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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended March 31, 2025

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 May 4, 2025, there were 79,257,019 shares of Common Stock, $0.001 par value per share, outstanding.

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FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Quarterly Report on Form 10-Q, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "might," "plan," "predict," "project," "target," "potential," "will," "would," "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:

- the outcome of pricing, coverage and reimbursement negotiations with third-party payors for our products or product candidates that we commercialize or may commercialize in the future;
- expectations with respect to sepiapterin for the treatment of phenylketonuria, including any regulatory submissions and potential approvals, commercialization, and the potential achievement of regulatory and sales milestones and contingent payments that we may be obligated to make
- our ability to maintain our marketing authorization of Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, in Brazil, Russia and other regions in which Translarna has been approved;
- the effect of the European Commission's adoption of the Committee for Medicinal Products for Human Use's negative opinion not to renew the conditional marketing authorization for Translarna in the European Economic Area on other regulatory bodies;
- our ability to use the results of Study 041, a randomized, 18-month, placebo-controlled clinical trial of Translarna for the treatment of nmDMD followed by an 18-month open-label extension, and from our international drug registry study to support a marketing approval for Translarna for the treatment of nmDMD in the United States;
- whether investigators agree with our interpretation of the results of clinical trials and the totality of clinical data from our trials of Translarna;
- expectations with respect to our license and collaboration agreement with Novartis Pharmaceuticals Corporation, or Novartis, including our right to receive any development, regulatory and sales milestones, and profit sharing and royalty payments from Novartis;
- expectations with respect to vatiquinone for the treatment of Friedreich's ataxia, including any regulatory submissions and potential approvals, commercialization, and the potential achievement of regulatory and sales milestones and contingent payments that we may be obligated to make;
- expectations with respect to Upstaza/Kebilidi, including commercialization, manufacturing capabilities, and the potential achievement of 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 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;
- 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;
- our ability to complete any post-marketing requirements imposed by regulatory agencies with respect to our products;
- our ability to satisfy our obligations under the terms of our lease agreements;
- 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;
- the timing and conduct of our ongoing, planned and potential future clinical trials and studies for our splicing and inflammation and ferroptosis 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 plans to advance our earlier stage programs and pursue research and development of other product candidates, including our splicing and inflammation and ferroptosis 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, 2024, 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, 2024 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, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 1,480,231	$ 779,709
Marketable securities	546,950	359,987
Trade and royalty receivables, net	210,495	158,554
Inventory, net	21,675	23,194
Prepaid expenses and other current assets	57,055	44,087
Total current assets	2,316,406	1,365,531
Fixed assets, net	59,459	60,970
Intangible assets, net	118,448	118,794
Goodwill	82,341	82,341
Operating lease ROU assets	57,317	56,685
Deposits and other assets	21,416	20,703
Total assets	$ 2,655,387	$ 1,705,024
Liabilities and stockholders' deficit		
Current liabilities:		
Accounts payable and accrued expenses	$ 317,807	$ 304,292
Deferred revenue	12,833	5,505
Operating lease liabilities- current	11,464	10,363
Finance lease liabilities- current	5,047	3,000
Liability for sale of future royalties- current	248,240	257,821
Total current liabilities	595,391	580,981
Long-term debt	285,712	285,412
Contingent consideration payable	—	800
Operating lease liabilities- noncurrent	73,559	74,947
Finance lease liabilities- noncurrent	13,833	15,574
Liability for sale of future royalties- noncurrent	1,850,223	1,823,955
Other long-term liabilities	22,429	21,426
Total liabilities	2,841,147	2,803,095
Stockholders' deficit:		
Common stock, $0.001 par value. Authorized 250,000,000 shares; issued and outstanding 79,225,276 shares at March 31, 2025. Authorized 250,000,000 shares; issued and outstanding 77,704,188 shares at December 31, 2024.	78	77
Additional paid-in capital	2,608,422	2,574,611
Accumulated other comprehensive loss	(13,949)	(25,886)
Accumulated deficit	(2,780,311)	(3,646,873)
Total stockholders' deficit	(185,760)	(1,098,071)
Total liabilities and stockholders' deficit	$ 2,655,387	$ 1,705,024

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,	
	2025	2024
Revenues:		
Net product revenue	$ 153,426	$ 177,604
Collaboration and license revenue	986,231	—
Royalty revenue	36,439	31,154
Manufacturing revenue	—	1,360
Total revenues	1,176,096	210,118
Operating expenses:		
Cost of product, collaboration and license sales, excluding amortization of acquired intangible assets	12,862	14,740
Amortization of acquired intangible assets	3,798	51,530
Research and development	108,973	116,129
Selling, general and administrative	80,961	73,272
Change in the fair value of contingent consideration	(800)	(100)
Tangible asset impairment and losses on transactions, net	77	—
Total operating expenses	205,871	255,571
Income (loss) from operations	970,225	(45,453)
Interest expense, net	(34,092)	(40,834)
Other (expense) income, net	(6,305)	1,591
Income (loss) before income tax expense	929,828	(84,696)
Income tax expense	(63,266)	(6,880)
Net income (loss) attributable to common stockholders	$ 866,562	$ (91,576)
Weighted-average shares outstanding:		
Basic (in shares)	78,115,836	76,496,127
Diluted (in shares)	86,385,922	76,496,127
Net income (loss) per share—basic (in dollars per share)	$ 11.09	$ (1.20)
Net income (loss) per share—diluted (in dollars per share)	$ 10.04	$ (1.20)

See accompanying unaudited notes.

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

	Three Months Ended March 31,	
	2025	2024
Net income (loss)	$ 866,562	$ (91,576)
Other comprehensive income (loss):		
Unrealized loss on marketable securities, net of tax	(88)	(444)
Foreign currency translation gain (loss), net of tax	12,025	(3,831)
Comprehensive income (loss)	$ 878,499	$ (95,851)

See accompanying unaudited notes.

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

Three months ended March 31, 2025	Common stock		Additional paid-in capital	Accumulated other comprehensive (loss) income	Accumulated deficit	Total stockholders' deficit
	Shares	Amount				
Balance, December 31, 2024	77,704,188	$ 77	$ 2,574,611	$ (25,886)	$ (3,646,873)	$ (1,098,071)
Exercise of options	431,089	—	15,751	—	—	15,751
Restricted stock vesting and issuance, net	1,089,999	1	—	—	—	1
Share-based compensation expense	—	—	18,060	—	—	18,060
Net income	—	—	—	—	866,562	866,562
Comprehensive income	—	—	—	11,937	—	11,937
Balance, March 31, 2025	79,225,276	$ 78	$ 2,608,422	$ (13,949)	$ (2,780,311)	$ (185,760)

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)

See accompanying unaudited notes.

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

	Three Months Ended March 31,	
	2025	2024
Cash flows from operating activities		
Net income (loss)	$ 866,562	$ (91,576)
Adjustments to reconcile net income (loss) to net cash provided by operating activities:		
Depreciation and amortization	7,221	55,395
Non-cash operating lease expense	1,790	1,792
Non-cash royalty revenue related to sale of future royalties	(32,974)	(25,228)
Non-cash interest expense on liability related to sale of future royalties	49,661	50,064
Change in valuation of contingent consideration	(800)	(100)
Loss on sale of fixed assets	77	—
Unrealized loss (gain) on ClearPoint Equity Investments	3,122	(9)
Unrealized gain on ClearPoint convertible debt security	—	(166)
Unrealized gain on marketable securities - equity investments	(774)	(710)
Disposal of asset	—	544
Deferred income taxes	2	(1)
Amortization of discounts on investments, net	(2,313)	(3,697)
Amortization of debt issuance costs	299	296
Share-based compensation expense	18,060	18,378
Unrealized foreign currency transaction losses, net	2,251	269
Changes in operating assets and liabilities:		
Inventory, net	2,191	120
Prepaid expenses and other current assets	(16,429)	93,904
Trade and royalty receivables, net	(47,792)	(39,500)
Deposits and other assets	(611)	11,192
Accounts payable and accrued expenses	18,502	(5,389)
Other liabilities	(458)	5,984
Deferred revenue	7,200	(801)
Payments on contingent consideration	(4,684)	—
Net cash provided by operating activities	$ 870,103	$ 70,761
Cash flows from investing activities		
Purchases of fixed assets	$ (1,724)	$ (9,588)
Proceeds from sale of fixed assets	70	—
Purchases of marketable securities- available for sale	(322,335)	(104,373)
Purchases of marketable securities- equity investments	(5,209)	(9,065)
Sale and redemption of marketable securities- available for sale	136,750	61,650
Sale and redemption of marketable securities- equity investments	8,896	1,207
Acquisition of product rights and licenses	(808)	(54,763)
Net cash used in investing activities	$ (184,360)	$ (114,932)
Cash flows from financing activities		
Proceeds from exercise of options	$ 15,751	$ 2,030
Payments on contingent consideration obligation	(6,341)	—
Payment of finance lease principal	—	(1,490)
Net cash provided by financing activities	$ 9,410	$ 540
Effect of exchange rate changes on cash	5,393	(2,030)
Net increase (decrease) in cash and cash equivalents	700,546	(45,661)
Cash and cash equivalents, and restricted cash beginning of period	795,316	610,284
Cash and cash equivalents, and restricted cash end of period	$ 1,495,862	$ 564,623
Supplemental disclosure of cash information		
Cash paid for interest	$ 2,156	$ 3,666
Cash paid for income taxes	3,040	2,042
Supplemental disclosure of non-cash investing and financing activity		
Unrealized loss on marketable securities, net of tax	$ (88)	$ (444)
Right-of-use assets obtained in exchange for operating lease obligations	850	1,723
Acquisition of product rights and licenses	2,185	2,296
Fixed asset additions through tenant improvement allowance	374	—
Milestone payable	—	2,500

See accompanying unaudited notes.

PTC Therapeutics, Inc.

Notes to Consolidated Financial Statements (unaudited)
March 31, 2025
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 that discovers, develops and commercializes clinically differentiated medicines that provide benefits to children and adults living 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 Russia for the treatment of nonsense mutation Duchenne muscular dystrophy ("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 previously had a marketing authorization for Translarna in the European Economic Area ("EEA"), which had been 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. 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. In January 2024, the CHMP issued a negative opinion for the renewal of the conditional marketing authorization following a re-examination procedure. In May 2024, the EC decided not to adopt the CHMP's negative opinion for the renewal of the conditional marketing authorization of Translarna and returned such opinion to the CHMP for re-evaluation. In June 2024, following the EC's request for re-review, the CHMP issued a negative opinion on the renewal of the conditional marketing authorization of Translarna for the treatment of nmDMD. In October 2024, the CHMP maintained its negative opinion for the renewal of the conditional marketing authorization following the requested reexamination procedure. On March 28, 2025, the EC adopted the opinion of the CHMP to not renew the authorization of Translarna for the treatment of nmDMD. While this action effectively removed the drug's conditional marketing authorization in the EEA, the EC indicated that individual countries within the European Union ("EU") can leverage Articles 117(3) and 5(1) of the EU Directive 2001/83 to allow continued commercial use of Translarna.

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 resubmission 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 resubmission. 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 resubmitted the NDA in July 2024, based on the results from Study 041 and from the Company's international drug registry

study for nmDMD patients receiving Translarna. In October 2024, the FDA accepted for review the resubmission of the NDA for Translarna for the treatment of nmDMD. As this was an NDA resubmission following a complete response letter to the NDA which was filed over protest in 2016, the FDA is not obligated to follow the review timelines under Prescription Drug User Fee Act guidelines and an action date has not been provided.

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. On November 13, 2024, the Company's biologics license application ("BLA") for its gene therapy treatment of AADC deficiency was approved by the FDA. This gene therapy is marketed under the brand name Kebilidi 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 the Company's product candidate for the treatment of phenylketonuria ("PKU"). 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. In April 2025, the CHMP issued a positive opinion on the MAA for Sephience™ (sepiapterin) for the treatment of children and adults living with PKU. The opinion includes a broad label inclusive of all ages and disease severities. The Company expects the EC to ratify the marketing authorization for Sephience in approximately two months. In July 2024 the Company submitted an NDA to the FDA for sepiapterin for the treatment of pediatric and adult patients with PKU, including the full spectrum of ages and disease subtypes, in the United States. In September 2024, the FDA accepted for filing the NDA, with a target regulatory action date of July 29, 2025. The Company also made a regulatory submission for sepiapterin for the treatment of PKU in Brazil in the third quarter of 2024, and in Japan in the fourth quarter of 2024, with a regulatory decision in Japan expected in the fourth quarter of 2025.

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 2024, the Company announced interim results from the full Phase 2 study of PTC518. At month 12, PTC518 treatment demonstrated durable dose-dependent lowering of mutant HTT ("mHTT") protein in the blood and dose-dependent lowering of mHTT protein in the cerebrospinal fluid in the interim cohort of stage 2 patients.

In addition, favorable trends were demonstrated on several relevant HD clinical assessments. Furthermore, following 12 months of treatment, PTC518 continued to be well tolerated. In September 2024, the FDA granted Fast Track designation to the PTC518 program for the treatment of HD. In December 2024, the Company held a Type C meeting with the FDA to discuss whether huntingtin protein lowering could be considered a surrogate endpoint for accelerated approval of PTC518. The FDA was aligned on the scientific rationale and asked to see additional data supportive of an association between huntingtin protein lowering and changes in clinical outcome scores. In May 2025, the Company announced that the Phase 2 study of PTC518 met its primary endpoints of blood HTT lowering and safety. The results on the full study population are consistent with the previously reported evidence of dose-dependent HTT lowering, favorable safety profile and early signals of dose-dependent clinical effect at 12 months in Stage 2 patients. In addition, at 24 months of treatment, there were continued trends of dose-dependent favorable clinical effect relative to a propensity-matched natural history cohort as well as dose-dependent NfL lowering. The Company plans to complete additional analyses and discuss next development and regulatory steps, including the potential for accelerated approval. In November 2024, the Company entered into a License and Collaboration Agreement (the "Novartis Agreement") with Novartis Pharmaceuticals Corporation ("Novartis"), relating to its PTC518 HD program which included related molecules. This transaction closed on January 11, 2025, and triggered a $1.0 billion upfront cash payment to the Company.

The Company's inflammation and ferroptosis 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 most advanced molecule in the Company's inflammation and ferroptosis platform is vatiquinone. The Company announced topline results from a registration-directed Phase 3 trial of vatiquinone in children and young adults with Friedreich's ataxia ("FA"), 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 October 2024, the Company announced that the pre-specified endpoint for two different FA long-term extension studies was met, with statistically significant evidence of durable treatment benefit on disease progression. In December 2024, the Company submitted an NDA to the FDA for vatiquinone for the treatment of children and adults living with FA. In February 2025, the FDA accepted for filing the NDA and granted priority review with a target regulatory action date of August 19, 2025.

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, 2025, the Company had an accumulated deficit of approximately $2,780.3 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 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 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 also relies on revenues associated with the Novartis Agreement. The Company expects that cash flows from the sales of its products, royalty payments from Roche, and milestone payments from Novartis, 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, 2024 included in the Company's Annual Report on Form 10-K filed with the Securities and Exchange Commission (the "SEC") on February 27, 2025 (the "2024 Form 10-K"). Selected significant accounting policies are discussed in further detail below.

Basis of presentation

The accompanying financial information as of March 31, 2025 and for the three months ended March 31, 2025 and 2024 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, 2024 and notes thereto included in the 2024 Form 10-K.

In the opinion of management, the unaudited financial information as of March 31, 2025 and for the three months ended March 31, 2025 and 2024 reflects all adjustments, which are normal recurring adjustments, necessary to present a fair statement of financial position, results of operations, stockholders' deficit, and cash flows. The results of operations for the three months ended March 31, 2025 are not necessarily indicative of the results to be expected for the year ended December 31, 2025 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, 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 of $5.0 million in connection with an amendment and restatement of the Company's lease in Hopewell Township, New Jersey. The letter of credit has the potential to be reduced to $3.0 million if after July 1, 2025, the Company is not in default of its lease. Restricted cash also contains an unconditional, irrevocable and transferable letter of credit of $10.0 million in connection with obligations for the Company's new facility lease in Warren, New Jersey. If after July 1, 2027, the Company is not in default of the lease agreement and meets certain creditworthiness guidelines, then the letter of credit will be reduced to $5.0 million. If after December 31, 2028, the Company is not in default of the lease agreement and meets certain creditworthiness guidelines, then the letter of credit will be further reduced to $2.5 million. Both letters of credit are classified within deposits and other assets on the consolidated balance sheet due to the long-term nature of the 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 consolidated statement of cash flows:

	End of period- March 31, 2025	Beginning of period- December 31, 2024
Cash and cash equivalents	$ 1,480,231	$ 779,709
Restricted cash included in deposits and other assets	15,631	15,607
Total Cash, cash equivalents and restricted cash per consolidated statement of cash flows	$ 1,495,862	$ 795,316

Segment information

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision maker ("CODM"), or decision-making group, in deciding how to allocate resources and in assessing performance. The Company's CODM consists of the chief executive officer, the chief financial

officer, and the chief business officer. The Company views its operations and manages its business in one operating and reporting segment: life science. The life science segment is focused on the discovery, development and commercialization of the Company's clinically differentiated medicines that provide benefits to patients with rare disorders. The Company is managed on a consolidated basis, and accordingly, the CODM assesses performance for the life science segment based on net income (loss), with a focus on revenues, research and development expense, and selling general, and administrative expense. Net income is reported on the income statement as consolidated net income (loss). The measure of segment assets is reported on the balance sheet as total consolidated assets. The Company derives its revenues through its worldwide net sales of its commercial products, collaboration agreements and license agreements, and royalty revenues. Refer to Note 11 for further segment information on revenues.

The Company expects to continue to incur significant expenses as it advances product candidates through all stages of development and clinical trials and, ultimately, seek regulatory approval. As such, the CODM uses cash forecast models in deciding how to invest into the life science segment. Such cash forecast models are reviewed to assess the entity-wide operating results and performance. Net income (loss) is used to monitor planned versus actual results. Monitoring planned versus actual results is used in assessing performance of the segment and in establishing management's compensation, along with the cash forecast models. Refer to Note 13 for segment information on significant segment expenses.

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, 2025 and 2024, no allowance was recorded for credit losses.

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

Inventory and cost of product, collaboration and license sales

Inventory

Inventories are stated at the lower of cost and net realizable value, utilizing standard costing, which approximates average costs 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, 2025	December 31, 2024
Raw materials	$ 2,102	$ 2,538
Work in progress	13,066	12,216
Finished goods	6,507	8,440
Total inventory	$ 21,675	$ 23,194

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, 2025 and 2024, the Company recorded inventory write-downs of $3.5 million and $3.7 million, respectively, primarily related to adjustments to inventory reserves and 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, collaboration and license sales. For the three months ended March 31, 2025 and 2024, these amounts were immaterial.

Cost of product, collaboration and license sales

Cost of product, collaboration and license 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, royalty payments to collaborative partners associated with royalty revenues and collaboration revenue related to milestones, and costs related to performance of services associated with license agreement revenues. Production costs are expensed as cost of product, collaboration and license 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 and if it is probable that a significant revenue reversal will not occur. 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.

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, license, 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.

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, 2025 and 2024, no allowance was recorded for credit losses. The allowance for doubtful accounts was $4.3 million as of March 31, 2025 and $2.3 million as of December 31, 2024. For the three months ended

March 31, 2025, bad debt expense was $1.9 million. For the three months ended March 31, 2024, bad debt expense was immaterial.

Liability for sale of future royalties

The Company has a royalty purchase agreement with Royalty Pharma Investments 2019 ICAV ("Royalty Pharma") in which the Company sold its right to receive sales-based royalty payments on worldwide net sales of Evrysdi in exchange for upfront cash consideration from Royalty Pharma. In accordance with the guidance in ASC 470-10-25-2, the Company determined that cash consideration obtained pursuant to the royalty purchase agreement should be classified as debt and is recorded as "liability for sale of future royalties-current" and "liability for sale of future royalties-noncurrent" on the Company's consolidated balance sheet based on the timing of the expected payments to be made to Royalty Pharma. The liability is 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 Royalty Pharma and the Company updates the effective interest rate on a quarterly basis. Refer to Note 9 for further details.

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 Pillar Two Framework that was supported by over 130 countries worldwide. The EU effective dates were 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 EU.

On December 22, 2017, the U.S. government enacted the 2017 Tax Cuts and Jobs Act ("TCJA"), 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 TCJA 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, 2025.

Since 2022, TCJA amendments to IRC Section 174 no longer permit 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 $45.3 million for the year ending December 31, 2025.

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.

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. Refer to Note 3 for further details.

Tangible asset impairment and losses on transactions, net

Tangible asset impairment and losses on transactions, net includes impairments identified on fixed assets, losses and gains on sales of fixed assets, and gains on lease terminations. For the three months ended March 31, 2025, these amounts consisted of a $0.1 million loss on sales of fixed assets.

Recently issued accounting standards

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, but expects the impact will be enhanced disclosures related to income taxes.

In November 2024, the FASB issued ASU 2024-03, Income Statement-Reporting Comprehensive Income- Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses. ASU 2024-03 enhances financial reporting by requiring additional information about specific expense categories in the notes to financial statements at interim and annual reporting periods. The guidance is effective for public business entities for annual periods beginning after December 15, 2026, and interim reporting periods beginning after December 15, 2027. 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 but expects the impact will be enhanced disclosures related to income statement expenses.

3. Leases

The Company's principal office space is a facility located in Warren, New Jersey, which is approximately 180,000 square feet. The Company also leases 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.

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, 2025, the balance of the finance lease liabilities-current and finance lease liabilities-noncurrent are $5.0 million and $13.8 million, respectively, and are directly related to the Company's MassBio agreement. As of December 31, 2024, the balance of the finance lease liabilities-current and finance lease liabilities-noncurrent were $3.0 million and $15.6 million, respectively. Additionally, the Company recorded finance lease costs of $0.3 million and $0.3 million related to interest on the lease liability during the three months ended March 31, 2025 and 2024, 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 0.5 years to 14.2 years and certain of the leases include renewal options to extend the lease for up to 15 years. For the three months ended March 31, 2025 and 2024, rent expense was $5.0 million and $6.9 million, respectively.

The components of operating lease expense were as follows:

	Three Months Ended March 31, 2025	Three Months Ended March 31, 2024
Operating Lease Cost		
Fixed lease cost	$ 3,372	$ 5,575
Variable lease cost	1,341	1,069
Short-term lease cost	328	214
Total operating lease cost	$ 5,041	$ 6,858

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 of March 31, 2025 and December 31, 2024:

	March 31, 2025	December 31, 2024
Weighted-average remaining lease terms - operating leases (years)	11.48	11.62
Weighted-average discount rate - operating leases	7.60 %	7.64 %
Weighted-average remaining lease terms - finance lease (years)	7.76	8.01
Weighted-average discount rate - finance lease	7.80 %	7.80 %

Supplemental cash flow information related to leases was as follows as of March 31, 2025 and 2024:

	Three Months Ended March 31, 2025	2024
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows from operating leases	$ 3,389	$ 3,948
Financing cash flows from finance lease	—	1,490
Operating cash flows from finance lease	—	1,510
Right-of-use assets obtained in exchange for lease obligations:		
Operating leases	$ 850	$ 1,723

Future minimum lease payments under non-cancelable leases as of March 31, 2025 were as follows:

	Operating Leases	Finance Lease
2025 (excludes the three months ended March 31, 2025)	$ 12,776	$ 3,000
2026	16,692	3,000
2027	14,134	3,000
2028	7,809	3,000
2029 and thereafter	74,003	12,000
Total lease payments	125,414	24,000
Less: Imputed Interest expense	40,391	5,120
Total	$ 85,023	$ 18,880

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,

2025 and December 31, 2024. 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. Other than the ClearPoint Equity Investments, no other items included in prepaids and other current assets on the consolidated balance sheets are fair valued.

In January 2020, the Company purchased a $10.0 million convertible note from ClearPoint that was convertible into ClearPoint shares at a conversion rate of $6.00 per share at any point throughout the term of the loan, with a maturity date five years from the purchase date. In August 2024, the outstanding principal amount of the convertible note, together with any accrued and unpaid interest thereon, was repaid in full by ClearPoint and therefore the balance at March 31, 2025 was $0. The Company determined that the convertible note represented an available for sale debt security and the Company had elected to record it at fair value under ASC 825. The Company classified its ClearPoint convertible debt security as a Level 2 asset within the fair value hierarchy, as the value was based on inputs other than quoted prices that are observable. The fair value of the ClearPoint convertible debt security was 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 was included as a component of deposits and other assets on the consolidated balance sheet.

The Company has an investment in mutual funds that is denominated in a foreign currency and is classified as marketable securities on the Company's consolidated balance sheets. This equity investment is reported at fair value, as it is readily available, and as such is classified as a Level 1 asset. Unrealized holding gains and losses for this equity investment are included as components of interest 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, 2025 and March 31, 2024:

	Ending Balance at December 31, 2024	Unrealized Gain/(Loss)	Foreign Currency Unrealized Gain	Investments Purchased	Redemptions/ Sale	Ending Balance at March 31, 2025
Marketable securities - equity investments	$ 29,034	774	2,066	5,209	(8,896)	28,187
ClearPoint Equity Investments	13,759	(3,122)	—	—	—	10,637
Total Fair Value	$ 42,793	$ (2,348)	$ 2,066	$ 5,209	$ (8,896)	$ 38,824

	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

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, 2025 and December 31, 2024:

	March 31, 2025			
	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	$ 518,763	$ —	$ 518,763	$ —
Marketable securities - equity investments	$ 28,187	$ 28,187	$ —	$ —
ClearPoint Equity Investments	$ 10,637	$ 10,637	$ —	$ —

	December 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	$ 330,953	$ —	$ 330,953	$ —
Marketable securities - equity investments	$ 29,034	$ 29,034	$ —	$ —
ClearPoint Equity Investments	$ 13,759	$ 13,759	$ —	$ —
Contingent consideration payable- net sales milestones	$ 800	$ —	$ —	$ 800

No transfers of assets between Level 1, Level 2, or Level 3 of the fair value measurement hierarchy occurred during the periods ended March 31, 2025 and December 31, 2024.

The following is a summary of marketable securities accounted for as available for sale debt securities at March 31, 2025 and December 31, 2024:

	March 31, 2025			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 102,613	$ 22	$ (7)	$ 102,628
Corporate debt securities	182,951	52	(47)	182,956
Government obligations	233,018	161	—	233,179
Total	$ 518,582	$ 235	$ (54)	$ 518,763

	December 31, 2024			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 44,780	$ —	$ (1)	$ 44,779
Corporate debt securities	89,320	76	(75)	89,321
Government obligations	196,584	269	—	196,853
Total	$ 330,684	$ 345	$ (76)	$ 330,953

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, 2025 and 2024, 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, 2025 and 2024, no allowance was recorded for credit losses. Unrealized gains and losses are reported as a component of accumulated other comprehensive loss in stockholders' deficit.

For the three months ended March 31, 2025 and 2024, 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, 2025 are as follows:

	March 31, 2025					
	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	$ (7)	33,667	—	—	(7)	$ 33,667
Corporate debt securities	$ (47)	91,351	—	—	(47)	$ 91,351
Total	$ (54)	$ 125,018	$ —	$ —	$ (54)	$ 125,018

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, 2024 are as follows:

	December 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	$ (1)	29,810	—	—	(1)	$ 29,810
Corporate debt securities	$ (75)	59,550	—	—	(75)	$ 59,550
Total	$ (76)	$ 89,360	$ —	$ —	$ (76)	$ 89,360

Available for sale debt securities at March 31, 2025 and December 31, 2024 mature as follows:

	March 31, 2025	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 102,628	$ —
Corporate debt securities	182,956	—
Government obligations	233,179	—
Total	$ 518,763	$ —

	December 31, 2024	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 44,779	$ —
Corporate debt securities	89,321	—
Government obligations	196,853	—
Total	$ 330,953	$ —

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, 2025 and December 31, 2024 was $340.6 million and $321.3 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. As of March 31, 2025, the probability of triggering the remaining contingent consideration was determined to be remote, and therefore the balance was written down to zero. The change in fair value of the contingent consideration for the three months ended March 31, 2025 is $0.8 million and is recorded in the consolidated statement of operations. Refer to Note 10 for additional details.

5. Accounts payable and accrued expenses

Accounts payable and accrued expenses at March 31, 2025 and December 31, 2024 consist of the following:

	March 31, 2025	December 31, 2024
Employee compensation, benefits, and related accruals	$ 32,091	$ 61,575
Income tax payable	64,622	4,701
Consulting and contracted research	16,497	19,909
Sales allowance	60,801	58,644
Sales rebates	102,443	101,613
Royalties	7,305	8,953
Accounts payable	15,150	17,274
Milestone payable	—	11,025
Other	18,898	20,598
Total	$ 317,807	$ 304,292

As of March 31, 2025, the Company does not have any accrued restructuring costs included above within employee compensation, benefits, and related accruals. As of December 31, 2024, there were $0.1 million 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, 2025 and 2024. 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, 2025.

7. Net income (loss) per share

Basic and diluted net income (loss) per share is computed by dividing net income (loss) by the weighted-average number of common shares outstanding.

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

	Three Months Ended March 31,	
	2025	2024
Numerator:		
Net income (loss), basic	$ 866,562	$ (91,576)
Add: Interest expense, net of tax, on the Company's 2026 Convertible Notes	1,081	—
Net income (loss), diluted	$ 867,643	$ (91,576)
Denominator:		
Weighted-average number of shares outstanding, basic	78,115,836	76,496,127
Effect of dilutive securities:		
Common stock issuable under the Company's equity incentive plans	2,795,971	—
Common stock issuable under the Company's 2026 Convertible Notes	5,474,115	—
Weighted-average common shares outstanding, diluted	86,385,922	76,496,127
Net income (loss) per common share, basic	$ 11.09	$ (1.20)*
Net income (loss) per common share, diluted	$ 10.04	$ (1.20)*

* In the three months ended March 31, 2024, the Company experienced a net loss and therefore did not report any dilutive share impact.

The following table shows historical common share equivalents outstanding, which are not included in the above calculation, as the effect of their inclusion is anti-dilutive during each period. The anti-dilutive shares are calculated as the unweighted outstanding shares as of the reporting period end date that are antidilutive using the treasury stock method.

	As of March 31,	
	2025	2024
Stock Options	2,490,078	9,530,185
Unvested restricted stock units	3,872,981	3,730,166
Total	6,363,059	13,260,351

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, 2025, awards for 4,984,677 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, 2025, awards for 1,894,422 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, 2025 through March 31, 2025, the Company issued a total of 802,745 stock options to various employees. Of those, 12,000 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, 2024	8,482,489	$ 42.29		
Granted	802,745	$ 46.74		
Exercised	(431,089)	$ 36.98		
Forfeited/Cancelled	(320,327)	$ 50.32		
Outstanding at March 31, 2025	8,533,818	$ 42.68	5.93 years	$ 91,932
Expected to vest at March 31, 2025	1,802,150	$ 38.47	8.81 years	$ 22,683
Exercisable at March 31, 2025	6,512,960	$ 43.96	5.02 years	$ 66,629

The fair value of grants made in the three months ended March 31, 2025 was contemporaneously estimated on the date of grant using the following assumptions:

	Three months ended March 31, 2025
Risk-free interest rate	4.12% - 4.44%
Expected volatility	55%
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, 2025 was $25.24 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 Units—Restricted stock units are granted subject to certain restrictions, including in some cases service or time conditions (restricted stock). The grant-date fair value of restricted stock units, which 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, 2025, through March 31, 2025, the Company issued a total of 1,537,675 restricted stock units to various employees. Of those, 29,420 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 units:

	Restricted Stock Units	
	Number of Shares	Weighted Average Grant Date Fair Value
Unvested at December 31, 2024	3,527,575	$ 33.39
Granted	1,537,675	46.76
Vested	(1,089,999)	36.71
Forfeited	(102,270)	33.26
Unvested at March 31, 2025	3,872,981	$ 37.77

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. In December 2024, the Company granted 25,000 PSUs to Dr. Matthew Klein, which will vest only if challenging performance goals relating to development, regulatory, or commercial goals are achieved over an approximately five year performance period. As of March 31, 2025, the achievements of the performance goals have not yet been deemed probable and therefore no expense has been recognized to date. An additional 31,250 PSUs with market conditions were granted in December 2024. The expense related to the PSUs with market conditions was immaterial for the three months ended March 31, 2025.

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, 2025, the Company recorded $0.5 million, in compensation expense related to the ESPP.

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

	Three Months Ended March 31,	
	2025	2024
Research and development	$ 8,663	$ 8,967
Selling, general and administrative	9,397	9,411
Total	$ 18,060	$ 18,378

As of March 31, 2025, there was approximately $170.3 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 2.9 years.

9. Debt

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 SMA License Agreement. 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 December 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 SMA 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, 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 SMA 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 in 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 utilized 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.

In June 2024, the Company, Royalty Pharma and Royalty Pharma plc, entered into an amendment to the A&R Royalty Purchase Agreement. Under the A&R Royalty Purchase Agreement, the Company exercised a put option in June 2024, resulting in the Company receiving $241.8 million in cash consideration. In connection with the put option exercise, the change in rights and obligations 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 after the put option exercise was not substantially different and was therefore determined to be a modification. The $241.8 million in cash consideration obtained was added to the liability for sale of future royalties and the annual effective interest rate under the A&R Royalty Purchase Agreement was determined to be 9.9%. The liability is being 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 Royalty Pharma and the Company updates the effective interest rate on a quarterly basis.

To date, the Company has sold to Royalty Pharma a total of 90.49% of the Royalty, which will be reduced to 83.33% (the "Assigned Royalty Rights") after Royalty Pharma receives $1.3 billion in aggregate payments (the "Assigned Royalty Cap") from the Royalty assigned at the closing of the Original Purchase Agreement. In exchange for the Assigned Royalty Rights, Royalty Pharma has paid to the Company upfront cash consideration totaling $1.9 billion, less Royalty payments received by the Company with respect to the Assigned Royalty Rights. The Company currently retains 9.51% of the Royalty, which increases to 16.67% after the Assigned Royalty Cap has been met. The Company has the option under the A&R Royalty Purchase Agreement to sell its retained portions of the Royalty to Royalty Pharma in up to three tranches for the following payments: (1) $100.0 million in exchange for 3.81% of the Royalty, which increases to 6.67% after the Assigned Royalty Cap has been met, (2) $100.0 million in exchange for 3.81% of the Royalty, which increases to 6.67% after the Assigned Royalty Cap has been met, and (3) $50.0 million in exchange for 1.90% of the Royalty, which increases to 3.33% after the Assigned Royalty Cap has been met, in each case less Royalty payments received by the Company with respect to the Assigned Royalty Rights. 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 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, 2025:

Liability for sale of future royalties- (current and noncurrent)	Three Months Ended March 31, 2025
Beginning balance as of December 31, 2024	$ 2,081,776
Less: Non-cash royalty revenue payable to Royalty Pharma	(32,974)
Plus: Non-cash interest expense recognized	49,661
Ending balance	$ 2,098,463
Effective interest rate as of March 31, 2025	9.3%

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

2026 Convertible Notes

In September 2019, the Company issued the 2026 Convertible Notes, 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, 2025	December 31, 2024
Principal	$ 287,500	$ 287,500
Less: Debt issuance costs	(1,788)	(2,088)
Net carrying amount	$ 285,712	$ 285,412

As of March 31, 2025, the remaining contractual life of the 2026 Convertible Notes is approximately 1.5 years.

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

	Three Months Ended March 31,	
	2025	2024
Contractual interest expense	$ 1,069	$ 1,076
Amortization of debt issuance costs	299	296
Total	$ 1,368	$ 1,372
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.

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 FA 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.

As of March 31, 2025, all of the milestones have either been paid or settled, with the exception of the regulatory milestones and net sales milestones related to FA and Angelman syndrome and the net sales milestones related to Upstaza/Kebilidi. 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 FA and Angelman syndrome. As a result, the Company does not expect the milestones related to FA and Angelman syndrome to be achieved. In addition, the Company does not expect to pay the 2%–6% royalties on annual net sales related to FA and Angelman syndrome. As of March 31, 2025, the remaining potential sales milestones related to Upstaza/Kebilidi is $50.0 million, however the Company has determined that the probability of triggering these milestones is remote.

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 CNS disorders. The lead program, vatiquinone, is in late stage development for FA 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, Censa Pharmaceuticals, Inc. ("Censa"), 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. As of March 31, 2025, $95.0 million of the $217.5 million in development and regulatory milestones have been paid to the former Censa securityholders.

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: life science.

During the three months ended March 31, 2025 and 2024, net product sales outside of the United States were $105.6 million and $120.1 million, respectively, consisting of sales of Translarna, Tegsedi, Waylivra, and Upstaza. Translarna net revenues made up $86.2 million and $103.6 million of the net product sales outside of the United States for the three months ended March 31, 2025 and 2024, respectively. During the three months ended March 31, 2025 and 2024, net product sales in the United States were $47.8 million and $57.5 million, respectively, consisting solely of sales of Emflaza. During the three months ended March 31, 2025, two countries, the United States and Russia, accounted for at least 10% of the Company's net product sales, representing $47.8 million and $38.5 million of the net product sales, respectively. 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. For the three months ended March 31, 2025 and 2024, two of the Company's distributors each accounted for over 10% of the Company's net product sales.

As of March 31, 2025 and December 31, 2024, 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 License revenue

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

Under the 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, 2025, 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, 2025 and 2024, the Company did not recognize collaboration revenue related to the SMA License Agreement with Roche.

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, 2025 and 2024, the Company has recognized $36.4 million and $31.2 million of royalty revenue, respectively, related to Evrysdi.

In November 2024, the Company entered into the Novartis Agreement with Novartis related to the Company's PTC518 HD program. The transaction contemplated by the Novartis Agreement closed in January 2025. Under the Novartis Agreement, and upon the closing of the transaction, the Company received an upfront nonrefundable payment of $1.0 billion on the effective date and can receive up to $1.9 billion in development, regulatory and sales milestones, a 40% share of U.S. profits and losses, and tiered double-digit royalties on ex-U.S. sales.

The Company evaluated the Novartis Agreement in order to determine the proper accounting treatment and concluded that the arrangement was not subject to ASC 730 or ASC 808, as the upfront payment was nonrefundable with no obligation for the Company to repay it and as the Company is not exposed to significant risks. The Company evaluated the arrangement under ASC 606, as a contract with a customer was determined to exist.

Pursuant to the Novartis Agreement, the Company was required to transfer the applicable licenses and related manufacturing and other know how to Novartis and to complete the ongoing Phase 2A Clinical Trial and continue the ongoing OLE Clinical Trial pursuant to its existing development plan, with the goal of transitioning the ongoing OLE Clinical Trial to Novartis within 12 months after the effective date of the Novartis Agreement. Novartis will be responsible

for all other development of licensed compounds and licensed products and the manufacture and commercialization of licensed compounds and licensed products worldwide. Therefore, the Company determined that there were three material and distinct performance obligations: the transfer of the licenses and know-how, completion of the Phase 2A clinical trial, and continuing the OLE Clinical Trial until transitioned to Novartis. As of March 31, 2025, the first two performance obligations were considered to be substantially complete. The OLE Clinical Trial is still ongoing and is expected to be fully transitioned to Novartis by the end of 2025.

The Company determined that the transaction included the fixed consideration of the $1.0 billion and variable consideration in the form of milestones, profit share, and royalties. Management evaluated the variable consideration under ASC 606 and determined that it would be fully constrained until the contingencies were resolved or any applicable sales occurred. Management allocated the transaction price of $1.0 billion to the performance obligations based on the guidance in ASC 606.

During the three months ended March 31, 2025, the Company recognized $989.8 million in license revenues related to performance obligations for Novartis already completed. The remaining $10.2 million was recorded as deferred revenue in the consolidated balance sheet and will be recognized over time as the work related to the OLE Clinical Trial is performed. Collaboration and license revenue during the three months ended March 31, 2025, was partially offset by $3.6 million related to a refund for a prior collaboration arrangement in relation to PTC518.

Manufacturing Revenue

For the three months ended March 31, 2025, the Company did not recognize any revenue related to the production of plasmid DNA and AAV vectors for gene therapy applications for external customers. For the three months ended March 31, 2024, the Company recognized $1.4 million of manufacturing revenue 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, 2025 and 2024.

As of March 31, 2025 and December 31, 2024, the Company does not have a contract liabilities balance or contract assets related to the production of plasmid DNA and AAV vectors for gene therapy applications for external customers.

In June 2024, the Company sold its gene therapy manufacturing business in Hopewell Township, New Jersey. Accordingly, the Company does not expect to have manufacturing revenue going forward.

12. Intangible assets and goodwill

Definite-lived intangibles

Definite-lived intangible assets consisted of the following at March 31, 2025 and December 31, 2024:

Definite-lived intangibles assets, gross	Ending Balance at December 31, 2024	Additions	Foreign currency translation	Ending Balance at March 31, 2025
Waylivra	12,397	1,791	495	14,683
Tegsedi	18,249	878	726	19,853
Kebilidi	10,731	—	—	10,731
Upstaza	106,937	—	—	106,937
Total definite-lived intangibles, gross	$ 148,314	$ 2,669	$ 1,221	$ 152,204

Definite-lived intangibles assets, accumulated amortization	Ending Balance at December 31, 2024	Amortization	Foreign currency translation	Ending Balance at March 31, 2025
Waylivra	(5,273)	(540)	(212)	(6,025)
Tegsedi	(5,609)	(806)	(226)	(6,641)
Kebilidi	(112)	(224)	—	(336)
Upstaza	(18,526)	(2,228)	—	(20,754)
Total definite-lived intangibles, accumulated amortization	$ (29,520)	$ (3,798)	$ (438)	$ (33,756)
Total definite-lived intangibles, net				$ 118,448

Akcea is 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, 2025, royalty payments of $0.9 million and $1.8 million were recorded for Tegsedi and Waylivra, respectively. As of March 31, 2025, a royalty payable of $0.7 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, 2025 and 2024, the Company recognized amortization expense of $3.8 million and $51.5 million, respectively, related to the Emflaza rights, Upstaza/ Kebilidi, Waylivra, and Tegsedi intangible assets. The estimated future amortization of the Upstaza/Kebilidi, Waylivra, and Tegsedi intangible assets is expected to be as follows:

	As of March 31, 2025
2025	$ 11,370
2026	15,162
2027	15,162
2028	15,162
2029 and thereafter	61,592
Total	$ 118,448

The weighted average remaining amortization period of the definite-lived intangibles as of March 31, 2025 is 8.9 years.

Goodwill

As a result of the Agilis Merger on August 23, 2018, the Company recorded $82.3 million of goodwill. As of March 31, 2025, 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, 2025 is $82.3 million.

13. Segment information

The Company views its operations and manages its business in one operating segment: life science. The table below summarizes the significant expense categories for the life science segment regularly reviewed by the CODM for the three months ended March 31, 2025 and 2024:

	Three months ended March 31,	
	2025	2024
Total revenues	$ 1,176,096	$ 210,118
Less:		
Cost of Product, Collaboration and License Sales	7,707	5,195
Program Spend	48,158	48,364
Employee Costs	67,699	63,997
Manufacturing Costs	14,950	18,081
Administrative Costs	18,414	16,872
Occupancy Costs	7,183	9,893
Other segment items (a)	145,423	139,292
Segment Net Income (Loss)	$ 866,562	$ (91,576)
Reconciliation of profit or loss		
Adjustments and reconciling items	—	—
Consolidated Net Income (Loss)	$ 866,562	$ (91,576)

(a) Other segment items includes the following:

	Three months ended March 31,	
	2025	2024
Interest income	$ (17,245)	$ (10,940)
Interest expense	51,337	51,774
Income tax expense	63,266	6,880
Depreciation	3,458	3,867
Amortization	3,798	51,530
All other (b)	40,809	36,181
Total other segment items	$ 145,423	$ 139,292

(b) All other includes COGS royalty, travel and expense, distribution costs, bad debt expense, finance costs, contract labor costs, stock compensation expense, change in the fair value of contingent consideration, and other (expense) income.

14. Subsequent events

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

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

The following discussion and analysis is meant to provide material information relevant to an assessment of the financial condition and results of operations of our company, including an evaluation of the amounts and certainty of cash flows from operations and from outside resources, so as to allow investors to better view our company from management's perspective. The following discussion of our financial condition and results of operations should be read in conjunction with our financial statements and the notes to those financial statements appearing elsewhere in this Quarterly Report on Form 10-Q and the audited consolidated financial statements and notes thereto and management's discussion and analysis of financial condition and results of operations for the year ended December 31, 2024 included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 27, 2025, or our 2024 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 2024 Annual Report, our actual results may differ materially from those anticipated in these forward-looking statements.

Our Company

We are a global biopharmaceutical company that discovers, develops and commercializes clinically differentiated medicines that provide benefits to children and adults living 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 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 has marketing authorization in Russia for the treatment of nonsense mutation Duchenne muscular dystrophy, or 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, 2025, we recognized $86.2 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, 2025, we recognized $47.8 million in net sales of Emflaza.

We previously had a marketing authorization for Translarna in the European Economic Area, or EEA, which had been 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. 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. In January 2024, the CHMP issued a negative opinion for the renewal of the conditional marketing authorization following a re-

examination procedure. In May 2024, the EC decided not to adopt the CHMP's negative opinion for the renewal of the conditional marketing authorization of Translarna and returned such opinion to the CHMP for re-evaluation. In June 2024, following the EC's request for re-review, the CHMP issued a negative opinion on the renewal of the conditional marketing authorization of Translarna for the treatment of nmDMD. In October 2024, the CHMP maintained its negative opinion for the renewal of the conditional marketing authorization following the requested reexamination procedure. On March 28, 2025, the EC adopted the opinion of the CHMP to not renew the authorization of Translarna for the treatment of nmDMD. While this action effectively removed the drug's conditional marketing authorization in the EEA, the EC indicated that individual countries within the European Union, or EU, can leverage Articles 117(3) and 5(1) of the EU Directive 2001/83 to allow continued commercial use of Translarna. There is a substantial risk that as a result of the EC's adoption of the CHMP's negative opinion we will lose all, or a significant portion of, our ability to generate revenue from sales of Translarna in the EEA.

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, or CRL, 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 CRL. 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 resubmission 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 resubmission of an NDA for Translarna. The FDA provided initial written feedback that Study 041 does not provide substantial evidence of effectiveness to support an NDA resubmission. 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 resubmitted the NDA in July 2024, based on the results from Study 041 and from our international drug registry study for nmDMD patients receiving Translarna. In October 2024, the FDA accepted for review the resubmission of the NDA for Translarna for the treatment of nmDMD. As this was an NDA resubmission following a complete response letter to the NDA which was filed over protest in 2016, the FDA is not obligated to follow the review timelines under Prescription Drug User Free Act guidelines and an action date has not been provided.

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 potentially 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) / Kebilidi™ (eladocagene exuparvovec-tneq)

Upstaza/Kebilidi is a gene therapy 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 November 2024, the FDA granted accelerated approval of our gene therapy for the treatment of children and adults with AADC deficiency, which is marketed with the brand name Kebilidi 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 our product candidate for the treatment of phenylketonuria, or PKU. 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. In April 2025, the CHMP issued a positive opinion on the MAA for Sephience™ (sepiapterin) for the treatment of children and adults living with PKU. The opinion includes a broad label inclusive of all ages and disease severities. We expect the EC to ratify the marketing authorization for Sephience in approximately two months. In July 2024, we submitted an NDA to the FDA for sepiapterin for the treatment of pediatric and adult patients with PKU, including the full spectrum of ages and disease subtypes, in the United States. In September 2024, the FDA accepted for filing the NDA, with a target regulatory action date of July 29, 2025. We also made regulatory submissions for sepiapterin for the treatment of PKU in Brazil in the third quarter of 2024, and in Japan in the fourth quarter of 2024, with a regulatory decision in Japan expected in the fourth quarter of 2025.

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 2024, we announced interim results from the full Phase 2 study of PTC518. At month 12, PTC518 treatment demonstrated durable dose-dependent lowering of mHTT protein in the blood and dose-dependent lowering of mHTT protein in the cerebrospinal fluid in the interim cohort of stage 2 patients. In addition, favorable trends were demonstrated on several relevant HD clinical assessments. Furthermore, following 12

months of treatment, PTC518 continued to be well tolerated. In September 2024, the FDA granted Fast Track designation to the PTC518 program for the treatment of HD. In December 2024, we held a Type C meeting with the FDA to discuss whether huntingtin protein lowering could be considered a surrogate endpoint for accelerated approval of PTC518. The FDA was aligned on the scientific rationale and asked to see additional data supportive of an association between huntingtin protein lowering and changes in clinical outcome scores. In May 2025, we announced that the Phase 2 study of PTC518 met its primary endpoints of blood HTT lowering and safety. The results on the full study population are consistent with the previously reported evidence of dose-dependent HTT lowering, favorable safety profile and early signals of dose-dependent clinical effect at 12 months in Stage 2 patients. In addition, at 24 months of treatment, there were continued trends of dose-dependent favorable clinical effect relative to a propensity-matched natural history cohort as well as dose-dependent NfL lowering. We plan to complete additional analyses and discuss next development and regulatory steps, including the potential for accelerated approval. In November 2024, we entered into the License and Collaboration Agreement, or the Novartis Agreement, with Novartis Pharmaceuticals Corporation, or Novartis, relating to our PTC518 program. This transaction closed in January 2025. Pursuant to the Novartis Agreement, we are responsible for completing the ongoing Phase 2A Clinical Trial and continuing the ongoing OLE Clinical Trial pursuant to its existing development plan, with the goal of transitioning the ongoing OLE Clinical Trial to Novartis within 12 months after the effective date of the Novartis Agreement. Novartis will be responsible for all other development of licensed compounds and licensed products and the manufacture and commercialization of licensed compounds and licensed products worldwide.

Inflammation and Ferroptosis Platform

Our inflammation and ferroptosis 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 most advanced molecule in our inflammation and ferroptosis platform is vatiquinone. We announced topline results from a registration-directed Phase 3 trial of vatiquinone in children and young adults with FA, 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 October 2024, we announced that the pre-specified endpoint for two different FA long-term extension studies was met, with statistically significant evidence of durable treatment benefit on disease progression. In December 2024, we submitted an NDA to the FDA for vatiquinone for the treatment of children and adults living with FA. In February 2025, the FDA accepted for filing the NDA and granted priority review with a target regulatory action date of August 19, 2025.

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 for 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 early access programs, or 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, we 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 and we have recognized license revenues related to performance obligations already completed pursuant to the Novartis Agreement.

We have financed our operations to date primarily through the private offerings of convertible senior notes, public and "at the market offerings" of common stock, proceeds from royalty purchase agreements, 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, revenue generated from net sales of Tegsedi and Waylivra in Latin America and the Caribbean, and license revenues related to performance obligations already completed pursuant to the Novartis Agreement.

In June 2024, we entered into an amendment with Royalty Pharma Investments 2019 ICAV, or Royalty Pharma, and Royalty Pharma plc, to the Amended and Restated Royalty Purchase Agreement, dated October 18, 2023, or the A&R Royalty Purchase Agreement, which amends and restated in its entirety the Royalty Purchase Agreement by and among us, RPI Intermediate Finance Trust, and for the limited purposes set forth in the agreement, Royalty Pharma plc, dated as of July 17, 2020, or the Original Royalty Purchase Agreement, and we exercised our first put option in exchange for $241.8 million in cash consideration. To date, Royalty Pharma has paid to us cash consideration of $1.9 billion (less payments on our right to receive sales-based royalty payments, or the Royalty, on worldwide net sales of Evrysdi and any other product in development pursuant to the SMA License Agreement received by us with respect to assigned Royalties, or the Assigned Royalty Rights) in exchange for 90.49% of the Royalty, which will be reduced to 83.33% after Royalty Pharma receives $1.3 billion in aggregate payments, or the Assigned Royalty Cap, from the Royalty assigned under the Original Royalty Purchase Agreement. We currently retain 9.51% of the Royalty, which increases to 16.67% after the Assigned Royalty Cap has been met. We have the option to sell our retained portions of the Royalty to Royalty Pharma in up to three tranches for the following payments: (1) $100.0 million in exchange for 3.81% of the Royalty, which increases to 6.67% after the Assigned Royalty Cap has been met, (2) $100.0 million in exchange for 3.81% of the Royalty, which increases to 6.67% after the Assigned Royalty Cap has been met, and (3) $50.0 million in exchange for 1.90% of the Royalty, which increases to 3.33% after the Assigned Royalty Cap has been met, in each case less Royalty payments received by us with respect to the Assigned Royalty Rights.

In November 2024, we entered into the Novartis Agreement relating to our PTC518 HD program which includes related molecules. Pursuant to the Novartis Agreement, we are responsible for completing the ongoing Phase 2A Clinical Trial and continuing the ongoing OLE Clinical Trial pursuant to its existing development plan, with the goal of transitioning the ongoing OLE Clinical Trial to Novartis within 12 months after the effective date. Novartis will be responsible for all other development of licensed compounds and licensed products and the manufacture and commercialization of licensed compounds and licensed products worldwide. Under the Novartis Agreement, and upon the closing of the transaction contemplated by the Novartis Agreement in January 2025, we received an upfront payment of $1.0 billion on the effective date and can receive up to $1.9 billion in development, regulatory and sales milestones, a 40% share of U.S. profits and losses, and tiered double-digit royalties on ex-U.S. sales.

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, 2025, 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, 2025.

As of March 31, 2025, we had an accumulated deficit of $2,780.3 million. We had a net income of $866.6 million and a net loss of $91.6 million for the three months ended March 31, 2025 and 2024, 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 our splicing and inflammation and ferroptosis 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 in which we do not currently have marketing authorization. We have submitted an MAA to the EMA for sepiapterin for the treatment of PKU in the EEA in March 2024, an NDA to the FDA for sepiapterin for the treatment of PKU in the United States in July 2024, and an NDA to the FDA for vatiquinione for the treatment of FA in the fourth 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.

With respect to our outstanding 1.50% convertible senior notes due September 15, 2026, or the 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 Pharmaceuticals, Inc., or Censa, securityholders of $57.5 million in the aggregate in cash upon the potential achievement in 2025 of certain 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.

Upon the potential achievement in 2025 of certain regulatory milestones relating to vatiquinone, which milestones would be payable in 2026, we expect to make payments to BioElectron Technology Corporation, or BioElectron, of $75.0 million in the aggregate, in cash or shares of our common stock, as determined by us, pursuant to an asset purchase agreement by and between the Company and BioElectron, dated October 1, 2019, or the BioElectron Asset Purchase Agreement.

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 2024 Annual Report. There were no material changes to these obligations and commitments during the period ended March 31, 2025. Furthermore, since we are a public company, we have incurred and expect to continue to incur additional costs associated with operating as such including significant legal, accounting, investor relations and other expenses.

We will need to generate significant revenues to achieve and sustain profitability, and we may never do so. 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 and if it is probable that a significant revenue reversal will not occur. 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, 2025 and 2024, net product sales outside of the United States were $105.6 million and $120.1 million, respectively, consisting of sales of Translarna, Tegsedi, Waylivra, and Upstaza. Translarna net revenues made up $86.2 million and $103.6 million of the net product sales outside of the United States for the three months ended March 31, 2025 and 2024, respectively. During the three months ended March 31, 2025 and 2024, net product sales in the United States were $47.8 million and $57.5 million, respectively, consisting solely of sales of Emflaza. During the three months ended March 31, 2025, two countries, the United States and Russia, accounted for at least 10% of the Company's net product sales, representing $47.8 million and $38.5 million of the net product sales, respectively. 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. For the three months ended March 31, 2025 and 2024, 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, 2025, we had recognized a total of $310.0 million in milestone payments and $582.0 million royalties on net sales pursuant to the SMA License Agreement. As of March 31, 2025, there are no remaining research and development event milestones that we can receive. The remaining potential sales milestones as of March 31, 2025 are $150.0 million upon achievement of certain sales events.

For the three months ended March 31, 2025 and 2024, we did not recognize collaboration revenue relating to the SMA License Agreement with Roche.

For the three months ended March 31, 2025 and 2024, we recognized $36.4 million and $31.2 million of royalty revenue, respectively, related to Evrysdi.

Novartis Collaboration for PTC518 HD. In November 2024, we entered into the Novartis Agreement with Novartis related to our PTC518 HD program. Upon the closing of the transaction contemplated by the Novartis Agreement in January 2025, we received an upfront payment of $1.0 billion on the effective date and can receive up to $1.9 billion in development, regulatory and sales milestones, a 40% share of U.S. profits and losses, and tiered double-digit royalties on ex-U.S. sales

For the three months ended March 31, 2025, we recognized $989.8 million in collaboration revenues related to the Novartis Agreement. For the three months ended March 31, 2024, we did not recognize collaboration revenue related to the Novartis Agreement.

Pursuant to the A&R Royalty Purchase Agreement, Royalty Pharma has paid to us aggregate cash consideration of $1.9 billion (less Royalty payments received by us with respect to the Assigned Royalty Rights) in exchange for 90.49% of the Royalty, which will be reduced to 83.33% of the Royalty after Royalty Pharma receives $1.3 billion in aggregate payments from the Royalty assigned at the closing of the Original Purchase Agreement. We currently retain 9.51% of the Royalty, which increases to 16.67% after the Assigned Royalty Cap has been met, and all economic rights to receive the remaining potential regulatory and sales milestone payments under the SMA License Agreement.

We have the option to sell our retained portions of the Royalty to Royalty Pharma in up to three tranches for the following payments: (1) $100.0 million in exchange for 3.81% of the Royalty, which increases to 6.67% after the Assigned Royalty Cap has been met, (2) $100.0 million in exchange for 3.81% of the Royalty, which increases to 6.67% after the Assigned Royalty Cap has been met, and (3) $50.0 million in exchange for 1.90% of the Royalty, which increases to 3.33% after the Assigned Royalty Cap has been met, in each case less Royalty payments received by us with respect to the Assigned Royalty Rights. 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 our activities under our splicing and inflammation and ferroptosis 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 tables provides research and development expense for our most advanced principal product development programs, for the three months ended March 31, 2025 and 2024.

	Three Months Ended March 31,	
	2025	2024
	(in thousands)	
Development	$ 47,849	$ 53,424
Research	13,847	13,499
Payroll, benefits, and share-based stock compensation	38,234	39,352
Facilities and other	9,043	9,854
Total research and development	$ 108,973	$ 116,129

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

For the three months ended March 31, 2025, compared to the three months ended March 31, 2024, the decrease in development expenses primarily reflected the decrease in program spend related to our continued focus of 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 the three months ended March 31, 2025, compared to the three months ended March 31, 2024, the change in research expenses was relatively flat. We continue to focus our resources on our differentiated, high potential research and development programs.

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

For the three months ended March 31, 2025 compared to the three months ended March 31, 2024, the decrease in payroll, benefits, and share-based stock compensation expenses primarily related to a decrease in share-based stock compensation for the three months ended March 31, 2025 compared to three months ended March 31, 2024.

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.

For the three months ended March 31, 2025 compared to the three months ended March 31, 2024, the decrease in facilities and other expenses primarily related to decreases in facility-based expenses at our facility in Hopewell Township, New Jersey as a result of an amendment and restatement of our lease for such facility in June 2024 and at our facility in Warren, New Jersey as a result of an amendment to our lease for such facility in December 2024.

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 A&R Royalty Purchase Agreement and the 2026 Convertible Notes outstanding, 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, 2025, there were no material changes to our critical accounting policies as reported in our 2024 Annual Report.

Results of operations

Three months ended March 31, 2025 compared to three months ended March 31, 2024

The following table summarizes revenues and selected expense and other income data for the three months ended March 31, 2025 and 2024.

(in thousands)	Three Months Ended March 31, 2025	2024	Change 2025 vs. 2024
Net product revenue	$ 153,426	$ 177,604	$ (24,178)
Collaboration and license revenue	986,231	—	986,231
Royalty revenue	36,439	31,154	5,285
Manufacturing revenue	—	1,360	(1,360)
Cost of product, collaboration and license sales, excluding amortization of acquired intangible assets	12,862	14,740	(1,878)
Amortization of acquired intangible assets	3,798	51,530	(47,732)
Research and development expense	108,973	116,129	(7,156)
Selling, general and administrative expense	80,961	73,272	7,689
Change in the fair value of contingent consideration	(800)	(100)	(700)
Tangible asset impairment and losses on transactions, net	77	—	77
Interest expense, net	(34,092)	(40,834)	6,742
Other (expense) income, net	(6,305)	1,591	(7,896)
Income tax expense	(63,266)	(6,880)	(56,386)

Net product revenue. Net product revenue was $153.4 million for the three months ended March 31, 2025, a decrease of $24.2 million, or 14%, from $177.6 million for the three months ended March 31, 2024. The decrease in net product revenue was primarily due to a decrease in net product sales of Emflaza and Translarna. Emflaza net product revenue was $47.8 million for the three months ended March 31, 2025, a decrease of $9.7 million, or 17%, compared to $57.5 million for the three months ended March 31, 2024. These results were driven by the expiration of Emflaza's orphan drug exclusivity in February 2024. Translarna net product revenues were $86.2 million for the three months ended March 31, 2025, a decrease of $17.4 million, or 17%, compared to $103.6 million for the three months ended March 31, 2024. These results were due to the timing of bulk patient orders.

Collaboration and license revenue. Collaboration and license revenue was $986.2 million for the three months ended March 31, 2025, an increase of $986.2 million, or 100%, from $0.0 million for the three months ended March 31, 2024. The increase in collaboration and license revenue was due to the receipt of the $1.0 billion upfront payment upon the effective date of the license and collaboration agreement with Novartis related to the Company's PTC518 HD program. For the three months ended March 31, 2025, we recognized $989.8 million related to license revenue from the Novartis Agreement which was partially offset by $3.6 million related to a refund for a prior collaboration arrangement in relation to PTC518. For the three months ended March 31, 2024, the Company did not recognize any collaboration or license revenue.

Royalty revenue. Royalty revenue was $36.4 million for the three months ended March 31, 2025, an increase of $5.3 million, or 17%, from $31.2 million for the three months ended March 31, 2024. The increase in royalty revenue was due to higher Evrysdi sales in the three months ended March 31, 2025 as compared to the three months ended March 31, 2024. In accordance with the SMA License Agreement, we are entitled to royalties on worldwide annual net sales of the product.

Manufacturing revenue. Manufacturing revenue was $0.0 million for the three months ended March 31, 2025, a decrease of $1.4 million, or 100%, from $1.4 million for the three months ended March 31, 2024. The decrease was due to the prior completion of all manufacturing services related to the production of plasmid DNA and AAV vectors for gene therapy applications for external customers. In June 2024, we sold our gene therapy manufacturing business in Hopewell Township, New Jersey. Accordingly, we do not expect to have manufacturing revenue going forward.

Cost of product, collaboration and license sales, excluding amortization of acquired intangible assets. Cost of product, collaboration and license sales, excluding amortization of acquired intangible assets were $12.9 million for the three months ended March 31, 2025, a decrease of $1.9 million, or 13%, from $14.7 million for the three months ended March 31, 2024. Cost of product, collaboration and license sales, excluding amortization of acquired intangible assets consisted primarily of royalty payments associated with Emflaza, Translarna, and Upstaza net product sales, costs associated with Emflaza, Translarna, and Upstaza products sold during the period, and inventory reserves. The decrease in cost of product, collaboration and license sales, excluding amortization of acquired intangible assets, was primarily due to decreases in royalty costs driven by Emflaza as a result of the completion of the royalty agreement with Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC), which was partially offset by Translarna inventory reserves.

Amortization of acquired intangible assets. Amortization of acquired intangible assets were $3.8 million for the three months ended March 31, 2025, a decrease of $47.7 million, or 93%, from $51.5 million for the three months ended March 31, 2024. These amounts are related to the Emflaza rights acquisition, as well as the Waylivra, Tegsedi, and Upstaza/Kebilidi intangible assets, which are all being amortized on a straight-line basis over their estimated useful lives. The amortization decrease was driven by the Emflaza rights intangible asset being fully amortized as of February 2024. As a result, there is no further amortization for Emflaza as of February 2024.

Research and development expense. Research and development expense was $109.0 million for the three months ended March 31, 2025, a decrease of $7.2 million, or 6%, from $116.1 million for the three months ended March 31, 2024. The decrease in research and development expenses related to decreases in program spend as we continued to focus our resources on our differentiated, high potential research and development programs.

Selling, general and administrative expense. Selling, general and administrative expense was $81.0 million for the three months ended March 31, 2025, an increase of $7.7 million, or 10%, from $73.3 million for the three months ended March 31, 2024. The increase reflected our continued investment to support our commercial activities including our expanding commercial portfolio.

Change in the fair value of contingent consideration. The change in the fair value of contingent consideration was a gain of $0.8 million for the three months ended March 31, 2025, an increase of $0.7 million, or over 100%, from a gain of $0.1 million for the three months ended March 31, 2024. As of March 31, 2025, the probability of triggering the remaining contingent consideration was determined to be remote, and therefore the balance was written down to zero.

Tangible asset impairment and losses on transactions, net. Tangible asset impairment and losses on transactions, net was $0.1 million for the three months ended March 31, 2025, an increase of $0.1 million, or 100%, from $0.0 million for the three months ended March 31, 2024. This increase was primarily related to losses on sales of fixed assets.

Interest expense, net. Interest expense, net was $34.1 million for the three months ended March 31, 2025, a decrease of $6.7 million, or 17%, from $40.8 million for the three months ended March 31, 2024. The decrease in interest expense, net was primarily related to investment income on our marketable securities – available for sale.

Other (expense) income, net. Other expense, net was $6.3 million for the three months ended March 31, 2025, a change of $7.9 million, or over 100%, from other income, net of $1.6 million for the three months ended March 31, 2024. The change in other (expense) income, net primarily related to net realized and unrealized losses on foreign currency of $6.4 million for the three months ended March 31, 2025, a change of $7.6 million, compared to net realized and unrealized gains on foreign currency of $1.2 million for the three months ended March 31, 2024.

Income tax expense. Income tax expense was $63.3 million for the three months ended March 31, 2025, an increase of $56.4 million, or over 100%, compared to income tax expense of $6.9 million for the three months ended March 31, 2024. The increase in income tax expense was attributable to the recognition of the revenue associated with the Novartis Agreement. 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

While we have generated net income in the three months ended March 31, 2025, we have historically 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 and Russia 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. In March 2025, the EC adopted the opinion of the CHMP to not renew the authorization of Translarna for the treatment of nmDMD. While this action effectively removed the drug's conditional marketing authorization in the EEA, the EC indicated that individual countries within the EU can leverage Articles 117(3) and 5(1) of the EU Directive 2001/83 to allow continued use of Translarna. 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 potentially 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 financed our operations to date primarily through the private offerings of convertible senior notes, public offerings and "at the market offerings" of common stock, proceeds from royalty purchase agreements, private placements of our 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 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 income and 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 2026 Convertible Notes, which included an option to purchase up to an additional $37.5 million in aggregate principal amount of the 2026 Convertible Notes, which was fully exercised 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. We received net proceeds of $279.3 million after deducting the initial purchasers' discounts and commissions and the offering expenses payable by us.

We have received fundings from Royalty Pharma under the A&R Royalty Purchase Agreement in July 2020, October 2023 and June 2024 totaling $1.9 billion (less Royalty payments received by us with respect to the Assigned Royalty Rights). In exchange for these fundings, we sold Royalty Pharma 90.49% of the Royalty, which will be reduced to 83.33% after Royalty Pharma receives the Assigned Royalty Cap from the Royalty assigned under the Original Royalty Purchase Agreement. We currently retain 9.51% of the Royalty, which increases to 16.67% after the Assigned Royalty Cap has been met. We have the option to sell our retained portions of the Royalty to Royalty Pharma in up to three tranches for the

following payments: (1) $100.0 million in exchange for 3.81% of the Royalty, which increases to 6.67% after the Assigned Royalty Cap has been met, (2) $100.0 million in exchange for 3.81% of the Royalty, which increases to 6.67% after the Assigned Royalty Cap has been met, and (3) $50.0 million in exchange for 1.90% of the Royalty, which increases to 3.33% after the Assigned Royalty Cap has been met, in each case less Royalty payments received by us with respect to the Assigned Royalty Rights.

In November 2024, we and Novartis entered into the Novartis Agreement relating to our PTC518 HD program which includes related molecules. Pursuant to the Novartis Agreement, we are responsible for completing the ongoing Phase 2A Clinical Trial and continuing the ongoing OLE Clinical Trial pursuant to its existing development plan, with the goal of transitioning the ongoing OLE Clinical Trial to Novartis within 12 months after the effective date. Novartis will be responsible for all other development of licensed compounds and licensed products and the manufacture and commercialization of licensed compounds and licensed products worldwide. Under the Novartis Agreement, and upon the closing of the transaction contemplated by the Novartis Agreement in January 2025, we received an upfront payment of $1.0 billion on the effective date and can receive up to $1.9 billion in development, regulatory and sales milestones, a 40% share of U.S. profits and losses, and tiered double-digit royalties on ex-U.S. sales.

Cash flows

As of March 31, 2025, we had cash, cash equivalents and marketable securities of $2.03 billion.

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

	Three Months Ended March 31,	
(in thousands)	**2025**	**2024**
Cash provided (used in) by:		
Operating activities	870,103	70,761
Investing activities	(184,360)	(114,932)
Financing activities	9,410	540

Net cash provided by operating activities was $870.1 million for the three months ended March 31, 2025, compared to $70.8 million for the three months ended March 31, 2024. The net cash provided by operating activities for the three months ended March 31, 2025, was primarily related to the upfront payment of $1.0 billion in cash received upon the closing of the transaction contemplated by the Novartis Agreement in January 2025, offset by spend supporting clinical development and commercial activities. The net cash provided by operating activities for the three months ended March 31, 2024 was primarily related to the cash received from the $100.0 million sales milestone for the achievement of $1.5 billion in worldwide net sales from Evrysdi, offset by spend supporting clinical development and commercial activities.

Net cash used in investing activities was $184.4 million for the three months ended March 31, 2025, compared to $114.9 million for the three months ended March 31, 2024. Cash used in investing activities for the three months ended March 31, 2025, was primarily related to the purchases of marketable securities and purchases of fixed assets, partially offset by net sales and redemption of marketable securities and proceeds from sales of fixed assets. 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, and purchases of fixed assets, partially offset by net sales and redemption of marketable securities.

Net cash provided by financing activities was $9.4 million for the three months ended March 31, 2025, compared to $0.5 million for the three months ended March 31, 2024. Cash provided by financing activities for the three months ended March 31, 2025 was primarily attributable to cash received from the exercise of options offset by payments on contingent consideration obligation. Cash provided by financing activities for the three months ended March 31, 2024 was 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 our splicing and inflammation and ferroptosis 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. We submitted an MAA to the EMA for sepiapterin for the treatment of PKU in the EEA in March 2024, an NDA to the FDA for sepiapterin for the treatment of PKU in July 2024, and an NDA to the FDA for vatiquinone for the treatment of FA in December 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 our products or product candidates in the United States or elsewhere;
- are required to take other steps to maintain our current marketing authorization in 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 inflammation and ferroptosis programs as well as studies in our products for maintaining authorizations, label extensions and additional indications;
- seek to discover and develop additional product candidates;
- seek to expand and diversify our product pipeline through strategic transactions;
- maintain, expand and protect our intellectual property portfolio; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and commercialization efforts.

We believe that our cash flows from product sales, together with existing cash and cash equivalents, 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 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 for 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 Translarna for the treatment of nmDMD in the United States;
- our ability to obtain marketing authorization for vatiquinone for the treatment of FA in the United States;
- 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 inflammation and ferroptosis 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 inflammation and ferroptosis programs and Translarna and Upstaza/Kebilidi 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 inflammation and ferroptosis programs;
- revenue received from commercial sales of our products or any of our product candidates;
- our ability to obtain additional and maintain existing reimbursed named patient and cohort EAP programs for Translarna for the treatment of nmDMD on adequate terms, or at all;
- the ability and willingness of patients and healthcare professionals to access Translarna through alternative means if pricing and reimbursement negotiations in the applicable territory do not have a positive outcome;
- the costs of preparing, filing and prosecuting patent applications, maintaining, and protecting our intellectual property rights and defending against intellectual property-related claims;
- the extent to which we acquire or invest in other businesses, products, product candidates, and technologies, including the success of any acquisition, in-licensing or other strategic transaction we may pursue, and the costs of subsequent development requirements and commercialization efforts, including with respect to our

acquisitions of Emflaza, Agilis, our inflammation and ferroptosis 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.

- the progress and results of activities for our PTC518 program, including our right to receive any development, regulatory and sales milestones, profit sharing and royalty payments from Novartis; and

- unexpected decreases in revenue or increase in expenses resulting from potential widespread outbreaks of contagious disease.

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 $57.5 million in the aggregate in cash upon the potential achievement in 2025 of certain regulatory milestones relating to sepiapterin pursuant to the Censa Merger Agreement.

Upon the potential achievement in 2025 of certain regulatory milestones relating to vatiquinone, which milestones would be payable in 2026, we expect to make payments to BioElectron of $75.0 million in the aggregate, in cash or shares of our common stock, as determined by us, pursuant to the BioElectron Asset Purchase Agreement.

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 2024 Annual Report. There were no material changes to these obligations and commitments during the period ended March 31, 2025.

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, 2025, 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 2024 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, 2025. 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, 2025, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

No change in our internal control over financial reporting occurred during the quarter ended March 31, 2025 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, 2024, 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
Allan Jacobson (Director)	Adoption (March 12, 2025)	Rule 10b5-1 trading arrangement	Sale	Until December 31, 2027, or such earlier date upon which all transactions are completed.	Up to 38,000 shares
Emma Reeve (Director)	Adoption (March 4, 2025)	Rule 10b5-1 trading arrangement	Sale	Until February 26, 2027, or such earlier date upon which all transactions are completed.	Up to 85,733 shares

Item 6. Exhibits.

Exhibit Number	Description of Exhibit
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.

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

SIGNATURES

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

PTC THERAPEUTICS, INC.

Date: May 6, 2025

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

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: May 6, 2025

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: May 6, 2025

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, 2025 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: May 6, 2025

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, 2025 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: May 6, 2025

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