiapterin and our splicing and ferroptosis and inflammation programs as well as studies in our products for maintaining authorizations, label extensions and additional indications. 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 are exploring other potential mechanisms in which we may provide Translarna to nmDMD patients in the EEA if the EC ratifies the CHMP's negative opinion for Translarna following a re-examination procedure. In March 2024, we submitted a BLA to the FDA for Upstaza for the treatment of AADC deficiency in the United States. Also in March 2024, we submitted an MAA to the EMA for sepiapterin for the treatment of PKU and we expect to submit an NDA to the FDA for sepiapterin for the treatment of PKU no later than the third quarter of 2024. These efforts may significantly impact the timing and extent of our commercialization and manufacturing expenses.

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

- seek to satisfy contractual and regulatory obligations that we assumed through our acquisitions and collaborations;
- execute our commercialization strategy for our products, including initial commercialization launches of our products, label extensions or entering new markets;

- 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;

- are required to take other steps to maintain our current marketing authorization in the EEA, Brazil and Russia for Translarna for the treatment of nmDMD or to obtain further marketing authorizations for Translarna for the treatment of nmDMD or other indications;

- initiate or continue the research and development of sepiapterin and our splicing and ferroptosis and inflammation programs as well as studies in our products for maintaining authorizations, label extensions and additional indications;

- continue to utilize our leased biologics manufacturing and laboratory space located in Hopewell Township, New Jersey, or the Hopewell Facility, to manufacture program materials for third parties;

- 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, 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 maintain our marketing authorization for Translarna for the treatment of nmDMD in the EEA following the CHMP's negative opinion on the conditional marketing authorization following a re-examination procedure or identify other potential mechanisms in which we may provide Translarna to nmDMD patients in the EEA;

- our ability to maintain the marketing authorization for Translarna and our other products in territories outside of the EEA;

- 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;

- the amount of generic drug competition that we face for Emflaza following its loss of orphan drug exclusivity related to the treatment of DMD in patients five years and older;

- our ability to obtain marketing authorization for sepiapterin for the treatment of PKU in the United States and EEA;

- our ability to obtain marketing authorization for Upstaza for the treatment of AADC deficiency in the United States;

- 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 support approval of Translarna for nmDMD in the United States;

- unexpected decreases in revenue or increase in expenses resulting from potential widespread outbreaks of contagious disease, such as COVID-19;

- our ability to successfully complete all post-marketing requirements imposed by regulatory agencies with respect to our products;

- the progress and results of activities for sepiapterin and our splicing and ferroptosis and inflammation programs as well as studies in our products for maintaining authorizations, label extensions and additional indications;

- the scope, costs and timing of our commercialization activities, including product sales, marketing, legal, regulatory, distribution and manufacturing, for any of our products and for any of our other product candidates that may receive marketing authorization or any additional territories in which we receive authorization to market Translarna;

- the costs, timing and outcome of regulatory review of sepiapterin and our splicing and ferroptosis and inflammation programs and Translarna and Upstaza in other territories;

- our ability to satisfy our obligations under the indenture governing the 2026 Convertible Notes;

- the timing and scope of any potential future 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 and ferroptosis and inflammation 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;

- our ability to continue to utilize the Hopewell Facility to manufacture program materials for third parties;

- 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 ferroptosis and inflammation 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 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.

We expect to make payments to the former Censa securityholders of $65.0 million in the aggregate in cash upon the potential achievement in 2024 of certain regulatory milestones relating to sepiapterin pursuant to the Censa Merger Agreement. We also expect to pay the former equityholders of Agilis $20.0 million in regulatory milestone payments upon the acceptance for filing by the FDA of a BLA for Upstaza for the treatment of AADC deficiency and $4.5 million in regulatory milestone payments upon the approval of the BLA from the FDA. The BLA for Upstaza to the FDA was submitted in March 2024. 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 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 2023 Annual Report. There were no material changes to these obligations and commitments during the period ended March 31, 2024.

We have never been profitable and 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, 2024, 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 2023 Annual Report.

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, 2024. 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, 2024, 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

During the quarter ended March 31, 2024, we implemented an enterprise resource planning ("ERP") system, SAP, on a worldwide basis, which is expected to improve the efficiency of certain financial and related transactional processes. This included the financial consolidation and reporting, sales order to cash, fixed assets, supplier management and indirect procure-to-pay processes, and we have revised and updated the related controls. These changes did not materially affect our internal control over financial reporting for the three months ended March 31, 2024. No other changes in our internal control over financial reporting occurred during the quarter ended March 31, 2024 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, 2023, 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 5. Other Information.

Director and Officer Trading Arrangements

A portion of the compensation of our directors and officers (as defined in Rule 16a-1(f) under the Securities Exchange Act of 1934, as amended, or the Exchange Act) is in the form of equity awards and, from time to time, directors and officers engage in open-market transactions with respect to the securities acquired pursuant to such equity awards or other Company securities, including to satisfy tax withholding obligations when equity awards vest or are exercised, and for diversification or other personal reasons.

Transactions in Company securities by directors and officers are required to be made in accordance with our insider trading policy, which requires that the transactions be in accordance with applicable U.S. federal securities laws that prohibit trading while in possession of material nonpublic information. Rule 10b5-1 under the Exchange Act provides an affirmative defense that enables directors and officers to prearrange transactions in Company securities in a manner that avoids concerns about initiating transactions while in possession of material nonpublic information.

The following table describes, for the quarterly period covered by this report, each trading arrangement for the sale or purchase of Company securities adopted or terminated by our directors and officers that is either (1) a contract, instruction

or written plan intended to satisfy the affirmative defense conditions of Rule 10b5-1(c), or a "Rule 10b5-1 trading arrangement", or (2) a "non-Rule 10b5-1 trading arrangement" (as defined in Item 408(c) of Regulation S-K):

Name (Title)	Action Taken (Date of Action)	Type of Trading Arrangement	Nature of Trading Arrangement	Duration of Trading Arrangement	Aggregate Number of Securities
Matthew Klein (Chief Executive Officer)	Adoption (February 15, 2024)	Rule 10b5-1 trading arrangement for sell-to-cover transactions for RSUs granted on February 15, 2024	Sale	Until final settlement of RSUs on or around February 15, 2028	Indeterminable (1)
Pierre Gravier (Chief Financial Officer)	Adoption (February 15, 2024)	Rule 10b5-1 trading arrangement for sell-to-cover transactions for RSUs granted on February 15, 2024	Sale	Until final settlement of RSUs on or around February 15, 2028	Indeterminable (1)
Pierre Gravier (Chief Financial Officer)	Adoption (February 15, 2024)	Rule 10b5-1 trading arrangement for sell-to-cover transactions for RSUs granted on February 15, 2024	Sale	Until final settlement of RSUs on or around February 15, 2026	Indeterminable (1)
Neil Almstead (Chief Technical Operations Officer)	Adoption (February 15, 2024)	Rule 10b5-1 trading arrangement for sell-to-cover transactions for RSUs granted on February 15, 2024	Sale	Until final settlement of RSUs on or around February 15, 2028	Indeterminable (1)
Neil Almstead (Chief Technical Operations Officer)	Adoption (February 15, 2024)	Rule 10b5-1 trading arrangement for sell-to-cover transactions for RSUs granted on February 15, 2024	Sale	Until final settlement of RSUs on or around February 15, 2026	Indeterminable (1)
Mark Boulding (EVP & Chief Legal Officer)	Adoption (February 15, 2024)	Rule 10b5-1 trading arrangement for sell-to-cover transactions for RSUs granted on February 15, 2024	Sale	Until final settlement of RSUs on or around February 15, 2028	Indeterminable (1)

Mark Boulding (EVP & Chief Legal Officer)	Adoption (February 15, 2024)	Rule 10b5-1 trading arrangement for sell-to-cover transactions for RSUs granted on February 15, 2024	Sale	Until final settlement of RSUs on or around February 15, 2026	Indeterminable (1)
Lee Golden (EVP & Chief Medical Officer)	Adoption (February 15, 2024)	Rule 10b5-1 trading arrangement for sell-to-cover transactions for RSUs granted on February 15, 2024	Sale	Until final settlement of RSUs on or around February 15, 2028	Indeterminable (1)
Lee Golden (EVP & Chief Medical Officer)	Adoption (February 15, 2024)	Rule 10b5-1 trading arrangement for sell-to-cover transactions for RSUs granted on February 15, 2024	Sale	Until final settlement of RSUs on or around February 15, 2026	Indeterminable (1)
Eric Pauwels (Chief Business Officer)	Adoption (February 15, 2024)	Rule 10b5-1 trading arrangement for sell-to-cover transactions for RSUs granted on February 15, 2024	Sale	Until final settlement of RSUs on or around February 15, 2028	Indeterminable (1)
Eric Pauwels (Chief Business Officer)	Adoption (February 15, 2024)	Rule 10b5-1 trading arrangement for sell-to-cover transactions for RSUs granted on February 15, 2024	Sale	Until final settlement of RSUs on or around February 15, 2026	Indeterminable (1)
Christine Utter (SVP, Chief Accounting Officer and Head of People Services)	Adoption (February 15, 2024)	Rule 10b5-1 trading arrangement for sell-to-cover transactions for RSUs granted on February 15, 2024	Sale	Until final settlement of RSUs on or around February 15, 2028	Indeterminable (1)

Christine Utter (SVP, Chief Accounting Officer and Head of People Services)	Adoption (February 15, 2024)	Rule 10b5-1 trading arrangement for sell-to-cover transactions for RSUs granted on February 15, 2024	Sale	Until final settlement of RSUs on or around February 15, 2026	Indeterminable (1)

(1) The number of shares subject to this RSU grant that will be sold to satisfy applicable tax withholding obligations upon vesting is unknown as the number will vary based on the extent to which vesting conditions are satisfied and the market price of the Company's common stock at the time of settlement. This trading arrangement provides for the automatic sale of shares that would otherwise be issuable on each settlement date of the RSU in an amount sufficient to satisfy the applicable withholding obligation, with the proceeds of the sale delivered to the Company in satisfaction of the applicable withholding obligation.

Item 6. Exhibits.

Exhibit Number	Description of Exhibit
10.1+*	Employment Agreement between PTC Therapeutics, Inc. and Lee Golden
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.

\+ Management contract, compensatory plan or arrangement.

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

SIGNATURES

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

PTC THERAPEUTICS, INC.

Date: April 25, 2024

By: /s/ Pierre Gravier
Pierre Gravier
Chief Financial Officer
(Principal Financial Officer and Duly Authorized Signatory)

Exhibit 10.1

EMPLOYMENT AGREEMENT

This EMPLOYMENT AGREEMENT (the "Agreement") is made as of May 4, 2020 (the "Effective Date"), by and between PTC Therapeutics, Inc., a Delaware corporation (the "Company") and Lee Golden ("Executive"). In consideration of the mutual covenants contained in this Agreement, the Company and Executive agree as follows:

1. Employment. The Company agrees to employ Executive and Executive agrees to be employed by the Company on the terms and conditions set forth in this Agreement.

(a) Capacity. Executive shall serve the Company as Senior Vice President, Head of Global Clinical Development, reporting to Matthew Klein, Chief Development Officer, or such senior executive as the Company shall specify. Executive shall have the responsibilities, duties and authority commensurate with the position of Senior Vice President, Head of Global Clinical Development. In addition to Executive's primary duties, Executive shall perform such other services for the Company that are consistent with his/her position as Senior Vice President, Head of Global Clinical Development as may be reasonably assigned to Executive from time to time by the individual to whom s/he reports or the Board of Directors of the Company (the "Board") or their respective designees. The principal location at which Executive shall perform such services shall be the Company's corporate headquarters currently located at 100 Corporate Court, Middlesex Business Center, South Plainfield, New Jersey, subject to Section 2(c)(i) of this Agreement.

(b) Devotion of Duties; Representations. During the Term (as defined below) of Executive's employment with the Company, Executive shall devote his/her best efforts and full business time and energies to the business and affairs of the Company, and shall endeavor to perform the duties and services contemplated hereunder to the reasonable satisfaction of the individual to whom s/he reports and the Board. During the Term, Executive shall adhere to, and perform all of Executive's duties in accordance with, all applicable laws, rules and regulations and all policies and procedures of the Company, as may be in effect from time to time. During the Term of Executive's employment with the Company, Executive shall not, without the prior written approval of the Company (by action of the Board), undertake any other employment from any person or entity or serve as a director of any other company; provided, however, that (i) the Company will entertain requests as to such other employment or directorships in good faith and (ii) Executive will be eligible to participate in any policy relating to outside activities that is applicable to the senior executives of the Company and approved by the Board after the date hereof.

2. Term of Employment.

(a) Executive's employment hereunder shall commence on the Effective Date. Executive's employment hereunder shall be terminated upon the first to occur of the following:

(i) Immediately upon Executive's death;

(ii) By the Company:

(A) By written notice to Executive effective the date of such notice, following the Disability of Executive. "Disability" means that Executive

(i) is unable to engage in any substantial gainful activity by reason of any medically determinable physical or mental impairment which can be expected to result in death or can be expected to last for a continuous period of not less than 12 months, or (ii) is, by reason of any medically determinable physical or mental impairment which can be expected to last for a continuous period of not less than 12 months, receiving income replacement benefits for a period of not less than three months under an accident and health plan covering employees of the Company. Such incapacity shall be determined by a physician chosen by the Company and reasonably satisfactory to Executive (or Executive's legal representative) upon examination requested by the Company (to which Executive hereby agrees to submit). Notwithstanding the foregoing, such Disability must result in Executive becoming "Disabled" within the meaning of Section 409A(a)(2)(C) of the Internal Revenue Code of 1986, as amended (the "Code") and the guidance issued thereunder. (In this Agreement we refer to Section 409A of the Code and any guidance issued thereunder as "Section 409A").

(B) By written notice to Executive, effective the date of such notice, for Cause (as defined below); or

(C) By written notice to Executive, effective ninety (90) days after the date of such notice and subject to Section 4 hereof, without Cause; or

(iii) By Executive:

(A) At any time by written notice to the Company, effective forty-five (45) days after the date of such notice; or

(B) By written notice to the Company for Good Reason (as defined below), effective on the date specified in such notice.

The term of Executive's employment by the Company under this Agreement is referred to herein as the "Term."

(b) Definition of "Cause". For purposes of this Agreement, "Cause" shall, pursuant to the reasonable good faith determination by a majority of the Board (excluding Executive) as documented in writing, include: (i) the willful and continued failure by Executive to substantially perform Executive's material duties or responsibilities under this Agreement (other than such a failure as a result of Disability); (ii) any action or omission by Executive involving willful misconduct or gross negligence with regard to the Company, which has a detrimental effect on the Company; (iii) Executive's conviction of a felony, either in connection with the performance of Executive's obligations to the Company or which otherwise shall adversely affect Executive's ability to perform such obligations or shall materially adversely affect the business activities, reputation, goodwill or image of the Company; (iv) the material breach of a fiduciary duty to the Company; or (v) the material breach by Executive of any of the provisions of this Agreement, provided that any breach of Executive's obligations with respect to Sections 5 or 6 of this Agreement, subject to the cure provision in the next sentence, shall be deemed "material." In respect of the events described in clauses (i) and (v) above, the Company shall give Executive notice of the failure of performance or breach, reasonable as to time, place and manner in the circumstances, and a 30-day opportunity to cure, provided that such failure of

performance or breach is reasonably amenable to cure as determined by the Board in its sole discretion.

(c) Definition of "Good Reason". For purposes of this Agreement, a "Good Reason" shall mean any of the following, unless (i) the basis for such Good Reason is cured within a reasonable period of time (determined in the light of the cure appropriate to the basis of such Good Reason, but in no event less than thirty (30) nor more than ninety (90) days) after the Company receives written notice (which must be received from Executive within ninety (90) days of the initial existence of the condition giving rise to such Good Reason) specifying the basis for such Good Reason or (ii) Executive has consented to the condition that would otherwise be a basis for Good Reason:

(i) A change in the principal location at which Executive provides services to the Company to a location more than fifty (50) miles from such principal location (and/or to a location in New York City (either of which change, the Company has reasonably determined as of the date hereof, would constitute a material change in the geographic location at which Executive provides services to the Company), provided that such a relocation shall not be deemed to occur under circumstances where Executive's responsibilities require him/her to work at a location other than the corporate headquarters for a reasonable period of time;

(ii) A material adverse change by the Company in Executive's duties, authority or responsibilities as Senior Vice President, Head of Global Clinical Development which causes Executive's position with the Company to become of materially less responsibility or authority than Executive's position immediately following the Effective Date. For purposes of this definition of "Good Reason," a "material adverse change" following a Corporate Change shall not include any diminution in authority, duties or responsibilities that is solely attributable to the change in the Company's ownership structure but does not otherwise change Executive's authority, duties or responsibilities (except in a positive manner) otherwise with respect to the Company's business.

(iii) A material reduction in Executive's base compensation (including Base Salary) except if the reduction is in connection with a general reduction of not more than 20% in compensation of senior executives of the Company generally that occurs prior to the effective date of any Corporate Change;

(iv) A material breach of this Agreement by the Company which has not been cured within thirty (30) days after written notice thereof by Executive; or

(v) Failure to obtain the assumption (assignment) of this Agreement by any successor to the Company.

(d) Definition of "Corporate Change" . For purposes of this Agreement, "Corporate Change" shall mean any circumstance in which (i) the Company is not the surviving entity in any merger, consolidation or other reorganization (or survives only as a subsidiary or affiliate of an entity other than a previously wholly-owned subsidiary of the Company); (ii) the Company sells, leases or exchanges or agrees to sell, lease or exchange all or substantially all of its assets to any other person or entity (other than a wholly-owned subsidiary of the Company);

(iii) any person or entity, including a "group" as contemplated by Section 13(d)(3) of the Securities Exchange Act of 1934 (excluding, for this purpose, the Company or any Subsidiary, or any employee benefit plan of the Company or any Subsidiary, or any "group" in which all or substantially all of its members or its members' affiliates are individuals or entities who are or were beneficial owners of the Company's outstanding shares prior to the initial public offering, if any, of the Company's stock), acquires or gains ownership or control (including, without limitations, powers to vote) of more than 50% of the outstanding shares of the Company's voting stock (based upon voting power); or (iv) as a result of or in connection with a contested election of directors, the persons who were directors of the Company before such election shall cease to constitute a majority of the Board of Directors of the Company. Notwithstanding the foregoing, a "Corporate Change" shall not occur as a result of an initial public offering of the Company's common stock, or as a result of a merger, consolidation, reorganization or restructuring after which either (1) a majority of the Board of Directors of the controlling entity consists of persons who were directors of the Company prior to the merger, consolidation, reorganization or restructuring or (2) Executive forms part of an executive management team that consists of substantially the same group of individuals and Executive is performing in a similar role, with similar authority and responsibility (other than changes solely attributable to the change in ownership structure), to that which existed prior to the reorganization or restructuring. Notwithstanding the foregoing, for any payments or benefits hereunder that are subject to Section 409A, the Corporate Change must constitute a "change in control event" within the meaning of Treasury Regulation Section 1.409A-3(i)(5)(i).

3. Compensation.

(a) Base Salary. Executive's minimum base salary during the Term shall be at the rate of $400.000.00 per year (the "Base Salary"). Base Salary shall be payable in substantially equal installments in accordance with the Company's payroll practices as in effect from time to time, less any amounts required to be withheld under applicable law. The Base Salary will be subject to adjustment from time to time in the sole discretion of the Board; provided that, the Company covenants that it shall not reduce the Base Salary below $400,000.00 or the Base Salary then in effect immediately prior to the reduction unless (i) Executive consents to such reduction, or (ii) the reduction is in connection with a general reduction of not more than 20% in compensation of senior executives of the Company generally that occurs prior to the effective date of any Corporate Change.

(b) Bonus. In addition to the Base Salary, the Company may pay Executive an annual bonus (the "Bonus") as determined by the Board, solely in its discretion (it being understood that Executive's target annual bonus shall be at 40% of the Base Salary, but may be higher or lower in any year in the Board's discretion). The Board's decision to issue a Bonus to Executive in any particular year shall have no effect on the absolute discretion of the Board to grant or not to grant a Bonus in subsequent years. Any Bonus for a particular year shall be paid or provided to Executive in a lump sum no later than March 15th of the calendar year following the calendar year in which the Bonus was earned. Executive will be eligible for a prorated 2020 Bonus.

(c) Equity Compensation. Executive will receive an inducement grant of (i) 40,500 options to purchase shares of common stock of PTC, and (ii) 2,850 restricted stock units, subject to formal approval by the Compensation Committee of the Board (or a majority of the Company's independent directors) (the "Inducement Grant") and to the terms of the applicable

grant agreements. The options will vest over four (4) years, with 25% vesting on the one-year anniversary of the Effective Date and 6.25% vesting every three month period thereafter over the following three (3) years. The restricted stock units will vest over four (4) years with 25% vesting annually on the anniversary of the Effective Date. Such award is granted pursuant to the inducement grant exception under NASDAQ rules and is intended to serve as a material inducement to Executive entering into employment with PTC. Executive shall be eligible to participate in PTC's annual equity and long term incentive plan(s) and may be eligible to receive discretionary awards under such plan(s), subject to the terms and conditions of such plan(s). Except as explicitly set forth below, Executive's rights with respect to equity (including stock options) shall be covered in PTC's equity and long term incentive plan(s) and separate stock option certificates or agreements for each grant.

(i) Accelerated Vesting.

(A) For the avoidance of doubt, in the event that Executive's employment hereunder is terminated by the Company without Cause or by Executive for Good Reason, neither the unvested portion of the Inducement Grant nor any unvested equity awards granted under the Company's equity and long- term incentive plan(s) shall be subject to any accelerated vesting except as otherwise provided for in the applicable award agreement or in Section 3(c)(i)(B) below.

(B) Except as otherwise provided in the applicable award, in the event that Executive's employment hereunder is terminated by the Company without Cause or by Executive for Good Reason within the period of three (3) months prior to (but only if negotiations relating to the particular Corporate Change that occurs are ongoing at the date of the notice of termination) or twelve (12) months after a Corporate Change that occurs during the Term (such fifteen- month period, the "Protected Period"), one hundred percent (100%) of the unvested portion of the Inducement Grant and all of Executive's outstanding unvested equity awards granted under the Company's equity and long-term incentive plan(s) shall vest immediately.

(d) Vacation. Executive is eligible for time off programs outlined in the Company's Time Off Policies. Executive shall accrue over the calendar year 160 hours of paid vacation. Executive may accrue up to 200 hours of vacation. Once Executive has reached the maximum accrual, no further vacation time will be accrued unless and until the Executive uses vacation time. Upon termination of employment, the value of Executive's current balance of accrued but unused vacation shall be paid out based on his/her Base Salary that was in effect immediately prior to his/her termination of employment.

(e) Fringe Benefits. Executive shall be entitled to participate in any employee benefit plans that the Company makes available to its senior executives (including, without limitation, group life, disability, medical, dental and other insurance, retirement, pension, profit- sharing and similar plans) (collectively, the "Fringe Benefits"), provided that the Fringe Benefits shall not include any stock option or similar plans relating to the grant of equity securities of the Company. These benefits may be modified or changed from time to time at the sole discretion of the Company. Where a particular benefit is subject to a formal plan (for example, medical or life insurance), eligibility to participate in and receive any particular benefit is governed solely by the

applicable plan document, and eligibility to participate in such plan(s) may be dependent upon, among other things, a physical examination.

(f) Reimbursement of Expenses. Executive shall be entitled to reimbursement for all ordinary and reasonable out-of-pocket business expenses that are reasonably incurred by him/her in furtherance of the Company's business in accordance with reasonable policies adopted from time to time by the Company for senior executives. The Company agrees to reimburse Executive for reasonable out-of-pocket fees related to maintaining Executive's medical license.

(g) Taxes and Withholdings. The Company shall deduct and withhold from all compensation and benefits under this Agreement all social security and other federal, state and local taxes and charges which currently are or which hereafter may be required by law to be so deducted and withheld.

4. Severance Compensation.

(a) In the event of any termination of Executive's employment for any reason the Company shall pay Executive (or Executive's estate) such portions of Executive's Base Salary as have accrued prior to such termination and have not yet been paid, together with (i) amounts for accrued unused vacation days (as provided above), (ii) any amounts for expense reimbursement which have been properly incurred or the Company has become obligated to pay prior to termination and have not been paid as of the date of such termination and (iii) the amount of any Bonus previously granted to Executive by the Board but not yet paid, which amount shall not include any pro rata portion of any Bonus which would have been earned if such termination had not occurred (the "Accrued Obligations").

(b) In the event that Executive's employment hereunder is terminated (i) by Executive for a Good Reason or (ii) by the Company without Cause, the Company shall pay to Executive the Accrued Obligations. In addition, the Company shall pay to Executive the severance benefits set forth in Section (b)(i) below for twelve (12) months following Executive's termination of employment (the "Severance Period"), and pay to Executive the severance benefits set forth in Section (b)(ii). The receipt of any severance benefits provided in this Section shall be dependent upon Executive's execution (and, as applicable, nonrevocation) of a standard separation agreement and general release of claims, substantially in the form attached hereto as Exhibit A (the "Release"). The Company will also consider in good faith (but without any binding commitment) requests from Executive that the Company include in the Release a release of Executive by the Company from matters specifically disclosed to the Company by Executive in writing in advance of execution of the Release and not involving any illegality, fraud, concealment, criminal acts or acts outside the scope of Executive's employment. The distribution of severance benefits in this Section 4 is subject to section (iii) of this Section 4(b).

(i) If Executive's employment is terminated (A) by Executive for a Good Reason or (B) by the Company without Cause, in either case before or after the Protected Period, the Company shall pay Executive his/her Base Salary, less any amounts required to be withheld under applicable law, for the Severance Period in substantially equal installments in accordance with the Company's payroll practices as in effect from time to time, commencing no later than sixty (60) days following the effective date of such termination. If Executive's employment is terminated (A) by Executive for a Good

Reason or (B) by the Company without Cause, in either case during the Protected Period, the Company shall pay Executive his/her Base Salary for the Severance Period, which total amount shall be payable in a lump sum no later than sixty (60) days following Executive's termination of employment. In each case, payments shall commence or be paid provided that the Release has been executed and any applicable revocation period has expired as of the 60th day following Executive's termination.

(ii) The Company shall provide Executive with a lump sum payment representing the net value of the contributions to Executive's current group health premiums that PTC would have paid on Executive's behalf (had Executive continued to be an employee of PTC) for the Severance Period, less any amounts required to be withheld under applicable law. Such payment shall be made no later than sixty (60) days following the effective date of Executive's termination; provided that the Release has been executed and any applicable revocation period has expired as of the 60th day following Executive's termination. The foregoing shall not be construed to extend any period of continuation coverage (e.g., COBRA) required by Federal law.

(iii) Compliance with Section 409A. Subject to the provisions in this Section 4(b)(iii), any severance payments or benefits under this Agreement shall begin only upon the date of Executive's "separation from service" (determined as set forth below) which occurs on or after the date of termination of Executive's employment. The following rules shall apply with respect to the distribution of the severance payments and benefits, if any, to be provided to Executive under this Agreement:

(1) It is intended that each installment of the severance payments and benefits provided under this Agreement shall be treated as a separate "payment" for purposes of Section 409A. Neither the Company nor Executive shall have the right to accelerate or defer the delivery of any such payments or benefits except to the extent specifically permitted or required by Section 409A.

(2) If, as of the date of Executive's "separation from service" from the Company, Executive is not a "specified employee" (within the meaning of Section 409A), then each installment of the severance payments and benefits shall be made on the dates and terms set forth in this Agreement.

(3) If, as of the date of Executive's "separation from service" from the Company, Executive is a "specified employee" (within the meaning of Section 409A), then:

(A) Each installment of the severance payments and benefits due under this Agreement that, in accordance with the dates and terms set forth herein, will in all circumstances, regardless of when the separation from service occurs, be paid within the short-term deferral period (as defined under Section 409A) shall be treated as a short-term deferral within the meaning of Treasury Regulation Section 1.409A-1(b)(4) to the maximum extent permissible under Section 409A and such payments and benefits shall be paid or provided on the dates and terms set forth in this Agreement; and

(B) Each installment of the severance payments and benefits due this Agreement that is not described in Section 4(b)(iii)(3)(A) above and that would, absent this subsection, be paid within the six-month period following Executive's "separation from service" from the Company shall not be paid until the date that is six months and one day after such separation from service (or, if earlier, Executive's death), with any such installments that are required to be delayed being accumulated during the six-month period and paid in a lump sum on the date that is six months and one day following Executive's separation from service and any subsequent installments, if any, being paid in accordance with the dates and terms set forth herein; provided, however, that the preceding provisions of this sentence shall not apply to any installment of severance payments and benefits if and to the maximum extent that such installment is deemed to be paid under a separation pay plan that does not provide for a deferral of compensation by reason of the application of Treasury Regulation 1.409A-1(b)(9)(iii) (relating to separation pay upon an involuntary separation from service). Any installments that qualify for the exception under Treasury Regulation Section 1.409A-1(b)(9)(iii) must be paid no later than the last day of Executive's second taxable year following the taxable year in which the separation from service occurs.

(4) The determination of whether and when Executive's separation from service from the Company has occurred shall be made in a manner consistent with, and based on the presumptions set forth in, Treasury Regulation Section 1.409A-1(h). Solely for purposes of this Section 4(b)(iii), "Company" shall include all persons with whom the Company would be considered a single employer under Section 414(b) and 414(c) of the Code.

(5) All reimbursements and in-kind benefits provided under this Agreement shall be made or provided in accordance with the requirements of Sections 409A to the extent that such reimbursements or in-kind benefits are subject to Section 409A, including, where applicable, the requirements that (i) any reimbursement is for expenses incurred during Executive's lifetime (or during a shorter period of time specified in this Agreement), (ii) the amount of expenses eligible for reimbursement during a calendar year may not affect the expenses eligible for reimbursement in any other calendar year, (iii) the reimbursement of an eligible expense will be made on or before the last day of the calendar year following the year in which the expense is incurred and (iv) the right to reimbursement is not subject to set off or liquidation or exchange for any other benefit.

(6) Notwithstanding anything herein to the contrary, the Company shall have no liability to Executive or to any other person if the payments and benefits provided hereunder that are intended to be exempt from or compliant with Section 409A are not so exempt or compliant.

(c) In the event that Executive's employment hereunder is terminated (i) by Executive for other than a Good Reason, or (ii) by the Company for Cause, or (iii) as a result of Executive's death or Disability, then the Company will pay to Executive the Accrued

Obligations. The Company shall have no obligation to pay Executive (or Executive's estate) any other compensation following such termination except as provided in Section 4(a).

(d) Modified Section 280G Cutback.

(i) Notwithstanding any other provision of this Agreement, except as set forth in Section 4(d)(ii), in the event that the Company undergoes a "Change in Ownership or Control" (as defined below), the Company shall not be obligated to provide to Executive a portion of any "Contingent Compensation Payments" (as defined below) that Executive would otherwise be entitled to receive to the extent necessary to eliminate any "excess parachute payments" (as defined in Section 280G(b)(1) of the Code) for Executive. For purposes of this Section 4(d), the Contingent Compensation Payments so eliminated shall be referred to as the "Eliminated Payments" and the aggregate amount (determined in accordance with Treasury Regulation Section 1.280G-1, Q/A-30 or any successor provision) of the Contingent Compensation Payments so eliminated shall be referred to as the "Eliminated Amount."

(ii) Notwithstanding the provisions of Section 4(d)(i), no such reduction in Contingent Compensation Payments shall be made if (1) the Eliminated Amount (computed without regard to this sentence) exceeds (2) 100% of the aggregate present value (determined in accordance with Treasury Regulation Section 1.280G-1, Q/A-31 and Q/A-32 or any successor provisions) of the amount of any additional taxes that would be incurred by Executive if the Eliminated Payments (determined without regard to this sentence) were paid to him/her (including, state and federal income taxes on the Eliminated Payments, the excise tax imposed by Section 4999 of the Code payable with respect to all of the Contingent Compensation Payments in excess of Executive's "base amount" (as defined in Section 280G(b)(3) of the Code), and any withholding taxes). The override of such reduction in Contingent Compensation Payments pursuant to this Section 4(d)(ii) shall be referred to as a "Section 4(d)(ii) Override." For purpose of this paragraph, if any federal or state income taxes would be attributable to the receipt of any Eliminated Payment, the amount of such taxes shall be computed by multiplying the amount of the Eliminated Payment by the maximum combined federal and state income tax rate provided by law.

(iii) For purposes of this Section 4(d) the following terms shall have the following respective meanings:

(1) "Change in Ownership or Control" shall mean a change in the ownership or effective control of the Company or in the ownership of a substantial portion of the assets of the Company determined in accordance with Section 280G(b)(2) of the Code.

(2) "Contingent Compensation Payment" shall mean any payment (or benefit) in the nature of compensation that is made or made available (under this Agreement or otherwise) to a "disqualified individual" (as defined in Section 280G(c) of the Code) and that is contingent (within the meaning of Section 280G(b)(2)(A)(i) of the Code) on a Change in Ownership or Control of the Company.

(iv) Any payments or other benefits otherwise due to Executive following a Change in Ownership or Control that could reasonably be characterized (as determined by the Company) as Contingent Compensation Payments (the "Potential Payments") shall not be made until the dates provided for in this Section 4(d)(iv). Within 30 days after each date on which Executive first becomes entitled to receive (whether or not then due) a Contingent Compensation Payment relating to such Change in Ownership or Control, the Company shall determine and notify Executive (with reasonable detail regarding the basis for its determinations) (1) which Potential Payments constitute Contingent Compensation Payments, (2) the Eliminated Amount and (3) whether the Section 4(d)(ii) Override is applicable. Within 30 days after delivery of such notice to Executive, Executive shall deliver a response to the Company (the "Executive Response") stating either (A) that s/he agrees with the Company's determination pursuant to the preceding sentence or (B) that s/he disagrees with such determination, in which case s/he shall set forth (x) which Potential Payments should be characterized as Contingent Compensation Payments, (y) the Eliminated Amount, and (z) whether the Section 4(d)(ii) Override is applicable. In the event that Executive fails to deliver an Executive Response on or before the required date, the Company's initial determination shall be final. If Executive states in the Executive Response that s/he agrees with the Company's determination, the Company shall make the Potential Payments to Executive within three business days following delivery to the Company of the Executive Response (except for any Potential Payments which are not due to be made until after such date, which Potential Payments shall be made on the date on which they are due). If Executive states in the Executive Response that s/he disagrees with the Company's determination, then, for a period of 60 days following delivery of the Executive Response, Executive and the Company shall use good faith efforts to resolve such dispute. If such dispute is not resolved within such 60-day period, such dispute shall be settled exclusively by arbitration in accordance with section 12(h) below. . Judgment may be entered on the arbitrator's award in any court having jurisdiction. The Company shall, within three business days following delivery to the Company of the Executive Response, make to Executive those Potential Payments as to which there is no dispute between the Company and Executive regarding whether they should be made (except for any such Potential Payments which are not due to be made until after such date, which Potential Payments shall be made on the date on which they are due). The balance of the Potential Payments shall be made within three business days following the resolution of such dispute.

(v) The Contingent Compensation Payments to be treated as Eliminated Payments shall be determined by the Company by determining the "Contingent Compensation Payment Ratio" (as defined below) for each Contingent Compensation Payment and then reducing the Contingent Compensation Payments in order beginning with the Contingent Compensation Payment with the highest Contingent Compensation Payment Ratio. For Contingent Compensation Payments with the same Contingent Compensation Payment Ratio, such Contingent Compensation Payment shall be reduced based on the time of payment of such Contingent Compensation Payments with amounts having later payment dates being reduced first. For Contingent Compensation Payments with the same Contingent Compensation Payment Ratio and the same time of payment, such Contingent Compensation Payments shall be reduced on a pro rata basis (but not below zero) prior to reducing Contingent Compensation Payment with a lower Contingent Compensation Payment Ratio. The term "Contingent

Compensation Payment Ratio" shall mean a fraction the numerator of which is the value of the applicable Contingent Compensation Payment that must be taken into account by Executive for purposes of Section 4999(a) of the Code, and the denominator of which is the actual amount to be received by Executive in respect of the applicable Contingent Compensation Payment. For example, in the case of an equity grant that is treated as contingent on the Change in Ownership or Control because the time at which the payment is made or the payment vests is accelerated, the denominator shall be determined by reference to the fair market value of the equity at the acceleration date, and not in accordance with the methodology for determining the value of accelerated payments set forth in Treasury Regulation Section 1.280G-1Q/A-24(b) or (c)).

(vi) The provisions of this Section 4(d) are intended to apply to any and all payments or benefits available to Executive under this Agreement or any other agreement or plan of the Company under which Executive receives Contingent Compensation Payments.

(vii) Notwithstanding Sections 4(d)(i)-(vi) hereof, until the closing of the first underwritten public offering of common stock of the Company, in the event that it shall be determined that any payment or benefit (including any accelerated vesting of options or other equity awards) made or provided, or to be made or provided, by the Company (or any successor thereto or affiliate thereof) to or for the benefit of Executive, whether pursuant to the terms of this Agreement, any other agreement, plan, program or arrangement of or with the Company (or any successor thereto or affiliate thereof) or otherwise, may be subject to the excise tax imposed by Section 4999 of the Code or any comparable tax imposed by any replacement or successor provision of United States tax law, then upon the request of Executive, the Company shall use reasonable efforts to procure a shareholder vote in satisfaction of the shareholder approval requirements described in Treas. Reg. Section 1.280G-1, Q&A-7.

5. Executive Covenants.

(a) Confidential Information. Executive recognizes and acknowledges the competitive and proprietary aspects of the business of the Company, and that as a result of Executive's employment, Executive recognizes and acknowledges that s/he will have access to, and will be involved in the development of, Confidential Information (as defined below) of the Company. As used herein, "Confidential Information" shall mean and include trade secrets, knowledge and other confidential information of the Company, which Executive has acquired, no matter from whom or on what matter such knowledge or information may have been acquired, heretofore or hereafter, concerning the content and details of the business of the Company, and which is not known to the general public, including but not limited to: (a) new products, product betterments and other inventions, formulas, processes, methods, materials, material combinations, manner of preparations, technical production procedures and information, alarm and security codes and procedures, sources of technology, and sources of supply of raw and finished materials and other products; (b) financial and accounting records; (c) the identity of employees, consultants, independent contractors, customers, business development partners, licensees, suppliers, creditors or other parties with which the Company has business dealings, the nature of the relationship with such persons, or any other information relating to such persons or the Company's dealings with such persons; and (d) computer software used by the Company or provided to the customers of the Company unless publicly available.

(i) For as long as Executive is employed and at all times thereafter, Executive shall not, directly or indirectly, communicate, disclose or divulge to any person or entity, or use for Executive's own benefit or the benefit of any person (other than the Company), any Confidential Information, except as permitted in subparagraph (iii) below. Upon termination of Executive's employment, or at any other time at the request of the Company, Executive agrees to deliver promptly to the Company all Confidential Information, including, but not limited to, customer and supplier lists, files and records, in Executive's possession or under Executive's control. Executive further agrees that s/he will not make or retain any copies of any of the foregoing and will so represent to the Company upon termination of Executive's employment.

(ii) Executive shall disclose immediately to the Company any trade secrets or other Confidential Information conceived or developed by Executive at any time during Executive's employment. Executive hereby assigns and agrees to assign to the Company Executive's entire right, title and interest in and to all Confidential Information. Such assignment shall include, without limitation, the rights to obtain patent or copyright protection, thereon in the United States and foreign countries. Executive agrees to provide all reasonable assistance to enable the Company to prepare and prosecute any application before any governmental agency for patent or copyright protection or any similar application with respect to any Confidential Information. Executive further agrees to execute all documents and assignments and to make all oaths necessary to vest ownership of such intellectual property rights in the Company, as the Company may request. These obligations shall apply whether or not the subject thereof was conceived or developed at the suggestion of the Company, and whether or not developed during regular hours of work or while on the premises of the Company. Executive shall at all times, both during and after termination of this Agreement by either Executive or the Company, maintain in confidence and shall not, without prior written consent of the Company, use, except in the course of performance of Executive's duties for the Company or as required by legal process (provided that Executive will promptly notify the Company of such legal process except with respect to any confidential government investigation), disclose or give to others any Confidential Information. In the event Executive is questioned by anyone not employed by the Company or by an employee of or a consultant to the Company not authorized to receive such information, in regard to any such information or any other secret or confidential work of the Company, or concerning any fact or circumstance relating thereto, Executive will promptly notify the Company.

(iii) Nothing in this Agreement, including but not limited to Section 5 (Executive Covenants), including sub-sections 5(a) (Confidential Information) and 5(b) (Non-Competition and Non-Solicitation) and Section 6 (Ownership of Ideas, Copyrights and Patents (Inventions), shall prohibit or restrict Executive, or be construed to prohibit or restrict Executive, from filing a charge or complaint with, reporting possible violations of any law or regulation, making disclosures to (including providing documents or other information), and/or participating in any investigation or proceeding conducted by any self-regulatory organization or governmental agency, authority or legislative body, including, but not limited to, the Securities and Exchange Commission and/or Equal Employment Opportunity Commission or as otherwise required by law.

(iv) Executive is hereby notified that under the Defend Trade Secrets Act: (a) no individual will be held criminally or civilly liable under Federal or State trade secret law for disclosure of a trade secret (as defined in the Economic Espionage Act) that is: (1) made in confidence to a Federal, State, or local government official, either directly or indirectly, or to an attorney, and made solely for the purpose of reporting or investigating a suspected violation of law; or, (2) made in a complaint or other document filed in a lawsuit or other proceeding, if such filing is made under seal so that it is not made public; and (b) an individual who pursues a lawsuit for retaliation by an employer for reporting a suspected violation of the law may disclose the trade secret to the attorney of the individual and use the trade secret information in the court proceeding, if the individual files any document containing the trade secret under seal, and does not disclose the trade secret, except as permitted by court order.

(b) Non-Competition and Non-Solicitation. Executive recognizes that the Company is engaged in a competitive business and that the Company has a legitimate interest in protecting its trade secrets, confidential business information, and customer, business development partner, licensee, supplier, and credit and/or financial relationships. Accordingly, in exchange for valuable consideration, including without limitation Executive's access to confidential business information and continued at-will employment, Executive agrees that, during the Term hereof and for a period of eighteen (18) months thereafter, Executive shall not

(i) directly or indirectly, whether for himself or for any other person or entity, and whether as a proprietor, principal, shareholder, partner, agent, employee, consultant, independent contractor, or in any other capacity whatsoever, undertake or have any interest in (other than the passive ownership of publicly registered securities representing an ownership interest of less than 1%), engage in or assume any role directly competitive with the Company's Field of Interest (or any portion thereof) or any other business in which the Company is engaged and for which the employee has rendered services while employed by the Company, or enter into any agreement to do any of the foregoing; or

(ii) initiate contact with (including without limitation phone calls, press releases and the sending or delivering of announcements), or in any manner solicit, directly or indirectly, any customers, business development partners, licensors, licensees, or creditors (including institutional lenders, bonding companies and trade creditors) of the Company in an attempt to induce or motivate them either to discontinue or modify their then prevailing or future relationship with the Company or to transfer any of their business with the Company to any person or entity other than the Company; or

(iii) initiate contact with, or in any manner solicit, directly or indirectly, any supplier of goods, services or materials to the Company in an attempt to induce or motivate them either to discontinue or modify their then prevailing or future relationship with the Company or to supply the same or similar inventory, goods, services or materials (except generally available inventory, goods, services or materials) to any person or entity other than the Company; or

(iv) directly or indirectly recruit, solicit or otherwise induce or influence any employee or independent contractor of the Company to discontinue or

modify his or her employment or engagement with the Company, or employ or contract with any such employee or contractor for the provision of services.

(c) Definition of "Field of Interest" . The term Company's "Field of Interest" shall mean the research, development and commercialization of products and strategies relating to: (i) therapies for genetic disorders or specific diseases within each of AADC deficiency, Friedreich Ataxia, Angelman, Mitochondrial diseases, Duchenne muscular dystrophy, and (ii) any other diseases or products, in each case, that Executive directly managed or supported during his/her employment with the Company.

(d) Definition of "Customer" . The term "customer" or "customers" shall include any person or entity (a) that is a current customer of the Company, (b) that was a customer of the Company at any time during the preceding twenty-four (24) months or (c) to which the Company made a written presentation for the solicitation of business at any time during the preceding twenty-four (24) months.

(e) Reasonableness of Restrictions. Executive further recognizes and acknowledges that (i) the types of employment which are prohibited by this Section 5 are narrow and reasonable in relation to the skills which represent Executive's principal salable asset both to the Company and to Executive's other prospective employers, and (ii) the broad geographical scope of the provisions of this Section 5 is reasonable, legitimate and fair to Executive in light of the global nature of the Company's business, particularly pharmaceutical research and development and in light of the limited restrictions on the type of employment prohibited herein compared to the types of employment for which Executive is qualified to earn Executive's livelihood.

(f) Remedies. Executive acknowledges that a breach of this Section 5 will cause great and irreparable injury and damage, which cannot be reasonably or adequately compensated by money damages. Accordingly, Executive acknowledges that the remedies of injunction and specific performance shall be available in the event of such a breach, in addition to money damages, costs and attorneys' fees, and other legal or equitable remedies, and that the Company shall be entitled as a matter of course to an injunction pending trial, without the posting of bond or other security. Any period of restriction set forth in this Section 5 shall be extended for a period of time equal to the duration of any breach or violation hereof.

(g) Notification. Any person employing Executive or evidencing any intention to employ Executive may be notified as to the existence and provisions of this Agreement.

(h) Modification of Covenants; Enforceability. In the event that any provision of this Section 5 is held to be in any respect an unreasonable restriction, then the court so holding may modify the terms thereof, including the period of time during which it operates or the geographic area to which it applies, or effect any other change to the extent necessary to render this section enforceable, it being acknowledged by the parties that the representations and covenants set forth herein are of the essence of this Agreement.

(i) Subsidiaries. For purposes of Sections 5 and 6 of this Agreement, "Company" shall include all direct and indirect subsidiaries of the Company. An entity shall be deemed to be a subsidiary of the Company if the Company directly or indirectly owns or controls 50% or more of the equity interest in such entity.

6. Ownership of Ideas, Copyrights and Patents.

(a) Property of the Company. Executive agrees that all ideas, discoveries, creations, manuscripts and properties, innovations, improvements, know-how, inventions, designs, developments, apparatus, techniques, methods, biological processes, cell lines, laboratory notebooks and formulae, whether patentable, copyrightable or not, which Executive may conceive, reduce to practice or develop, alone or in conjunction with another, or others, whether during or out of regular business hours, and whether at the request or upon the suggestion of the Company, or otherwise, in the course of performing services for the Company in any capacity, whether heretofore or hereafter, (collectively, "the Inventions") are and shall be the sole and exclusive property of the Company, and that Executive shall not publish any of the Inventions without the prior written consent of the Company. Executive hereby assigns to the Company all of Executive's right, title and interest in and to all of the foregoing. Executive further represents and agrees that to the best of Executive's knowledge and belief none of the Inventions will violate or infringe upon any right, patent, copyright, trademark or right of privacy, or constitute libel or slander against or violate any other rights of any person, firm or corporation and that Executive will use his/her best efforts to prevent any such violation.

(b) Cooperation. At any time during or after the Term, Executive agrees that s/he will fully cooperate with the Company, its attorneys and agents in the preparation and filing of all papers and other documents as may be required to perfect the Company's rights in and to any of such Inventions, including, but not limited to, executing any lawful document (including, but not limited to, applications, assignments, oaths, declarations and affidavits) and joining in any proceeding to obtain letters patent, copyrights, trademarks or other legal rights of the United States and of any and all other countries on such Inventions, provided that any patent or other legal right so issued to Executive, personally, shall be assigned by Executive to the Company without charge by Executive. Executive further designates the Company as his/her agent for, and grants to the Company a power of attorney with full power of substitution, which power of attorney shall be deemed coupled with an interest, for the purpose of effecting the foregoing assignments from Executive to the Company. Company will bear the reasonable expenses which it causes to be incurred in Executive's assisting and cooperating hereunder. Executive waives all claims to moral rights in any Inventions.

7. Disclosure to Future Employers. The Company may provide in its discretion, a copy of this Agreement (in whole or in part, including the covenants contained in Sections 5 and 6 of this Agreement) to: (a) any business or enterprise which Executive may directly, or indirectly, own, manage, operate, finance, join, control or in which Executive participates in the ownership, management, operation, financing, or control, or with which Executive may be connected as an officer, director, employee, partner, principal, agent, representative, consultant or otherwise, or (b) any third party who may be affected by the restrictive covenants in this Agreement.

8. Records. Upon termination of Executive's relationship with the Company, and at any time requested by the Company, Executive shall deliver to the Company any property of the

Company which may be in Executive's possession including products, materials, memoranda, notes, records, reports, or other documents or photocopies of the same.

9. Insurance. The Company, in its sole discretion, may apply for and procure in its own name (whether or not for its own benefit) policies of insurance insuring Executive's life. Executive agrees to submit to reasonable medical or other examinations and to execute and deliver any applications or other instruments in writing that are reasonably necessary to effectuate such insurance. No adverse employment actions may be based upon the results of any such exam or the failure by the Company to obtain such insurance.

10. No Conflicting Agreements. Executive hereby represents and warrants that Executive has no commitments or obligations inconsistent with this Agreement.

11. "Market Stand-Off" Agreement. Executive agrees, if requested by the Company and an underwriter of common stock (or other securities) of the Company, not to sell or otherwise transfer or dispose of any common stock (or other securities) of the Company held by Executive during a period not to exceed one hundred and eighty (180) days following the effective date of the first underwritten public offering of common stock of the Company, offered on a firm commitment basis pursuant to a registration statement filed with the Securities and Exchange Commission (or any successor agency of the Federal government administrating the Securities Act of 1933, as amend, and the Securities Exchange Act of 1934, as amended) under the Securities Act of 1933, as amended, on Form S-1 or its then equivalent, and to enter into an agreement to such effect. The Company may impose stop-transfer instructions with respect to the shares (or securities) subject to the foregoing restriction until the end of said period.

12. General.

(a) Notices. All notices, requests, consents and other communications hereunder shall be in writing, shall be addressed to the receiving party's address as follows:

If to the Company: PTC Therapeutics Inc.
100 Corporate Court
South Plainfield, NJ 07080
USA
Attention: Legal Department
Telephone: (908) 222-7000

With an email copy to: legal@ptcbio.com

If to Executive: Lee S. Golden, M.D.

or to such other address as a party may designate by notice hereunder, and shall be either (i) delivered by hand, (ii) sent by overnight courier, or (iii) sent by registered or certified mail, return receipt requested, postage prepaid. All notices, requests, consents and other communications hereunder shall be deemed to have been given either (i) if by hand, at the time of the delivery thereof to the receiving party at the address of such party set forth above, (ii) if sent by overnight courier, on the next business day following the day such notice is delivered to

the courier service, or (iii) if sent by registered or certified mail, on the fifth (5th) business day following the day such mailing is made.

(b) Entire Agreement. This Agreement embodies the entire agreement and understanding between the parties hereto with respect to the subject matter hereof and supersedes all prior oral or written agreements and understandings relating to the subject matter hereof, except with respect to the equity and fringe benefit arrangements referred to in Subsections 3(c) and (e) above. No statement, representation, warranty, covenant or agreement of any kind not expressly set forth in this Agreement shall affect, or be used to interpret, change or restrict, the express terms and provisions of this Agreement.

(c) Modifications and Amendments. The terms and provisions of this Agreement may be modified or amended only by written agreement executed by the parties hereto.

(d) Waivers and Consents. The terms and provisions of this Agreement may be waived, or consent for the departure therefrom granted, only by written document executed by the party entitled to the benefits of such terms or provisions. No such waiver or consent shall be deemed to be or shall constitute a waiver or consent with respect to any other terms or provisions of this Agreement, whether or not similar. Each such waiver or consent shall be effective only in the specific instance and for the purpose for which it was given, and shall not constitute a continuing waiver or consent.

(e) Assignment. The Company shall assign its rights and obligations hereunder to any person or entity that succeeds to all or substantially all of the Company's business or that aspect of the Company's business in which Executive is principally involved. Executive may not assign Executive's rights and obligations under this Agreement without the prior written consent of the Company.

(f) Benefit. All statements, representations, warranties, covenants and agreements in this Agreement shall be binding on the parties hereto and shall inure to the benefit of the respective successors and permitted assigns of each party hereto. Nothing in this Agreement shall be construed to create any rights or obligations except among the parties hereto, and no person or entity shall be regarded as a third-party beneficiary of this Agreement.

(g) Governing Law. This Agreement and the rights and obligations of the parties hereunder shall be construed in accordance with and governed by the laws of The State of New Jersey, without giving effect to the conflict of law principles thereof.

(h) Arbitration. Executive and the Company agree that any legal dispute or controversy arising out of, relating to, or concerning the formation, existence, scope, validity, enforceability or breach of this Agreement or Executive's employment with the Company ("Arbitrable Claims"), shall be resolved by final and binding arbitration in accordance with the JAMS Employment Arbitration Rules & Procedures ("JAMS Rules") then in effect, and not by court or jury trial, to be held (unless the parties agree in writing otherwise) within 45 miles of and in the same state where the Executive was last employed by the Company. The arbitrator shall be an attorney experienced in arbitrating employment law disputes or a retired judge. The JAMS Rules may be found at www.jamsadr.com or by searching for "JAMS Employment Arbitration Rules" using a service such as www.google.com. If for any reason the JAMS will not

administer the arbitration, either party may apply to a court of competent jurisdiction with authority over the location where the arbitration will be conducted for appointment of a neutral Arbitrator Executive understands and agrees that notwithstanding the foregoing, the Company may pursue legal or equitable relief against Executive in the event of a breach of a restrictive covenant as per Section 5(f) above.

(i) Severability. The parties intend this Agreement to be enforced as written. However, (i) if any portion or provision of this Agreement shall to any extent be declared illegal or unenforceable by a duly authorized court having jurisdiction, then the remainder of this Agreement, or the application of such portion or provision in circumstances other than those as to which it is so declared illegal or unenforceable, shall not be affected thereby, and each portion and provision of this Agreement shall be valid and enforceable to the fullest extent permitted by law; and (ii) if any provision, or part thereof, is held to be unenforceable because of the duration of such provision or the geographic area covered thereby or otherwise, the Company and Executive agrees that the court or arbitrator making such determination shall have the power to reduce the duration and/or geographic area of such provision, and/or to delete specific words and phrases ("blue-penciling"), and in its reduced or blue-penciled form such provision shall then be enforceable and shall be enforced.

(j) Headings and Captions; Interpretation. The headings and captions of the various subdivisions of this Agreement are for convenience of reference only and shall in no way modify, or affect the meaning or construction of any of the terms or provisions hereof. The provisions of the following Sections of this Agreement are in addition to, and do not limit, each other: Sections 6 and 5(a); Sections 7 and 5(g); Sections 12(k) and 12(f); and Sections 12(l) and 12(d).

(k) Injunctive Relief. Executive hereby expressly acknowledges that any breach or threatened breach of any of the terms and/or conditions set forth in Section 5 or 6 of this Agreement will result in substantial, continuing and irreparable injury to the Company. Therefore, Executive hereby agrees that, in addition to any other remedy that may be available to the Company, the Company shall be entitled to injunctive or other equitable relief by a court of appropriate jurisdiction.

(l) No Waiver of Rights, Powers and Remedies. No failure or delay by a party hereto in exercising any right, power or remedy under this Agreement, and no course of dealing between the parties hereto, shall operate as a waiver of any such right, power or remedy of the party. No single or partial exercise of any right, power or remedy under this Agreement by a party hereto, nor any abandonment or discontinuance of steps to enforce any such right, power or remedy, shall preclude such party from any other or further exercise thereof or the exercise of any other right, power or remedy hereunder. The election of any remedy by a party hereto shall not constitute a waiver of the right of such party to pursue other available remedies. No notice to or demand on a party not expressly required under this Agreement shall entitle the party receiving such notice or demand to any other or further notice or demand in similar or other circumstances or constitute a waiver of the rights of the party giving such notice or demand to any other or further action in any circumstances without such notice or demand.

(m) Counterparts. This Agreement may be executed in one or more counterparts, and by different parties hereto on separate counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument.

(n) Survival. The provisions of Sections 4, 5, 6, 7, 8, 11 and 12 shall survive the termination of this Agreement and Executive's employment hereunder in accordance with their terms.

(o) Knowing and Voluntary Nature of Agreement. Executive acknowledges and agrees that Executive is executing this Agreement knowingly and voluntarily and without any duress or undue influence by PTC or anyone else. Executive further acknowledges and agrees that Executive has carefully read this Agreement and fully understands it, including that Executive is waiving the right to a jury trial. Executive further agrees that Executive has been provided an opportunity to seek the advice of an attorney of Executive's choice before signing this Agreement.

IN WITNESS THEREOF, the parties hereto have executed this Agreement as of the day and year first above written.

PTC Therapeutics, Inc.

/s/ Mark E. Boulding
Name: Mark E. Boulding
Title: EVP, Chief Legal Officer

Agreed and Accepted

/s/ Lee S. Golden, M.D.
Name: Lee S. Golden, M.D.

EXHIBIT A

Sample Separation and Release Agreement

[Insert Date]

[Insert Employee Name]
[Insert Employee Address]

Dear **[Insert Employee Name]**:

In connection with the termination of your employment with PTC Therapeutics, Inc. (the "Company") on **[Termination Date]**, you are eligible to receive the Severance Compensation as described in Section 4 of the Employment Agreement executed between you and the Company on [**Insert Date**] (the "Employment Agreement") if you sign and return this letter agreement to me by **[Return Date – e.g., 21 days from date of receipt of this letter agreement]** and it becomes binding between you and the Company. By signing and returning this letter agreement [and not revoking your acceptance], you will be agreeing to the terms and conditions set forth in the numbered paragraphs below, including the release of claims set forth in paragraph 3. Therefore, you are advised to consult with an attorney before signing this letter agreement and you may take up to [twenty-one (21) days] to do so. [If you sign this letter agreement, you may change your mind and revoke your agreement during the seven (7) day period after you have signed it by notifying me in writing. If you do not so revoke, this letter agreement will become a binding agreement between you and the Company upon the expiration of the seven (7) day period.]

If you choose not to sign and return this letter agreement by **[Return Date-Same as Above][**, or if you timely revoke your acceptance in writing], you shall not receive any Severance Compensation from the Company. You will, however, receive payment for your final wages and any unused vacation time accrued through the Termination Date, as defined below. Also, regardless of signing this letter agreement, you may elect to continue receiving group medical insurance pursuant to the federal "COBRA" law, 29 U.S.C. § 1161 et seq. If you so elect, you shall pay all premium costs on a monthly basis for as long as, and to the extent that, you remain eligible for COBRA continuation. You should consult the COBRA materials to be provided by the Company for details regarding these benefits. All other benefits will cease upon your Termination Date in accordance with the plan documents.

The following numbered paragraphs set forth the terms and conditions that will apply if you timely sign and return this letter agreement and do not revoke it in writing within the seven (7) day period.

1. **Termination Date** – Your effective date of termination from the Company is **[Insert Date]** (the "Termination Date").

2. **Release** – In consideration of the payment of the Severance Compensation, which you acknowledge you would not otherwise be entitled to receive, you hereby fully, forever, irrevocably and unconditionally release, remise and discharge the Company, its affiliates, subsidiaries, parent companies, predecessors, and successors, and all of their respective past and present officers, directors, stockholders, partners, members, employees, agents,

representatives, plan administrators, attorneys, insurers and fiduciaries (each in their individual and corporate capacities) (collectively, the "Released Parties") from any and all claims, charges, complaints, demands, actions, causes of action, suits, rights, debts, sums of money, costs, accounts, reckonings, covenants, contracts, agreements, promises, doings, omissions, damages, executions, obligations, liabilities, and expenses (including attorneys' fees and costs), of every kind and nature that you ever had or now have against any or all of the Released Parties, including, but not limited to, any and all claims arising out of or relating to your employment with and/or separation from the Company, including, but not limited to, all claims under Title VII of the Civil Rights Act of 1964, 42 U.S.C. § 2000e et seq., the Americans With Disabilities Act of 1990, 42 U.S.C. § 12101 et seq., the Age Discrimination in Employment Act, 29 U.S.C. § 621 et seq., the Genetic Information Nondiscrimination Act of 2008, 42 U.S.C. § 2000ff et seq., the Family and Medical Leave Act, 29 U.S.C. § 2601 et seq., the Worker Adjustment and Retraining Notification Act ("WARN"), 29 U.S.C. § 2101 et seq., the Rehabilitation Act of 1973, 29 U.S.C. § 701 et seq., Executive Order 11246, Executive Order 11141, the Fair Credit Reporting Act, 15 U.S.C. § 1681 et seq., and the Employee Retirement Income Security Act of 1974 ("ERISA"), 29 U.S.C. § 1001 et seq., all as amended; all claims arising out of the New Jersey Law Against Discrimination, N.J. Stat. Ann. § 10:5-1 et seq., the New Jersey Family Leave Act, N.J. Stat. Ann. § 34:11B-1 et seq., the New Jersey Conscientious Employee Protection Act, N.J. Stat. Ann. § 34:19-1 et seq., and the N.J. Stat. Ann. § 34:11-56.1 et seq. (New Jersey equal pay law), all as amended; all common law claims including, but not limited to, actions in defamation, intentional infliction of emotional distress, misrepresentation, fraud, wrongful discharge, and breach of contract, including without limitation, all claims arising from the Employment Agreement; all state and federal whistleblower claims to the maximum extent permitted by law; all claims to any non-vested ownership interest in the Company, contractual or otherwise; and any claim or damage arising out of your employment with and/or separation from the Company (including a claim for retaliation) under any common law theory or any federal, state or local statute or ordinance not expressly referenced above; provided, however, that nothing in this letter agreement shall (i) prevent you from filing a charge with, cooperating with, or participating in any proceeding before the Equal Employment Opportunity Commission or a state fair employment practices agency (except that you acknowledge that you may not recover any monetary benefits in connection with any such claim, charge or proceeding) or (ii) deprive you of any rights you may have to be indemnified by the Company as provided in any agreement between the Company and you or pursuant to the Company's Certificate of Incorporation or by-laws.

3. **Non-Disclosure, Non-Competition, Confidential Information and Non- Solicitation and Inventions**– You acknowledge and reaffirm your obligations to keep confidential and not disclose all non-public information concerning the Company with respect to Confidential Information, non-solicitation, and Inventions and its clients that you acquired during the course of your employment with the Company, as stated more fully in Sections 5 and 6 of the Employment Agreement, which remains in full force and effect.

4. **Return of Company Property** – You acknowledge and reaffirm your obligations to the Company with respect to Company property, as stated more fully in Section 6 and 8 of

the Employment Agreement. You confirm that you have returned to the Company all keys, files, records (and copies thereof), equipment (including, but not limited to, computer hardware, software and printers, wireless handheld devices, cellular phones, smartphones, tablets, etc.), Company identification, and any other Company-owned property in your possession or control and have left intact all electronic Company documents, including but not limited to those which you developed or helped to develop during your employment. You further confirm that you have cancelled all accounts for your benefit, if any, in the Company's name, including but not limited to, credit cards, telephone charge cards, cellular phone and/or wireless data accounts and computer accounts.

5. **Business Expenses and Final Compensation** – You acknowledge that you have been reimbursed by the Company for all business expenses incurred in conjunction with the performance of your employment and that no other reimbursements are owed to you. You further acknowledge that you have received payment in full for all services rendered in conjunction with your employment by the Company, including payment for all wages, bonuses and accrued, unused vacation time, and that no other compensation is owed to you except as provided herein.

6. **Non-Disparagement** – To the extent permitted by law, you understand and agree that as a condition for payment to you of the Severance Compensation herein described, you shall not make any false, disparaging or derogatory statements to any person or entity, including any media outlet, regarding the Company or any of its directors, officers, employees, agents or representatives or about the Company's business affairs and financial condition.

7. **Continued Assistance** – You acknowledge and reaffirm your obligations to the Company with respect to cooperation, as stated more fully in Section 6 of the Employment Agreement. You agree that after the Termination Date you will provide all reasonable cooperation to the Company, including but not limited to, assisting the Company transition your job duties, assisting the Company in defending against and/or prosecuting any litigation or threatened litigation, and performing any other tasks as reasonably requested by the Company.

8. **Cooperation** – To the extent permitted by law, you agree to cooperate fully with the Company in the defense or prosecution of any claims or actions which already have been brought, are currently pending, or which may be brought in the future against or on behalf of the Company**,** whether before a state or federal court, any state or federal government agency, or a mediator or arbitrator. Your full cooperation in connection with such claims or actions shall include, but not be limited to, being available to meet with counsel to prepare its claims or defenses, to prepare for trial or discovery or an administrative hearing or a mediation or arbitration and to act as a witness when requested by the Company at reasonable times designated by the Company. You agree that you will notify the Company promptly in the event that you are served with a subpoena or in the event that you are asked to provide a third party with information concerning any actual or potential complaint or claim against the Company.

9. **Amendment and Waiver** – This letter agreement shall be binding upon the parties and may not be modified in any manner, except by an instrument in writing of concurrent or subsequent date signed by duly authorized representatives of the parties hereto. This letter agreement is binding upon and shall inure to the benefit of the parties and their respective agents, assigns, heirs, executors, successors and administrators. No delay or omission by the Company in exercising any right under this letter agreement shall operate as a waiver of that or any other right. A waiver or consent given by the Company on any one occasion shall be effective only in that instance and shall not be construed as a bar to or waiver of any right on any other occasion.

10. **Validity** – Should any provision of this letter agreement be declared or be determined by any court of competent jurisdiction to be illegal or invalid, the validity of the remaining parts, terms or provisions shall not be affected thereby and said illegal or invalid part, term or provision shall be deemed not to be a part of this letter agreement.

11. **Confidentiality** – To the extent permitted by law, you understand and agree that as a condition for payment to you of the Severance Compensation herein described, the terms and contents of this letter agreement, and the contents of the negotiations and discussions resulting in this letter agreement, shall be maintained as confidential by you and your agents and representatives and shall not be disclosed except to the extent required by federal or state law or as otherwise agreed to in writing by the Company.

12. **Nature of Agreement** – You understand and agree that this letter agreement is a severance agreement and does not constitute an admission of liability or wrongdoing on the part of the Company.

13. **Acknowledgments –** You acknowledge that you have been given at least [twenty-one (21) days] to consider this letter agreement, and that the Company advised you to consult with an attorney of your own choosing prior to signing this letter agreement. [You understand that you may revoke this letter agreement for a period of seven (7) days after you sign this letter agreement by notifying me in writing, and the letter agreement shall not be effective or enforceable until the expiration of this seven (7) day revocation period.] You understand and agree that by entering into this agreement, you are waiving any and all rights or claims you might have under the Age Discrimination in Employment Act, as amended by the Older Workers Benefits Protection Act, and that you have received consideration beyond that to which you were previously entitled.

14. **Voluntary Assent** – You affirm that no other promises or agreements of any kind have been made to or with you by any person or entity whatsoever to cause you to sign this letter agreement, and that you fully understand the meaning and intent of this letter agreement. You state and represent that you have had an opportunity to fully discuss and review the terms of this letter agreement with an attorney. You further state and represent that you have carefully read this letter agreement, understand the contents herein, freely and voluntarily assent to all of the terms and conditions hereof and sign your name of your own free act.

15. **Protected Conduct** – Nothing in this Agreement shall prohibit or restrict you, or be construed to prohibit or restrict you, from filing a charge or complaint with, reporting

possible violations of any law or regulation, making disclosures to (including providing documents or other information), and/or participating in any investigation or proceeding conducted by any self-regulatory organization or governmental agency, authority or legislative body, including, but not limited to, the Securities and Exchange Commission and/or Equal Employment Opportunity Commission or as otherwise required by law.

16. **Applicable Law** – This letter agreement shall be interpreted and construed by the laws of the State of New Jersey, without regard to conflict of laws provisions. You hereby irrevocably submit to and acknowledge and recognize the jurisdiction of the courts of the State of New Jersey, or if appropriate, a federal court located in the State of New Jersey (which courts, for purposes of this letter agreement, are the only courts of competent jurisdiction), over any suit, action or other proceeding arising out of, under or in connection with this letter agreement or the subject matter hereof.

17. **Entire Agreement** – This letter agreement contains and constitutes the entire understanding and agreement between the parties hereto with respect to your Severance Compensation and the settlement of claims against the Company and cancels all previous oral and written negotiations, agreements and commitments in connection therewith, except as otherwise set forth herein. For example, nothing in this paragraph shall modify, cancel or supersede your obligations set forth in paragraph 3 herein.

18. **Tax Acknowledgement** – In connection with the payments and consideration provided to you pursuant to this letter agreement, the Company shall withhold and remit to the tax authorities the amounts required under applicable law, and you shall be responsible for all applicable taxes with respect to such payments and consideration under applicable law. You acknowledge that you are not relying upon the advice or representation of the Company with respect to the tax treatment of any of the Severance Compensation set forth in Section 4 of the Employment Agreement.

If you have any questions about the matters covered in this letter agreement, please call me at **[Insert Phone Number]**.

Very truly yours,

By: _____
[Name]
[Title]

I hereby agree to the terms and conditions set forth above. I have been given at least [twenty-one (21) days] to consider this letter agreement and I have chosen to execute this on the date below. I intend that this letter agreement will become a binding agreement between me and the Company [if I do not revoke my acceptance in seven (7) days].

_____ _____
[Insert Employee Name] Date

To be returned to me by **[Return Date – e.g., 21 days from date of receipt of this letter]**.

January 6, 2022

Lee Golden

Dear Lee,

Congratulations on your promotion to Chief Medical Officer. Your success with PTC has been impressive and we look forward to your continued contributions to PTC's success. The effective date of your promotion is January 7, 2022. In this new role you will continue to report to Matthew Klein.

Outlined below are details of your promotion:

- Your new annual base salary is $450,000.00 annually, subject to deductions for taxes and other withholdings as required by law. This represents an increase of 8.17% over your current base salary. Please allow 1 to 2 pay periods for your new base salary to be reflected in your paycheck.

- Your new bonus target will continue to be 40% of your annual salaried earnings paid in accordance with the terms of conditions of PTC's incentive compensation plan.

- You will receive a one-time grant of 10,000 Stock Options and 6,000 RSUs, each of which will vest according to the normal vesting schedule.

- The terms and conditions of your existing Employment Agreement remain in effect.

On behalf of PTC, let me again congratulate you on your promotion. We look forward to this next step in your career. Please feel free to contact me if you have any questions concerning your promotion.

Sincerely,

/s/ Karen Koubek
Karen Koubek
Executive Director, Human Resources
Cc: Matthew Klein

PTC Therapeutics, Inc.
100 Corporate Court
South Plainfield, NJ 07080
908.222.7000
www.ptcbio.com

April 28, 2023

Lee Golden

Dear Lee,

Congratulations on your promotion to EVP & Chief Medical Officer of PTC Therapeutics, Inc. Your success with PTC has been impressive and we look forward to your continued contributions to PTC's business and mission. The effective date of your promotion is April 1, 2023, following receipt of a signed copy of this letter from you indicating your acceptance. In this new role you will report to our CEO Matt Klein

Outlined below are details of your promotion:

- Your annual base salary will be increased to $525,000 annually, subject to deductions for taxes and other withholdings as required by law. Please allow 1 to 2 pay periods for your new base salary to be reflected in your paycheck.
- Your bonus target will increase to 45.00% of your annual salaried earnings paid in accordance with the terms of conditions of PTC's annual incentive compensation plan.
- You will receive a one-time grant of 21,000 stock options to purchase shares of common stock of PTC, and 8,400 restricted stock units**,** subject to formal approval by the Compensation Committee of the Board of Directors (or a majority of PTC's independent directors) and to the terms of the applicable grant agreements. The options will vest over four years, with 25% vesting on the one-year anniversary of your grant date and 6.25% vesting every three-month period thereafter over the following three years. The restricted stock units will vest over four years, with 25% vesting annually on the anniversary of the grant date.
- This letter supersedes any other recent discussions or communications from PTC with respect to changes in your pay, title, role in the organization, or equity grants. All other terms of your employment with PTC will remain consistent with existing signed employment and equity agreements and applicable policies.

On behalf of PTC, let me again congratulate you on your promotion. Please return a signed copy of this letter to me by close of business on Friday, May 5th, 2023. Feel free to contact me if you have any questions concerning this letter.

Sincerely,

/s/ Hege Sollie-Zetlamyer
Hege Sollie-Zetlmayer
SVP, Human Resources

Accepted by,

/s/ Lee Golden
Lee Golden
EVP, Chief Medical Officer

Cc: Matt Klein

Exhibit 31.1

CERTIFICATIONS

I, Matthew B. Klein, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: April 25, 2024

By: /s/ MATTHEW B. KLEIN
Matthew B. Klein
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATIONS

I, Pierre Gravier, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: April 25, 2024

By: /s/ PIERRE GRAVIER
Pierre Gravier
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, 2024 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Matthew B. Klein, Chief Executive Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his knowledge:

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

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

Date: April 25, 2024

By: /s/ MATTHEW B. KLEIN
Matthew B. Klein
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, 2024 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Pierre Gravier, Chief Financial Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his knowledge:

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

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

Date: April 25, 2024

By: /s/ PIERRE GRAVIER
Pierre Gravier
Chief Financial Officer
(Principal Financial Officer)