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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 June 30, 2026

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 July 29, 2026, there were 83,460,802 shares of Common Stock, $0.001 par value per share, outstanding.

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PTC Therapeutics, Inc.

FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Quarterly Report on Form 10-Q, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "might," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," "aim," 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 Sephience™ (sepiapterin) for the treatment of phenylketonuria, or PKU, including commercialization and the potential achievement of sales milestones and contingent payments that we may be obligated to make;
- our ability to maintain marketing authorization of Translarna™ (ataluren) for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, in geographies in which it has approval, including the effect of the European Commission's adoption of the negative opinion from the Committee for Medicinal Products for Human Use on Translarna, and the impact of the withdrawal of the Translarna new drug application in the United States, on existing approvals;
- the ability and willingness of individual countries within the European Union, or EU, to leverage Articles 117(3) and 5(1) of the EU Directive 2001/83 to allow continued commercial use of Translarna;
- expectations with respect to our license and collaboration agreement with Novartis Pharmaceuticals Corporation, or Novartis, for votoplam for the treatment of Huntington's disease, including our right to receive development, regulatory and sales milestones, profit sharing and royalty payments from Novartis, the design and expected timing of clinical trials and studies, the availability of data, and regulatory submissions and responses, including potential accelerated approval;
- expectations with respect to vatiquinone for the treatment of Friedreich's ataxia, including with respect to the design and expected timing of clinical trials and studies, the availability of data, and regulatory submissions and responses and potential approvals and other matters;
- expectations with respect to Upstaza™/Kebilidi™ (eladocagene exuparvovec)/(eladocagene exuparvovec-tneq) for the treatment of Aromatic L-Amino Decarboxylase deficiency, 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 commercialization of Evrysdi® (risdiplam) for the treatment of spinal muscular atrophy, or SMA, under our program directed against SMA 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 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;
- significant business effects, including the effects of industry, market, economic, political or regulatory conditions;

- changes in tax and other laws, regulations, rates and policies;
- 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;
- 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 scientific approach and general development progress;
- 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 0% convertible senior notes due 2031;
- 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;
- the sufficiency of our cash resources and our ability to obtain adequate financing in the future on favorable terms or at all, for our foreseeable and unforeseeable operating expenses and capital expenditure requirements;
- 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, as well as in Part I, Item 1A. Risk Factors in our Annual Report on Form 10-K for the year ended December 31, 2025, 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, 2025 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)

	June 30, 2026	December 31, 2025
Assets		
Current assets:		
Cash and cash equivalents	$ 1,049,887	$ 984,648
Marketable securities	1,179,195	960,723
Trade and royalty receivables, net	239,044	181,621
Inventory, net	91,354	79,647
Prepaid expenses and other current assets	57,879	67,309
Total current assets	2,617,359	2,273,948
Fixed assets, net	51,487	55,371
Intangible assets, net	402,926	388,753
Goodwill	82,341	82,341
Operating lease ROU assets	74,783	80,053
Deposits and other assets	21,581	18,301
Total assets	$ 3,250,477	$ 2,898,767
Liabilities and stockholders' deficit		
Current liabilities:		
Accounts payable and accrued expenses	$ 382,245	$ 381,272
Current portion of long-term debt	55,451	286,631
Deferred revenue	1,192	2,040
Operating lease liabilities- current	11,965	12,484
Finance lease liabilities- current	6,000	3,000
Liability for sale of future royalties- current	314,215	283,000
Total current liabilities	771,068	968,427
Long-term debt	535,519	—
Operating lease liabilities- noncurrent	87,533	93,583
Finance lease liabilities- noncurrent	—	3,000
Liability for sale of future royalties- noncurrent	2,007,219	2,025,366
Other long-term liabilities	14,501	13,704
Total liabilities	3,415,840	3,104,080
Stockholders' deficit:		
Common stock, $0.001 par value. Authorized 250,000,000 shares; issued and outstanding 83,327,286 shares at June 30, 2026. Authorized 250,000,000 shares; issued and outstanding 81,474,366 shares at December 31, 2025.	82	80
Additional paid-in capital	2,712,080	2,748,335
Accumulated other comprehensive income	6,017	10,501
Accumulated deficit	(2,883,542)	(2,964,229)
Total stockholders' deficit	(165,363)	(205,313)
Total liabilities and stockholders' deficit	$ 3,250,477	$ 2,898,767

See accompanying unaudited notes.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
Revenues:				
Net product revenue	$ 238,819	$ 118,329	$ 464,392	$ 271,755
Collaboration and license revenue	50,595	2,941	50,738	989,172
Royalty revenue	71,105	57,605	117,940	94,044
Total revenues	360,519	178,875	633,070	1,354,971
Operating expenses:				
Cost of product, collaboration and license sales, excluding amortization of acquired intangible assets	19,921	11,420	47,949	24,282
Amortization of acquired intangible assets	11,841	4,061	23,422	7,859
Research and development	99,150	112,990	200,023	221,963
Selling, general and administrative	80,630	85,262	166,813	166,223
Change in the fair value of contingent consideration	—	—	—	(800)
Tangible asset impairment and losses on transactions, net	—	99	927	176
Total operating expenses	211,542	213,832	439,134	419,703
Income (loss) from operations	148,977	(34,957)	193,936	935,268
Interest expense, net	(48,481)	(30,358)	(97,511)	(64,450)
Other expense, net	(2,951)	(5,737)	(1,342)	(12,042)
Income (loss) before income tax (expense) benefit	97,545	(71,052)	95,083	858,776
Income tax (expense) benefit	(14,049)	6,203	(14,396)	(57,063)
Net income (loss) attributable to common stockholders	$ 83,496	$ (64,849)	$ 80,687	$ 801,713
Weighted-average shares outstanding:				
Basic (in shares)	83,006,808	78,151,240	82,765,248	78,438,830
Diluted (in shares)	92,020,009	78,151,240	91,824,273	86,502,578
Net income (loss) per share—basic (in dollars per share)	$ 1.01	$ (0.83)	$ 0.97	$ 10.22
Net income (loss) per share—diluted (in dollars per share)	$ 0.92	$ (0.83)	$ 0.90	$ 9.29

See accompanying unaudited notes.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
Net income (loss)	$ 83,496	$ (64,849)	$ 80,687	$ 801,713
Other comprehensive (loss) income:				
Unrealized loss on marketable securities, net of tax	(633)	(186)	(2,150)	(274)
Foreign currency translation (loss) gain, net of tax	(1,816)	21,491	(2,334)	33,516
Comprehensive income (loss)	$ 81,047	$ (43,544)	$ 76,203	$ 834,955

See accompanying unaudited notes.

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

Three months ended June 30, 2026	Common stock		Additional paid-in capital	Accumulated other comprehensive income (loss)	Accumulated deficit	Total stockholders' deficit
	Shares	Amount				
Balance, March 31, 2026	82,882,024	$ 81	$ 2,778,026	$ 8,466	$ (2,967,038)	$ (180,465)
Exercise of options	344,594	1	13,233	—	—	13,234
Restricted stock vesting and issuance, net	48,675	—	—	—	—	—
Repurchase of 2026 Convertible Notes	—	—	(102,734)	—	—	(102,734)
Conversion of 2026 Convertible Notes	3,506	—	(2,899)	—	—	(2,899)
Issuance of common stock in connection with an employee stock purchase plan	48,487	—	3,163	—	—	3,163
Share-based compensation expense	—	—	23,291	—	—	23,291
Net income	—	—	—	—	83,496	83,496
Comprehensive loss	—	—	—	(2,449)	—	(2,449)
Balance, June 30, 2026	83,327,286	$ 82	$ 2,712,080	$ 6,017	$ (2,883,542)	$ (165,363)

Three months ended June 30, 2025	Common stock		Additional paid-in capital	Accumulated other comprehensive (loss) income	Accumulated deficit	Total stockholders' deficit
	Shares	Amount				
Balance, March 31, 2025	79,225,276	$ 78	$ 2,608,422	$ (13,949)	$ (2,780,311)	$ (185,760)
Exercise of options	36,196	—	1,134	—	—	1,134
Restricted stock vesting and issuance, net	44,190	—	—	—	—	—
Issuance of common stock in connection with an employee stock purchase plan	72,483	—	2,827	—	—	2,827
Share-based compensation expense	—	—	18,543	—	—	18,543
Receivable from investor	—	—	254	—	—	254
Net loss	—	—	—	—	(64,849)	(64,849)
Comprehensive income	—	—	—	21,305	—	21,305
Balance, June 30, 2025	79,378,145	$ 78	$ 2,631,180	$ 7,356	$ (2,845,160)	$ (206,546)

Six months ended June 30, 2026	Common stock		Additional paid-in capital	Accumulated other comprehensive income (loss)	Accumulated deficit	Total stockholders' deficit
	Shares	Amount				
Balance, December 31, 2025	81,474,366	$ 80	$ 2,748,335	$ 10,501	$ (2,964,229)	$ (205,313)
Exercise of options	506,463	1	19,636	—	—	19,637
Restricted stock vesting and issuance, net	1,294,464	1	—	—	—	1
Repurchase of 2026 Convertible Notes	—	—	(102,734)	—	—	(102,734)
Conversion of 2026 Convertible Notes	3,506	—	(2,899)	—	—	(2,899)
Issuance of common stock in connection with an employee stock purchase plan	48,487	—	3,163	—	—	3,163
Share-based compensation expense	—	—	46,712	—	—	46,712
Receivable from investor	—	—	(133)	—	—	(133)
Net income	—	—	—	—	80,687	80,687
Comprehensive loss	—	—	—	(4,484)	—	(4,484)
Balance, June 30, 2026	83,327,286	$ 82	$ 2,712,080	$ 6,017	$ (2,883,542)	$ (165,363)

Six months ended June 30, 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	467,285	—	16,885	—	—	16,885
Restricted stock vesting and issuance, net	1,134,189	1	—	—	—	1
Issuance of common stock in connection with an employee stock purchase plan	72,483	—	2,827	—	—	2,827
Share-based compensation expense	—	—	36,603	—	—	36,603
Receivable from investor	—	—	254	—	—	254
Net income	—	—	—	—	801,713	801,713
Comprehensive income	—	—	—	33,242	—	33,242
Balance, June 30, 2025	79,378,145	$ 78	$ 2,631,180	$ 7,356	$ (2,845,160)	$ (206,546)

See accompanying unaudited notes.

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

	Six Months Ended June 30,	
	2026	**2025**
Cash flows from operating activities		
Net income	$ 80,687	$ 801,713
Adjustments to reconcile net income to net cash provided by operating activities:		
Depreciation and amortization	31,287	14,804
Non-cash operating lease expense	3,553	3,634
Non-cash royalty revenue related to sale of future royalties	(117,940)	(85,099)
Non-cash interest expense on liability related to sale of future royalties	131,008	99,329
Change in valuation of contingent consideration	—	(800)
Tangible asset impairment and losses on transactions, net	927	176
Inducement expense related to repurchase of 2026 Convertible Notes	3,386	—
Unrealized loss on ClearPoint Equity Investments	—	3,078
Unrealized gain on marketable securities - equity investments	(1,914)	(1,538)
Amortization of discounts on investments, net	(8,270)	(6,869)
Amortization of debt issuance costs	689	601
Share-based compensation expense	46,712	36,603
Unrealized foreign currency transaction losses, net	458	4,939
Changes in operating assets and liabilities:		
Inventory, net	(13,217)	(8,591)
Prepaid expenses and other current assets	9,544	(12,197)
Trade and royalty receivables, net	(60,118)	(25,891)
Deposits and other assets	(579)	(827)
Accounts payable and accrued expenses	(31,213)	6,864
Other liabilities	(4,125)	(17,320)
Deferred revenue	(804)	3,845
Payments on contingent consideration	—	(4,684)
Net cash provided by operating activities	$ 70,071	$ 811,770
Cash flows from investing activities		
Purchases of fixed assets	$ (3,389)	$ (2,971)
Purchases of marketable securities- available for sale	(921,643)	(847,164)
Purchases of marketable securities- equity investments	(32,976)	(17,029)
Sale of marketable securities- available for sale	709,325	247,151
Sale of marketable securities- equity investments	37,499	17,129
Acquisition of product rights and licenses	(5,108)	(3,485)
Net cash used in investing activities	$ (216,292)	$ (606,369)
Cash flows from financing activities		
Proceeds from exercise of options	$ 19,637	$ 16,885
Proceeds from employee stock purchase plan	3,163	2,827
Repurchase of the 2026 Convertible Notes	(327,890)	—
Conversion of the 2026 Convertible Notes	(12,879)	—
Debt issuance costs related to 2031 Convertible Notes	(13,750)	—
Proceeds from issuance of 2031 Convertible Notes	550,000	—
Payments on contingent consideration obligation	—	(6,341)
Net cash provided by financing activities	$ 218,281	$ 13,371
Effect of exchange rate changes on cash	(4,062)	18,981
Net increase in cash and cash equivalents	67,998	237,753
Cash and cash equivalents, and restricted cash beginning of period	998,334	795,316
Cash and cash equivalents, and restricted cash end of period	$ 1,066,332	$ 1,033,069
Supplemental disclosure of cash information		
Cash paid for interest	$ 3,017	$ 2,156
Total income taxes paid, net of refunds received:		
Federal	—	
State:		
Illinois	4,270	
Florida	1,300	
Other State	53	
Foreign:	487	
Total income taxes paid, net of refunds received	$ 6,110	$ 39,188
Supplemental disclosure of non-cash investing and financing activity		
Unrealized loss on marketable securities, net of tax	$ (2,150)	$ (274)
Right-of-use assets obtained in exchange for operating lease obligations	—	850
Decrease in right-of-use assets related to lease modifications and termination	1,466	—
Decrease in operating lease liabilities due to lease modifications and termination	1,547	—
Acquisition of product rights and licenses	4,566	2,285
Fixed asset additions through tenant improvement allowance	—	385
Accrued debt issuance fees for 2031 Convertible Notes	850	—
Capital expenditures unpaid at the end of the period	593	—
Milestone payable	30,000	25,150

See accompanying unaudited notes.

PTC Therapeutics, Inc.

Notes to Consolidated Financial Statements (unaudited)
June 30, 2026
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 dedicated to the discovery, development and commercialization of clinically differentiated medicines for children and adults living with rare disorders. PTC is advancing a robust and diversified pipeline of transformative medicines as part of its mission to provide access to best-in-class treatments for patients with unmet medical needs. PTC's strategy is to leverage its scientific expertise and global commercial infrastructure to optimize value for its patients and other 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 developed Sephience™ (sepiapterin), a product for the treatment of phenylketonuria ("PKU"), a rare inherited metabolic disease characterized by the body's inability to break down an essential amino acid called phenylalanine, and which can result in neurological and other symptoms. In June 2025, Sephience was granted marketing authorization by the European Commission ("EC") for the treatment of children and adults living with PKU within the European Economic Area ("EEA"). In July 2025, Sephience was approved by the U.S. Food and Drug Administration ("FDA") for the treatment of pediatric and adult patients living with PKU in the United States age one month and above. In December 2025, Sephience was approved by the Japanese Ministry of Health, Labor, and Welfare ("MHLW") for the treatment of children and adults living with PKU in Japan. In February 2026, Sephience was approved by ANVISA, the Brazilian health regulatory authority, for the treatment of children and adults living with PKU in Brazil. Sephience is also approved in additional geographies.

The Company has two products, Translarna™ (ataluren) and Emflaza® (deflazacort), for the treatment of Duchenne muscular dystrophy ("DMD"), a rare, life-threatening disorder. While Translarna previously had conditional approval in the EEA, in March 2025, the EC adopted the negative opinion of the Committee for Medicinal Products for Human Use, or CHMP, of the European Medicines Agency, or EMA, to not renew the conditional marketing authorization of Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy ("nmDMD"). However, the EC indicated that individual countries within the European Union, can leverage Articles 117(3) and 5(1) of the EU Directive 2001/83 to allow continued commercial use of Translarna. Translarna has marketing authorization in additional geographies outside of the EEA, but the EC adoption of the CHMP negative opinion may affect future reauthorizations. Emflaza is approved in the United States for the treatment of DMD in patients two years and older.

Translarna is an investigational new drug in the United States. In 2017, the Company filed a new drug application ("NDA") for Translarna for the treatment of nmDMD over protest with the FDA and the Office of Drug Evaluation I of the FDA issued a complete response letter for the NDA, stating that it was unable to approve the application in its current form. The Company re-submitted the NDA in July 2024 and in October 2024, the FDA accepted for review the resubmission of the NDA for Translarna for the treatment of nmDMD. Following feedback from the FDA, the Company withdrew the NDA resubmission for Translarna in February 2026. Further development of Translarna for the treatment of nmDMD in the United States is not planned.

The Company has developed Upstaza™ (eladocagene exuparvovec), a gene therapy used for the treatment of Aromatic L-Amino Acid Decarboxylase ("AADC") deficiency ("AADC deficiency"), a rare central nervous system ("CNS") disorder arising from reductions in the enzyme AADC that results from mutations in the dopa decarboxylase gene. In July 2022, the EC approved Upstaza for the treatment of AADC deficiency for patients 18 months and older within the EEA. In November 2022, the Medicines and Healthcare Products Regulatory Agency approved Upstaza for the treatment of AADC deficiency for patients 18 months and older within the United Kingdom. In November 2024, the Company's biologics license application 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. Waylivra has also received marketing authorization in the EU for the treatment of FCS. In December 2022, ANVISA approved Waylivra for the treatment of familial partial lipodystrophy.

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.

In addition to the Company's SMA program, the Company's splicing platform also includes votoplam (PTC518), which is being developed for the treatment of Huntington's disease ("HD"). The Company initiated a Phase 2 study of votoplam for the treatment of HD in the first quarter of 2022, which consisted 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 votoplam biomarker effect. In June 2024, the Company announced interim results from the full Phase 2 study of votoplam. At month 12, votoplam 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, votoplam continued to be well tolerated. In September 2024, the FDA granted Fast Track designation to the votoplam 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 votoplam. 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 votoplam 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 Neurofilament light ("NfL") lowering. In November 2024, the Company entered into a License and Collaboration Agreement (the "Novartis Agreement") with Novartis Pharmaceuticals Corporation ("Novartis"), relating to its votoplam HD program which included related molecules. This transaction closed in January 2025, triggering a $1.0 billion upfront cash payment to the Company. In April 2026, Novartis announced that it had commenced the global Phase 3 clinical trial, which is expected to enroll approximately 770 individuals with early symptomatic disease, randomized 3:2 to receive votoplam 10 milligrams or placebo, and includes an interim analysis. Pursuant to the Novartis Agreement, the initiation of the Phase 3 clinical trial triggered a $50.0 million milestone payment to the Company. The $50.0 million development milestone is recorded as collaboration and license revenue for the three and six months ending June 30, 2026. Also in April 2026, the Company reported positive topline results from the 24-month interim analysis of the PIVOT-HD long-term extension study, with favorable dose-dependent effects on disease progression for Stage 2 HD patients following 24 months of votoplam treatment compared to an external natural history cohort, with 52% slowing of disease progression on the Composite Unified Huntington's Disease Rating Scale at the 10 milligram dose level. While the Phase 3 clinical trial remains the base case for votoplam approval, Novartis and the Company are finalizing a plan to engage with FDA to discuss the 24-month results in the second half of 2026.

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 and non-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. In August 2025, the FDA issued a complete response letter related to the NDA stating that substantial evidence of efficacy was not demonstrated for vatiquinone and that an additional adequate and well-controlled study would be needed to support NDA resubmission. The Company met with the FDA in the fourth quarter of 2025 to discuss the vatiquinone development program, at which time the FDA suggested an additional study be conducted to support NDA resubmission. In April 2026, the Company again met with FDA to discuss the design of a new trial to provide additional data to support NDA resubmission. Based on the meeting discussion and written feedback, the Company plans to initiate the PROVE-FA open label study using matched natural history control in the third quarter of 2026. This study is expected to enroll approximately 120 patients ages 7 to 21 and the study primary endpoint is the change in mFARS from baseline to month 24.

During the quarter ended June 30, 2026, the Company initiated a Phase 1 study of PTC612, its oral NLRP3 inhibitor, and completed several of the single and multiple ascending dose treatment cohorts. Notably, this healthy volunteer study includes a cohort of individuals with obesity and cardiovascular disease, which the Company expects will provide an early view of pharmacokinetics and pharmacodynamics. The Company expects to initiate a Phase 2a study of PTC844, its next-generation DHODH inhibitor, in the third quarter of 2026. The PTC844 study will be a 12-week pharmacokinetics and pharmacodynamics study in which the Company will assess treatment effect on biomarkers related to T-cell and B-cell immunity. The Company expects that the results of this study will help inform the ultimate target indications for PTC844.

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 June 30, 2026, the Company had an accumulated deficit of approximately $2,883.5 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 areas addressed by the Company's product candidates. The Company has also relied on revenue generated from net sales of its products, the 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, and the revenues associated with the Novartis Agreement. The Company expects that cash flows from the sales of its products, 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 after the issuance date of these financial statements.

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, 2025 included in the Company's Annual Report on Form 10-K filed with the Securities and Exchange Commission (the "SEC") on February 19, 2026 (the "2025 Form 10-K"). There were no changes to significant accounting policies for the three and six months ended June 30, 2026. Selected significant accounting policies are discussed in further detail below.

Basis of presentation

The accompanying financial information as of June 30, 2026 and for the three and six months ended June 30, 2026 and 2025 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, 2025 and notes thereto included in the 2025 Form 10-K.

In the opinion of management, the unaudited financial information as of June 30, 2026 and for the three and six months ended June 30, 2026 and 2025 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 and six months ended June 30, 2026 are not necessarily indicative of the results to be expected for the year ended December 31, 2026 or for any other interim period or for any other future year.

Use of estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. Significant estimates in these consolidated financial statements have been made in connection with the calculation of net product sales, certain accruals related to the Company's research and development expenses, valuation procedures for 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 $3.0 million in connection with an amendment and restatement of the Company's lease in Hopewell Township, New Jersey. Restricted cash also contains an unconditional, irrevocable and transferable letter of credit of $10.0 million in connection with obligations for the Company's 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 collateral account of $2.8 million in connection with the Company's corporate card program. The collateral account is classified within deposits and other assets on the consolidated balance sheet due to its long-term nature. Restricted cash also includes a bank guarantee of $0.7 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- June 30, 2026	Beginning of period- December 31, 2025
Cash and cash equivalents	$ 1,049,887	$ 984,648
Restricted cash included in deposits and other assets	16,445	13,686
Total Cash, cash equivalents and restricted cash per consolidated statement of cash flows	$ 1,066,332	$ 998,334

Revenue recognition

Net product revenue

The Company's net product revenue primarily consists of sales of Sephience for the treatment of PKU. Net product revenues also includes 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 obligation with

its customers has been satisfied. The Company's performance obligation is to provide products based on customer orders from distributors, hospitals, specialty pharmacies or retail pharmacies. The performance obligation is 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 reassess 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 and six months ended June 30, 2026 and 2025, no allowance was recorded for credit losses. The allowance for doubtful accounts was $15.6 million as of June 30, 2026 and $15.2 million as of December 31, 2025. For the three and six months ended June 30, 2026, bad debt expense was $0.1 million and $0.8 million, respectively. For the three and six months ended June 30, 2025, bad debt expense was $5.9 million and $7.8 million, respectively.

Income Taxes

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 June 30, 2026.

On December 15, 2022, the European Union 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.

Since 2022, TCJA amendments to Internal Revenue Code ("IRC") Section 174 have no longer permitted an immediate deduction for research and development expenditures in the tax year that such costs were incurred. Instead, these IRC Section 174 development costs have had to be capitalized and amortized over a 5-year or 15-year period for domestic and foreign expenditures, respectively. On July 4, 2025, the U.S. government enacted the One Big Beautiful Bill Act ("OBBBA") which generally extended the tax provisions previously enacted by the TCJA that were set to expire, as well as made other changes to federal tax law for multinational corporations. Effective beginning with PTC's 2025 tax year, the OBBBA restored immediate deductibility of domestic expenditures, while foreign expenditures will continue to be capitalized and amortized over 15 years. Additionally, IRC Section 174A, enacted as part of the OBBBA, allows for the immediate expensing of all remaining unamortized domestic costs either upfront in 2025, or ratably in 2025 and 2026.

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.

Recently issued accounting standards

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.

In September 2025, the FASB issued ASU 2025-06, Intangibles—Goodwill and Other— Internal-Use Software (Subtopic 350-40): Targeted Improvements to the Accounting for Internal-Use Software. ASU 2025-06 enhances accounting for software costs that are accounted for under Subtopic 350-40, Intangibles—Goodwill and Other—Internal-Use Software (referred to as "internal-use software"). The guidance is effective for public business entities for annual periods beginning after December 15, 2027, and interim reporting periods within those annual reporting periods. Entities have three transition options: prospective, retrospective, or modified prospective adoption. Early adoption is permitted as of the beginning of an annual reporting period. Instead of following prescriptive project stages for capitalization of internal-use software, entities will capitalize costs when management has authorized and committed funding for the software project and it is probable that the project will be completed and used as intended. The Company is assessing the impact of the adoption on the Company's consolidated financial statements and accompanying footnotes.

In December 2025, the FASB issued ASU 2025-11, Interim Reporting (Topic 270): Narrow-Scope Improvements. ASU 2025-11 clarifies and reorganizes existing guidance related to interim financial reporting and disclosures. The guidance is effective for public business entities for interim reporting periods with annual reporting periods beginning after December 15, 2027. The Company is assessing the impact of the adoption on the Company's consolidated financial statements and accompanying footnotes but expects the guidance will not have a material impact, as the guidance does not change the recognition or measurement of financial statement amounts.

In December 2025, the FASB issued ASU 2025-12, Codification Improvements. ASU 2025-12 makes targeted improvements to the FASB Accounting Standards Codification by clarifying language, correcting technical errors, and addressing unintended codification applications across various topics in the U.S. GAAP. The guidance is effective for public business entities for interim reporting periods with annual reporting periods beginning after December 15, 2026. The Company is assessing the impact of the adoption on the Company's consolidated financial statements and accompanying footnotes but expects the guidance will not have a material impact, as the guidance does not change the recognition or measurement of financial statement amounts.

Impact of recently adopted accounting pronouncements

In July 2025, the FASB issued ASU 2025-05, Financial Instruments-Credit Losses (Topic 326): Measurement of Credit Losses for Accounts Receivable and Contract Assets. ASU 2025-05 addresses challenges encountered when applying the guidance in Topic 326, Financial Instruments—Credit Losses, to current accounts receivable and current contract assets arising from transactions accounted for under Topic 606, Revenue from Contracts with Customers. The guidance is effective for public business entities for annual periods beginning after December 15, 2025, and interim reporting periods within those annual reporting periods. Early adoption is permitted in both interim and annual reporting periods in which financial statements have not yet been issued or made available for issuance. In developing reasonable and supportable

forecasts as part of estimating expected credit losses, the guidance provides that all entities may elect a practical expedient that assumes that current conditions as of the balance sheet date do not change for the remaining life of the asset. The Company adopted this guidance January 1, 2026. The adoption of this guidance did not have a material impact on the Company's consolidated financial statements and accompanying footnotes.

In April 2024, the FASB issued ASU 2024-04, Debt—Debt with Conversion and Other Options (Subtopic 470-20): Induced Conversions of Convertible Debt Instruments. ASU 2024-04 improves the relevance and consistency in application of the induced conversion guidance in Subtopic 470-20, Debt—Debt with Conversion and Other Options. The guidance is effective for all entities for annual reporting periods beginning after December 15, 2025, and interim reporting periods within those annual reporting periods. Early adoption is permitted for all entities that have adopted the amendments in Update 2020-06. The Company adopted this guidance January 1, 2026. Refer to Note 9 for additional details and the impact on the Company's consolidated financial statements.

3. Inventory

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

	June 30, 2026	December 31, 2025
Raw materials	$ 10,521	$ 2,291
Work in progress	57,775	55,686
Finished goods	23,058	21,670
Total inventory	$ 91,354	$ 79,647

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 and six months ended June 30, 2026, the Company recorded inventory write-downs of $0.9 million and $2.7 million, respectively, primarily related to adjustments to inventory reserves and product approaching expiration. For the three and six months ended June 30, 2025, the Company recorded inventory write-downs of $0.8 million and $4.3 million, respectively. 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 and six months ended June 30, 2026 and 2025, these amounts were immaterial. For the six months ended June 30, 2026, the Company recorded a $0.8 million loss related to inventory impairments, which is recorded as tangible asset impairment and losses on transactions, net on the statements of operations. No inventory impairments were recorded for the three months ended June 30, 2026 and for the three and six months ended June 30, 2025.

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's marketable securities consist of both debt securities and equity investments. The Company previously owned 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") represented financial instruments, and therefore, were recorded at fair value, which was readily determinable. As of December 31, 2025, the Company sold all of its ClearPoint Equity Investments.

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 tables presented below are a summary of changes in the fair value for the Company's marketable securities – equity investments and ClearPoint Equity Investments for the three and six months ended June 30, 2026 and June 30, 2025:

	Ending Balance at March 31, 2026	Unrealized Gain	Foreign Currency Unrealized Gain	Investments Purchased	Sales	Ending Balance at June 30, 2026
Marketable securities - equity investments	$ 48,443	700	1,083	9,019	(27,614)	$ 31,631
Total Fair Value	$ 48,443	$ 700	$ 1,083	$ 9,019	$ (27,614)	$ 31,631

	Ending Balance at March 31, 2025	Unrealized Gain	Foreign Currency Unrealized Gain	Investments Purchased	Sales	Ending Balance at June 30, 2025
Marketable securities - equity investments	$ 28,187	764	1,665	11,820	(8,233)	$ 34,203
ClearPoint Equity Investments	10,637	44	—	—	—	10,681
Total Fair Value	$ 38,824	$ 808	$ 1,665	$ 11,820	$ (8,233)	$ 44,884

	Ending Balance at December 31, 2025	Unrealized Gain	Foreign Currency Unrealized Gain	Investments Purchased	Sales	Ending Balance at June 30, 2026
Marketable securities - equity investments	$ 31,596	1,914	2,644	32,976	(37,499)	$ 31,631
Total Fair Value	$ 31,596	$ 1,914	$ 2,644	$ 32,976	$ (37,499)	$ 31,631

	Ending Balance at December 31, 2024	Unrealized Gain/(Loss)	Foreign Currency Unrealized Gain	Investments Purchased	Sales	Ending Balance at June 30, 2025
Marketable securities - equity investments	$ 29,034	1,538	3,731	17,029	(17,129)	$ 34,203
ClearPoint Equity Investments	13,759	(3,078)	—	—	—	10,681
Total Fair Value	$ 42,793	$ (1,540)	$ 3,731	$ 17,029	$ (17,129)	$ 44,884

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 June 30, 2026 and December 31, 2025:

	June 30, 2026			
	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	$ 1,147,564	$ —	$ 1,147,564	$ —
Marketable securities - equity investments	$ 31,631	$ 31,631	$ —	$ —

	December 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	$ 929,127	$ —	$ 929,127	$ —
Marketable securities - equity investments	$ 31,596	$ 31,596	$ —	$ —

No transfers of assets between Level 1, Level 2, or Level 3 of the fair value measurement hierarchy occurred during the three and six months ended June 30, 2026 and year ended December 31, 2025.

The following is a summary of marketable securities accounted for as available for sale debt securities at June 30, 2026 and December 31, 2025:

	June 30, 2026			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 134,989	$ 1	$ (82)	$ 134,908
Corporate debt securities	510,870	2	(797)	510,075
Government obligations	503,068	5	(492)	502,581
Total	$ 1,148,927	$ 8	$ (1,371)	$ 1,147,564

	December 31, 2025			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 93,113	$ 8	$ (9)	$ 93,112
Corporate debt securities	279,090	181	(9)	279,262
Government obligations	556,137	616	—	556,753
Total	$ 928,340	$ 805	$ (18)	$ 929,127

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 and six months ended June 30, 2026 and 2025, 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 and six months ended June 30, 2026 and 2025, no allowance was recorded for credit losses. Unrealized gains and losses are reported as a component of accumulated other comprehensive income (loss) in stockholders' deficit.

For the three and six months ended June 30, 2026 and 2025, realized gains from the sale of available for sale debt securities were immaterial. 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 June 30, 2026 are as follows:

	June 30, 2026					
	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	$ (82)	120,039	—	—	(82)	$ 120,039
Corporate debt securities	$ (797)	493,847	—	—	(797)	$ 493,847
Government obligations	$ (492)	454,099	—	—	(492)	$ 454,099
Total	$ (1,371)	$ 1,067,985	$ —	$ —	$ (1,371)	$ 1,067,985

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, 2025 are as follows:

	December 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	$ (9)	50,306	—	—	(9)	$ 50,306
Corporate debt securities	$ (9)	45,068	—	—	(9)	$ 45,068
Total	$ (18)	$ 95,374	$ —	$ —	$ (18)	$ 95,374

Available for sale debt securities at June 30, 2026 and December 31, 2025 mature as follows:

	June 30, 2026	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 134,908	$ —
Corporate debt securities	498,443	11,632
Government obligations	502,581	—
Total	$ 1,135,932	$ 11,632

	December 31, 2025	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 93,112	$ —
Corporate debt securities	279,262	—
Government obligations	556,753	—
Total	$ 929,127	$ —

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 June 2026, the Company issued, at par value, $550.0 million aggregate principal amount of 0% convertible senior notes due 2031 (the "2031 Convertible Notes"), which included an option to purchase up to an additional $50.0 million in aggregate principal amount of the 2031 Convertible Notes, which was exercised in full by the initial purchasers. The Company accounted for the 2031 Convertible Notes as a single liability measured at amortized cost, as further discussed in Note 9. The fair value of the 2031 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 2031 Convertible Notes observed in market trading which are Level 2 inputs. The estimated fair value of the 2031 Convertible Notes at June 30, 2026 was $579.2 million.

In September 2019, the Company issued $287.5 million aggregate principal amount of 1.50% convertible senior notes due September 15, 2026 (the "2026 Convertible Notes"). In June 2026, the Company used a portion of the proceeds of the 2031 Convertible Notes to repurchase $222.0 million aggregate principal amount of its 2026 Convertible Notes for approximately $328.8 million, inclusive of accrued interest, as further discussed in Note 9. Additionally, during the three months ended June 30, 2026, a holder converted $10.0 million principal amount of the 2026 Convertible Notes in exchange for $12.9 million in cash and 3,506 shares of the Company's common stock. Refer to Note 9 for further information. As of June 30, 2026, the remaining aggregate principal of the 2026 Convertible Notes is $55.5 million. 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 June 30, 2026 and December 31, 2025 was $85.5 million and $424.5 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. In 2025, the probability of triggering the remaining contingent consideration was determined to be remote, and therefore the balance was written down to zero. Refer to Note 10 for additional details.

5. Accounts payable and accrued expenses

Accounts payable and accrued expenses at June 30, 2026 and December 31, 2025 consist of the following:

	June 30, 2026	December 31, 2025
Employee compensation, benefits, and related accruals	$ 39,303	$ 73,737
Income tax payable	—	4,677
Consulting and contracted research	16,395	25,007
Sales allowance	119,562	129,197
Sales rebates	109,741	76,704
Royalties	13,580	9,574
Accounts payable	40,447	45,487
Milestone payable	30,000	416
Other	13,217	16,473
Total	$ 382,245	$ 381,272

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 and six months ended June 30, 2026 and 2025. 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 June 30, 2026.

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 June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
Numerator:				
Net income (loss), basic	$ 83,496	$ (64,849)	$ 80,687	$ 801,713
Add: Interest expense, net of tax, on the Company's 2026 and 2031 Convertible Notes	959	—	2,045	2,161
Net income (loss), diluted	$ 84,455	$ (64,849)	$ 82,732	$ 803,874
Denominator:				
Weighted-average number of shares outstanding, basic	83,006,808	78,151,240	82,765,248	78,438,830
Effect of dilutive securities:				
Common stock issuable under the Company's equity incentive plans	3,551,404	—	3,591,103	2,589,633
Common stock issuable under the Company's 2026 and 2031 Convertible Notes	5,461,797	—	5,467,922	5,474,115
Weighted-average common shares outstanding, diluted	92,020,009	78,151,240	91,824,273	86,502,578
Net income (loss) per common share, basic	$ 1.01	$ (0.83)*	$ 0.97	$ 10.22
Net income (loss) per common share, diluted	$ 0.92	$ (0.83)*	$ 0.90	$ 9.29

* In the three months ended June 30, 2025, 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 on a year to date basis using the treasury stock method.

	As of June 30,	
	2026	2025
Stock options	807,769	3,494,565
Unvested restricted stock units	1,206,529	83,657
Unvested performance based restricted stock units	93,478	—
Total	2,107,776	3,578,222

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 June 30, 2026, awards for 3,408,235 shares of common stock are available for issuance under the Amended 2013 LTIP.

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 June 30, 2026, awards for 1,754,494 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.

Stock option awards—From January 1, 2026 through June 30, 2026, the Company issued a total of 757,556 stock options to various employees. Of those, 25,755 were inducement grants for non-statutory stock options, all of which were made pursuant to the 2020 Inducement Stock Incentive Plan. A summary of stock option activity is as follows:

	Number of options	Weighted-average exercise price	Weighted-average remaining contractual term	Aggregate intrinsic value(in thousands)
Outstanding at December 31, 2025	6,396,202	$ 43.02		
Granted	757,556	$ 76.44		
Exercised	(506,463)	$ 38.80		
Forfeited/Cancelled	(21,132)	$ 43.72		
Outstanding at June 30, 2026	6,626,163	$ 47.16	5.84 years	$ 227,986
Expected to vest at June 30, 2026	1,593,265	$ 54.41	8.62 years	$ 43,276
Exercisable at June 30, 2026	4,855,637	$ 44.32	4.81 years	$ 180,891

The fair value of grants made in the six months ended June 30, 2026, was contemporaneously estimated on the date of grant using the following assumptions:

	Six months ended June 30, 2026
Risk-free interest rate	3.78% - 4.21%
Expected volatility	54%
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 six months ended June 30, 2026 was $40.21 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, 2026, through June 30, 2026, the Company issued a total of 1,293,798 restricted stock units to various employees. Of those, 30,610 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, including performance-based restricted stock units ("PSUs") for which the performance conditions were satisfied:

	Restricted Stock Units	
	Number of Shares	Weighted Average Grant Date Fair Value
Unvested at December 31, 2025	3,667,130	$ 38.20
Granted	1,293,798	76.53
Performance based PSUs with conditions satisfied	25,000	46.37
Vested	(1,294,464)	38.16
Forfeited	(169,015)	45.16
Unvested at June 30, 2026	3,522,449	$ 52.02

Performance-based Restricted Stock Units—The Company has granted Chief Executive Officer, Dr. Matthew Klein PSUs which will vest only if challenging performance goals relating to development and regulatory milestones are achieved which are considered "PSUs with performance conditions". The Company also granted Dr. Klein PSUs, which will vest only if challenging performance goals relating to stock price goals are achieved. In addition, the Company granted PSUs to Dr. Klein for which the number of PSUs that vest shall be determined based upon the Company's total shareholder return ("TSR") over the period beginning on November 14, 2025 and ending on December 31, 2028 relative to the TSR of the group of companies in the Nasdaq Biotechnology Index. Together, the PSUs with stock price goals and the PSUs related to the TSR are considered the "PSUs with market conditions".

During the six months ended June 30, 2026, 25,000 of the PSUs with performance conditions granted to Dr. Klein in December 2024 were satisfied due to the Company's achievement of corporate goals and are included in the restricted stock units table above. Also, during the six months ended June 30, 2026, the Company granted Dr. Klein 37,500 PSUs with performance conditions which will only vest if challenging performance goals relating to regulatory milestones are achieved over an approximately three-year performance period. The expenses related to satisfied PSUs were $0.7 million and $1.2 million for the three and six months ended June 30, 2026, respectively. The achievement of the remaining performance goals for the PSUs with performance conditions granted to Dr. Klein has not yet been deemed probable and therefore no expense has been recognized to date. The expenses related to the PSUs with market conditions granted to Dr. Klein were $1.1 million and $2.3 million for the three and six months ended June 30, 2026, respectively, and $0.3 million for each of the three and six months ended June 30, 2025.

The following table summarizes information regarding the Company's PSUs granted to Dr. Klein:

	Performance-based Restricted Stock Units

	Performance Conditions	Market Conditions	Total
Total PSUs granted for which the performance or market conditions were not yet satisfied at December 31, 2025	25,000	115,625	140,625
PSUs with performance or market conditions granted during the period	37,500	—	37,500
PSUs with performance or market conditions satisfied during the period	(25,000)	—	(25,000)
Total PSUs granted for which the performance or market conditions were not yet satisfied at June 30, 2026	37,500	115,625	153,125

Employee Stock Purchase Plan—In June 2016, the Company established an Employee Stock Purchase Plan (as amended, the "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 and six months ended June 30, 2026, the Company recorded $0.5 million and $1.0 million, respectively, 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, performance-based restricted stock units and the ESPP as follows:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
Research and development	$ 10,547	$ 9,030	$ 21,677	$ 17,693
Selling, general and administrative	12,744	9,513	25,035	18,910
Total	$ 23,291	$ 18,543	$ 46,712	$ 36,603

As of June 30, 2026, there was approximately $206.8 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

The Company has a royalty purchase agreement, (the "A&R Royalty Purchase Agreement"), with Royalty Pharma. Under the A&R Royalty Purchase Agreement, Royalty Pharma has provided funding to the Company totaling $2.1 billion. In exchange for these fundings, the Company sold Royalty Pharma 100% of the Company's rights to receive sales-based royalty payments on worldwide net sales of Roche's Evrysdi® (risdiplam) product and any other product developed pursuant to the SMA collaboration with the Company, Roche, and the SMA Foundation. Pursuant to A&R Royalty Purchase Agreement, the Company is entitled to three potential additional purchase price payments of $20.0 million each conditioned upon receipt by Royalty Pharma of more than $347.0 million of Assigned Royalty Payments (as defined in the A&R Royalty Purchase Agreement) in respect of Calendar Year Net Sales (as defined in the A&R Royalty Purchase Agreement) arising in 2027, $363.0 million of Assigned Royalty Payments in respect of Calendar Year Net Sales arising in 2028, and $379.0 million of Assigned Royalty Payments in respect of Calendar Year Net Sales arising in 2029, respectively.

Pursuant to the guidance in ASC 470-10-25-2, the Company determined that these fundings should be classified as debt and are 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 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.

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 six months ended June 30, 2026:

Liability for sale of future royalties- (current and noncurrent)	Six Months Ended June 30, 2026
Beginning balance as of December 31, 2025	$ 2,308,366
Less: Non-cash royalty revenue payable to Royalty Pharma	(117,940)
Plus: Non-cash interest expense recognized	131,008
Ending balance	$ 2,321,434
Effective interest rate as of June 30, 2026	11.0%

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

2031 Convertible Notes

In June 2026, the Company issued, at par value, $550.0 million aggregate principal amount of 0% convertible senior notes due 2031 (the "2031 Convertible Notes"), which reflects the exercise in full by the initial purchasers of their option to purchase up to an additional $50.0 million in aggregate principal amount of the 2031 Convertible Notes. The net proceeds to the Company from the offering were approximately $535.4 million after deducting the initial purchasers' discounts and commissions and the estimated offering expenses payable by the Company. The 2031 Convertible Notes do not bear regular interest and the principal amounts of the 2031 Convertible Notes will not accrete.

The 2031 Convertible Notes are governed by an indenture (the "2031 Convertible Notes Indenture") with U.S Bank Trust Company National Association as trustee (the "2031 Convertible Notes Trustee").

The 2031 Convertible Notes may bear special interest under specified circumstances relating to the Company's failure to comply with its reporting obligations under the 2031 Convertible Notes Indenture or if the 2031 Convertible Notes are not freely tradeable as required by the 2031 Convertible Notes Indenture.

Special interest, if any, will be payable semiannually in arrears on June 15 and December 15 of each year, beginning on December 15, 2026 (if and to the extent that special interest is payable). The 2031 Convertible Notes will mature on June 15, 2031, unless earlier converted, redeemed or repurchased pursuant to their terms.

The initial conversion rate of the 2031 Convertible Notes is 9.3042 shares of the Company's common stock, per $1,000 principal amount of 2031 Convertible Notes (which is equivalent to an initial conversion price of approximately $107.48 per share). The conversion rate will be subject to adjustment upon the occurrence of certain specified events but will not be adjusted for any accrued and unpaid special interest. In addition, upon the occurrence of a make-whole fundamental change (as defined in the 2031 Convertible Notes Indenture) or an issuance of a notice of redemption, the Company will, in certain circumstances, increase the conversion rate by a number of additional shares for a holder that elects to convert its 2031 Convertible Notes in connection with such make-whole fundamental change or notice of redemption.

Holders may convert all or any portion of their 2031 Convertible Notes at their option at any time prior to the close of business on the business day immediately preceding March 15, 2031 only under the following circumstances: (1) during any calendar quarter commencing after the calendar quarter ending on September 30, 2026 (and only during such calendar quarter), if the last reported sale price of the common stock for at least 20 trading days (whether or not consecutive) during a period of 30 consecutive trading days ending on, and including, 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; (2) during the five business day period after any five consecutive trading day period (the "measurement period") in which the trading price per $1,000 principal amount of 2031 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 common stock and the conversion rate on each such trading day; (3) if the Company calls any or all of the 2031 Convertible Notes for redemption, at any time prior to the close of business on the second scheduled trading day immediately preceding the relevant redemption date; or (4) upon the occurrence of specified corporate events. On or after March 15, 2031 until the close of business on the second scheduled trading day immediately preceding the maturity date, holders may convert all or any portion of their 2031 Convertible Notes at any time, regardless of the foregoing circumstances. Upon conversion, the Company will satisfy its conversion obligation by paying or delivering, as the case may be, cash, shares of Common Stock or a combination of cash and shares of common stock, at the Company's election.

The Company may not redeem the 2031 Convertible Notes prior to June 20, 2029. The Company may redeem for cash all or any portion of the 2031 Convertible Notes, at the Company's option, on or after June 20, 2029 if the last reported sale price of the common stock has been at least 130% of the conversion price then in effect for at least 20 trading days (whether or not consecutive) during any 30 consecutive trading day period (including the last trading day of such period) ending on, and including, the trading day immediately preceding the date on which the Company provides a written notice of redemption at a redemption price equal to 100% of the principal amount of the 2031 Convertible Notes to be redeemed, *plus* any accrued and unpaid special interest to, but excluding, the redemption date. No "sinking fund" is provided for the 2031 Convertible Notes.

If the Company undergoes a fundamental change (as defined in the 2031 Convertible Notes Indenture) prior to the maturity date, then, subject to certain conditions, holders of 2031 Convertible Notes may require the Company to repurchase for cash all or any portion of their 2031 Convertible Notes at a fundamental change repurchase price equal to 100% of the principal amount of the 2031 Convertible Notes to be repurchased, plus any accrued and unpaid special interest to, but excluding, the fundamental change repurchase date.

The 2031 Convertible Notes are the Company's general unsecured, senior obligations and will rank senior in right of payment to any of its indebtedness that is expressly subordinated in right of payment to the 2031 Convertible Notes; equal in right of payment with all of the Company's existing and future unsecured indebtedness that is not so subordinated (including any of the Company's outstanding 2026 Convertible Notes); effectively junior in right of payment to any of the Company's senior, secured indebtedness to the extent of the value of the assets securing such indebtedness; and structurally junior to all indebtedness and other liabilities (including trade payables) of the Company's current or future subsidiaries.

The 2031 Convertible Notes Indenture contains customary events of default with respect to the 2031 Convertible Notes, including that upon certain events of default (including the Company's failure to make any payment of principal or any special interest on the Notes when due and payable) occurring and continuing, the 2031 Convertible Notes Trustee by written notice to the Company, or the holders of at least 25% in principal amount of the outstanding 2031 Convertible Notes by notice to the Company and the 2031 Convertible Notes Trustee, may (subject to the provisions of the 2031 Convertible Notes Indenture) declare 100% of the principal of and accrued and unpaid special interest, if any, on all the 2031 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 special interest, if any, on the 2031 Convertible Notes will automatically become due and payable without any further act or declaration on the part of the holders or the 2031 Convertible Notes Trustee. Upon such a declaration of acceleration, such principal and accrued and unpaid special interest, if any, will be due and payable immediately.

The Company accounted for the 2031 Convertible Notes as a single liability measured at amortized cost. The debt issuance costs were recorded as a direct deduction from the face value of the 2031 Convertible Notes and will be amortized to interest expense over the five-year term of the 2031 Convertible Notes using the effective interest rate method.

The 2031 Convertible Notes consist of the following:

	June 30, 2026	December 31, 2025
Principal	$ 550,000	$ —
Less: Debt issuance costs	(14,481)	—

Net carrying amount	$ 535,519	$ —

As of June 30, 2026, the remaining contractual life of the 2031 Convertible Notes is approximately 5.0 years.

The following table sets forth total interest expense recognized related to the 2031 Convertible Notes:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
Amortization of debt issuance costs	$ 119	$ —	$ 119	$ —
Total	$ 119	$ —	$ 119	$ —
Effective interest rate	0.5 %	— %	0.5 %	— %

2026 Convertible Notes

In September 2019, the Company issued, at par value, $287.5 million aggregate principal amount of 1.50% convertible senior notes due 2026, which included an option to purchase up to an additional $37.5 million in aggregate principal amount of the 2026 Convertible Notes, which was exercised in full by the initial purchasers. The 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 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 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").

Beginning 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. As of June 30, 2026, the 2026 Convertible Notes are convertible. 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. As of June 30, 2026, the 2026 Convertible Notes were redeemable.

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 (including any of the Company's outstanding 2031 Convertible Notes), 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) will, 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.

Following the issuance of the 2031 Convertible Notes, in June 2026 the Company used approximately $328.8 million of the net proceeds of the 2031 Convertible Notes to repurchase for cash $222.0 million aggregate principal amount of its 2026 Convertible Notes pursuant to privately negotiated transactions with certain holders entered into concurrently with the pricing of the offering of the 2031 Convertible Notes. The repurchase of the 2026 Convertible Notes was accounted for as an induced conversion in accordance with ASC-470-20. The excess of the fair value of the repurchase price over the if-converted value was $3.4 million and was recorded as inducement expense within other expense, net on the consolidated statements of operations. The net carrying amount of the repurchased 2026 Convertible Notes was derecognized, and the difference between the if-converted value and the net carrying amount of $102.7 million was recognized as a reduction in additional paid-in capital within the consolidated statement of stockholders' deficit.

During the three months ended June 30, 2026, a holder converted a portion of the 2026 Convertible Notes with a principal amount of $10.0 million in exchange for $12.9 million in cash and 3,506 shares of the Company's common stock. The Company recorded a $10.0 million reduction to the carrying value of the 2026 Convertible Notes, and the excess of $2.9 million was recognized as a reduction in additional paid-in capital within the Company's statement of stockholders' deficit.

The 2026 Convertible Notes consist of the following:

	June 30, 2026	December 31, 2025
Principal	$ 287,500	$ 287,500
Conversion of the 2026 Convertible Notes	(10,000)	—
Repurchase of the 2026 Convertible Notes	(222,000)	—
Less: Debt issuance costs	(49)	(869)
Net carrying amount	$ 55,451	$ 286,631

As of June 30, 2026, the remaining contractual life of the 2026 Convertible Notes is approximately 0.2 years.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
Contractual interest expense	$ 830	$ 1,067	$ 1,899	$ 2,135
Amortization of debt issuance costs	265	301	570	601
Total	$ 1,095	$ 1,368	$ 2,469	$ 2,736
Effective interest rate	1.9 %	1.9 %	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 Agreement and Plan of Merger, dated as of July 19, 2018 by and among the Company, Agilis Biotherapeutics, Inc. ("Agilis") and Agility Merger Sub, Inc. (such merger pursuant thereto, the "Agilis Merger"), 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 June 30, 2026, 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 June 30, 2026, 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 inflammation and ferroptosis 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. As of June 30, 2026, no contingent milestones have been achieved, and therefore, the remaining potential contingent milestone payments is $200.0 million.

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.

On August 5, 2025, the Company, certain former securityholders of Censa, and, for the limited purposes set forth in the agreement, Shareholder Representative Services LLC, entered into a Rights Satisfaction Agreement (the "Rights Satisfaction Agreement") pursuant to which such former securityholders of Censa (the "Participating Rightsholders") agreed to the cancellation and forfeiture of their rights to receive certain contingent payments from the Company based on worldwide annual net sales by the Company of products containing sepiapterin ("Net Sales of Product") under the Censa Merger Agreement in exchange for the consideration set forth in the Rights Satisfaction Agreement and further detailed below.

Pursuant to the terms of the Rights Satisfaction Agreement, the Participating Rightsholders canceled and forfeited their rights under the Censa Merger Agreement to receive a percentage of annual net sales during the applicable payment term equal to (i) 8% of annual Net Sales of Product for that portion of annual Net Sales of Product less than or equal to $250.0 million, (ii) 10% of annual Net Sales of Product for that portion of annual Net Sales of Product greater than $250.0 million but less than $500.0 million and (iii) 12% of annual Net Sales of Product for that portion of annual Net Sales of Product greater than $500.0 million (collectively, the "Net Sales Payments").

In consideration of the foregoing, the Company agreed to pay to the Participating Rightsholders an aggregate amount in cash up to $250.0 million (the "Upfront Consideration") upon the consummation of the transactions contemplated by the Rights Satisfaction Agreement and potential milestone payments (each an "Additional Milestone Payment") of up to $100.0 million each (or up to $500.0 million in the aggregate) based on the achievement of specified Net Sales Thresholds (as defined in the Censa Merger Agreement). The amount of the Upfront Consideration and the Additional Milestone Payments was subject to adjustment in the Rights Satisfaction Agreement based on the number of Participating Rightsholders.

At the consummation of the transactions contemplated by the Rights Satisfaction Agreement, based on the participation of former Censa securityholders holding approximately 90% of Censa's equity securities prior to the consummation of the transactions contemplated by the Censa Merger Agreement, the Company paid an aggregate amount of Upfront Consideration in cash of $225.1 million. Additionally, the Company is obligated to make Additional Milestone Payments in an amount equal to approximately $90.0 million upon achievement of each of the (i) first occurrence of a three or fewer consecutive calendar year period in which aggregate Net Sales of Product are greater than $3.0 billion, (ii) first occurrence of a five or fewer consecutive calendar year period in which aggregate Net Sales of Product are greater than $5.0 billion, (iii) first occurrence of a seven or fewer consecutive calendar year period in which aggregate Net Sales of Product are greater than $7.0 billion, (iv) first occurrence of a nine or fewer consecutive calendar year period in which aggregate Net Sales of Product are greater than $9.0 billion and (v) first occurrence of an 11 or fewer consecutive calendar year period in which aggregate Net Sales of Product are greater than $11.0 billion. If, after the consummation of the transactions contemplated by the Rights Satisfaction Agreement, any additional former securityholder of Censa executes and delivers a joinder to the Rights Satisfaction Agreement and becomes a party thereto, the Company will pay such former securityholder an amount in cash equal to such former securityholder's applicable pro rata share of the Upfront Consideration (less any Net Sale Payments previously received) and any Additional Milestone Payments that become payable to Participating Rightsholders under the Rights Satisfaction Agreement.

The Rights Satisfaction Agreement has no effect on the Censa Merger Agreement other than to provide for the cancellation and forfeiture of the Participating Rightsholders' rights to receive the Net Sales Payments described above. As a result, all other rights and obligations under the Censa Merger Agreement remain in effect pursuant to their terms, including, without limitation, the Company's obligation to pay certain contingent payments upon the achievement of certain development and Net Sales of Product milestones.

In June 2025, Sephience was granted marketing authorization by the EC for the treatment of children and adults living with PKU. Pursuant to the Censa Merger Agreement, the acceptance triggered a $25.0 million regulatory milestone payment to the former Censa securityholders. In July 2025, Sephience was granted FDA approval for the treatment of children and adults living with PKU. Pursuant to the Censa Merger Agreement, the approval triggered a $32.5 million milestone payment to the former Censa securityholders. These milestones were recorded as intangible assets and are being amortized to cost of product sales over their expected useful lives on a straight-line basis. As of June 30, 2026, $152.5 million of the $217.5 million in development and regulatory milestones have been paid to the former Censa securityholders.

As of the quarter ended June 30, 2026, aggregate Sephience global net sales in the prior four consecutive quarters exceeded $250.0 million, which, pursuant to the Censa Merger Agreement, triggered a $30.0 million net sales milestone payment to the former Censa securityholders. This milestone was recorded in accounts payable and accrued expenses and intangible assets on the Company's consolidated balance sheet as of June 30, 2026 and is being amortized to cost of product sales over its expected useful live on a straight-line basis. For the six months ended June 30, 2026, royalties of $2.2 million related payable to the former Censa securityholders were recorded on the consolidated balance sheet within intangible assets, net. Refer to Note 12 for additional details.

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. For the six months ended June 30, 2026, royalties of $3.2 million and $2.9 million related to Tegsedi and Waylivra, respectively, were recorded on the consolidated balance sheet within intangible assets, net. Refer to Note 12 for additional details.

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, Sephience, and Upstaza/Kebilidi 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

The Company views its operations and manages its business in one operating 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 derives its revenues through its worldwide net product sales, collaboration and license agreements, and royalty revenues.

Net product sales

During the three and six months ended June 30, 2026 and 2025, net product revenues consisted of the following:

	Three Months Ended June 30,					
	2026			2025		
(in thousands)	United States	International	Total	United States	International	Total
Sephience	$ 127,550	$ 23,760	$ 151,310	$ —	$ —	$ —
Translarna	—	42,217	42,217	—	59,470	59,470
Emflaza	24,634	—	24,634	36,353	—	36,353
Upstaza/Kebilidi	—	11,163	11,163	—	11,889	11,889
All other products	—	9,495	9,495	—	10,617	10,617
Total net product revenue	$ 152,184	$ 86,635	$ 238,819	$ 36,353	$ 81,976	$ 118,329

	Six Months Ended June 30,					
	2026			2025		
	United States	International	Total	United States	International	Total
Sephience	$ 239,590	$ 36,271	$ 275,861	$ —	$ —	$ —
Translarna	—	101,193	101,193	—	145,624	145,624
Emflaza	46,112	—	46,112	84,142	—	84,142
Upstaza/Kebilidi	2,998	17,759	20,757	—	20,547	20,547
All other products	—	20,469	20,469	—	21,442	21,442
Total net product revenue	$ 288,700	$ 175,692	$ 464,392	$ 84,142	$ 187,613	$ 271,755

Disaggregated net product revenues by country for the three and six months ended June 30, 2026 and 2025 are as follows:

	Three Months Ended June 30,		Six Months Ended June 30,	
(in thousands)	2026	2025	2026	2025
United States	$ 152,184	$ 36,353	$ 288,700	$ 84,142
Russia	25,581	18,124	28,830	56,638
Brazil	8,210	40,951	49,422	50,456
All other countries	52,844	22,901	97,440	80,519
Total net product revenue	$ 238,819	$ 118,329	$ 464,392	$ 271,755

For three and six months ended June 30, 2026, three of the Company's distributors each accounted for over 10% of the Company's net product sales. For three and six months ended June 30, 2025, three and two of the Company's distributors, respectively, each accounted for over 10% of the Company's net product sales.

As of June 30, 2026 and December 31, 2025, 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 June 30, 2026, 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 and six months ended June 30, 2026 and 2025, 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 recognize royalties on worldwide annual net sales of a commercial product under the SMA License Agreement. For the three and six months ended June 30, 2026, the Company has recognized $71.1 million and $117.9 million of royalty revenue related to Evrysdi, respectively. For the three and six months ended June 30, 2025 the Company has recognized $57.6 million and $94.0 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 votoplam 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 determined that there were three material and distinct performance obligations: the transfer of the licenses and know-how, the completion of the Phase 2A clinical trial, and continuing the ongoing open label extension ("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. All such performance obligations have been completed. 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.

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 and six months ended June 30, 2026, the Company recognized $50.6 million and $50.7 million in collaboration and license revenues, respectively, primarily related to a development milestone pursuant to the Novartis Agreement for Novartis's initiation of the first Phase 3 clinical trial for a Licensed Product (as defined in the Novartis Agreement), which triggered a $50.0 million milestone payment to the Company. During the three and six months ended June 30, 2025, the Company recognized $2.9 million and $992.7 million, respectively, in collaboration and license revenues related to performance obligations completed pursuant to the Novartis Agreement. Collaboration and license revenue during the six months ended June 30, 2025 was partially offset by $3.5 million related to a refund for a prior collaboration arrangement in relation to votoplam.

12. Intangible assets and goodwill

Definite-lived intangibles

Definite-lived intangible assets consisted of the following at June 30, 2026 and December 31, 2025:

Definite-lived intangible assets, gross	Ending Balance at December 31, 2025	Additions	Foreign currency translation	Ending Balance at June 30, 2026
Waylivra	18,598	2,933	(578)	20,953
Tegsedi	24,825	3,186	(757)	27,254
Kebilidi	10,731	—	—	10,731
Upstaza	106,937	—	—	106,937
Sephience	283,500	32,190	—	315,690
Total definite-lived intangibles, gross	$ 444,591	$ 38,309	$ (1,335)	$ 481,565

Definite-lived intangible assets, accumulated amortization	Ending Balance at December 31, 2025	Amortization	Foreign currency translation	Ending Balance at June 30, 2026
Waylivra	(8,689)	(1,853)	284	(10,258)
Tegsedi	(10,295)	(2,455)	337	(12,413)
Kebilidi	(1,006)	(972)	—	(1,978)
Upstaza	(27,437)	(7,950)	—	(35,387)
Sephience	(8,411)	(10,192)	—	(18,603)
Total definite-lived intangibles, accumulated amortization	$ (55,838)	$ (23,422)	$ 621	$ (78,639)
Total definite-lived intangibles, net				$ 402,926

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 six months ended June 30, 2026, royalties of $3.2 million and $2.9 million related to Tegsedi and Waylivra, respectively, were recorded on the consolidated balance sheet within intangible assets, net. As of June 30, 2026, a royalty payable of $2.0 million and $0.4 million for Tegsedi and Waylivra, respectively, was recorded on the consolidated balance sheet within accounts payable and accrued expenses.

As of the quarter ended June 30, 2026, aggregate Sephience global net sales in the prior four consecutive quarters exceeded $250.0 million, which, pursuant to the Censa Merger Agreement, triggered a $30.0 million net sales milestone payment to the former Censa securityholders. The $30.0 million milestone was recorded in accounts payable and accrued expenses on our consolidated balance sheet as of June 30, 2026. These milestones were recorded as intangible assets and are being amortized to cost of product sales over their expected useful lives on a straight-line basis.

The former Censa securityholders may also be entitled to receive other contingent payments subject to certain terms set forth in the Censa Merger Agreement related to sales of Sephience. In accordance with the guidance for an asset acquisition, the Company will record such payments when they become payable to the former Censa securityholders and increase the cost basis for the Sephience intangible asset. For the six months ended June 30, 2026, royalties of $2.2 million were recorded for Sephience. As of June 30, 2026, a royalty payable of $2.2 million for Sephience was recorded on the consolidated balance sheet within accounts payable and accrued expenses.

For the three months ended June 30, 2026 and 2025, the Company recognized amortization expense of $11.8 million and $4.1 million, respectively, related to its intangible assets. For the six months ended June 30, 2026 and 2025, the Company recognized amortization expense of $23.4 million and $7.9 million, respectively, related to its intangible assets.

The estimated future amortization of the Company's intangible assets is expected to be as follows:

	As of June 30, 2026
2026	$ 24,790
2027	49,581
2028	49,581
2029	43,571
2030 and thereafter	235,403
Total	$ 402,926

The weighted average remaining amortization period of the definite-lived intangibles as of June 30, 2026 is 10.7 years.

Goodwill

As a result of the Agilis Merger on August 23, 2018, the Company recorded $82.3 million of goodwill. As of June 30, 2026, there have been no changes to the balance of goodwill since the date of the Agilis Merger. Accordingly, the goodwill balance as of June 30, 2026 is $82.3 million.

13. Segment information

The Company views its operations and manages its business in one operating segment: life science. 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. The table below summarizes the significant expense categories for the life science segment regularly reviewed by the chief operating decision maker for the three and six months ended June 30, 2026 and 2025:

	Three months ended June 30,		Six months ended June 30,	
	2026	2025	2026	2025
Total revenues	$ 360,519	$ 178,875	$ 633,070	$ 1,354,971
Less:				
Cost of product, collaboration and license sales	10,933	8,093	30,611	15,800
Program spend	42,580	49,705	80,125	97,863
Employee costs	68,348	65,593	144,469	133,292

Manufacturing costs	6,232	19,519	15,317	34,470
Administrative costs	16,547	17,896	33,236	36,310
Occupancy costs	7,493	6,643	15,848	13,825
Other segment items (a)	124,890	76,275	232,777	221,698
Segment net income (loss)	$ 83,496	$ (64,849)	$ 80,687	$ 801,713
Reconciliation of profit or loss				
Adjustments and reconciling items	—	—	—	—
Consolidated net income (loss)	$ 83,496	$ (64,849)	$ 80,687	$ 801,713

(a) Other segment items includes the following:

	Three months ended June 30,		Six months ended June 30,	
	2026	2025	2026	2025
Interest income	$ (18,119)	$ (20,991)	$ (36,104)	$ (38,236)
Interest expense	66,600	51,349	133,615	102,686
Income tax expense (benefit)	14,049	(6,203)	14,396	57,063
Depreciation	4,009	3,573	7,815	7,031
Amortization	11,841	4,061	23,422	7,859
All other (b)	46,510	44,486	89,633	85,295
Total other segment items	$ 124,890	$ 76,275	$ 232,777	$ 221,698

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

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, 2025 included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 19, 2026, or our 2025 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 I, Item 1A. (Risk Factors) of our 2025 Annual Report, our actual results may differ materially from those anticipated in these forward-looking statements.

Our Company

We are a global biopharmaceutical company dedicated to the discovery, development and commercialization of clinically differentiated medicines for children and adults living with rare disorders. We are advancing a robust and diversified pipeline of transformative medicines as part of our mission to provide access to best-in-class treatments for patients with unmet medical needs. Our strategy is to leverage our scientific expertise and global commercial infrastructure to optimize value for our patients and other stakeholders. 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

Sephience™ (sepiapterin)

Sephience (sepiapterin) is a product for the treatment of phenylketonuria, or PKU, a rare inherited metabolic disease characterized by the body's inability to break down an essential amino acid called phenylalanine, and which can result in neurological and other symptoms. In June 2025, Sephience was granted marketing authorization by the European Commission, or EC, for the treatment of children and adults living with PKU within the European Economic Area, or EEA. In July 2025, Sephience was approved by the U.S. Food and Drug Administration, or FDA, for the treatment of pediatric and adult patients living with PKU in the United States age one month and above. In December 2025, Sephience was approved by the Japanese Ministry of Health, Labor and Welfare, or MHLW, for the treatment of children and adults living with PKU in Japan, where the label includes individuals of all ages and the full spectrum of disease severity. In February 2026, Sephience was approved by ANVISA, the Brazilian health regulatory authority, for the treatment of children and adults living with PKU in Brazil. Sephience is also approved in additional geographies. During the three months ended June 30, 2026, we recognized $151.3 million in net sales of Sephience.

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. While Translarna previously had conditional approval in the EEA, in March 2025, the EC adopted the negative opinion of the Committee of Medicinal Products for Human Use, or CHMP, of the European Medicines Agency, or EMA, to not renew the conditional marketing authorization of Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD. However, 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.

Translarna is an investigational new drug in the United States. In 2017, we filed a new drug application, or NDA, for Translarna for the treatment of nmDMD over protest with the FDA and in October 2017, the Office of Drug Evaluation I of the FDA issued a complete response letter for the NDA, stating that it was unable to approve the application in its current form. We re-submitted the NDA in July 2024 and in October 2024, the FDA accepted for review the resubmission of the NDA for Translarna for the treatment of nmDMD. Following feedback from the FDA, we decided to withdraw the NDA resubmission for Translarna in February 2026. Further development of Translarna for the treatment of nmDMD in the United States is not planned.

Translarna has marketing authorization in additional geographies outside of the EEA, though the EC adoption of the CHMP negative opinion and the withdrawal of the Translarna NDA in the United States may affect future reauthorizations. During the three months ended June 30, 2026, we recognized $42.2 million in net sales for Translarna. Emflaza is approved in the United States for the treatment of DMD in patients two years and older. During the three months ended June 30, 2026, we recognized $24.6 million in net sales for Emflaza.

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 for the treatment of DMD in patients five years and older, which expired in February 2024. With the expiration of this orphan drug exclusivity, we have seen an increase in competition from generics, which has, and we expect will continue to have, a 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 expired 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, 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. Waylivra has also received marketing authorization in the EU for the treatment of FCS. In December 2022, ANVISA approved Waylivra for the treatment of familial partial lipodystrophy.

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

Splicing Platform

In addition to our SMA program, our splicing platform also includes votoplam, which is being developed for the treatment of Huntington's disease, or HD. We announced the results from our Phase 1 study of votoplam in healthy volunteers in September 2021 demonstrating dose-dependent lowering of huntingtin messenger ribonucleic acid and protein levels, that votoplam efficiently crosses blood brain barrier at significant levels and that votoplam was well tolerated. We initiated a Phase 2 study of votoplam for the treatment of HD in the first quarter of 2022, which consisted 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 votoplam biomarker effect. In September 2024, the FDA granted Fast Track designation to the votoplam program for the treatment of HD.

In November 2024, we entered into a License and Collaboration Agreement with Novartis Pharmaceuticals Corporation, or Novartis, relating to our votoplam program, or the Novartis Agreement, which included related molecules. While Novartis has taken over responsibility for the further development of the votoplam program, we continue to collaborate with Novartis on next steps. In May 2025, we announced that the Phase 2 study of votoplam 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. In April 2026, Novartis announced that it had commenced the global Phase 3 clinical trial, which is expected to enroll approximately 770 individuals with early symptomatic disease, randomized 3:2 to receive votoplam 10 milligrams or placebo, and includes an interim analysis. Also in April 2026, we reported positive topline results from the 24-month interim analysis of the PIVOT-HD long-term extension study, with favorable dose-dependent effects on disease progression for Stage 2 HD patients following 24 months of votoplam treatment compared to an external natural history cohort, with 52% slowing of disease progression on the Composite Unified Huntington's Disease Rating Scale at the 10 milligram dose level. While the Phase 3 clinical trial remains the base case for votoplam approval, we are working with Novartis to finalize a plan to engage with FDA to discuss the 24-month results in the second half of 2026.

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 and non-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 Friedreich's ataxia, or FA, called MOVE-FA, in May 2023. While the trial 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, 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 August 2025, the FDA issued a complete response letter related to the NDA stating that substantial evidence of efficacy was not demonstrated for vatiquinone and that an additional adequate and well-controlled study would be needed to support NDA resubmission. We met with the FDA in the fourth quarter of 2025 to discuss the vatiquinone development program, at which time the FDA suggested an additional study be conducted to support NDA resubmission. In April 2026, we again met with FDA to discuss the design of a new trial to provide additional data to support NDA resubmission. Based on the meeting discussion and written feedback, we plan to initiate the PROVE-FA open label study using matched natural history control in the third quarter of 2026. This study is expected to enroll approximately 120 patients ages 7 to 21 and the study primary endpoint is the change in mFARS from baseline to month 24.

During the quarter ended June 30, 2026, we initiated a Phase 1 study of PTC612, our oral NLRP3 inhibitor, and completed several of the single and multiple ascending dose treatment cohorts. Notably, this healthy volunteer study includes a cohort of individuals with obesity and cardiovascular disease, which we expect will provide an early view of pharmacokinetics and pharmacodynamics. We also expect to initiate a Phase 2a study of PTC844, our next-generation DHODH inhibitor, in

the third quarter of 2026. The PTC844 study will be a 12-week pharmacokinetics and pharmacodynamics study in which we will assess treatment effect on biomarkers related to T-cell and B-cell immunity. We expect that the results of this study will help inform the ultimate target indications for PTC844.

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 were primarily generated from sales of Sephience for the treatment of PKU in the U.S. and EEA, 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. There is a substantial risk that as a result of the EC's adoption of the CHMP's negative opinion we will lose a significant portion of our ability to generate revenue from sales of Translarna in the EEA. We also generated revenue from sales of Upstaza/Kebilidi for the treatment of AADC deficiency in the EEA and in the U.S., and have recognized revenue associated with milestone and royalty payments from Roche pursuant to a License and Collaboration Agreement, or the SMA License Agreement, by and among us, Roche and, for the limited purposes set forth therein, the SMA Foundation, under our SMA program and we have recognized license revenues related to performance obligations 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 areas addressed by our product candidates. We have relied on revenue generated from net sales of our products. 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 with RPI Intermediate Finance Trust, or the Immediate Finance Trust, or the Original Purchase Agreement, and we exercised our first put option in exchange for $241.8 million in cash consideration. In December 2025, we, Royalty Pharma, and, for the limited purposes set forth in Amendment No. 2 (as defined below), Royalty Pharma plc, entered into an Amendment No. 2 to Amended and Restated Royalty Purchase Agreement, or Amendment No. 2, which amends that certain A&R Royalty Purchase Agreement, as amended. Under Amendment No. 2, we sold to Royalty Pharma a certain portion of our right to receive sales-based royalty payments on worldwide net sales of Roche's Evrysdi® (risdiplam) product and any other product developed pursuant to the License and Collaboration Agreement, dated as of November 23, 2011, by and among us, F. Hoffman-La Roche Ltd, Hoffman-La Roche Inc., together with F. Hoffman-La Roche Ltd, Roche, and, for the limited purposes set forth therein, the Spinal Muscular Atrophy Foundation, such payments, the Royalty. Pursuant to Amendment No. 2, on December 29, 2025, we sold to Royalty Pharma its retained interest in the Royalty in exchange for $240.0 million in upfront cash consideration, and three potential additional cash purchase price payments of $20.0 million each conditioned upon receipt by Royalty Pharma of more than $347.0 million of Assigned Royalty Payments (as defined in the A&R Royalty Purchase Agreement) in respect of Calendar Year Net Sales (as defined in the A&R Royalty Purchase Agreement) arising in 2027, $363.0 million of Assigned Royalty Payments in respect of Calendar Year Net Sales arising in 2028, and $379.0 million of Assigned Royalty Payments in respect of Calendar Year Net Sales arising in 2029, respectively. The retained interest sold by us to Royalty Pharma pursuant to the Amendment No. 2 is equal to 9.5111% of the Royalty before the 2020 Assigned Royalty Cap (as defined in the A&R Royalty Purchase Agreement) has been met,

and 16.6666% of the Royalty from and after such time as the 2020 Assigned Royalty Cap has been met. As a result of the sale, Royalty Pharma owns 100% of the Royalty and we own 0% of the Royalty.

In November 2024, we entered into the Novartis Agreement relating to our votoplam HD program which includes related molecules. Novartis is responsible for all other development of licensed compounds and licensed products and the manufacture and commercialization of licensed compounds and licensed products worldwide. While Novartis has taken over responsibility for the further development of the votoplam program, we continue to collaborate with Novartis on next steps. 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 we are eligible to 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. We have also recognized revenue associated with milestone payments from Novartis pursuant to the Novartis Agreement. In April 2026, Novartis notified us that it had initiated the first Phase 3 clinical trial for a Licensed Product (as defined in the Novartis Agreement), which pursuant to the Novartis Agreement, triggered a $50.0 million milestone payment to us. The $50.0 million development milestone is recorded as collaboration and license revenue for the three and six months ended June 30, 2026.

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 and six months ended June 30, 2026, 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 June 30, 2026.

In June 2026, we issued $550.0 million aggregate principal amount of 0% convertible senior notes due 2031, or the 2031 Convertible Notes, which reflects the exercise in full by the initial purchasers of their option to purchase up to an additional $50.0 million in aggregate principal amount of the 2031 Convertible Notes. The 2031 Convertible Notes are governed by an indenture, or the 2031 Convertible Notes Indenture, with U.S. Bank Trust Company, National Association as trustee. The 2031 Convertible Notes bear no regular interest and the principal amounts of the 2031 Convertible Notes will not accrete. The 2031 Convertible Notes may bear special interest under specified circumstances relating to our failure to comply with our reporting obligations under the 2031 Convertible Notes Indenture or if the 2031 Convertible Notes are not freely tradeable as required by the 2031 Convertible Notes Indenture. Special interest, if any, will be payable semiannually in arrears on June 15 and December 15 of each year, beginning on December 15, 2026 (if and to the extent that special interest is payable). The 2031 Convertible Notes will mature on June 15, 2031, unless earlier converted, redeemed or repurchased pursuant to their terms. We received net proceeds of approximately $535.4 million after deducting the initial purchasers’ discounts and commissions and the offering expenses payable by us.

Following the issuance of the 2031 Convertible Notes, we used approximately $328.8 million of the net proceeds of the 2031 Convertible Notes to repurchase for cash $222.0 million aggregate principal amount of our outstanding 1.50% convertible senior notes due September 15, 2026, or the 2026 Convertible Notes, pursuant to privately negotiated transactions with certain holders entered into concurrently with the pricing of the offering of the 2031 Convertible Notes. Cash interest payments on the 2026 Convertible Notes were payable on a semi-annual basis in arrears, which will require remaining funding of $0.4 million. The 2026 Convertible Notes are currently convertible at the option of the holders and will mature and become due and payable on September 15, 2026, unless earlier repurchased or converted.

As of the quarter ended June 30, 2026, aggregate Sephience global net sales in the prior four consecutive quarters exceeded $250.0 million, which, pursuant to the Agreement and Plan of Merger, dated as of May 6, 2020, or the Censa Merger Agreement, by and among us and Censa Pharmaceuticals, Inc., or Censa, triggered a $30.0 million net sales milestone to the former Censa securityholders. This milestone payment was recorded in accounts payable and accrued expenses on our consolidated balance sheet as of June 30, 2026.

As of June 30, 2026, we had an accumulated deficit of $2,883.5 million. We had net income of $80.7 million and $801.7 million for the six months ended June 30, 2026 and 2025, 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, Japan 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 may seek to expand and diversify our product pipeline through opportunistically in-licensing or acquiring the rights to products, product candidates or technologies and we may incur expenses, including with respect to transaction costs, subsequent development costs or any upfront, milestone or other payments or other financial obligations associated with any such transaction, which would increase our future capital requirements.

We 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 2025 Annual Report. There were no material changes to these obligations and commitments during the period ended June 30, 2026. 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 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. Our net product revenues primarily consist of sales of Sephience for the treatment of PKU. Our net product revenues also consist 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 and six months ended June 30, 2026 and 2025, net product revenues consisted of the following:

	Three Months Ended June 30,

(in thousands)	2026 United States	2026 International	2026 Total	2025 United States	2025 International	2025 Total
Sephience	$ 127,550	$ 23,760	$ 151,310	$ —	$ —	$ —
Translarna	—	42,217	42,217	—	59,470	59,470
Emflaza	24,634	—	24,634	36,353	—	36,353
Upstaza/Kebilidi	—	11,163	11,163	—	11,889	11,889
All other products	—	9,495	9,495	—	10,617	10,617
Total net product revenue	$ 152,184	$ 86,635	$ 238,819	$ 36,353	$ 81,976	$ 118,329

	Six Months Ended June 30,					
	2026			2025		
	United States	International	Total	United States	International	Total
Sephience	$ 239,590	$ 36,271	$ 275,861	$ —	$ —	$ —
Translarna	—	101,193	101,193	—	145,624	145,624
Emflaza	46,112	—	46,112	84,142	—	84,142
Upstaza/Kebilidi	2,998	17,759	20,757	—	20,547	20,547
All other products	—	20,469	20,469	—	21,442	21,442
Total net product revenue	$ 288,700	$ 175,692	$ 464,392	$ 84,142	$ 187,613	$ 271,755

Disaggregated net product revenues by country for the three and six months ended June 30, 2026 and 2025, are as follows:

(in thousands)	Three Months Ended June 30, 2026	Three Months Ended June 30, 2025	Six Months Ended June 30, 2026	Six Months Ended June 30, 2025
United States	$ 152,184	$ 36,353	$ 288,700	$ 84,142
Russia	25,581	18,124	28,830	56,638
Brazil	8,210	40,951	49,422	50,456
All other countries	52,844	22,901	97,440	80,519
Total net product revenue	$ 238,819	$ 118,329	$ 464,392	$ 271,755

For three and six months ended June 30, 2026, three of our distributors each accounted for over 10% of our net product sales. For three and six months ended June 30, 2025, three and two of our distributors, respectively, each accounted for over 10% of our 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 June 30, 2026, we had recognized a total of $310.0 million in milestone payments and $907.7 million in royalties on net sales pursuant to the SMA License Agreement. As of June 30, 2026, there are no remaining research and development event milestones that we can receive. The remaining potential sales milestones as of June 30, 2026 are $150.0 million upon achievement of certain sales events.

For the three and six months ended June 30, 2026 and 2025, we did not recognize collaboration revenue related to the SMA License Agreement with Roche.

For the three and six months ended June 30, 2026, we recognized $71.1 million and $117.9 million of royalty revenue, respectively, related to Evrysdi. For the three and six months ended June 30, 2025, we recognized $57.6 million and $94.0 million of royalty revenue, respectively, related to Evrysdi.

Novartis Collaboration for votoplam HD. In November 2024, we entered into the Novartis Agreement with Novartis related to our votoplam 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 are eligible to 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. During the three and six months ended June 30, 2026, we recognized $50.6 million and $50.7 million in license revenues, respectively, primarily related to a development milestone pursuant to our Novartis Agreement for Novartis's initiation of the first Phase 3 clinical trial for a Licensed Product (as defined in the Novartis Agreement), which triggered a $50.0 million milestone payment to us. During the three and six months ended June 30, 2025, we recognized $2.9 million and $992.7 million in license revenues, respectively, related to performance obligations completed pursuant to the Novartis Agreement. Collaboration and license revenue during the three months ended June 30, 2025, was partially offset by $3.5 million related to a refund for a prior collaboration arrangement in relation to votoplam.

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 candidate manufacturing costs.

The following table provides research and development expense for our most advanced principal product development programs, for the three and six months ended June 30, 2026 and 2025.

	Three Months Ended June 30,	
	2026	2025
	(in thousands)	
Sephience	$ 22,078	$ 26,741
Inflammation & Ferroptosis platform	5,571	9,186
Global DMD	2,988	8,318
Gene Therapy	169	3,465
Other development programs	224	3,440
Total Development	31,030	51,150
Research	18,331	15,691
Payroll, benefits, and share-based stock compensation	40,096	36,995
Facilities and other indirect costs	9,693	9,154
Total research and development	**$ 99,150**	**$ 112,990**

	Six Months Ended June 30,	
	2026	2025
	(in thousands)	
Sephience	$ 44,704	$ 50,882
Inflammation & Ferroptosis platform	11,235	15,290
Global DMD	4,358	15,695
Gene Therapy	3,842	8,246
Other development programs	1,170	8,886
Total Development	65,309	98,999
Research	31,213	29,538
Payroll, benefits, and share-based stock compensation	83,554	75,229
Facilities and other indirect costs	19,947	18,197
Total research and development	**$ 200,023**	**$ 221,963**

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

For the three and six months ended June 30, 2026, compared to the three and six months ended June 30, 2025, the changes reflect progressing through different phases of studies as we continue to focus our resources on our differentiated, high potential research and development programs. The decrease is primarily due to a decrease in costs relating to Global DMD, Gene Therapy, Splicing platform, Inflammation & Ferroptosis platform, and Sephience related development.

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

For the three and six months ended June 30, 2026, compared to the three and six months ended June 30, 2025, the increase in research expenses primarily related to increased investment in research programs and advancement of the clinical pipeline.

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 and six months ended June 30, 2026, compared to the three and six months ended June 30, 2025, the increase in payroll, benefits, and share-based stock compensation expenses primarily related to an increase in share-based stock compensation, and increases in salaries due to annual merit increases for the three and six months ended June 30, 2026 compared to the three and six months ended June 30, 2025.

Facilities and other indirect costs. 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 June 30, 2026, compared to the three months ended June 30, 2025, the change in facilities and other indirect costs was relatively flat. For the six months ended June 30, 2026, compared to the six months ended June 30, 2025, the change in facilities and other indirect costs was related to new leases that commenced in the second half of 2025.

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 the 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, the 2026 Convertible Notes outstanding, and the 2031 Convertible Notes outstanding, partially 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 and six months ended June 30, 2026, there were no material changes to our critical accounting policies as reported in our 2025 Annual Report.

Results of operations

Three months ended June 30, 2026 compared to the three months ended June 30, 2025

The following table summarizes revenues and selected expense and other income data for the three months ended June 30, 2026 and 2025.

(in thousands)	Three Months Ended June 30, 2026	2025	Change 2026 vs. 2025
Net product revenue	$ 238,819	$ 118,329	$ 120,490
Collaboration and license revenue	50,595	2,941	47,654
Royalty revenue	71,105	57,605	13,500
Cost of product, collaboration and license sales, excluding amortization of acquired intangible assets	19,921	11,420	8,501
Amortization of acquired intangible assets	11,841	4,061	7,780
Research and development expense	99,150	112,990	(13,840)
Selling, general and administrative expense	80,630	85,262	(4,632)
Tangible asset impairment and losses on transactions, net	—	99	(99)
Interest expense, net	(48,481)	(30,358)	(18,123)
Other expense, net	(2,951)	(5,737)	2,786
Income tax (expense) benefit	(14,049)	6,203	(20,252)

Net product revenue. Net product revenue was $238.8 million for the three months ended June 30, 2026, an increase of $120.5 million, or over 100%, from $118.3 million for the three months ended June 30, 2025. The increase in net product revenue was primarily due to an increase in net product sales of $151.3 million for Sephience, which is in the first year of its launch, partially offset by a decrease in net product sales of $11.7 million for Emflaza and $17.3 million for Translarna. The decrease in Emflaza sales is primarily driven by additional generic competition. The decrease in Translarna sales is primarily due to the EC's adoption of the CHMP's negative opinion.

Collaboration and license revenue. Collaboration and license revenue was $50.6 million for the three months ended June 30, 2026, an increase of $47.7 million, or over 100%, from $2.9 million for the three months ended June 30, 2025. For the three months ended June 30, 2026, we recognized $50.6 million, primarily related to a development milestone. In April 2026, Novartis notified the Company that it had initiated the first Phase 3 clinical trial for a Licensed Product (as defined in the Novartis Agreement). Pursuant to the Novartis Agreement, this triggered a $50.0 million milestone payment to us. For the three months ended June 30, 2025, we recognized $2.9 million related to license revenue from the Novartis Agreement for performance obligations completed during the period.

Royalty revenue. Royalty revenue was $71.1 million for the three months ended June 30, 2026, an increase of $13.5 million, or 23%, from $57.6 million for the three months ended June 30, 2025. The increase in royalty revenue was due to higher Evrysdi sales in the three months ended June 30, 2026 as compared to the three months ended June 30, 2025. In

accordance with the SMA License Agreement, we are entitled to recognize royalties on worldwide annual net sales of the product. See "Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations—Overview—Corporate Updates—Funding."

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 was $19.9 million for the three months ended June 30, 2026, an increase of $8.5 million, or 74%, from $11.4 million for the three months ended June 30, 2025. Cost of product, collaboration and license sales, excluding amortization of acquired intangible assets consists primarily of the costs associated with the Novartis agreement, royalty payments associated with Sephience and Upstaza/Kebilidi net product sales, costs associated with Sephience, Translarna, Upstaza/Kebilidi, and Emflaza product sold during the period, as well as the production costs associated with these products. The increase was primarily driven by the increase in net product sales, which impacted the cost for products sold and royalty expense for the period.

Amortization of acquired intangible assets. Amortization of acquired intangible assets was $11.8 million for the three months ended June 30, 2026, an increase of $7.8 million, or over 100%, from $4.1 million for the three months ended June 30, 2025. The increase to the intangible assets balance was primarily related to Upstaza/Kebildi and Sephience intangible assets recorded as a result of the regulatory approvals and net sales milestones as well as the Censa Rights Satisfaction Agreement, which increased the corresponding amortization for those assets.

Research and development expense. Research and development expense was $99.2 million for the three months ended June 30, 2026, a decrease of $13.8 million, or 12%, from $113.0 million for the three months ended June 30, 2025. The decrease in research and development expenses primarily related to decreases in development 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 $80.6 million for the three months ended June 30, 2026, a decrease of $4.6 million, or 5%, from $85.3 million for the three months ended June 30, 2025. The decrease is primarily due to a decrease in selling expenses related to prelaunch activities for Sephience.

Tangible asset impairment and losses on transactions, net. Tangible asset impairment and losses on transactions, net decreased $0.1 million, or 100%, from $0.1 million for the three months ended June 30, 2025. The decrease in tangible asset impairment and losses on transactions primarily related to no impairments and gains or losses during the three months ended June 30, 2026 as compared to $0.1 million related to fixed asset impairments in the three months ended June 30, 2025.

Interest expense, net. Interest expense, net was $48.5 million for the three months ended June 30, 2026, an increase of $18.1 million, or 60%, from $30.4 million for the three months ended June 30, 2025. The increase in interest expense, net was primarily due to an increase in interest expense related to the liability for the sale of future royalties related to the A&R Royalty Purchase Agreement.

Other expense, net. Other expense, net was $3.0 million for the three months ended June 30, 2026, a decrease of $2.8 million, or 49%, from other expense, net of $5.7 million for the three months ended June 30, 2025. The decrease in other expense, net, primarily relates to net realized and unrealized gains from foreign currency of $0.4 million for the three months ended June 30, 2026, compared to net realized and unrealized losses from foreign currency of $8.7 million for the three months ended June 30, 2025. This decrease was partially offset by an inducement expense of $3.4 million related to the repurchase of a portion of the 2026 Convertible Notes and other items of $2.8 million.

Income tax (expense) benefit. Income tax expense was $14.0 million for the three months ended June 30, 2026, a change of $20.3 million, or over 100%, compared to income tax benefit of $6.2 million for the three months ended June 30, 2025. The change in income tax (expense) benefit was driven by the recognition of revenue associated with the A&R Royalty Purchase Agreement.

Six months ended June 30, 2026 compared to the six months ended June 30, 2025

The following table summarizes revenues and selected expense and other income data for the six months ended June 30, 2026 and 2025.

(in thousands)	Six Months Ended June 30, 2026	2025	Change 2026 vs. 2025
Net product revenue	$ 464,392	$ 271,755	$ 192,637
Collaboration and license revenue	50,738	989,172	(938,434)
Royalty revenue	117,940	94,044	23,896
Cost of product, collaboration and license sales, excluding amortization of acquired intangible assets	47,949	24,282	23,667
Amortization of acquired intangible assets	23,422	7,859	15,563
Research and development expense	200,023	221,963	(21,940)
Selling, general and administrative expense	166,813	166,223	590
Change in the fair value of contingent consideration	—	(800)	800
Tangible asset impairment and losses on transactions, net	927	176	751
Interest expense, net	(97,511)	(64,450)	(33,061)
Other expense, net	(1,342)	(12,042)	10,700
Income tax expense	(14,396)	(57,063)	42,667

Net product revenue. Net product revenue was $464.4 million for the six months ended June 30, 2026, an increase of $192.6 million, or 71%, from $271.8 million for the six months ended June 30, 2025. The increase in net product revenue was primarily due to an increase in net product sales of $275.9 million for Sephience, which is in the first year of its launch, partially offset by a decrease in net product sales of $38.0 million for Emflaza and $44.4 million for Translarna. The decrease in Emflaza sales is primarily driven by additional generic competition. The decrease in Translarna sales is primarily due to the EC's adoption of the CHMP's negative opinion.

Collaboration and license revenue. Collaboration and license revenue was $50.7 million for the six months ended June 30, 2026, a decrease of $938.4 million, or 95%, from $989.2 million for the six months ended June 30, 2025. The decrease 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 our votoplam HD program for the six months ended June 30, 2025. For the six months ended June 30, 2026, we recognized $50.7 million primarily related to a development milestone pursuant to our Novartis Agreement for Novartis's initiation of the first Phase 3 clinical trial for a Licensed Product (as defined in the Novartis Agreement), which triggered a $50.0 million milestone payment to us. For the six months ended June 30, 2025, we recognized $992.7 million related to license revenue from the Novartis Agreement which was partially offset by $3.5 million related to a refund for a prior collaboration arrangement in relation to votoplam.

Royalty revenue. Royalty revenue was $117.9 million for the six months ended June 30, 2026, an increase of $23.9 million, or 25%, from $94.0 million for the six months ended June 30, 2025. The increase in royalty revenue was due to higher Evrysdi sales in the six months ended June 30, 2026 as compared to the six months ended June 30, 2025. In accordance with the SMA License Agreement, we are entitled to recognize royalties on worldwide annual net sales of the product.

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 was $47.9 million for the six months ended June 30, 2026, an increase of $23.7 million, or 97%, from $24.3 million for the six months ended June 30, 2025. Cost of product, collaboration and license sales, excluding amortization of acquired intangible assets consists primarily of the costs associated with the Novartis agreement, royalty payments associated with Sephience and Upstaza/Kebilidi net product sales, costs associated with Sephience, Translarna, Upstaza/Kebilidi, and Emflaza product sold during the period, as well as the production costs associated with these products. The increase was primarily driven by the increase in net product sales, which impacted the cost for products sold and royalty expense for the period.

Amortization of acquired intangible assets. Amortization of acquired intangible assets was $23.4 million for the six months ended June 30, 2026 an increase of $15.6 million, or over 100%, from $7.9 million for the six months ended June 30, 2025.

The increase to the intangible assets balance was primarily related to Upstaza/Kebildi and Sephience intangible assets recorded as a result of the regulatory approvals and net sales milestones as well as the Censa Rights Satisfaction Agreement, which increased the corresponding amortization for those assets.

Research and development expense. Research and development expense was $200.0 million for the six months ended June 30, 2026, a decrease of $21.9 million, or 10%, from $222.0 million for the six months ended June 30, 2025. The decrease in research and development expenses related to decreases in development 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 $166.8 million for the six months ended June 30, 2026, an increase of $0.6 million, or 0%, from $166.2 million for the six months ended June 30, 2025. The increase reflected our continued investment to support our commercial activities including our expanding commercial portfolio.

Change in the fair value of contingent consideration. There was no change in the fair value of contingent consideration for the six months ended June 30, 2026, a change of $0.8 million, or 100%, from a gain of $0.8 million for the six months ended June 30, 2025. During the first quarter of 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 $0.9 million for the six months ended June 30, 2026, an increase of $0.8 million, or over 100%, from $0.2 million for the six months ended June 30, 2025. The increase was primarily driven by a $0.8 million loss related to inventory impairments during the six months ended June 30, 2026. During the six months ended June 30, 2025, we recorded $0.1 million related to fixed asset impairments and $0.1 million related to losses on the sale of fixed assets.

Interest expense, net. Interest expense, net was $97.5 million for the six months ended June 30, 2026, an increase of $33.1 million, or 51%, from $64.5 million for the six months ended June 30, 2025. The increase in interest expense, net was primarily due to an increase in interest expense related to the liability for the sale of future royalties related to the A&R Royalty Purchase Agreement.

Other expense, net. Other expense, net was $1.3 million for the six months ended June 30, 2026, a decrease of $10.7 million, or 89%, from other expense, net of $12.0 million for the six months ended June 30, 2025. The decrease in other expense, net, primarily relates to net realized and unrealized gains from foreign currency of $1.8 million for the six months ended June 30, 2026, compared to net realized and unrealized losses from foreign currency of $15.0 million for the six months ended June 30, 2025. This decrease was partially offset by an inducement expense of $3.4 million related to the repurchase of a portion of the 2026 Convertible Notes and other items of $2.7 million.

Income tax expense. Income tax expense was $14.4 million for the six months ended June 30, 2026, a decrease of $42.7 million, or 75%, compared to income tax expense of $57.1 million for the six months ended June 30, 2025. The decrease in income tax expense was driven by the projected utilization of additional tax attributes in 2026 as a result of the provisions within the One Big Beautiful Bill Act.

Liquidity and capital resources

Sources of liquidity

While we have generated net income in the six months ended June 30, 2026 and 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. Our product revenue primarily consists of sales of Sephience for the treatment of PKU. Our product revenues also consist 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 ability to generate product revenue

from any of our products will largely depend on the coverage and reimbursement levels set by governmental authorities, private health insurers and other third-party payors, as the case may be, depending on the country in which our products are marketed, and the rate and degree of market acceptance and clinical utility of any of our products.

Additionally, for Emflaza, its seven-year period of orphan drug exclusivity related to the treatment of DMD in patients five years and older expired in February 2024. With the expiration of this orphan drug exclusivity, we have seen an increase in competition from generics, which has, and we expect will continue to have, a 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 expired in June 2026.

Additionally, for Translarna, 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 other geographies 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. While Translarna previously had conditional approval in the EEA, in March 2025, the EC adopted the negative opinion of the CHMP of the EMA to not renew the conditional marketing authorization of Translarna for the treatment of nmDMD. However, 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 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 a significant portion of our ability to generate revenue from sales of Translarna in the EEA. Additionally, the loss of the Translarna marketing authorization in the EEA and the withdrawal of the Translarna NDA in the United States may influence regulatory entities in other jurisdictions in which Translarna has been approved to reassess such approvals. There is substantial risk that we will be unable to maintain our marketing authorizations in these countries. Even in countries where our marketing authorization is maintained, there may be an impact on pricing and reimbursement of Translarna within those countries.

We have financed our operations to date primarily through 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 areas addressed by our product candidates. We expect to continue to incur significant expenses 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—Overview—Corporate Updates — Funding" for additional information.

We have received fundings from Royalty Pharma under the A&R Royalty Purchase Agreement in July 2020, October 2023, June 2024 and December 2025, totaling $2.1 billion. In exchange for these fundings, we sold Royalty Pharma 100% of our right to receive sales-based royalty payments on worldwide net sales of Roche's Evrysdi® (risdiplam) product and any other product developed pursuant to the SMA collaboration with us, Roche, and the SMA Foundation.

In November 2024, we and Novartis entered into the Novartis Agreement relating to our votoplam HD program which includes related molecules. Pursuant to the Novartis Agreement, we were responsible for conducting the Phase 2A clinical trial of votoplam, which is complete, and have transitioned sponsorship of the ongoing open-label extension clinical trial to Novartis. 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 are eligible 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 April 2026, Novartis notified us that it had initiated the first Phase 3 clinical trial for a Licensed Product (as defined in the Novartis Agreement), which, pursuant to the Novartis Agreement, triggered a $50.0 million milestone payment to us.

In September 2019, we issued $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 exercised in full by the initial purchasers. We received net proceeds of $279.3 million after deducting the initial purchasers' discounts and commissions and the offering expenses payable by us. 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 2026 Convertible Notes are currently convertible at the option of the holder.

During the three months ended June 30, 2026, a holder converted $10.0 million principal value of 2026 Convertible Notes in exchange for $12.9 million in cash and 3,506 shares of our common stock. We recorded a $10.0 million reduction to the carrying value of the convertible notes, and the excess of $2.9 million was recognized as a reduction in additional paid-in capital within our statement of stockholders' deficit.

In June 2026 we closed a private offering of $550.0 million aggregate principal amount of 0% convertible senior notes due 2031, which reflects the exercise in full by the initial purchasers of their option to purchase up to an additional $50.0 million in aggregate principal amount of the 2031 Convertible Notes. The 2031 Convertible Notes bear no regular interest and the principal amounts of the 2031 Convertible Notes will not accrete. The 2031 Convertible Notes may bear special interest under specified circumstances relating to our failure to comply with our reporting obligations under the 2031 Convertible Notes Indenture or if the 2031 Convertible Notes are not freely tradeable as required by the 2031 Convertible Notes Indenture. Special interest, if any, will be payable semiannually in arrears on June 15 and December 15 of each year, beginning on December 15, 2026 (if and to the extent that special interest is payable). The 2031 Convertible Notes will mature on June 15, 2031, unless earlier converted, redeemed or repurchased pursuant to their terms. We received net proceeds of approximately $535.4 million after deducting the initial purchasers' discounts and commissions and the offering expenses payable by us.

Following the issuance of the 2031 Convertible Notes, we used approximately $328.8 million of the net proceeds of the 2031 Convertible Notes to repurchase for cash $222.0 million aggregate principal amount of our 2026 Convertible Notes pursuant to privately negotiated transactions with certain holders entered into concurrently with the pricing of the offering of the 2031 Convertible Notes. After giving effect to the repurchases of the 2026 Convertible Notes, the aggregate principal amount outstanding of 2026 Convertible Notes is $55.5 million. The repurchase of the 2026 Convertible Notes was accounted for as an induced conversion. The excess of the fair value of the repurchase price over the if-converted value was $3.4 million and was recorded as inducement expense within other expense, net on the consolidated statements of operations. The net carrying amount of the repurchased 2026 Convertible Notes was derecognized, and the difference between the if-converted value and the net carrying amount of $102.7 million was recognized as a reduction in additional paid-in capital within the consolidated statement of stockholders' deficit.

Cash flows

As of June 30, 2026, we had cash, cash equivalents and marketable securities of $2.23 billion.

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

	Six Months Ended June 30,	
(in thousands)	**2026**	**2025**
Cash provided by (used in):		
Operating activities	70,071	811,770
Investing activities	(216,292)	(606,369)
Financing activities	218,281	13,371

Net cash provided by operating activities was $70.1 million for the six months ended June 30, 2026, and $811.8 million for the six months ended June 30, 2025. The net cash provided by operating activities for the six months ended June 30, 2026, primarily relates to the $50.0 million in cash received from Novartis for initiating the first Phase 3 clinical trial for a Licensed Product. The net cash provided by operating activities for the six months ended June 30, 2025, was

primarily related to the upfront payment of $1.0 billion in cash received upon the closing of the Novartis Agreement in January 2025, partially offset by spend supporting clinical development and commercial activities.

Net cash used in investing activities was $216.3 million for the six months ended June 30, 2026, compared to $606.4 million for the six months ended June 30, 2025. Cash used in investing activities for the six months ended June 30, 2026 and 2025, was primarily related to the purchases of marketable securities, acquisition of product rights, and purchases of fixed assets, offset by sales of marketable securities.

Net cash provided by financing activities was $218.3 million for the six months ended June 30, 2026, compared to $13.4 million for the six months ended June 30, 2025. Cash provided by financing activities for the six months ended June 30, 2026, was primarily attributable to cash received from the exercise of options, proceeds from employee stock purchase plan, proceeds from the issuance of the 2031 Convertible notes, offset by the debt issuance costs related to the 2031 convertible notes, and the repurchase and conversion of the 2026 Convertible Notes. Cash provided by financing activities for the six months ended June 30, 2025, was primarily attributable to proceeds from our employee stock purchase plan and proceeds from the exercise of options, partially offset by payments on contingent consideration obligation.

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, Japan 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. These efforts may significantly impact the timing and extent of our commercialization and manufacturing expenses. We met with the FDA in the fourth quarter of 2025 to discuss the vatiquinone development program, at which time the FDA suggested an additional study be conducted to support NDA resubmission. In April 2026, we again met with FDA to discuss the design of a new trial to provide additional data to support NDA resubmission. Based on the meeting discussion and written feedback, we plan to initiate the PROVE-FA open label study using matched natural history control in the third quarter of 2026.

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;
- initiate or continue the research and development of 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 and milestone payments from Novartis, 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 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 product candidates;
- our plans for vatiquinone including with respect to the expected timing of clinical trials and studies, availability of data, regulatory submissions and responses, meetings with regulatory agencies, and other matters;
- 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 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;
- the costs, timing and outcome of regulatory review of our splicing and inflammation and ferroptosis programs and Sephience, Translarna and Upstaza/Kebilidi in other territories;
- our ability to satisfy our obligations under the indenture governing the 2026 Convertible Notes;
- our ability to satisfy our obligations under the indenture governing the 2031 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;

- 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 votoplam 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 geopolitical events, global economic developments and public health pandemics or epidemics.

With respect to our outstanding 2026 Convertible Notes, cash interest payments are payable on a semi-annual basis in arrears, which will require remaining funding of $0.4 million through maturity.

As of the quarter ended June 30, 2026, aggregate Sephience global net sales in the prior four consecutive quarters exceeded $250.0 million, which, pursuant to the Censa Merger Agreement, triggered a $30.0 million net sales milestone payment to the former Censa securityholders. This milestone payment was recorded in accounts payable and accrued expenses on our consolidated balance sheet as of June 30, 2026.

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 2025 Annual Report. There were no material changes to these obligations and commitments during the period ended June 30, 2026.

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 stockholders 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 June 30, 2026, 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 2025 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 June 30, 2026. 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 June 30, 2026, 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 June 30, 2026 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, 2025, 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
Michael Schmertzler (Director) (by Section Six Partners, L.P., of which Mr. Schmertzler is a general partner and limited partner)	Adoption (May 14, 2026)	Rule 10b5-1 trading arrangement	Sale	Until February 1, 2027, or until such earlier date upon which all transactions are completed	Up to 170,000 shares

Item 6. Exhibits.

Exhibit Number	Description of Exhibit
4.1	Indenture (including Form of Note), dated June 18, 2026, between PTC Therapeutics, Inc. and U.S. Bank Trust Company, National Association. (incorporated by reference to Exhibit 4.1 to the Current Report on Form 8-K filed by the Registrant on June 18, 2026)
10.1	Consulting Services Agreement between the Registrant and Alethia Young (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed by the Registrant on June 9, 2026)
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: July 30, 2026

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: July 30, 2026

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: July 30, 2026

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 June 30, 2026 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: July 30, 2026

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 June 30, 2026 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: July 30, 2026

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