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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 September 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 October 27, 2026, there were 83,544,284 shares of Common Stock, $0.001 par value per share, outstanding.

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FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Quarterly Report on Form 10-Q, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "might," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," "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 ST-920 (isaralgagene civaparvovec) for the treatment of Fabry disease, including the expected timing of regulatory submissions and potential approvals and the potential achievement of regulatory milestones and contingent payments that we may be obligated to make;
- expectations with respect to Upstaza™/Kebilidi™ (eladocagene exuparvovec)/(eladocagene exuparvovec-tneq) for the treatment of Aromatic L-Amino Acid 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 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)

	September 30, 2026	December 31, 2025
Assets		
Current assets:		
Cash and cash equivalents	$ 508,894	$ 984,648
Marketable securities	1,619,024	960,723
Trade and royalty receivables, net	238,069	181,621
Inventory, net	109,332	79,647
Prepaid expenses and other current assets	62,465	67,309
Total current assets	2,537,784	2,273,948
Fixed assets, net	49,325	55,371
Intangible assets, net	443,686	388,753
Goodwill	82,341	82,341
Operating lease ROU assets	72,953	80,053
Deposits and other assets	21,626	18,301
Total assets	$ 3,207,715	$ 2,898,767
Liabilities and stockholders' deficit		
Current liabilities:		
Accounts payable and accrued expenses	$ 440,811	$ 381,272
Current portion of long-term debt	—	286,631
Deferred revenue	1,975	2,040
Operating lease liabilities- current	11,212	12,484
Finance lease liabilities- current	3,000	3,000
Liability for sale of future royalties- current	319,078	283,000
Total current liabilities	776,076	968,427
Long-term debt	536,268	—
Operating lease liabilities- noncurrent	85,554	93,583
Finance lease liabilities- noncurrent	—	3,000
Liability for sale of future royalties- noncurrent	1,986,525	2,025,366
Other long-term liabilities	14,501	13,704
Total liabilities	3,398,924	3,104,080
Stockholders' deficit:		
Common stock, $0.001 par value. Authorized 250,000,000 shares; issued and outstanding 83,535,767 shares at September 30, 2026. Authorized 250,000,000 shares; issued and outstanding 81,474,366 shares at December 31, 2025.	82	80
Additional paid-in capital	2,741,391	2,748,335
Accumulated other comprehensive income	2,553	10,501
Accumulated deficit	(2,935,235)	(2,964,229)
Total stockholders' deficit	(191,209)	(205,313)
Total liabilities and stockholders' deficit	$ 3,207,715	$ 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 September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenues:				
Net product revenue	$ 275,894	$ 130,956	$ 740,286	$ 402,711
Collaboration and license revenue	229	9,258	50,967	998,430
Royalty revenue	81,684	70,793	199,624	164,837
Total revenues	357,807	211,007	990,877	1,565,978
Operating expenses:				
Cost of product, collaboration and license sales, excluding amortization of acquired intangible assets	23,664	15,781	71,613	40,063
Amortization of acquired intangible assets	12,595	7,534	36,017	15,393
Research and development	105,353	100,158	305,376	322,121
Selling, general and administrative	80,081	84,046	246,894	250,269
Change in the fair value of contingent consideration	—	—	—	(800)
Tangible asset impairment and losses on transactions, net	—	472	927	648
Acquired in-process research and development	111,000	—	111,000	—
Total operating expenses	332,693	207,991	771,827	627,694
Income from operations	25,114	3,016	219,050	938,284
Interest expense, net	(45,866)	(32,592)	(143,377)	(97,042)
Other (expense) income, net	(1,073)	6,502	(2,415)	(5,540)
Loss on extinguishment of debt, net	(19,263)	—	(19,263)	—
(Loss) income before income tax (expense) benefit	(41,088)	(23,074)	53,995	835,702
Income tax (expense) benefit	(10,605)	38,970	(25,001)	(18,093)
Net (loss) income attributable to common stockholders	$ (51,693)	$ 15,896	$ 28,994	$ 817,609
Weighted-average shares outstanding:				
Basic (in shares)	83,476,100	78,471,119	83,004,625	78,642,478
Diluted (in shares)	83,476,100	87,115,369	92,253,675	86,899,727
Net (loss) income per share—basic (in dollars per share)	$ (0.62)	$ 0.20	$ 0.35	$ 10.40
Net (loss) income per share—diluted (in dollars per share)	$ (0.62)	$ 0.20	$ 0.34	$ 9.45

See accompanying unaudited notes.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Net (loss) income	$ (51,693)	$ 15,896	$ 28,994	$ 817,609
Other comprehensive (loss) income:				
Unrealized (loss) gain on marketable securities, net of tax	(2,631)	676	(4,781)	402
Foreign currency translation (loss) gain, net of tax	(833)	(2,102)	(3,167)	31,414
Comprehensive (loss) income	$ (55,157)	$ 14,470	$ 21,046	$ 849,425

See accompanying unaudited notes.

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

Three months ended September 30, 2026	Common stock		Additional paid-in capital	Accumulated other comprehensive income (loss)	Accumulated deficit	Total stockholders' deficit
	Shares	Amount				
Balance, June 30, 2026	83,327,286	$ 82	$ 2,712,080	$ 6,017	$ (2,883,542)	$ (165,363)
Exercise of options	141,755	—	5,349	—	—	5,349
Restricted stock vesting and issuance, net	66,726	—	—	—	—	—
Share-based compensation expense	—	—	23,827	—	—	23,827
Receivable from investor	—	—	135	—	—	135
Net loss	—	—	—	—	(51,693)	(51,693)
Comprehensive loss	—	—	—	(3,464)	—	(3,464)
Balance, September 30, 2026	83,535,767	$ 82	$ 2,741,391	$ 2,553	$ (2,935,235)	$ (191,209)

Three months ended September 30, 2025	Common stock		Additional paid-in capital	Accumulated other comprehensive income (loss)	Accumulated deficit	Total stockholders' deficit
	Shares	Amount				
Balance, June 30, 2025	79,378,145	$ 78	$ 2,631,180	$ 7,356	$ (2,845,160)	$ (206,546)
Exercise of options	486,129	1	16,829	—	—	16,830
Restricted stock vesting and issuance, net	67,492	—	—	—	—	—
Share-based compensation expense	—	—	19,426	—	—	19,426
Receivable from investor	—	—	62	—	—	62
Net income	—	—	—	—	15,896	15,896
Comprehensive loss	—	—	—	(1,426)	—	(1,426)
Balance, September 30, 2025	79,931,766	$ 79	$ 2,667,497	$ 5,930	$ (2,829,264)	$ (155,758)

Nine months ended September 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	648,218	1	24,985	—	—	24,986
Restricted stock vesting and issuance, net	1,361,190	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	—	—	70,539	—	—	70,539
Receivable from investor	—	—	2	—	—	2
Net income	—	—	—	—	28,994	28,994
Comprehensive loss	—	—	—	(7,948)	—	(7,948)
Balance, September 30, 2026	83,535,767	$ 82	$ 2,741,391	$ 2,553	$ (2,935,235)	$ (191,209)

Nine months ended September 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	953,414	1	33,714	—	—	33,715
Restricted stock vesting and issuance, net	1,201,681	1	—	—	—	1
Issuance of common stock in connection with an employee stock purchase plan	72,483	—	2,827	—	—	2,827
Share-based compensation expense	—	—	56,029	—	—	56,029
Receivable from investor	—	—	316	—	—	316
Net income	—	—	—	—	817,609	817,609
Comprehensive income	—	—	—	31,816	—	31,816
Balance, September 30, 2025	79,931,766	$ 79	$ 2,667,497	$ 5,930	$ (2,829,264)	$ (155,758)

See accompanying unaudited notes.

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

	Nine Months Ended September 30,	
	2026	**2025**
Cash flows from operating activities		
Net income	$ 28,994	$ 817,609
Adjustments to reconcile net income to net cash provided by operating activities:		
Depreciation and amortization	48,104	25,913
Non-cash operating lease expense	5,358	5,635
Non-cash royalty revenue related to sale of future royalties	(199,624)	(149,160)
Non-cash interest expense on liability related to sale of future royalties	196,861	149,305
Change in valuation of contingent consideration	—	(800)
Tangible asset impairment and losses on transactions, net	927	648
Inducement expense related to repurchase of 2026 Convertible Notes	3,386	—
Unrealized gain on ClearPoint Equity Investments	—	(5,734)
Unrealized gain on marketable securities - equity investments	(3,018)	(3,160)
Disposal of asset	—	163
Deferred income taxes	—	(4)
Amortization of discounts on investments, net	(13,600)	(12,972)
Amortization of debt issuance costs	1,459	906
Loss on extinguishment of debt	19,263	—
Share-based compensation expense	70,539	56,029
Unrealized foreign currency transaction losses, net	637	4,768
Changes in operating assets and liabilities:		
Inventory, net	(31,332)	(27,117)
Prepaid expenses and other current assets	4,858	(13,876)
Trade and royalty receivables, net	(60,076)	(32,365)
Deposits and other assets	(635)	1,501
Accounts payable and accrued expenses	(20,521)	(36,341)
Other liabilities	(6,827)	(26,497)
Deferred revenue	7	(4,298)
Payments on contingent consideration	—	(4,684)
Net cash provided by operating activities	$ 44,760	$ 745,469
Cash flows from investing activities		
Purchases of fixed assets	$ (6,046)	$ (6,486)
Proceeds from sale of fixed assets	—	70
Purchases of marketable securities- available for sale	(1,674,893)	(1,051,267)
Purchases of marketable securities- equity investments	(38,864)	(46,537)
Sale of marketable securities- available for sale	1,029,918	424,351
Sale of marketable securities- equity investments	39,655	40,112
Acquisition of product rights and licenses	(8,474)	(256,235)
Net cash used in investing activities	$ (658,704)	$ (895,992)
Cash flows from financing activities		
Proceeds from exercise of options	$ 24,986	$ 33,715
Proceeds from employee stock purchase plan	3,163	2,827
Repurchase and final settlement of the 2026 Convertible Notes	(402,653)	—
Conversion of the 2026 Convertible Notes	(12,879)	—
Debt issuance costs related to 2031 Convertible Notes	(14,572)	—
Proceeds from issuance of 2031 Convertible Notes	550,000	—
Payments on contingent consideration obligation	—	(6,341)
Payments on finance lease principal	(3,000)	(1,611)
Net cash provided by financing activities	$ 145,045	$ 28,590
Effect of exchange rate changes on cash	(4,100)	12,917
Net decrease in cash and cash equivalents	(472,999)	(109,016)
Cash and cash equivalents, and restricted cash beginning of period	998,334	795,316
Cash and cash equivalents, and restricted cash end of period	$ 525,335	$ 686,300
Supplemental disclosure of cash information		
Cash paid for interest	$ 3,433	$ 5,702
Total income taxes paid, net of refunds received:		
Federal	—	
State:		
Illinois	6,570	
Florida	1,300	
Other State	153	
Foreign:	950	
Total income taxes paid, net of refunds received	$ 8,973	$ 40,124
Supplemental disclosure of non-cash investing and financing activity		
Unrealized (loss) gain on marketable securities, net of tax	$ (4,781)	$ 402
Right-of-use assets obtained in exchange for operating lease obligations	—	27,675
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	4,657	2,221
Fixed asset additions through tenant improvement allowance	—	766
Milestone payable	80,000	32,500

See accompanying unaudited notes.

PTC Therapeutics, Inc.

Notes to Consolidated Financial Statements (unaudited)
September 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 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 ("CHMP"), of the European Medicines Agency ("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 (the "EU") 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 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. Hoffmann-La Roche Ltd and Hoffmann-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 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 nine months ended September 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, the Company is working with Novartis to finalize a plan to engage with FDA to discuss the 24-month results. Due to FDA scheduling, a Type C meeting to discuss votoplam Phase 2 data and potential accelerated approval path is scheduled for the first quarter of 2027.

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 initiated 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 second quarter of 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 initiated 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 September 2026, the Company acquired from Sangamo Therapeutics, Inc. ("Sangamo") all of Sangamo's rights, title and interest in and to the assets primarily related to ST-920 (isaralgagene civaparvovec), a one-time administered AAV gene therapy product candidate for Fabry disease. A rolling biologics license application, or BLA, submission to FDA for accelerated approval of ST-920 is expected to be completed in the fourth quarter of 2026. The BLA for accelerated approval will be based on evidence of durable α-Gal A activity, reduction of lyso Gb3 levels and early signals of clinical benefit on renal function as well as other clinical aspects of disease. In addition, ST-920 has demonstrated an encouraging safety and tolerability profile and there is no requirement for routine prophylactic or post-infusion systemic immunosuppressive agents. The Company also expects to pursue regulatory approval outside of the United States. Refer to Note 3 for further details regarding the acquisition.

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 September 30, 2026, the Company had an accumulated deficit of approximately $2,935.2 million. The Company has financed its operations to date primarily through the private offerings of convertible senior notes (see Note 10), public and "at the market" offerings of common stock, proceeds from royalty purchase agreements (see Note 10), 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 nine months ended September 30, 2026. Selected significant accounting policies are discussed in further detail below.

Basis of presentation

The accompanying financial information as of September 30, 2026 and for the three and nine months ended September 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 September 30, 2026 and for the three and nine months ended September 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 nine months ended September 30, 2026 are not necessarily indicative of the results to be expected for the year ending 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- September 30, 2026	Beginning of period- December 31, 2025
Cash and cash equivalents	$ 508,894	$ 984,648
Restricted cash included in deposits and other assets	16,441	13,686
Total Cash, cash equivalents and restricted cash per consolidated statement of cash flows	$ 525,335	$ 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 revenue 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 nine months ended September 30, 2026 and 2025, no allowance was recorded for credit losses. The allowance for doubtful accounts was $15.3 million as of September 30, 2026 and $15.2 million as of December 31, 2025. For the three months ended September 30, 2026 bad debt expense was immaterial. For the nine months ended September 30, 2026, bad debt expense was $0.7 million. For the three and nine months ended September 30, 2025, bad debt expense was $0.8 million and $8.6 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 September 30, 2026.

On December 15, 2022, the EU member states formally adopted the EU's Pillar Two Directive, which generally provides for a minimum effective tax rate of 15%, as established by the Organization for Economic Co-operation and Development Pillar Two Framework that was supported by over 130 countries worldwide. The EU effective dates were January 1, 2024, and January 1, 2025, for different aspects of the directive. A significant number of other countries are also implementing similar legislation. As a result, the tax laws in the U.S. and other countries in which PTC and its affiliates do business could change on a prospective or retroactive basis and any such changes could materially adversely affect the Company's business. The Company is continuing to evaluate the potential impact on future periods of the Pillar Two Framework, pending legislative adoption by additional individual countries, including those within the EU.

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 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 10 for additional details and the impact on the Company's consolidated financial statements.

3. Acquisitions

Sangamo Asset Acquisition

On August 25, 2026, the Company entered into an Asset Purchase Agreement with Sangamo (the "Sangamo Purchase Agreement") to acquire from Sangamo, subject to the terms and conditions set forth in the Sangamo Purchase Agreement, all of Sangamo's right, title and interest in and to the assets primarily related to ST-920, a one-time administered AAV gene therapy product candidate for Fabry disease, which Sangamo had been developing and investigating in clinical trials (the "Sangamo Asset Acquisition").

Upon the closing of the Sangamo Asset Acquisition on September 17, 2026 and pursuant to the Sangamo Purchase Agreement, the Company paid upfront cash consideration of $111.0 million and may become obligated to make up to an additional $100.0 million in contingent milestone payments upon the achievement of specified regulatory approval milestones.

As ST-920 was the only asset acquired and no liabilities were assumed, the Company determined that all of the fair value was concentrated in ST-920 and accounted for the transaction as an asset acquisition under ASC 805-50. As ST-920 is an in-process research and development asset, the Company concluded that it did not have any alternative future use, and accordingly, the upfront cash consideration of $111.0 million was recorded to acquired in-process research and development within operating expenses on the Company's statement of operations during the three and nine months ended September 30, 2026. The contingent milestone payments were not included in the initial cost of the acquired assets and

will be capitalized and amortized over their expected useful lives when the related contingencies are resolved and the amounts become payable.

4. Inventory

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

	September 30, 2026	December 31, 2025
Raw materials	$ 10,512	$ 2,291
Work in progress	74,731	55,686
Finished goods	24,089	21,670
Total inventory	$ 109,332	$ 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 months ended September 30, 2026, the inventory write-downs were immaterial. For the nine months ended September 30, 2026, the Company recorded inventory write-downs of $2.6 million primarily related to adjustments to inventory reserves and product approaching expiration. For the three and nine months ended September 30, 2025, the Company recorded inventory write-downs of $0.5 million and $4.8 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 nine months ended September 30, 2026 and 2025, these amounts were immaterial. For the nine months ended September 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 September 30, 2026, and for the three and nine months ended September 30, 2025.

5. 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 nine months ended September 30, 2026 and September 30, 2025:

	Ending Balance at June 30, 2026	Unrealized Gain	Foreign Currency Unrealized Loss	Investments Purchased	Sales	Ending Balance at September 30, 2026
Marketable securities - equity investments	$ 31,631	1,104	(363)	5,887	(2,156)	$ 36,103
Total Fair Value	$ 31,631	$ 1,104	$ (363)	$ 5,887	$ (2,156)	$ 36,103

	Ending Balance at June 30, 2025	Unrealized Gain	Foreign Currency Unrealized Gain	Investments Purchased	Sales	Ending Balance at September 30, 2025
Marketable securities - equity investments	$ 34,203	1,622	1,605	29,508	(22,982)	$ 43,956
ClearPoint Equity Investments	10,681	8,812	—	—	—	19,493
Total Fair Value	$ 44,884	$ 10,434	$ 1,605	$ 29,508	$ (22,982)	$ 63,449

	Ending Balance at December 31, 2025	Unrealized Gain	Foreign Currency Unrealized Gain	Investments Purchased	Sales	Ending Balance at September 30, 2026
Marketable securities - equity investments	$ 31,596	3,018	2,280	38,864	(39,655)	$ 36,103
Total Fair Value	$ 31,596	$ 3,018	$ 2,280	$ 38,864	$ (39,655)	$ 36,103

	Ending Balance at December 31, 2024	Unrealized Gain	Foreign Currency Unrealized Gain	Investments Purchased	Sales	Ending Balance at September 30, 2025
Marketable securities - equity investments	$ 29,034	3,160	5,337	46,537	(40,112)	$ 43,956
ClearPoint Equity Investments	13,759	5,734	—	—	—	19,493
Total Fair Value	$ 42,793	$ 8,894	$ 5,337	$ 46,537	$ (40,112)	$ 63,449

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 September 30, 2026 and December 31, 2025:

	September 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	$ 1,582,921	$ —	$ 1,582,921	$ —
Marketable securities - equity investments	$ 36,103	$ 36,103	$ —	$ —

	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 nine months ended September 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 September 30, 2026 and December 31, 2025:

	September 30, 2026			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 122,351	$ —	$ (206)	$ 122,145
Corporate debt securities	679,965	—	(2,162)	677,803
Government obligations	784,599	3	(1,629)	782,973
Total	$ 1,586,915	$ 3	$ (3,997)	$ 1,582,921

	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 nine months ended September 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 nine months ended September 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 nine months ended September 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 September 30, 2026 are as follows:

	September 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	$ (206)	122,145	—	—	(206)	$ 122,145
Corporate debt securities	$ (2,162)	677,803	—	—	(2,162)	$ 677,803
Government obligations	$ (1,629)	734,956	—	—	(1,629)	$ 734,956
Total	$ (3,997)	$ 1,534,904	$ —	$ —	$ (3,997)	$ 1,534,904

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 September 30, 2026 and December 31, 2025 mature as follows:

	September 30, 2026	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 122,145	$ —
Corporate debt securities	611,756	66,047
Government obligations	730,227	52,746
Total	$ 1,464,128	$ 118,793

	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 10. 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 September 30, 2026 was $506.8 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"). As of September 30, 2026, the 2026 Convertible Notes were all either converted, repurchased, or extinguished. Refer to Note 10 for details. The fair value of the 2026 Convertible Notes, which differed from their carrying values, was influenced by interest rates, the Company's stock price and stock price volatility and was 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 December 31, 2025 was $424.5 million.

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 11 for additional details.

6. Accounts payable and accrued expenses

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

	September 30, 2026	December 31, 2025
Employee compensation, benefits, and related accruals	$ 48,410	$ 73,737
Income tax payable	—	4,677
Consulting and contracted research	15,204	25,007
Sales allowance	100,322	129,197
Sales rebates	120,636	76,704
Royalties	16,970	9,574
Accounts payable	41,591	45,487
Milestone payable	80,000	416
Other	17,678	16,473
Total	$ 440,811	$ 381,272

7. 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 nine months ended September 30, 2026 and 2025. The remaining shares of the Company's 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 September 30, 2026.

8. Net (loss) income per share

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

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Numerator:				
Net (loss) income, basic	$ (51,693)	$ 15,896	$ 28,994	$ 817,609
Add: Interest expense, net of tax, on the Company's 2026 and 2031 Convertible Notes	—	1,096	2,789	3,257
Net (loss) income, diluted	$ (51,693)	$ 16,992	$ 31,783	$ 820,866
Denominator:				
Weighted-average number of shares outstanding, basic	83,476,100	78,471,119	83,004,625	78,642,478
Effect of dilutive securities:				
Common stock issuable under the Company's equity incentive plans	—	3,170,135	3,601,228	2,783,134
Common stock issuable under the Company's 2026 and 2031 Convertible Notes	—	5,474,115	5,647,822	5,474,115
Weighted-average common shares outstanding, diluted	83,476,100	87,115,369	92,253,675	86,899,727
Net (loss) income per common share, basic	$ (0.62)*	$ 0.20	$ 0.35	$ 10.40
Net (loss) income per common share, diluted	$ (0.62)*	$ 0.20	$ 0.34	$ 9.45

* In the three months ended September 30, 2026, 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 September 30,	
	2026	2025
Stock options	875,727	3,246,389
Unvested restricted stock units	5,100	90,900
Unvested performance based restricted stock units	75,893	—
Total	956,720	3,337,289

9. 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 September 30, 2026, awards for 3,449,684 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 September 30, 2026, awards for 1,667,243 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 September 30, 2026, the Company issued a total of 827,789 stock options to various employees. Of those, 77,025 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	827,789	$ 75.87		
Exercised	(648,218)	$ 38.55		
Forfeited/Cancelled	(21,999)	$ 44.05		
Outstanding at September 30, 2026	6,553,774	$ 47.61	5.65 years	$ 123,254
Expected to vest at September 30, 2026	1,529,433	$ 56.39	8.51 years	$ 20,798
Exercisable at September 30, 2026	4,867,971	$ 44.40	4.65 years	$ 100,949

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

	Nine months ended September 30, 2026
Risk-free interest rate	3.78% - 4.85%
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 nine months ended September 30, 2026 was $39.97 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 September 30, 2026, the Company issued a total of 1,341,523 restricted stock units to various employees. Of those, 68,605 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	1,341,523	76.21
Performance based PSUs with conditions satisfied	25,000	46.37
Vested	(1,361,190)	38.04
Forfeited	(202,804)	46.15
Unvested at September 30, 2026	3,469,659	$ 52.55

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 nine months ended September 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 nine months ended September 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. During the nine months ended September 30, 2026, all 37,500 PSUs with performance conditions were forfeited due to the regulatory performance conditions not being satisfied before the performance deadline. The expenses related to satisfied PSUs were $0.4 million and $1.6 million for the three and nine months ended September 30, 2026, respectively, and $1.0 million for the three and nine months ended September 30, 2025. The expenses related to the PSUs with market conditions granted to Dr. Klein were $1.1 million and $3.4 million for the three and nine months ended September 30, 2026, respectively, and $0.1 and $0.5 million for the three and nine months ended September 30, 2025, respectively.

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)
PSUs with performance or market conditions not satisfied and forfeited during the period	(37,500)	—	(37,500)
Total PSUs granted for which the performance or market conditions were not yet satisfied at September 30, 2026	—	115,625	115,625

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 nine months ended September 30, 2026, the Company recorded $0.5 million and $1.6 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 September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Research and development	$ 10,479	$ 9,133	$ 32,155	$ 26,827
Selling, general and administrative	13,348	10,293	38,384	29,202
Total	$ 23,827	$ 19,426	$ 70,539	$ 56,029

As of September 30, 2026, there was approximately $188.2 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 2.8 years.

10. 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 the 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 nine months ended September 30, 2026:

Liability for sale of future royalties- (current and noncurrent)	Nine Months Ended September 30, 2026
Beginning balance as of December 31, 2025	$ 2,308,366
Less: Non-cash royalty revenue payable to Royalty Pharma	(199,624)
Plus: Non-cash interest expense recognized	196,861
Ending balance	$ 2,305,603
Effective interest rate as of September 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 2031 Convertible 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:

	September 30, 2026	December 31, 2025
Principal	$ 550,000	$ —
Less: Debt issuance costs	(13,732)	—
Net carrying amount	$ 536,268	$ —

As of September 30, 2026, the remaining contractual life of the 2031 Convertible Notes is approximately 4.7 years.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Amortization of debt issuance costs	$ 720	$ —	$ 839	$ —
Total	$ 720	$ —	$ 839	$ —
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 bore 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 matured on September 15, 2026.

The 2026 Convertible Notes were 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 could convert their 2026 Convertible Notes at any time. Upon conversion, the Company was to 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 19.0404 shares of the Company's common stock per $1,000 principal amount of the 2026 Convertible Notes, which was equivalent to an initial conversion price of approximately $52.52 per share of the Company's common stock. The conversion rate was subject to adjustment in some events but was not to 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 could 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 provided 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 was provided for the 2026 Convertible Notes, which meant that the Company was not required to redeem or retire the 2026 Convertible Notes periodically.

If the Company underwent a "fundamental change" (as defined in the 2026 Convertible Notes Indenture), subject to certain conditions, holders of the 2026 Convertible Notes could 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 represented senior unsecured obligations and ranked senior in right of payment to the Company's future indebtedness expressly subordinated in right of payment to the notes, equal in right of payment to the Company's existing and future unsecured indebtedness 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 contained 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, could, and the 2026 Convertible Notes Trustee at the request of such holders (subject to the provisions of the 2026 Convertible Notes Indenture) would, 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 would automatically become due and payable. Upon such a declaration of acceleration, such principal and accrued and unpaid interest, if any, would be due and payable immediately.

In April 2026, the Company settled a holder's conversion 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 accounted for the transaction as an induced conversion under ASC 470-20 and recorded a $10.0 million reduction to the carrying value of the 2026 Convertible Notes with the excess of $2.9 million recognized as a reduction in additional paid-in capital within the Company's statement of stockholders' deficit.

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.

On July 20, 2026, the Company provided an irrevocable final settlement notice to the 2026 Convertible Notes Trustee stating that the Company elected a combination settlement with a specified dollar amount of $1,460, together with cash, if applicable, in lieu of delivering any fractional share of the Company's common stock pursuant to the 2026 Convertible Notes Indenture for the final conversion of any 2026 Convertible Notes surrendered prior to close of business on the business day immediately preceding the maturity date.

In September 2026, $55.5 million aggregate principal of the 2026 Convertible Notes were converted, which the Company settled by paying cash of $74.8 million pursuant to the 2026 Convertible Notes Indenture on September 15, 2026. Additionally, the Company paid accrued interest of $0.4 million pursuant to the 2026 Convertible Notes Indenture on September 15, 2026. The Company evaluated the transaction noting that as the settlement notice was irrevocable and the Company elected to settle all or a portion of the conversion value in excess of the principal in cash the Company concluded that the conversion option no longer met the own-equity exception in ASC 815-10-15-74(a) and therefore should be bifurcated with the conversion option recorded at fair value. The Company accounted for the conversions under extinguishment accounting in accordance with ASC 470-50, whereby the additional cash required to be paid in excess of the principal, an amount of $19.3 million, was recorded as a loss on extinguishment of debt within the consolidated statements of operations. The $19.3 million loss is being presented net of a gain on the change in the fair value of the conversion option of $7.7 million.

The 2026 Convertible Notes consisted of the following:

	September 30, 2026	December 31, 2025
Principal	$ 287,500	$ 287,500
Conversion of the 2026 Convertible Notes	(10,000)	—
Repurchase and final settlement of the 2026 Convertible Notes	(277,500)	—
Less: Debt issuance costs	—	(869)
Net carrying amount	$ —	$ 286,631

As of September 30, 2026, there is no contractual life remaining as the 2026 Convertible Notes has been satisfied and discharged.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Contractual interest expense	$ 172	$ 1,081	$ 2,071	$ 3,217
Amortization of debt issuance costs	49	306	620	906
Total	$ 221	$ 1,387	$ 2,691	$ 4,123
Effective interest rate	1.9 %	1.9 %	1.9 %	1.9 %

11. 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 September 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 September 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 September 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 September 30, 2026, $152.5 million of the $217.5 million in development and regulatory milestones have been paid to the former Censa securityholders.

In 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 September 30, 2026 and is being amortized to cost of product sales over its expected useful life on a straight-line basis. As of the quarter ended September 30, 2026, aggregate Sephience global net sales in the prior four consecutive quarters exceeded $500.0 million, which, pursuant to the Censa Merger Agreement, triggered a $50.0 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 September 30, 2026 and is being amortized to cost of product sales over its expected useful life on a straight-line basis. For the nine months ended September 30, 2026, royalties of $4.1 million related payable to the former Censa securityholders were recorded on the consolidated balance sheet within intangible assets, net. Refer to Note 13 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 nine months ended September 30, 2026, royalties of $4.3 million and $3.4 million related to Tegsedi and Waylivra, respectively, were recorded on the consolidated balance sheet within intangible assets, net. Refer to Note 13 for additional details.

On August 25, 2026, the Company entered into the Sangamo Purchase Agreement to acquire from Sangamo, subject to the terms and conditions set forth in the Sangamo Purchase Agreement, all of Sangamo's right, title and interest in and to the assets primarily related to ST-920, a one-time administered AAV gene therapy product candidate for Fabry disease, which Sangamo had been developing and investigating in clinical trials. Subject to the terms and conditions of the Sangamo Purchase Agreement, Sangamo may become entitled to receive contingent payments from the Company based on the achievement of the following milestone events, (i) a one-time, non-refundable payment of $80.0 million (the "Accelerated Milestone Payment"), upon accelerated approval by the FDA (the "Accelerated Milestone Event") of a BLA for ST-920 for the treatment of Fabry disease based on Sangamo's Phase 1/2 STAAR study and related long-term follow-up clinical study (the "STAAR Studies"), and (ii) a one-time, non-refundable payment of $20.0 million (the "Full Milestone Payment") upon full approval by the FDA of a BLA for ST-920 for the treatment of Fabry disease based on the STAAR Studies, either as part of the original BLA submission or if an accelerated approval is subsequently converted into a full approval (the "Full Milestone Event"). The Company is required to notify Sangamo in writing within 15 business days following the achievement of a milestone event, and any applicable payment must be made no later than 20 business days following such notice. If the Full Milestone Event is achieved and the Accelerated Milestone Event was not previously

achieved, the Company will be required to make both the Accelerated Milestone Payment and the Full Milestone Payment within 20 business days following notice of the achievement of the Full Milestone Event. The aggregate milestone payments made by the Company to Sangamo under the Sangamo Purchase Agreement will not exceed $100.0 million. The Company will record the milestone payments when the applicable milestone events are achieved and they become payable. Milestone payments will be capitalized and amortized over their expected useful lives, since at the time of achievement there will be alternative future use.

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.

12. 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 nine months ended September 30, 2026 and 2025, net product revenues consisted of the following:

	Three Months Ended September 30,					
	2026			2025		
(in thousands)	United States	International	Total	United States	International	Total
Sephience	$ 144,795	$ 37,090	$ 181,885	$ 14,365	$ 5,198	$ 19,563
Translarna	—	48,511	48,511	—	50,665	50,665
Emflaza	25,094	—	25,094	35,160	—	35,160
Upstaza/Kebilidi	3,960	10,593	14,553	6,898	8,837	15,735
All other products	—	5,851	5,851	—	9,833	9,833
Total net product revenue	$ 173,849	$ 102,045	$ 275,894	$ 56,423	$ 74,533	$ 130,956

	Nine Months Ended September 30,					
	2026			2025		
	United States	International	Total	United States	International	Total
Sephience	$ 384,385	$ 73,361	$ 457,746	$ 14,365	$ 5,198	$ 19,563
Translarna	—	149,704	149,704	—	196,289	196,289
Emflaza	71,206	—	71,206	119,303	—	119,303
Upstaza/Kebilidi	6,958	28,351	35,309	6,898	29,385	36,283
All other products	—	26,321	26,321	—	31,273	31,273
Total net product revenue	$ 462,549	$ 277,737	$ 740,286	$ 140,566	$ 262,145	$ 402,711

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
(in thousands)	2026	2025	2026	2025
United States	$ 173,849	$ 56,423	$ 462,549	$ 140,566
Russia	33,011	25,107	61,841	81,745
Brazil	1,590	9,222	51,012	59,678
All other countries	67,444	40,204	164,884	120,722
Total net product revenue	$ 275,894	$ 130,956	$ 740,286	$ 402,711

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

As of September 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 September 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 nine months ended September 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 nine months ended September 30, 2026, the Company has recognized $81.7 million and $199.6 million of royalty revenue related to Evrysdi, respectively. For the three and nine months ended September 30, 2025 the Company has recognized $70.8 million and $164.8 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 nine months ended September 30, 2026, the Company recognized $0.2 million and $51.0 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 nine months ended September 30, 2025, the Company recognized $9.3 million and $1.0 billion, respectively, in collaboration and license revenues related to performance obligations completed pursuant to the Novartis Agreement. Collaboration and license revenue during the nine months ended September 30, 2025 was partially offset by $3.5 million related to a refund for a prior collaboration arrangement in relation to votoplam.

13. Intangible assets and goodwill

Definite-lived intangibles

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

Definite-lived intangible assets, gross	Ending Balance at December 31, 2025	Additions	Foreign currency translation	Ending Balance at September 30, 2026
Waylivra	18,598	3,414	(691)	21,321
Tegsedi	24,825	4,297	(902)	28,220
Kebilidi	10,731	—	—	10,731
Upstaza	106,937	—	—	106,937
Sephience	283,500	84,053	—	367,553
Total definite-lived intangibles, gross	$ 444,591	$ 91,764	$ (1,593)	$ 534,762

Definite-lived intangible assets, accumulated amortization	Ending Balance at December 31, 2025	Amortization	Foreign currency translation	Ending Balance at September 30, 2026
Waylivra	(8,689)	(2,848)	353	(11,184)
Tegsedi	(10,295)	(3,877)	426	(13,746)
Kebilidi	(1,006)	(1,459)	—	(2,465)
Upstaza	(27,437)	(11,925)	—	(39,362)
Sephience	(8,411)	(15,908)	—	(24,319)
Total definite-lived intangibles, accumulated amortization	$ (55,838)	$ (36,017)	$ 779	$ (91,076)
Total definite-lived intangibles, net				$ 443,686

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 increases the cost basis for the Waylivra and Tegsedi intangible assets.

For the nine months ended September 30, 2026, royalties of $4.3 million and $3.4 million related to Tegsedi and Waylivra, respectively, were recorded on the consolidated balance sheet within intangible assets, net. As of September 30, 2026, a royalty payable of $1.1 million and $0.5 million for Tegsedi and Waylivra, respectively, was recorded on the consolidated balance sheet within accounts payable and accrued expenses.

As of the three months 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 and intangible assets on the consolidated balance sheet as of September 30, 2026. As of the three months ended September 30, 2026, aggregate Sephience global net sales in the prior four consecutive quarters exceeded $500.0 million, which, pursuant to the Censa Merger Agreement, triggered a $50.0 million net sales milestone payment to the former Censa securityholders. The $50.0 million milestone was recorded in accounts payable and accrued expenses and intangible assets on the Company's consolidated balance sheet as of September 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 nine months ended September 30, 2026, royalties of $4.1 million were recorded for Sephience. As of September 30, 2026, a royalty payable of $3.1 million for Sephience was recorded on the consolidated balance sheet within accounts payable and accrued expenses.

For the three months ended September 30, 2026 and 2025, the Company recognized amortization expense of $12.6 million and $7.5 million, respectively, related to its intangible assets. For the nine months ended September 30, 2026 and 2025, the Company recognized amortization expense of $36.0 million and $15.4 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 September 30, 2026
2026	$ 13,534
2027	54,133
2028	54,133
2029	47,781
2030 and thereafter	274,105
Total	$ 443,686

The weighted average remaining amortization period of the definite-lived intangibles as of September 30, 2026 is 10.8 years.

Goodwill

As a result of the Agilis Merger on August 23, 2018, the Company recorded $82.3 million of goodwill. As of September 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 September 30, 2026 is $82.3 million.

14. 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 nine months ended September 30, 2026 and 2025:

	Three months ended September 30,		Nine months ended September 30,	
	2026	2025	2026	2025
Total revenues	$ 357,807	$ 211,007	$ 990,877	$ 1,565,978
Less:				
Cost of product, collaboration and license sales	12,740	12,441	43,351	28,241
Program spend	39,275	45,940	119,400	143,803
Employee costs	65,245	64,470	209,714	197,762
Manufacturing costs	11,632	11,048	26,949	45,517
Administrative costs	22,982	18,614	56,221	54,924
Occupancy costs	7,195	7,852	23,042	21,678
Acquired in-process research and development	111,000	—	111,000	—
Other segment items (a)	139,431	34,746	372,206	256,444
Segment net (loss) income	$ (51,693)	$ 15,896	$ 28,994	$ 817,609
Reconciliation of profit or loss				
Adjustments and reconciling items	—	—	—	—
Consolidated net (loss) income	$ (51,693)	$ 15,896	$ 28,994	$ 817,609

(a) Other segment items include the following:

	Three months ended September 30,		Nine months ended September 30,	
	2026	2025	2026	2025
Interest income	$ (20,928)	$ (19,089)	$ (57,032)	$ (57,325)
Interest expense	66,794	51,681	200,409	154,367
Income tax expense (benefit)	10,605	(38,970)	25,001	18,093
Depreciation	4,222	3,569	12,037	10,600
Amortization	12,595	7,534	36,017	15,393
All other (b)	66,143	30,021	155,774	115,316
Total other segment items	$ 139,431	$ 34,746	$ 372,206	$ 256,444

(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, loss on extinguishment of debt, net, and other expense.

15. 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

Sangamo Asset Acquisition

In September 2026, we acquired from Sangamo Therapeutics, Inc., or Sangamo, all rights, title and interest in and to the assets primarily related to ST-920 (isaralgagene civaparvovec), a one-time administered AAV gene therapy product candidate for Fabry disease. A rolling biologics license application, or BLA, submission to the U.S. Food and Drug Administration, or FDA, for accelerated approval of ST-920 is expected to be completed in Q4 2026. The BLA for accelerated approval will be based on evidence of durable α-Gal A activity, reduction of lyso Gb3 levels and early signals of clinical benefit on renal function as well as other clinical aspects of disease. In addition, ST-920 has demonstrated an encouraging safety and tolerability profile and there is no requirement for routine prophylactic or post-infusion systemic immunosuppressive agents. We also expect to pursue regulatory approval of ST-920 outside of the United States.

Subject to the terms and conditions of the Sangamo Purchase Agreement, Sangamo may become entitled to receive contingent payments from the Company based on the achievement of certain regulatory milestone events up to $100.0 million.

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 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 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 September 30, 2026, we recognized $181.9 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 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, 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 September 30, 2026, we recognized $48.5 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 September 30, 2026, we recognized $25.1 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, when commercializing Emflaza for the treatment of DMD in patients five years and older, which expired in February 2024, and for the treatment of DMD in patients two years of age to less than five, which expired in June 2026. 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.

Upstaza™ (eladocagene exuparvovec) / Kebilidi™ (eladocagene exuparvovec-tneq)

Upstaza/Kebilidi is a gene therapy for the treatment of Aromatic L Amino Acid 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 dated August 1, 2018, by and between us and Akcea Therapeutics, Inc., 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. Hoffmann-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 Neurofilament light 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. Due to FDA scheduling, a Type C meeting to discuss votoplam Phase 2 data and potential accelerated approval path is scheduled for the first quarter of 2027.

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, in the third quarter of 2026 we initiated the PROVE-FA open label study using matched natural history control. 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.

In the second quarter of 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.

In the third quarter of 2026, we also initiated a Phase 2a study of PTC844, our next-generation DHODH inhibitor. 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 EAPs, 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, 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. Hoffmann-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 nine months ended September 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 nine months ended September 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 September 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.

In the three months 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 and intangible assets on our consolidated balance sheet as of September 30, 2026. In the three months ended September 30, 2026, aggregate Sephience global net sales in the prior four consecutive quarters exceeded $500.0 million, which, pursuant to the Censa Merger Agreement, triggered a $50.0 million net sales milestone payment to the former Censa securityholders. This milestone was recorded in accounts payable and accrued expenses and intangible assets on our consolidated balance sheet as of September 30, 2026.

As of September 30, 2026, we had an accumulated deficit of $2,935.2 million. We had net income of $29.0 million and $817.6 million for the nine months ended September 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 September 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 nine months ended September 30, 2026 and 2025, net product revenues consisted of the following:

	Three Months Ended September 30,					
	2026			2025		
(in thousands)	United States	International	Total	United States	International	Total
Sephience	$ 144,795	$ 37,090	$ 181,885	$ 14,365	$ 5,198	$ 19,563
Translarna	—	48,511	48,511	—	50,665	50,665
Emflaza	25,094	—	25,094	35,160	—	35,160
Upstaza/Kebilidi	3,960	10,593	14,553	6,898	8,837	15,735
All other products	—	5,851	5,851	—	9,833	9,833
Total net product revenue	$ 173,849	$ 102,045	$ 275,894	$ 56,423	$ 74,533	$ 130,956

	Nine Months Ended September 30,					
	2026			2025		
	United States	International	Total	United States	International	Total
Sephience	$ 384,385	$ 73,361	$ 457,746	$ 14,365	$ 5,198	$ 19,563
Translarna	—	149,704	149,704	—	196,289	196,289
Emflaza	71,206	—	71,206	119,303	—	119,303
Upstaza/Kebilidi	6,958	28,351	35,309	6,898	29,385	36,283
All other products	—	26,321	26,321	—	31,273	31,273
Total net product revenue	$ 462,549	$ 277,737	$ 740,286	$ 140,566	$ 262,145	$ 402,711

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
(in thousands)	2026	2025	2026	2025
United States	$ 173,849	$ 56,423	$ 462,549	$ 140,566
Russia	33,011	25,107	61,841	81,745
Brazil	1,590	9,222	51,012	59,678
All other countries	67,444	40,204	164,884	120,722
Total net product revenue	$ 275,894	$ 130,956	$ 740,286	$ 402,711

For the three and nine months ended September 30, 2026, three of our distributors each accounted for over 10% of our net product sales. For the three and nine months ended September 30, 2025, four and three 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 September 30, 2026, we had recognized a total of $310.0 million in milestone payments and $989.4 million in royalties on net sales pursuant to the SMA License Agreement. As of September 30, 2026, there are no remaining research and development event milestones that we can receive. The remaining potential sales milestones as of September 30, 2026 are $150.0 million upon achievement of certain sales events.

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

For the three and nine months ended September 30, 2026, we recognized $81.7 million and $199.6 million of royalty revenue, respectively, related to Evrysdi. For the three and nine months ended September 30, 2025, we recognized $70.8 million and $164.8 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 months ended September 30, 2026, we recognized $0.2 million in revenues related to work performed for Novartis. During the nine months ended September 30, 2026, we recognized $51.0 million in license revenues, 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 nine months ended September 30, 2025, we recognized $9.3 million and $1.0 billion in license revenues, respectively, related to performance obligations completed pursuant to the Novartis Agreement. Collaboration and license revenue during the nine months ended September 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 nine months ended September 30, 2026 and 2025.

	Three Months Ended September 30,	
	2026	2025
	(in thousands)	
Sephience	$ 27,695	$ 27,618
Inflammation & Ferroptosis platform	13,637	10,991
Gene Therapy	4,920	2,749
Other development programs	138	2,700
Total Development	46,390	44,058
Research	10,137	9,680
Payroll, benefits, and share-based stock compensation	38,831	36,613
Facilities and other indirect costs	9,995	9,807
Total research and development	**$ 105,353**	**$ 100,158**

	Nine Months Ended September 30,	
	2026	2025
	(in thousands)	
Sephience	$ 72,399	$ 78,500
Inflammation & Ferroptosis platform	24,872	26,282
Global DMD	4,821	17,450
Gene Therapy	8,762	10,984
Other development programs	845	9,828
Total Development	111,699	143,044
Research	41,350	39,230
Payroll, benefits, and share-based stock compensation	122,385	111,843
Facilities and other indirect costs	29,942	28,004
Total research and development	**$ 305,376**	**$ 322,121**

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

For the three and nine months ended September 30, 2026, compared to the three and nine months ended September 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. For the three months ended September 30, 2026, compared to the three months ended September 30, 2025, the increase is primarily due to an increase in costs relating to Inflammation and Ferroptosis platform and Gene Therapy. For the nine months ended September 30, 2026, compared to the nine months ended September 30, 2025, the decrease is primarily due to a decrease in costs relating to Global DMD, Gene Therapy, Inflammation and Ferroptosis platform, and Sephience related development.

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

For the three and nine months ended September 30, 2026, compared to the three and nine months ended September 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 nine months ended September 30, 2026, compared to the three and nine months ended September 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 nine months ended September 30, 2026 compared to the three and nine months ended September 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 September 30, 2026, compared to the three months ended September 30, 2025, the change in facilities and other indirect costs was relatively flat. For the nine months ended September 30, 2026, compared to the nine months ended September 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 may 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, 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 nine months ended September 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 September 30, 2026 compared to the three months ended September 30, 2025

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

(in thousands)	Three Months Ended September 30, 2026	2025	Change 2026 vs. 2025
Net product revenue	$ 275,894	$ 130,956	$ 144,938
Collaboration and license revenue	229	9,258	(9,029)
Royalty revenue	81,684	70,793	10,891
Cost of product, collaboration and license sales, excluding amortization of acquired intangible assets	23,664	15,781	7,883
Amortization of acquired intangible assets	12,595	7,534	5,061
Research and development expense	105,353	100,158	5,195
Selling, general and administrative expense	80,081	84,046	(3,965)
Tangible asset impairment and losses on transactions, net	—	472	(472)
Acquired in-process research and development	111,000	—	111,000
Interest expense, net	(45,866)	(32,592)	(13,274)
Other (expense) income, net	(1,073)	6,502	(7,575)
Loss on extinguishment of debt, net	(19,263)	—	(19,263)
Income tax (expense) benefit	(10,605)	38,970	(49,575)

Net product revenue. Net product revenue was $275.9 million for the three months ended September 30, 2026, an increase of $144.9 million, or over 100%, from $131.0 million for the three months ended September 30, 2025. The increase in net product revenue was primarily due to an increase in net product sales of $162.3 million for Sephience, which is in the first year of its launch, partially offset by decreases in net product sales of $10.1 million for Emflaza and $2.2 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 $0.2 million for the three months ended September 30, 2026, a decrease of $9.0 million, or 98%, from $9.3 million for the three months ended September 30, 2025. For the three months ended September 30, 2026, we recognized $0.2 million in revenues related to work performed for Novartis. For the three months ended September 30, 2025, we recognized $9.3 million related to license revenue from the Novartis Agreement for performance obligations completed during the period.

Royalty revenue. Royalty revenue was $81.7 million for the three months ended September 30, 2026, an increase of $10.9 million, or 15%, from $70.8 million for the three months ended September 30, 2025. The increase in royalty revenue was due to higher Evrysdi sales in the three months ended September 30, 2026 as compared to the three months ended September 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 $23.7 million for the three months ended September 30, 2026, an increase of $7.9 million, or 50%, from $15.8 million for the three months ended September 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 $12.6 million for the three months ended September 30, 2026, an increase of $5.1 million, or 67%, from $7.5 million for the three months ended September 30, 2025. The increase to the intangible assets balance was primarily related to Upstaza/Kebilidi 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 $105.4 million for the three months ended September 30, 2026, an increase of $5.2 million, or 5%, from $100.2 million for the three months ended September 30, 2025. The increase in research and development expenses primarily related to increases in development program spend as we continued to invest our resources on our differentiated, high potential research and development programs.

Selling, general and administrative expense. Selling, general and administrative expense was $80.1 million for the three months ended September 30, 2026, a decrease of $4.0 million, or 5%, from $84.0 million for the three months ended September 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 was $0.0 for the three months ended September 30, 2026, a decrease of $0.5 million, or 100%, from $0.5 million for the three months ended September 30, 2025. The decrease in tangible asset impairment and losses on transactions, net primarily related to fixed asset impairments of $0.4 million, and a $0.1 million loss related to sales of fixed assets during the three months ended September 30, 2025. There were no tangible asset impairment and losses during the three months ended September 30, 2026.

Acquired in-process research and development. Acquired in-process research and development was $111.0 million for the three months ended September 30, 2026, an increase of $111.0 million, or 100%, from $0.0 million for the three months ended September 30, 2025. The increase in acquired in-process research and development is due to the Sangamo Asset Acquisition. On August 25, 2026, we entered into an Asset Purchase Agreement with Sangamo, or the Sangamo Purchase Agreement, to purchase the Fabry asset from Sangamo. Upon the closing of the Sangamo Asset Acquisition on September 17, 2026, we paid upfront cash consideration of $111.0 million and may become obligated to make up to an additional $100.0 million in contingent milestone payments upon the achievement of specified regulatory approval milestones pursuant to the Sangamo Purchase Agreement.

Interest expense, net. Interest expense, net was $45.9 million for the three months ended September 30, 2026, an increase of $13.3 million, or 41%, from $32.6 million for the three months ended September 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 and the interest expense related to the 2031 Convertible Notes, offset by an increase of investment income on our marketable securities-available for sale.

Other (expense) income, net. Other expense, net was $1.1 million for the three months ended September 30, 2026, a change of $7.6 million, or over 100%, from other income, net of $6.5 million for the three months ended September 30, 2025. Other expense, net primarily relates to net realized and unrealized foreign exchange losses of $1.2 million for the three months ended September 30, 2026. Other income, net primarily related to net unrealized gains of $8.8 million on our ClearPoint Neuro, Inc. equity investments, partially offset by net realized and unrealized foreign exchange losses of $2.4 million for the three months ended September 30, 2025.

Loss on extinguishment of debt, net. Loss on extinguishment of debt, net was $19.3 million for the three months ended September 30, 2026, an increase of $19.3 million, or 100%, from $0.0 million for the three months ended September 30, 2025. The increase in loss on extinguishment of debt, net is due to the conversion of the $55.5 million remaining aggregate principal of the 2026 Convertible Notes. We accounted for the conversions under extinguishment accounting in accordance with ASC 470-50, whereby the additional cash required to be paid in excess of the principal, an amount of $19.3 million, was recorded as a loss on extinguishment of debt within the consolidated statements of operations. The $19.3 million loss is being presented net of a gain on the change in the fair value of the conversion option of $7.7 million.

Income tax (expense) benefit. Income tax expense was $10.6 million for the three months ended September 30, 2026, a change of $49.6 million, or over 100%, compared to income tax benefit of $39.0 million for the three months ended September 30, 2025. The change in income tax (expense) benefit was driven by the recognition of revenue associated with the A&R Royalty Purchase Agreement.

Nine months ended September 30, 2026 compared to the nine months ended September 30, 2025

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

(in thousands)	Nine Months Ended September 30, 2026	2025	Change 2026 vs. 2025
Net product revenue	$ 740,286	$ 402,711	$ 337,575
Collaboration and license revenue	50,967	998,430	(947,463)
Royalty revenue	199,624	164,837	34,787
Cost of product, collaboration and license sales, excluding amortization of acquired intangible assets	71,613	40,063	31,550
Amortization of acquired intangible assets	36,017	15,393	20,624
Research and development expense	305,376	322,121	(16,745)
Selling, general and administrative expense	246,894	250,269	(3,375)
Change in the fair value of contingent consideration	—	(800)	800
Tangible asset impairment and losses on transactions, net	927	648	279
Acquired in-process research and development	111,000	—	111,000
Interest expense, net	(143,377)	(97,042)	(46,335)
Other expense, net	(2,415)	(5,540)	3,125
Loss on extinguishment of debt, net	(19,263)	—	(19,263)
Income tax expense	(25,001)	(18,093)	(6,908)

Net product revenue. Net product revenue was $740.3 million for the nine months ended September 30, 2026, an increase of $337.6 million, or 84%, from $402.7 million for the nine months ended September 30, 2025. The increase in net product revenue was primarily due to an increase in net product sales of $438.2 million for Sephience, which is in the first year of its launch, partially offset by decreases in net product sales of $48.1 million for Emflaza and $46.6 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 $51.0 million for the nine months ended September 30, 2026, a decrease of $947.5 million, or 95%, from $998.4 million for the nine months ended September 30, 2025. For the nine months ended September 30, 2026, we recognized $51.0 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 nine months ended September 30, 2025, we recognized $1.0 billion 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 $199.6 million for the nine months ended September 30, 2026, an increase of $34.8 million, or 21%, from $164.8 million for the nine months ended September 30, 2025. The increase in royalty revenue was due to higher Evrysdi sales in the nine months ended September 30, 2026 as compared to the nine months ended September 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 $71.6 million for the nine months ended September 30, 2026, an increase of $31.6 million, or 79%, from $40.1 million for the nine months ended September 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 $36.0 million for the nine months ended September 30, 2026 an increase of $20.6 million, or over 100%, from $15.4 million for the nine months ended September 30, 2025. The increase to the intangible assets balance was primarily related to Upstaza/Kebilidi 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 $305.4 million for the nine months ended September 30, 2026, a decrease of $16.7 million, or 5%, from $322.1 million for the nine months ended September 30, 2025. The decrease in research and development expenses related to decreases in development program spend relating to Global DMD, Gene Therapy, Inflammation and Ferroptosis platform, and Sephience related development 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 $246.9 million for the nine months ended September 30, 2026, a decrease of $3.4 million, or 1%, from $250.3 million for the nine months ended September 30, 2025. The decrease is primarily due to a decrease in selling expenses related to prelaunch activities for Sephience.

Change in the fair value of contingent consideration. There was no change in the fair value of contingent consideration for the nine months ended September 30, 2026, reflecting a change of $0.8 million, or 100%, from a gain of $0.8 million for the nine months ended September 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 nine months ended September 30, 2026, an increase of $0.3 million, or 43%, from $0.6 million for the nine months ended September 30, 2025. The increase was primarily driven by a $0.8 million loss related to inventory impairments during the nine months ended September 30, 2026. During the nine months ended September 30, 2025, we recorded fixed asset impairments of $0.5 million, as well as a $0.1 million loss related to sales of fixed assets.

Acquired in-process research and development. Acquired in-process research and development was $111.0 million for the nine months ended September 30, 2026, an increase of $111.0 million, or 100%, from $0.0 million for the nine months ended September 30, 2025. The increase in acquired in-process research and development is due to the Sangamo Asset Acquisition. On August 25, 2026, we entered into the Sangamo Purchase Agreement. Upon the closing of the Sangamo Asset Acquisition on September 17, 2026, we paid upfront cash consideration of $111.0 million and may become obligated to make up to an additional $100.0 million in contingent milestone payments upon the achievement of specified regulatory approval milestones pursuant to the Sangamo Purchase Agreement.

Interest expense, net. Interest expense, net was $143.4 million for the nine months ended September 30, 2026, an increase of $46.3 million, or 48%, from $97.0 million for the nine months ended September 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 and the interest expense related to the 2031 Convertible Notes.

Other expense, net. Other expense, net was $2.4 million for the nine months ended September 30, 2026, a decrease of $3.1 million, or 56%, from other expense, net of $5.5 million for the nine months ended September 30, 2025. Other expense, net, for the nine months ended September 30, 2026, was primarily related to an inducement expense of $3.4 million related to the repurchase of a portion of the 2026 Convertible Notes partially offset by net realized and unrealized gains from foreign currency of $0.6 million and other items of $0.4 million. Other expense, net, for the nine months ended September 30, 2025, was primarily related to net realized and unrealized losses from foreign currency of $17.4 million, partially offset by unrealized gains on our ClearPoint Neuro, Inc. equity investments of $5.7 million and $6.0 million related to other income items.

Loss on extinguishment of debt, net. Loss on extinguishment of debt, net was $19.3 million for the nine months ended September 30, 2026, an increase of $19.3 million, or 100%, from $0.0 million for the nine months ended September 30, 2025. The increase in loss on extinguishment of debt, net is due to the conversion of the $55.5 million remaining aggregate principal of the 2026 Convertible Notes. We accounted for the conversions under extinguishment accounting in accordance with ASC 470-50, whereby the additional cash required to be paid in excess of the principal, an amount of $19.3 million, was recorded as a loss on extinguishment of debt within the consolidated statements of operations. The $19.3 million loss is being presented net of a gain on the change in the fair value of the conversion option of $7.7 million.

Income tax expense. Income tax expense was $25.0 million for the nine months ended September 30, 2026, an increase of $6.9 million, or 38%, compared to income tax expense of $18.1 million for the nine months ended September 30, 2025. The increase in income tax expense was driven by the recognition of revenue associated with the A&R Royalty Purchase Agreement.

Liquidity and capital resources

Sources of liquidity

While we have generated net income in the nine months ended September 30, 2026, 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, and its orphan drug exclusivity related to the treatment of DMD in patients two years of age to less than five expired in June 2026. 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.

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—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 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. 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 outstanding 2026 Convertible Notes matured on September 15, 2026, unless earlier repurchased or converted, as further described below.

In April 2026, we settled a holder's conversion with a principal amount of $10.0 million in exchange for $12.9 million in cash and 3,506 shares of our common stock. We accounted for the transaction as an induced conversion under ASC 470-20 and recorded a $10.0 million reduction to the carrying value of the 2026 Convertible Notes with the excess of $2.9 million 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 was $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.

On July 20, 2026, we provided an irrevocable final settlement notice to U.S. Bank National Association, as trustee of the 2026 Convertible Notes, stating that we elected a combination settlement with a specified dollar amount of $1,460, together with cash, if applicable, in lieu of delivering any fractional share of our common stock pursuant to the indenture governing the 2026 Convertible Notes, or the 2026 Convertible Notes Indenture for the final conversion of any 2026 Convertible Notes surrendered prior to close of business on the business day immediately preceding the maturity date of the 2026 Convertible Notes.

In September 2026, $55.5 million aggregate principal of the 2026 Convertible Notes were converted, which we settled by paying cash of $74.8 million pursuant to the 2026 Convertible Notes Indenture on September 15, 2026. Additionally, we paid accrued interest of $0.4 million pursuant to the 2026 Convertible Notes Indenture on September 15, 2026. We evaluated the transaction noting that as the settlement notice was irrevocable and we elected to settle all or a portion of the conversion value in excess of the principal in cash, we concluded that the conversion option no longer met the own-equity exception in ASC 815-10-15-74(a) and therefore should be bifurcated with the conversion option recorded at fair value. We accounted for the conversions under extinguishment accounting in accordance with ASC 470-50, whereby the additional cash required to be paid in excess of the principal, an amount of $19.3 million, was recorded as a loss on extinguishment of debt within our consolidated statements of operations. The $19.3 million loss is being presented net of a gain on the change in the fair value of the conversion option of $7.7 million.

Cash flows

As of September 30, 2026, we had cash, cash equivalents and marketable securities of $2.13 billion.

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

(in thousands)	Nine Months Ended September 30, 2026	2025
Cash provided by (used in):		
Operating activities	44,760	745,469
Investing activities	(658,704)	(895,992)
Financing activities	145,045	28,590

Net cash provided by operating activities was $44.8 million for the nine months ended September 30, 2026, and $745.5 million for the nine months ended September 30, 2025. The net cash provided by operating activities for the nine months ended September 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 offset by cash used in operating activities primarily related to supporting clinical development and commercial activities. The net cash provided by operating activities for the nine months ended September 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 $658.7 million for the nine months ended September 30, 2026, compared to $896.0 million for the nine months ended September 30, 2025. Cash used in investing activities for the nine months ended September 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 $145.0 million for the nine months ended September 30, 2026, compared to $28.6 million for the nine months ended September 30, 2025. Cash provided by financing activities for the nine months ended September 30, 2026, was primarily attributable to cash received from the exercise of options, proceeds from employee stock purchase plan, and proceeds from the issuance of the 2031 Convertible Notes, partially offset by the debt issuance costs related to the 2031 Convertible Notes, repurchase and final settlement, and conversion of the 2026 Convertible Notes, and payment on finance lease principal. Cash provided by financing activities for the nine months ended September 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 and payments on our finance lease principal.

Funding requirements

We anticipate that we will continue to incur significant expenses in connection with our commercialization efforts in the United States, the EEA, Latin America, 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, in the third quarter of 2026 we initiated the PROVE-FA open label study using matched natural history control.

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 plans for ST-920, including with respect to the expected timing of regulatory submissions and responses, meetings with regulatory agencies, and other matters, and the potential achievement of regulatory milestones and related contingent payments that we may be obligated to make under the Sangamo Purchase Agreement;
- 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, inflammation and ferroptosis, and ST-920 programs and Sephience, Translarna and Upstaza/Kebilidi in other territories;
- 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, inflammation and ferroptosis, and ST-920 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 EAPs 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, Censa and ST-920 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.

In the three months 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 and intangible assets on our consolidated balance sheet as of September 30, 2026. In the three months ended September 30, 2026, aggregate Sephience global net sales in the prior four consecutive quarters exceeded $500.0 million, which, pursuant to the Censa Merger Agreement, triggered a $50.0 million net sales milestone payment to the former Censa securityholders. This milestone was recorded in accounts payable and accrued expenses and intangible assets on our consolidated balance sheet as of September 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 September 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 September 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 September 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 September 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 September 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
Jerome Zeldis (Director)	Adoption (August 14, 2026)	Rule 10b5-1 trading arrangement	Sale	Until January 3, 2028, or until such earlier date upon which all transactions are completed	Up to 26,000 shares
Michael Schmertzler (Director)	Adoption (August 26, 2026)	Rule 10b5-1 trading arrangement	Sale	Until January 4,2027, or until such earlier date upon which all transactions are completed	Up to 24,000 shares
Hege Sollie-Zetlmayer (Director)	Adoption (September 16, 2026)	Rule 10b5-1 trading arrangement	Sale	Until June 16, 2027, or until such earlier date upon which all transactions are completed	Up to 177,043 shares

Item 6. Exhibits.

Exhibit Number	Description of Exhibit
2.1*	Asset Purchase Agreement, dated August 25, 2026, by and between PTC Therapeutics, Inc. and Sangamo Therapeutics, Inc.
31.1*	Certification of Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2*	Certification of Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1*	Certification of Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
32.2*	Certification of Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.INS*	Inline XBRL Instance Document
101.SCH*	Inline XBRL Taxonomy Extension Schema Document
101.CAL*	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.LAB*	Inline XBRL Taxonomy Extension Label Linkbase Database
101.PRE*	Inline XBRL Taxonomy Extension Presentation Linkbase Document
101.DEF*	Inline XBRL Taxonomy Extension Definition Linkbase Document
104	The cover page from this Quarterly Report on Form 10-Q, formatted in Inline XBRL

* Submitted electronically herewith.

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: October 29, 2026

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

Exhibit 2.1

EXECUTION VERSION

ASSET PURCHASE AGREEMENT

by and between

PTC THERAPEUTICS, INC.

and

SANGAMO THERAPEUTICS, INC.

dated as of

August 25, 2026

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ASSET PURCHASE AGREEMENT

This Asset Purchase Agreement (this "**Agreement**"), dated as of August 25, 2026, is entered into by and between Sangamo Therapeutics, Inc., a Delaware corporation ("**Seller**"), and PTC Therapeutics, Inc., a Delaware corporation ("**Buyer**"). Seller and Buyer are each sometimes referred to herein as a "**Party**" and collectively as the "**Parties**."

RECITALS

WHEREAS, on June 23, 2026 (the "**Petition Date**"), Seller as debtor and debtor in possession (the "**Debtor**") sought relief under Chapter 11 of Title 11 of the United States Code (as amended, the "**Bankruptcy Code**") by filing a case (the "**Chapter 11 Case**") in the United States Bankruptcy Court for the District of Delaware (the "**Bankruptcy Court**"), which is being administered under Case No. 26-10989 (CTG);

WHEREAS, Seller has been developing and investigating in clinical trials a gene therapy product for the treatment of Fabry disease (the "**Business**");

WHEREAS, Seller wishes to sell and assign to Buyer, and Buyer wishes to purchase and assume from Seller, substantially all the assets of the Business, subject to the terms and conditions set forth herein; and

WHEREAS, subject to the terms and conditions set forth in this Agreement and the entry of the Sale Order, the Parties desire to enter into this Agreement, pursuant to which Seller shall sell, assign, transfer, and convey to Buyer, and Buyer shall purchase and acquire from Seller, all of Seller's right, title and interest in and to the Purchased Assets, and Buyer shall assume all of the Assumed Liabilities, and the Parties intend to effectuate the transactions contemplated by this Agreement, upon the terms and conditions hereinafter set forth in a sale authorized by the Bankruptcy Court pursuant to, *inter alia*, Sections 105, 363 and 365 of the Bankruptcy Code, all on the terms and subject to the conditions set forth in this Agreement and subject to entry of the Sale Order.

NOW, THEREFORE, in consideration of the mutual covenants and agreements hereinafter set forth and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the Parties agree as follows:

ARTICLE I
DEFINITIONS

Section 1.01 Definitions. The following terms have the meanings specified or referred to in this ARTICLE I:

"**Accelerated Milestone Event**" means the first accelerated approval by the FDA of a US BLA for a Company Product for the treatment of Fabry disease based on the STAAR Studies.

"**Accelerated Milestone Payment**" has the meaning set forth in Section 2.09(a).

"**Action**" means any action, cause of action, claim, demand, litigation, suit, summons, subpoena, inquiry, investigation, audit, hearing, originating application to a tribunal, arbitration or other legal proceeding of any nature, civil, criminal, regulatory, administrative or otherwise, whether in equity or at law, in contract, in tort or otherwise, excluding the Chapter 11 Case.

"**Affiliate**" means, with respect to any Person, any other Person directly or indirectly controlling, controlled by or under common control with such Person. For the purpose of this definition, "control" (including, with its correlative meanings, "controlled by" and "under common control with") shall mean the possession, directly or indirectly, of the power to direct or cause the direction of management or policies of a Person, whether through the ownership of voting securities or partnership or other ownership interests, by Contract or otherwise.

"**Agreement**" has the meaning set forth in the preamble.

"**Ancillary Documents**" means the Bill(s) of Sale, the Assignment and Assumption Agreement(s), Intellectual Property Assignment(s), the Transition Services Agreement and any other agreements, instruments and documents required to be delivered at the Closing.

"**Apportioned Taxes**" has the meaning set forth in Section 2.10(c).

"**Assigned Contracts**" has the meaning set forth in Section 2.01(c).

"**Assignment and Assumption Agreement**" has the meaning set forth in Section 3.02(a)(ii).

"**Assumed Liabilities**" has the meaning set forth in Section 2.03.

"**Auction**" means the auction contemplated by the Bidding Procedures Order.

"**Avoidance Action Claims**" means, collectively, any and all avoidance, recovery, subordination, or other claims, actions, rights, or remedies that may be brought by or on behalf of Seller or its estate or other authorized parties in interest under the Bankruptcy Code or applicable non-bankruptcy Law, including remedies, claims or causes of action arising under or pursuant to chapter 5 of the Bankruptcy Code or any other similar provision of applicable Law.

"**Backup Bidder**" means the party whose bid is determined to be the next highest bid after the bid of the Successful Bidder at the Auction.

"**Bankruptcy Code**" has the meaning set forth in the recitals.

"**Bankruptcy Court**" has the meaning set forth in the recitals.

"**Bankruptcy Period**" has the meaning set forth in Section 10.10(b).

"**Bidding Procedures**" means the bidding procedures approved by the Bankruptcy Court pursuant to the Bidding Procedures Order, in the form of Exhibit B attached hereto.

"**Bidding Procedures Order**" means an order of the Bankruptcy Court approving, among other things, the Bidding Procedures for conducting a sale and auction of the Purchased Assets, and authorizing Seller's performance of its obligations under this Agreement, in the form of Exhibit A attached hereto.

"**Bill of Sale**" has the meaning set forth in Section 3.02(a)(i).

"**Books and Records**" has the meaning set forth in Section 2.01(l).

"**Business**" has the meaning set forth in the recitals.

"**Business Data**" has the meaning set forth in Section 4.12(c).

"**Business Day**" means a day, other than Saturday, Sunday or other day on which commercial banks in Tokyo, Japan or Wilmington, Delaware are authorized or required by applicable Law to close.

"**Buyer**" has the meaning set forth in the preamble; *provided* that PTC Therapeutics, Inc. may designate an affiliate as the "Buyer" under this Agreement by providing written notice to Seller by no later than the date that is two Business Days prior to the Closing Date; *provided further* that in the event of and notwithstanding any such designation, Buyer shall remain liable for all of its obligations under this Agreement.

"**Buyer's Closing Certificate**" has the meaning set forth in Section 8.03(c).

"**Buyer Material Adverse Effect**" means any Effect that, individually or in the aggregate, has had, or would reasonably be expected to have, a material adverse effect on Buyer's ability to consummate the transactions contemplated by this Agreement on or before the End Date or to timely perform any of its obligations under this Agreement.

"**Chapter 11 Case**" has the meaning set forth in the recitals.

"**Closing**" has the meaning set forth in Section 3.01.

"**Closing Consideration**" has the meaning set forth in Section 2.08.

"**Closing Date**" has the meaning set forth in Section 3.01.

"**COBRA**" means, collectively, the Consolidated Omnibus Budget Reconciliation Act of 1985, as amended, and any similar state or local law.

"**Code**" means the Internal Revenue Code of 1986, as amended.

"**Company Product**" means isaralgagene civaparvovec, code named by Seller as ST-920, a gene therapy product consisting of an engineered, liver-targeted adeno-associated viral vector (rAAV 2/6) encoding a copy of the human *GLA* gene, as described in U.S. IND #18733, and any other gene therapy candidate developed by Seller consisting of an AAV vector encoding a version (human or otherwise) of the *GLA* gene.

"**Competing Transaction**" has the meaning set forth in Section 6.01.

"**Consulting Agreement**" means consulting, independent contractor or other non-employee service agreement between Seller or any of its Subsidiaries, on the one hand, and any Specified Individual, on the other hand.

"**Contracts**" means, with respect to any Person, any legally binding contract, subcontract, agreement, lease, sublease, license, sublicense, commitment, sale or purchase order, indenture, note, bond, loan, mortgage, deed of trust, insurance policy, instrument, or other arrangement, commitment, undertaking or understanding, whether written or oral, to which such Person is a party or by which such Person or such Person's properties or assets are bound, and which is primarily related to the Business.

"**Copyrights**" means any writings and other works (including software, databases, literary, pictorial and graphic works), whether copyrightable or not, published or unpublished, in any jurisdiction (domestic and foreign), and any and all copyright rights, whether registered or not, moral rights, database rights and similar rights therein, and registrations or applications for registration of copyrights in any jurisdiction (domestic and foreign), and any renewals or extensions thereof.

"**Cure Costs**" means, with respect to any Assigned Contract, the Liabilities that must be paid or otherwise satisfied to cure all monetary defaults under such Assigned Contracts to the extent required under Section 365(b) of the Bankruptcy Code in connection with the assignment and assumption of such Assigned Contract.

"**Debtor**" has the meaning set forth in the recitals.

"**Designated Contract**" has the meaning set forth in Section 2.05(i).

"**DIP Order**" means an order from the Bankruptcy Court approving, among other things, Seller's entry into a debtor-in-possession financing facility on an interim or final basis.

"**Disclosure Schedules**" means the Stalking Horse APA Disclosure Schedules provided by Seller to Buyer prior to the execution of this Agreement by Buyer, as may be supplemented or modified from time to time in accordance with the terms of this Agreement, and thereafter any such supplements or modifications shall be part of the Disclosure Schedules; *provided* that Seller or Buyer may, with the written consent of the other in its sole discretion, supplement or modify the Disclosure Schedules hereto at any time prior to Closing; *provided further* that, notwithstanding anything to the contrary set forth in this Agreement, the consent described in the immediately preceding proviso shall not apply to any supplements or modifications of the Disclosure Schedules expressly contemplated by the provisions of this Agreement, including without limitation, Section 2.05 hereof.

"**Dollars**" or "**$**" means the lawful currency of the United States.

"**e-mail**" has the meaning set forth in Section 10.01.

"**Effect**" means any event, state of facts, circumstance, change, occurrence, development, condition, result or effect.

"**Employee**" means any employee of Seller or any of its Subsidiaries whose services for Seller and its Subsidiaries are primarily in connection with the Business.

"**Employee Plan**" means with respect to current and former Employees, any (i) "employee benefit plan" as defined in Section 3(3) of ERISA, (ii) compensation, employment, consulting, independent contractor, severance, termination protection, change in control, indemnification, loan, transaction bonus, retention or similar plan, agreement, arrangement, program or policy or (iii) other plan, agreement, arrangement, program or policy providing for compensation, bonuses, profit-sharing, equity or equity-based compensation or other forms of incentive or deferred compensation, vacation or time-off benefits, insurance (including any self-insured arrangement), medical, dental, vision, hospitalization, prescription or fringe benefits, life insurance, relocation or expatriate benefits, perquisites, disability or sick leave benefits, employee assistance program, supplemental unemployment benefits or post-employment or retirement benefits (including compensation, pension, employee stock ownership, stock purchase, stock option, health, medical or insurance benefits, and any plan maintained under Section 401(k) of the Code), in each case (A) whether or not written, and (B) that is sponsored, maintained, administered, contributed to, required to be contributed to or entered into by Seller or any of its ERISA Affiliates or for which Seller or any of its Subsidiaries has or would be reasonably expected to have any current or future liability. The term "Employee Plan" includes any PEO Plan, programs, policies and arrangements sponsored or maintained by a PEO in which current or former employees, officers or directors of Seller or any of its ERISA Affiliates are eligible to participate in connection with their service to Seller or any of its ERISA Affiliates.

"**Employment Agreement**" means each management, employment, severance, retention, relocation, repatriation, expatriation, change in control or similar agreement or offer letter between Seller or any of its Subsidiaries, on the one hand, and any Specified Individual, on the other hand.

"**End Date**" has the meaning set forth in Section 9.01(b)(ii).

"**ERISA**" means the Employee Retirement Income Security Act of 1974, as amended.

"**ERISA Affiliate**" means any person which is (or at any relevant time was or will be) a member of a "controlled group of corporations" with, under "common control" with, or a member of an "affiliated service group" with Seller as such terms are defined in Sections 414(b), (c), (m) or (o) of the Code.

"**Essential Counterparties**" has the meaning set forth in Section 2.05(i).

"**Exchange Act**" means the Securities Exchange Act of 1934, as amended.

"**Excluded Assets**" has the meaning set forth in Section 2.02.

"**Excluded Business**" means the business conducted by Seller and its Subsidiaries using the assets of Seller and its Subsidiaries unrelated to the Business.

"**Excluded Contracts**" has the meaning set forth in Section 2.02(a).

"**Excluded Liabilities**" has the meaning set forth in Section 2.04.

"**FCPA**" has the meaning set forth in Section 4.10(b).

"**FDA**" means the United States Food and Drug Administration.

"**Final Order**" means an order of the Bankruptcy Court (or any other court of competent jurisdiction) entered by the clerk of the Bankruptcy Court (or such other court) on the docket in the Chapter 11 Case (or the docket of such other court), (a) as to which no appeal, notice of appeal, motion to amend or make additional findings of fact, motion to alter or amend judgment, motion for rehearing or motion for new trial, request for stay, motion or petition for reconsideration, application or request for review, or other similar motion, application, notice or request (collectively, a "**Challenge**") has been timely filed, or, if a Challenge has been timely filed, it has been disposed of in a manner that upholds and affirms the subject order in all respects without the possibility for further Challenge thereon; (b) as to which the time for instituting or filing a Challenge shall have expired; and (c) as to which no stay is in effect.

"**Full Milestone Event**" means the first full approval by the FDA of a US BLA for a Company Product for the treatment of Fabry disease based on the STAAR Studies, either as part of the original BLA submission or if an accelerated approval under section 506(c) of the FD&C Act is converted into a full approval.

"**Full Milestone Payment**" has the meaning set forth in Section 2.09(a).

"**Fundamental Representations**" means those representations and warranties of Seller set forth in Section 4.01 (*Organization and Qualification of Seller*), Section 4.02 (*Authority of Seller*), Section 4.03(a) (*No Conflicts with Organizational Documents*), Section 4.06 (*Intellectual Property*) and Section 4.17 (*Finders' Fees*).

"**GAAP**" means generally accepted accounting principles in the United States as in effect on the date of this Agreement.

"**Good Clinical Practices**" or "**GCP**" means the then-current applicable requirements and standards for designing, conducting, recording, and reporting clinical trials (including all applicable requirements relating to protection of human subjects), including as set forth in (i) the FD&C Act and applicable regulations promulgated thereunder (including, for example, 21 C.F.R. Parts 50, 54, 56, and 312), (ii) applicable guidelines from the International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use, including ICH E6(R3) and ICH E3, (iii) Regulation (EU) No 536/2014, and (iv) comparable standards of good clinical practice (including all applicable requirements relating to protection of human subjects) as are required by Governmental Authorities in any other country or jurisdiction.

"**Good Laboratory Practices**" or "**GLP**" means the then-current standards for good laboratory practices for pharmaceuticals, including as set forth in (i) the FDA's GLP regulations at 21 C.F.R. Part 58, (ii) Directive 2004/10/EC, (iii) the GLP principles of the Organisation for Economic Co-Operation and Development (OECD), and (iv) such standards of good laboratory

practice as are required by other organizations and Governmental Authorities in countries in which any nonclinical study is conducted, to the extent such standards are not less stringent than the FDA's GLP regulations.

"**Good Manufacturing Practices**" or "**GMP**" means the then-current good practices and standards for the manufacture and testing of pharmaceutical products and their components, including as set forth in (i) the FD&C Act, (ii) 21 C.F.R. Parts 210, 211, 600, and 610, and FDA guidance issued thereunder, and (iii) comparable applicable Law related to the manufacture and testing of pharmaceutical materials in jurisdictions outside the United States. "Good Manufacturing Practices," or "GMP" also means the quality guidelines promulgated by the ICH, including the ICH Q7A, titled "Q7A Good Manufacturing Practice Guidance for Active Pharmaceutical Ingredients" and the policies promulgated thereunder.

"**Governmental Authority**" means any transnational, domestic or foreign federal, state, provincial, local or other governmental, regulatory or administrative authority, department, court, mediator or other tribunal or arbitral body (public or private), agency, board, commission or official, including any political subdivision thereof, or any other governmental or quasi-governmental (including self-regulatory) authority or instrumentality.

"**Governmental Order**" means any order, writ, judgment, injunction, decree, stipulation, determination, award, verdict, or other decision issued, promulgated or entered by or with any Governmental Authority.

"**Health Care Laws**" means all Laws applicable to the research, nonclinical and clinical testing, development, manufacturing, ownership, operation, storage, import, export, distribution, marketing, pricing, sale, promotion, document retention, warehousing, packaging, labeling, and handling of any compound or Company Product, including the Federal Food, Drug, and Cosmetic Act ("**FD&C Act**"), the Public Health Service Act, and the implementing regulations, and any comparable state and foreign Laws, including the Laws, regulations, and guidance GCP, GLP and GMP, including guidance from the International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use.

"HSR Act" means the Hart-Scott-Rodino Antitrust Improvements Act of 1976 and the rules and regulations promulgated thereunder, and any successor to such statute, rules or regulations.

"**Independent Contractor**" means any natural person who is an independent contractor, advisor, director, consultant or other service provider (other than an employee) who provides personal services to Seller or any of its Subsidiaries in connection with the Business.

"**Insurance Policies**" has the meaning set forth in Section 4.16.

"**Intellectual Property Agreements**" means all agreements relating to Intellectual Property Rights owned by a Third Party and licensed or sublicensed, whether exclusively or non-exclusively, to Seller or any of its Subsidiaries primarily relating to the Business, including consent to use agreements, settlements, coexistence agreements, covenants not to sue, waivers, releases, grants of immunity, rights of reference, permissions and other Contracts, whether written or oral,

relating to any such Intellectual Property Rights to which Seller is a party, beneficiary or otherwise bound.

"**Intellectual Property Assets**" means all Intellectual Property Rights that are owned by Seller and are primarily relating to the Company Product or primarily held for use in the conduct of the Business as currently conducted or proposed to be conducted, together with all (i) royalties, fees, income, payments, and other proceeds now or hereafter due or payable to Seller with respect to such Intellectual Property Rights; and (ii) claims and causes of action with respect to such Intellectual Property Rights, whether accruing before, on, or after the date hereof, including all rights to and claims for (including the right to recover and retain) damages, restitution, attorneys' fees, and injunctive and other legal or equitable relief for past, present, or future infringement, misappropriation, or other violation thereof; provided that Intellectual Property Assets shall not include any Intellectual Property Rights set forth in Section 2.02(d) of the Disclosure Schedules.

"**Intellectual Property Assignment**" has the meaning set forth in Section 3.02(a)(iii).

"**Intellectual Property Registrations**" means all Intellectual Property Assets that are subject to any issuance, registration, recordation or application by or with any Governmental Authority or authorized private registrar in any jurisdiction, including issued Patents, registered Trademarks, domain names and Copyrights, and pending applications for any of the foregoing.

"**Intellectual Property Rights**" means any and all (i) Trademarks, (ii) Patents, (iii) Trade Secrets, information, knowledge, experience, skills, drawings, blueprints, utility models, technology, inventions, discoveries and improvements, (iv) Copyrights, (v) moral rights, data and database rights, design rights, industrial property rights, publicity rights and privacy rights, (vi) all forms and types of computer software (including source code, object code, firmware, development tools, APIs, files, records and data and all documentation related to any of the foregoing), (vii) any other intellectual property or proprietary rights and (viii) all rights under or relating to any of the foregoing granted under any Contract.

"**Inventory**" has the meaning set forth in Section 2.01(d).

"**IRS**" means the U.S. Internal Revenue Service.

"**IND**" means any Investigational New Drug application submitted to the FDA pursuant to 21 C.F.R. Part 312 or any similar application or submission submitted to a Regulatory Authority outside the United States for the purpose of obtaining permission to conduct clinical trials, including a clinical trial application.

"**IT Assets**" means computers, mobile devices, computer software, firmware, middleware, servers, workstations, routers, hubs, switches, storage devices, data communications lines and all other information technology, network, security and data assets, equipment, systems, cloud infrastructure, services, and all associated documentation and materials owned by Seller or any of its Subsidiaries or licensed, subscribed to, operated, held for use, or leased by Seller or any of its Subsidiaries, in each case, that are primarily related to the Business.

"**Key Suppliers**" has the meaning set forth in Section 4.15(a).

"**Knowledge of Seller", "Seller's Knowledge**" or any other similar knowledge qualification, means the actual knowledge of the individuals listed in Section 1.01(a) of the Disclosure Schedules, in each case after making reasonable inquiry, together with the knowledge such individuals would reasonably be expected to have had if they had made such due inquiry.

"**Law**" means any federal, state, local or foreign law, treatise, decree, statute, code, constitution, principle of common law, ordinance, rule, regulation, guidance, guideline, judgment, decree, injunction, ruling, order, procedure, decision or other requirement of any Governmental Authority, including GLP, GMP, GCP, Health Care Laws and Privacy Laws.

"**Liabilities**" means liabilities, obligations, damages, costs, expenses or commitments of any nature whatsoever, asserted or unasserted, direct or indirect, known or unknown, absolute or contingent, accrued or unaccrued, matured or unmatured or otherwise.

"**Lien**" shall have the meaning set forth in Section 101(37) of the Bankruptcy Code and shall include any pledge, option, charge, lien, license, debentures, trust deeds, hypothecation, easement, security interest, right-of-way, encroachment, mortgage, deed of trust, defect of title, restriction on transferability, restriction on use or other encumbrance, in each case whether imposed by agreement, law, equity or otherwise.

"**Marketing Application**" means an application for the approval, license, or authorization to market a pharmaceutical compound or product in any country or group of countries, as defined in the Laws and filed with the Governmental Authority of a given country or group of countries, and all additions, amendments, supplements, extensions and modifications thereto, including a US BLA.

"**Milestone Events**" means the Full Milestone Event and the Accelerated Milestone Event.

"**Milestone Payments**" means the Full Milestone Payment and the Accelerated Milestone Payment.

"**Necessary Consent**" has the meaning set forth in Section 2.06(b).

"**Non-Recourse Parties**" has the meaning set forth in Section 10.13(a).

"**Parties**" has the meaning set forth in the preamble.

"**Party**" has the meaning set forth in the preamble.

"**Patent**" means any national or multinational statutory invention registrations, patents and patent applications issued or applied for in any jurisdiction, including all certificates of invention, provisionals, nonprovisionals, substitutions, divisionals, continuations, continuations-in-part, reissues, renewals, extensions, supplementary protection certificates, reexaminations, patents of addition, utility models, inventors' certificates, patent term adjustments, patent term extensions and the equivalents of any of the foregoing in any jurisdiction, and all inventions disclosed in each such registration, patent or patent application.

"**PEO**" means a professional employer organization, employer of record or similar Third Party.

"**Permits**" means each grant, license, franchise, permit, easement, variance, exception, exemption, waiver, consent, certificate, certification, registration, accreditation, approval, authorization, concession, decree, confirmation, qualification, consent, variance, Governmental Order or other similar authorization of any Governmental Authority.

"**Permitted Liens**" means (i) Liens for Taxes not yet due or delinquent or the validity or amount of which is being contested in good faith by appropriate proceedings and for which adequate reserves have been established in accordance with GAAP and which would not, individually or in the aggregate, have a material impact on the Purchased Assets or the Business, (ii) materialmen's, mechanics', carriers', workers', warehousemen's, repairers' and similar Liens arising in the ordinary course of business, securing obligations as to which there is no default and which are not yet due and payable, or the validity or amount of which is being contested in good faith by appropriate proceedings which have the effect of preventing the forfeiture or sale of the property subject thereto and for which adequate reserves have been established in accordance with GAAP and which would not, individually or in the aggregate, have a material impact on the Purchased Assets or the Business, (iii) Liens to secure payment of workers' compensation, unemployment insurance, social security or other social security legislation (other than Liens imposed by ERISA), (iv) with respect to leased or licensed personal property, the terms and conditions of the lease, license, sublease or other agreement applicable thereto (excluding any license of Intellectual Property Rights), (v) any non-exclusive licenses granted to service providers and vendors in the ordinary course of business in connection with the Business, and (vi) solely prior to Closing, any Lien that will be removed or released by operation of the Sale Order or any other order of the Bankruptcy Court.

"**Person**" means an individual, corporation, partnership, limited liability company, association, trust or other entity or organization, including a government or political subdivision or a Governmental Authority.

"**Personal Information**" means any information relating to an identified or identifiable individual or household, or that is otherwise regulated under applicable Law.

"**Petition Date**" has the meaning set forth in the recitals.

"**Plan Sponsor Alternative**" has the meaning ascribed to such term in the Bidding Procedures.

"**Post-Closing Tax Period**" has the meaning set forth in Section 2.10(c).

"**Pre-Closing Tax Period**" has the meaning set forth in Section 2.10(c).

"**Previously Omitted Contract**" has the meaning set forth in Section 2.05(e).

"**Previously Omitted Contract Designation**" has the meaning set forth in Section 2.05(e).

"**Previously Omitted Contract Notice**" has the meaning set forth in Section 2.05(e).

"**Privacy Laws**" has the meaning set forth in Section 4.12(a).

"**Process**" or "**Processing**" means any operation or set of operations whether or not by automatic means, including collection, recording, organization, storage, retention, access, adaptation, alteration, retrieval, consultation, use, disclosure, dissemination, making available, alignment, combination, blocking, deleting, erasure, or destruction.

"**Product Data and Records**" means all original or, if originals do not exist, true, correct and complete copies of, data, files (including data files), reports, plans, operating records, protocols, work papers and other documents in any form (including in paper or electronically stored formats and including drafts where final copies are not available) that, in each case, are owned or controlled by Seller or its Subsidiaries and are primarily related to a Company Product, including (a) all data, files (including data files), reports, plans, operating records, protocols, work papers, and all other documents related to research, preclinical and clinical testing and studies (including in vivo and in vitro studies and indication development data) conducted by or on behalf of Seller or any of its Subsidiaries primarily related to any Company Product (or earlier versions of any Company Product), including scientific, preclinical and clinical data, laboratory notebooks, procedures, tests, dosage information, criteria for patient selection, safety and efficacy and study protocols; (b) all manufacturing data and records; (c) investigator's brochures; and (d) all pharmacovigilance and other safety records, in each case, in all forms, including in paper or electronically stored formats, in which they are stored or maintained, and all data and information included or referenced therein.

"**Purchase Price**" has the meaning set forth in Section 2.08.

"**Purchased Assets**" has the meaning set forth in Section 2.01.

"**Reference Date**" means January 1, 2020.

"**Regulatory Authority**" means any national (e.g., the FDA), supra national (e.g., the European Commission, the Council of the European Union, or the EMA), regional, state or local regulatory agency, department, bureau, commission, council or other Governmental Authority involved in the granting of approval of Marketing Applications or pricing approvals (or other pricing or reimbursement approvals) for pharmaceutical products.

"**Regulatory Documentation**" means, collectively: (a) all (i) INDs or other filings needed to initiate clinical testing of any pharmaceutical product, (ii) Marketing Applications, establishment license applications and drug master files, (iii) applications for designation as an "Orphan Licensed Product(s)" under the Orphan Drug Act, (iv) applications for "Fast Track" status, "Breakthrough Therapy" status or "Regenerative Medicine Advanced Therapy Designation" under Section 506 of the FD&C Act (21 U.S.C. § 356) or (v) requests for a Special Protocol Assessment under Section 505(b)(5)(B) and (C) of the FD&C Act (21 U.S.C. § 355(b)(5)(B) and (C)) and all other similar filings (including counterparts of any of the foregoing in any jurisdiction); (b) any applications for pricing approval (or other pricing or reimbursement approvals) and other applications, filings, dossiers or similar documents submitted to a Regulatory Authority in any jurisdiction for the purpose of obtaining approval of a Marketing Application or a pricing approval (or other pricing or reimbursement approvals) from that Regulatory Authority,

including any pediatric investigation plan submitted to any Regulatory Authority; (c) any supplements and amendments to any of the foregoing; and (d) any correspondence with any Regulatory Authority relating to any of the foregoing, including any pre-IND or INTERACT (or similar) meeting materials or minutes; in each case of the foregoing (a) through (d), that are owned or controlled by Seller or its Subsidiaries, and (i) are referenced in any IND for any Company Product (e.g., right of reference to another IND or a drug master file), or (ii) are otherwise primarily related to any Company Product.

"**Released Party**" has the meaning set forth in Section 10.13(b).

"**Releasing Party**" has the meaning set forth in Section 10.13(b).

"**Representatives**" means with respect to any Person, such Person's officers, directors, employees, investment bankers, attorneys, accountants, consultants, agents and other advisors or representatives.

"**Sale Hearing**" means the hearing conducted by the Bankruptcy Court to approve the transactions contemplated by this Agreement and entry of the Sale Order.

"**Sale Motion**" has the meaning set forth in Section 6.02(a).

"**Sale Order**" means an order entered by the Bankruptcy Court, substantially in the form of the Stalking Horse APA Sale Order attached hereto as Exhibit C, subject to the following modifications: (a) "Buyer" shall refer to "Buyer" as defined in this Agreement; (b) all references to the "Astellas Stalking Horse APA" shall be replaced with references to this Agreement; (c) conforming changes to reflect the terms of this Agreement; (d) immaterial modifications or clarifications; and (e) any other modifications agreed to by Seller and Buyer.

"**Sanctions**" has the meaning set forth in Section 4.10(b).

"**Security Incident**" means any unauthorized access, acquisition, interruption, alteration or modification, loss, theft, corruption or other unauthorized Processing of Personal Information or Business Data.

"**Seller**" has the meaning set forth in the preamble.

"**Seller's Closing Certificate**" has the meaning set forth in Section 8.02(i).

"**Seller Material Adverse Effect**" means any Effect that has had, or is reasonably likely to have, individually or in the aggregate, a material adverse effect on: (i) the assets, Liabilities, business, operations, properties, financial condition or results of operations of the Business, taken as a whole; provided that, for purposes of the foregoing clause (i), no such Effect to the extent resulting from, attributable to or arising out of any of the following occurring after the date hereof shall be taken into account in determining whether a Seller Material Adverse Effect has occurred or would reasonably be expected to occur: (A) changes in general economic conditions in the United States, (B) changes in securities or financial market conditions in the United States, (C) changes in general conditions in the industry in which Seller and its Subsidiaries operate, (D) acts of war, sabotage or terrorism (whether the commencement or escalation thereof) or pandemics or

natural disasters involving the United States, (E) changes in general political or social conditions in the United States, (F) changes in Laws affecting Seller or the Business (it being understood and agreed that this clause (F) shall not apply with respect to any representation or warranty the purpose of which is to address compliance with applicable Laws), (G) changes in GAAP affecting Seller (it being understood and agreed that this clause (G) shall not apply with respect to any representation or warranty the purpose of which is to address compliance with GAAP), (H) the failure to meet any internal or published budgets, projections, forecasts or predictions of financial performance for any period, in and of itself (it being understood and agreed that this clause (H) shall not apply with respect to any Effects giving rise or contributing to such change that are not otherwise excluded from this definition of "Seller Material Adverse Effect" or with respect to the underlying cause of such failure, unless such cause would otherwise be excepted by clauses (A) through (G) or clauses (I) through (M) of this definition), (I) the announcement of this Agreement or the transactions contemplated hereby in accordance with the terms hereof (it being understood and agreed that this clause (I) shall not apply with respect to any representation or warranty the purpose of which is to address the consequences of the execution and delivery of this Agreement, the consummation of the transactions contemplated hereby or the performance of obligations hereunder), (J) the commencement of the Chapter 11 Case, (K) the sale process of the Business to the extent in accordance with the terms of the Bidding Procedures Order, (L) any objections in the Bankruptcy Court to (x) this Agreement or any of the transactions contemplated hereby, (y) the Bidding Procedures Order, or (z) the assumption or rejection of any Assigned Contract otherwise in compliance with this Agreement; or (M) any order of the Bankruptcy Court entered in compliance with this Agreement, except in the cases of clauses (A) through (G), to the extent such Effect has a disproportionate effect on the Business, taken as a whole, relative to other participants in the industry in which Seller and its Subsidiaries operate the Business; or (ii) Seller's ability to perform its obligations or consummate the transactions contemplated by this Agreement in accordance with the terms and subject to the conditions hereof and on or before the End Date.

"**Specified Individual**" has the meaning set forth in Section 7.04(a).

"**STAAR Studies**" means the clinical studies: (a) titled "A Phase I/II, Multicenter, Open-Label, Single-Dose, Dose-Ranging Study to Assess the Safety and Tolerability of ST-920, an AAV2/6 Human Alpha Galactosidase A Gene Therapy, in Subjects With Fabry Disease (STAAR))," coded as ST-920-201 with ClinicalTrials.gov ID NCT04046224; and (b) titled "Long-Term Follow-up of Fabry Disease Subjects Who Were Treated With ST-920, an AAV2/6 Human Alpha Galactosidase A Gene Therapy," coded as ST-920-LT01, with ClinicalTrials.gov ID NCT05039866, in each case of (a) and (b), without further patient enrollment or amendment to the protocol as it exists as of the date hereof.

"**Stalking Horse APA Disclosure Schedules**" means the disclosure schedules to that certain Asset Purchase Agreement by and between Astellas Gene Therapies, Inc. and Sangamo Therapeutics, Inc., dated as of June 22, 2026, which disclosure schedule was delivered by Sangamo Therapeutics, Inc. to Astellas Gene Therapies, Inc. concurrently with the execution and delivery of the aforementioned Asset Purchase Agreement.

"**Stalking Horse APA Sale Order**" means that certain Astellas Sale Order filed at Docket No. 13-5 in the Chapter 11 Case.

"**Subsidiary**" means, with respect to any Person, any other Person of which such Person or any of its Subsidiaries (i) is a general partner or holds a majority of the voting interests of a partnership or (ii) holds securities or other ownership interests having ordinary voting power to elect a majority of the board of directors or other corporate bodies performing similar functions (or, if there are no such ownership interests having ordinary voting power, 50% or more of the equity interests) which are at any time, directly or indirectly, owned or controlled by such Person.

"**Successful Bidder**" has the meaning set forth in the Bidding Procedures.

"**Tax**" means any federal, state, local, foreign or supranational income, gains, alternative or add-on minimum tax, base erosion and anti-abuse, minimum, diverted profits, gross income, withholding, estimated, gross receipts, sales, use, *ad valorem*, value added, goods and services, transfer, franchise, fringe benefit, capital stock, profits, license, registration, payroll, social security (or equivalent) or any other applicable social contribution, employment, unemployment, workers' compensation, disability, excise, severance, stamp, occupation, premium, property (real, tangible or intangible), commercial rent, financial transaction, stamp, stamp duty, environmental, windfall profit, unclaimed property and other tax of any kind, any customs duties, escheat obligations and any other fees, contributions, governmental charges, levies, excises, duties or assessments of any kind whatsoever in the nature of a tax, together with any interest, penalty, inflation linkage or addition thereto imposed by applicable Law.

"**Tax Law**" means all applicable Laws relating to or regulating the assessment, determination, reporting, collection or imposition of Taxes.

"**Tax Return**" means any report, return, declaration, statement, information return, claim for refund, declaration of estimated payments, voucher or other document, together with any schedule and attachment thereto and any amendment thereof, filed or required to be filed with any Taxing Authority in connection with Taxes, including the determination, assessment, reporting, withholding, collection or payment of any Taxes.

"**Taxing Authority**" means any Governmental Authority having jurisdiction over or responsibility for the assessment, determination, collection or administration of Taxes or Tax Law.

"**Third Party**" means any Person, including as defined in Section 13(d) of the Exchange Act, other than Buyer or any of its Affiliates.

"**Trade Secrets**" means trade secrets, proprietary information, and all other confidential know-how and confidential information and rights in any jurisdiction (domestic and foreign), including confidential recipes, ideas, formulae, formulations, compositions, specifications, techniques, assays, specifications, data, results, methods, processes, protocols, schematics, prototypes, models, designs, customer lists and supplier lists.

"**Trademarks**" means trademarks, service marks, trade names, service names, business marks, brand names, certification marks, trade dress, logos, slogans, social media identifiers and handles, corporate names, trade styles, domain names and other indications of origin, whether registered or unregistered, together with all common law rights, statutory rights and other rights arising under the Laws of any jurisdiction, the goodwill associated with the foregoing and

registrations in any jurisdiction of, and applications (including intent-to-use applications) in any jurisdiction to register, the foregoing, including any extension, modification or renewal of any such registration or application.

"**Transfer Taxes**" has the meaning set forth in Section 2.10(b).

"**Transition Services Agreement**" means the transition services agreement in respect of the services (including the term and rates) described in Exhibit D, in form and substance reasonably acceptable to Buyer.

"**US BLA**" means a biologics license application as defined in section 351 of the Public Health Service Act (PHSA) (41 USC § 262) and 21 C.F.R. Part 601.

"**WARN Act**" means the federal Worker Adjustment and Retraining Notification Act of 1988, and similar state, local and foreign laws related to advance notice or consent requirements in advance of plant closings, mass layoffs and employment losses.

Section 1.02 Other Definitional and Interpretative Provisions. The words "hereof," "herein" and "hereunder" and words of like import used in this Agreement shall refer to this Agreement as a whole and not to any particular provision of this Agreement. The captions herein are included for convenience of reference only and shall be ignored in the construction or interpretation hereof. References to Articles, Sections, Exhibits, Annexes and Schedules are to Articles, Sections, Exhibits, Annexes and Schedules of this Agreement unless otherwise specified. All Exhibits, Annexes and Schedules annexed hereto or referred to herein are hereby incorporated in and made a part of this Agreement as if set forth in full herein. Any capitalized terms used in any Exhibit, Annex or Schedule but not otherwise defined therein shall have the meaning as defined in this Agreement. Any singular term in this Agreement shall be deemed to include the plural, and any plural term the singular. Whenever the words "include," "includes" or "including" are used in this Agreement, they shall be deemed to be followed by the words "without limitation," whether or not they are in fact followed by those words or words of like import. "Writing," "written" and comparable terms refer to printing, typing and other means of reproducing words (including electronic media) in a visible form. "Extent" in the phrase "to the extent" means the degree to which a subject or other thing extends, and such phrase does not mean simply "if." References to any applicable Law shall be deemed to refer to such applicable Law as amended from time to time and to any rules, regulations or interpretations promulgated thereunder. References to any Contract are to that Contract as amended, modified, supplemented, extended or renewed from time to time in accordance with the terms hereof and thereof; provided that with respect to any Contract listed on the Disclosure Schedules, all such amendments, modifications, supplements, extensions and renewals must also be listed in the appropriate Section thereof. References to any Person include the successors and permitted assigns of that Person. References from or through any date mean, unless otherwise specified, from and including or through and including, respectively. The terms "ordinary course" or "ordinary course of business" or words of similar import when used in this Agreement mean "ordinary course of business consistent with past practice". The word "or" is used in the inclusive sense of "and/or." References to "law," "laws" or to a particular statute or law shall be deemed also to include any applicable Law. References to "foreign" or words of similar import shall be deemed to refer to any jurisdictions outside the United States. Unless otherwise specifically indicated, all references to "dollars" and

"$" will be deemed references to the lawful currency of the United States of America. Unless otherwise specified, the words "made available to Buyer" or "provided to Buyer" mean the documents that were, as of at least two Business Days prior to the date hereof, (a) posted to the data room maintained by Seller or its Representatives in connection with the transactions contemplated by this Agreement (provided that Buyer or its Representatives had access to such documents in such data room and such documents were not removed from such data room prior to the date hereof), (b) otherwise provided to Buyer or its Representatives in response to a diligence request from Buyer or its Representatives or (c) included as an exhibit to Seller's documents that were filed by Seller with the U.S. Securities and Exchange Commission on or after January 1, 2024.

ARTICLE II
PURCHASE AND SALE

Section 2.01 Purchase and Sale of Assets. Subject to the entry of the Sale Order and upon terms and conditions set forth herein and the Sale Order, at the Closing, Seller shall sell, assign, transfer, convey and deliver to Buyer, or cause to be sold, assigned, transferred, conveyed and delivered to Buyer, and Buyer shall purchase from Seller or its applicable Subsidiary, free and clear of any Liens other than Permitted Liens, all of Seller's and its Subsidiaries' right, title and interest in, to and under all of the following assets, properties and rights of every kind and nature, whether real, personal or mixed, tangible or intangible, wherever located and whether now existing or hereafter acquired (other than the Excluded Assets), which primarily relate to, or are primarily used or primarily held for use in connection with, the Business (collectively, the "**Purchased Assets**"):

(a) the Company Products;

(b) all Intellectual Property Assets;

(c) subject to Section 2.05, all Contracts set forth on Section 2.01(c) of the Disclosure Schedules (including Intellectual Property Agreements), and in connection with which an order has been entered by the Bankruptcy Court (which may be the Sale Order), authorizing the assumption and assignment of such Contracts by Buyer (the "**Assigned Contracts**");

(d) all inventory, finished goods, raw materials, work in progress, packaging, supplies, parts and other inventories primarily related to the Business or any Purchased Asset, including all inventory of the Company Products ("**Inventory**");

(e) all laboratory equipment, devices, machinery and tools primarily related to, primarily used in or primarily held for use by the Business or any Purchased Asset;

(f) all Permits related to the Business which are held by Seller or any of its Subsidiaries and all of Seller's and its Subsidiaries' pending applications for Permits related to the Business and the STAAR Studies, including all Permits and other applications for Permits related to the Business as currently conducted or to the ownership and use of the other Purchased Assets;

(g) the IT Assets;

(h) all rights to any Actions of any nature available to or being pursued by Seller or any of its Subsidiaries to the extent related to the Business, any Purchased Assets or the Assumed Liabilities, whether arising by way of counterclaim or otherwise, including Avoidance Action Claims, all other claims, counterclaims, defenses, credits, rebates (including any vendor or supplier rebates), demands, allowances, refunds, rights of set off, rights under or with respect to express or implied guarantees, warranties, representations, covenants, indemnities, exculpation, advancement, reimbursement of expenses or contract renewal rights and other similar rights, in each case, whether direct or derivative, known or unknown, liquidated or unliquidated, contingent or otherwise, relating to or arising against counterparties to any Assigned Contract or related to the Business, other than the rights to Actions specifically set forth on Section 2.02(d) of the Disclosure Schedules;

(i) all prepaid expenses, credits, advance payments, claims, security, refunds, rights of recovery, rights of set-off, rights of recoupment, deposits, charges, sums and fees to the extent related to the Business or any Purchased Asset including any such item relating to the payment of Taxes;

(j) all of Seller's and its Subsidiaries' rights under warranties, indemnities and all similar rights against Third Parties to the extent related to any Purchased Asset;

(k) all insurance benefits, including rights and proceeds, to the extent arising from or relating to the Business or any of the Purchased Assets or the Assumed Liabilities; provided, however, any insurance policies for D&O or other fiduciary coverage shall be Excluded Assets;

(l) originals, or where not available, copies, of all books and records, to the extent related to the Company Products or Business, including (i) books of account, ledgers and general, financial and accounting records, machinery and equipment maintenance files, personnel files (except for such files the disclosure of which would violate applicable Law), lists of prospective customers, vendors and suppliers, purchase orders and invoices, production data, quality control records and procedures, patient complaints and inquiry files, research and development files, product development and stability records; (ii) Product Data and Records; (iii) the Regulatory Documentation; (iv) strategic plans, internal financial statements, marketing and promotional surveys, material and research and files, (v) all files relating to the filing, prosecution, issuance, maintenance, enforcement or defense of any Intellectual Property Assets (including attorney-client privileged materials); (vi) records, files (including data files) and documents evidencing any Intellectual Property Assets; and (vii) documentation covering, containing, describing, memorializing or otherwise pertaining to or used for any exploitation activity in connection with any Company Product, in all forms, including in paper or electronically stored formats, in which they are stored or maintained, and all data and information included or referenced therein ("**Books and Records**"); and

(m) all goodwill associated with the Business or any Purchased Asset, including the Intellectual Property Assets.

Section 2.02 Excluded Assets. Notwithstanding any provision of this Agreement to the contrary, Buyer shall acquire no right, title, or interest in, to or under the following assets, properties, rights, interests, or claims in any assets, properties, rights, interests or claims of any kind or description of Seller or any of its Subsidiaries that are not Purchased Assets (collectively, the "**Excluded Assets**"), including:

(a) assets, properties, rights, interests or claims of any kind or description of Seller or any of its Subsidiaries that are primarily related to the Excluded Business;

(b) Contracts, including Intellectual Property Agreements, that are not Assigned Contracts (the "**Excluded Contracts**");

(c) the corporate seals, organizational documents, minute books, stock books, Tax Returns, books of account or other records having to do with the corporate organization of Seller or any of its Subsidiaries;

(d) the assets, properties and rights specifically set forth on Section 2.02(d) of the Disclosure Schedules;

(e) all equity and other ownership interests in Seller and any of its Subsidiaries;

(f) all Tax assets of Seller and its Subsidiaries, including any net operating loss carryforwards, Tax credits, Tax refunds, and similar attributes (and any foreign equivalents thereof); and

(g) the rights which accrue or will accrue to Seller or any of its Subsidiaries under this Agreement and the Ancillary Documents.

Section 2.03 Assumed Liabilities. Subject to the entry of the Sale Order and upon the terms and conditions set forth herein and the Sale Order, Buyer shall assume and agree to pay, perform and discharge only the following Liabilities of Seller or its applicable Subsidiary (collectively, the "**Assumed Liabilities**"), and no other Liabilities:

(a) all Liabilities from the ownership or operation of the Purchased Assets by Buyer solely to the extent such Liabilities arise after the Closing and relate solely to such post-Closing operations by Buyer (which shall exclude, for the avoidance of doubt, Cure Costs);

(b) all Liabilities in respect of the Assigned Contracts but solely to the extent that such Liabilities thereunder arise after the Closing and relate solely to such post-Closing operations by Buyer (which shall exclude, for the avoidance of doubt, Cure Costs), and do not relate to any breach, default or violation by Seller or any of its Subsidiaries on or prior to the Closing; and

(c) all Liabilities for (i) 50% of any Transfer Taxes and (ii) Taxes arising out of, in respect of or relating to the Business or the Purchased Assets for all Post-Closing Tax Periods.

Section 2.04 Excluded Liabilities. Notwithstanding anything to the contrary set forth herein, Seller expressly acknowledges and agrees that Buyer will not assume, be obligated to pay, perform or otherwise discharge or in any other manner be liable or responsible for any Liabilities of Seller or any of its Subsidiaries, whether existing on the Closing Date or arising thereafter, including on the basis of any Law imposing successor liability, other than the Assumed Liabilities and the obligations of Buyer under this Agreement (all such Liabilities that Buyer are not assuming being referred to collectively as the "**Excluded Liabilities**"). Without limiting the generality of the foregoing, the Excluded Liabilities shall include the following:

(a) any Liabilities to the extent relating to or arising out of the Excluded Assets;

(b) any Liabilities under the Excluded Contracts or any other Contracts, including Intellectual Property Agreements, (i) which are not validly and effectively assigned to Buyer pursuant to this Agreement; or (ii) to the extent such Liabilities arise out of or relate to a breach by Seller or any of its Subsidiaries of such Contracts prior to Closing;

(c) all Liabilities with respect to the Purchased Assets and Assigned Contracts that arose or were required to be performed prior to the Closing Date, including Cure Costs;

(d) any Liabilities of Seller or any of its ERISA Affiliates with respect to any present or former employees, officers, directors, retirees, independent contractors or consultants of Seller or any of its ERISA Affiliates, including any Liabilities associated with any claims for wages or other compensation, benefits, bonuses, accrued vacation, workers' compensation, severance, retention, termination or other payments;

(e) any Liabilities with respect to any Employee Plan, including with respect to any obligations to provide continued coverage under COBRA in connection with prior participation in any Employee Plan;

(f) any Liabilities to indemnify, reimburse or advance amounts to any present or former officer, director, employee, independent contractor, consultant, or agent of Seller or any of its Subsidiaries (including with respect to any breach of fiduciary obligations by same);

(g) any Liabilities associated with debt, loans or credit facilities of Seller or any of its Subsidiaries, the Business or the Purchased Assets owing to financial institutions;

(h) any accounts payable of Seller or any of its Subsidiaries (i) which constitute intercompany payables owing to Affiliates of Seller or any of its Subsidiaries or any third parties other than those arising under the Assigned Contracts; (ii) which constitute debt, loans or credit facilities to financial institutions; or (iii) which did not arise in the ordinary course of business;

(i) any Liability for (i) Taxes of Seller or any of its Subsidiaries (or any stockholder or Affiliate of Seller), including any Taxes that arise out of the consummation of the transactions contemplated hereby imposed by applicable Tax Law on Seller or any

of its Subsidiaries, (ii) Taxes relating to the Business, the Purchased Assets or the Assumed Liabilities for any Pre-Closing Tax Period; (iii) 50% of any Transfer Taxes; and (iv) other Taxes of Seller or any of its Subsidiaries (or any stockholder or Affiliate of Seller) of any kind or description (including any Liability for Taxes of Seller or any of its Subsidiaries (or any stockholder or Affiliate of Seller) that become Liabilities of Buyer under any common law doctrine of de facto merger or transferee or successor liability or otherwise by operation of contract or Law);

(j) any Liabilities in respect of any pending or threatened Action arising out of, relating to or otherwise in respect of the operation of the Business or the Purchased Assets to the extent such Action relates to Seller or its Subsidiaries' operation of the Business prior to the Closing Date;

(k) any Liabilities arising out of, in respect of or in connection with the failure by Seller or any of its Affiliates to comply with any Law or Governmental Order; and

(l) any obligations or Liabilities arising out of, in respect of or in connection with any DIP Order.

Section 2.05 Assignment of Contracts; Cure Costs

(a) Section 2.01(c) of the Disclosure Schedules sets forth a list of all Contracts to which Seller or a Subsidiary thereof is a party and which are to be included in the Assigned Contracts. Seller shall make such deletions to Section 2.01(c) of the Disclosure Schedules as Buyer shall, in its sole discretion, request in writing at any time up to the date that is two Business Days prior to the Closing Date. Any such deleted Contract shall be deemed to no longer be an Assigned Contract. Seller shall add any Contracts to Section 2.01(c) of the Disclosure Schedules as Buyer shall, in its sole discretion, request in writing at any time up to the date that is two Business Days prior to the effective date of any Chapter 11 plan; provided that the counterparty to such Contract is provided notice and an opportunity to object to such assumption and assignment in accordance with the Bidding Procedures Order or any other relevant order of the Bankruptcy Court.

(b) Subject to Section 2.05(a) and Section 2.05(e), any Contract of Seller or any of its Subsidiaries that is not listed on Section 2.01(c) of the Disclosure Schedules shall not be considered an Assigned Contract and shall be deemed an Excluded Contract. Buyer and Seller acknowledge and agree that there shall be no reduction or increase in the Purchase Price if Buyer elects to add or delete any Contracts from Section 2.01(c) of the Disclosure Schedules.

(c) Seller shall provide timely and proper written notice of a proposed Sale Order to all parties to any executory Contracts to which Seller or any of its Subsidiaries is a party that are related to the Business and take all actions required to assume and assign the Assigned Contracts to Buyer, including using commercially reasonable efforts to facilitate any negotiations with the counterparties to such Contracts and to obtain a finding under the Sale Order that the proposed assumption and assignment of the Assigned

Contracts to Buyer satisfy all applicable requirements of Section 365 of the Bankruptcy Code.

(d) At the Closing, (i) Seller shall, pursuant to the Sale Order and the Assignment and Assumption Agreement(s), as applicable, assign (or cause to be assigned) to Buyer (the consideration for which is included in the Purchase Price) each of the Assigned Contracts that is capable of being assigned under applicable Law and (ii) Buyer shall assume and discharge the Assumed Liabilities (if any) under the Assigned Contracts, pursuant to the Assignment and Assumption Agreement(s).

(e) If prior to or following Closing, it is discovered that a Contract should have been listed in Section 4.07 of the Disclosure Schedules but was not so listed (any such Contract, a "**Previously Omitted Contract**"), Seller shall, promptly (but in no event later than two Business Days following the discovery thereof) notify Buyer in writing of such Previously Omitted Contract. Buyer shall deliver written notice to Seller promptly thereafter, designating such Previously Omitted Contract as "Assigned" or "Excluded" (a "**Previously Omitted Contract Designation**"). A Previously Omitted Contract designated as "Excluded," or with respect to which Buyer fails to deliver a Previously Omitted Contract Designation, shall be an Excluded Contract. If Buyer designates a Previously Omitted Contract as "Assigned", Section 2.01(c) of the Disclosure Schedules shall be amended to include such Previously Omitted Contract and Seller shall serve a notice (the "**Previously Omitted Contract Notice**") on the counterparties to such Previously Omitted Contract notifying such counterparties of Seller's intention to assign and Buyer's intention to assume such Previously Omitted Contract in accordance with this Agreement. The Previously Omitted Contract Notice shall provide the counterparties to such Previously Omitted Contract with ten Business Days to object, in writing to Seller and Buyer, to the assumption of their Contract. If the counterparties, Seller and Buyer are unable to reach a consensual resolution with respect to the objection, Seller will seek an expedited hearing before the Bankruptcy Court to approve the assumption. If no objection is timely served on Seller and Buyer, Seller shall obtain an order of the Bankruptcy Court approving the assumption of the Previously Omitted Contract. Seller and Buyer shall execute, acknowledge and deliver such other instruments and take commercially reasonable efforts as are reasonably practicable for Buyer to assume the rights and obligations under such Previously Omitted Contract.

(f) Seller shall give written notice to Buyer prior to the submission by Seller of any motion in the Chapter 11 Case to reject any Contract used or held for use in the Business and provide Buyer with an opportunity to designate such Contract as either an Assigned Contract or Excluded Contract; provided that in no event will Seller reject or seek to reject any Contract used or held for use in the Business prior to the date that is five Business Days prior to the anticipated Closing Date unless prior written approval has been obtained from Buyer.

(g) Seller shall be responsible for payment of all Cure Costs, if any, necessary to cure all defaults under the Assigned Contracts in connection with the assumption and assignment thereof to Buyer pursuant to Section 365 of the Bankruptcy Code.

(h) In the event of any objection by a counterparty to an Assigned Contract to (i) the Cure Costs asserted by Seller, (ii) the adequate assurance of future performance to be provided by Buyer, or (iii) any other aspect of the proposed assumption and assignment of such Assigned Contract (each, a "**Cure/Assumption Objection**"), Seller shall use reasonable best efforts to resolve any such Cure/Assumption Objection (with the prior written consent of Buyer, not to be unreasonably withheld); provided that Buyer shall reasonably cooperate with Seller in connection with any objection relating to adequate assurance of future performance. In no event shall Seller settle any Cure/Assumption Objection without the express prior written consent of Buyer. Seller shall litigate before the Bankruptcy Court any Cure/Assumption Objection that cannot be settled with the prior written consent of Buyer and shall request that the Bankruptcy Court hear and determine such objection on an expedited basis.

(i) Except with respect to any Assigned Contracts to which any party set forth on Schedule 2.05(i) or an Affiliate thereof (such Persons set forth on Schedule 2.05(i) and its Affiliates, the "**Essential Counterparties**") is party, if any Cure/Assumption Objection with respect to an Assigned Contract has not been resolved prior to the Closing (whether by order of the Bankruptcy Court or by agreement with the applicable counterparty), (i) Buyer may, in its sole discretion, by written notice to Seller, elect to remove such Assigned Contract from Schedule 2.01(c) of the Disclosure Schedules, in which case such Contract shall be an Excluded Contract for all purposes of this Agreement and no Liabilities arising thereunder shall be assumed or borne by Buyer, or (ii) Buyer may elect to temporarily treat such Contract as a "**Designated Contract**" and proceed to Closing without any modification to Purchase Price, without the assignment of such Designated Contract, and determine within seven Business Days after resolution of such objection (whether by order of the Bankruptcy Court or by agreement with the applicable counterparty) whether to treat such Designated Contract as an Assigned Contract or an Excluded Contract. Upon resolution of any Cure/Assumption Objection following the Closing, Seller shall promptly assume and assign such Contract to Buyer in accordance with Section 365 of the Bankruptcy Code.

Section 2.06 Assignment of Assets.

(a) Except as otherwise provided in Section 2.05, notwithstanding anything in this Agreement to the contrary, at any time prior to the date that is five Business Days prior to the anticipated Closing Date, Buyer may, in its sole discretion by written notice to Seller, designate any of the Purchased Assets as additional Excluded Assets, which notice shall set forth in reasonable detail the Purchased Assets so designated. Buyer and Seller acknowledge and agree that there shall be no reduction in the Purchase Price if Buyer elects to designate any Purchased Assets as Excluded Assets. Notwithstanding any other provision hereof, the Liabilities of Seller or any of its Subsidiaries under or related to any Purchased Asset excluded under this paragraph will constitute Excluded Liabilities.

(b) Notwithstanding any other provision of this Agreement to the contrary, this Agreement will not constitute an agreement to assign or transfer and will not effect the assignment or transfer of any Purchased Asset (including any Assigned Contract) if (i) (A) prohibited by applicable Law, (B) an attempted assignment or transfer thereof would

reasonably likely subject Buyer, its Affiliates or any of their respective Representatives to civil or criminal Liability or (C) an attempted assignment or transfer thereof, without the approval, authorization or consent of, or granting or issuance of any license or permit by, any Third Party thereto (each such action, a "**Necessary Consent**"), would constitute a breach, default or violation thereof or of any Law or in any way adversely affect the rights of Buyer thereunder or (ii) the Bankruptcy Court has not entered an order approving such assignment or transfer. In such event, such assignment or transfer is subject to such Necessary Consent being obtained and Seller and Buyer will use their commercially reasonable efforts to obtain the Necessary Consents with respect to any such Purchased Asset (including any Assigned Contract) or any claim or right or any benefit arising thereunder for the assignment or transfer thereof to Buyer as Buyer may reasonably request. If such Necessary Consent is not obtained, or if an attempted assignment or transfer thereof would give rise to any of the circumstances described in clauses (i) or (ii) of the first sentence of this section, be ineffective or would adversely affect the rights of Buyer to such Purchased Asset following the Closing, (x) Seller and Buyer will, and will cause their respective Affiliates to, (1) use commercially reasonable efforts (including cooperating with one another to obtain such Necessary Consents, to the extent feasible) as may be necessary so that Buyer would obtain the benefits and assume the obligations thereunder in accordance with this Agreement, (2) complete any such assignments or transfers as soon as reasonably practicable, and (3) upon receipt of any applicable Necessary Consents, to transfer or assign the applicable Purchased Asset to Buyer, and (y) Seller will, and will cause its respective Affiliates to, cooperate with Buyer in good faith without further consideration in any arrangement reasonably acceptable to Buyer and Seller intended to provide Buyer with the benefit of any such Purchased Assets.

Section 2.07 Further Conveyances and Assumptions. From time to time following the Closing, Seller and Buyer will, and will cause their respective Affiliates to, execute, acknowledge and deliver all such further conveyances, notices, assumptions, assignments, releases and other instruments, and will take such further actions, as may be reasonably necessary or appropriate to assure fully to Buyer and their successors or assigns, all of the properties, rights, titles, interests, estates, remedies, powers and privileges intended to be conveyed to Buyer under this Agreement and to assure fully to Seller and its Affiliates and their respective successors and assigns, the assumption of the liabilities and obligations intended to be assumed by Buyer under this Agreement, and to otherwise make effective the transactions contemplated herein; provided that nothing in this section will require Buyer or any of its Affiliates to assume any Liabilities other than the Assumed Liabilities.

Section 2.08 Purchase Price. The purchase price for the Purchased Assets shall be the aggregate of (i) $111,000,000 (the "**Closing Consideration**") and (ii) in the manner and at the time and subject to the terms and conditions set forth in Section 2.09, the Milestone Payments (together with the Closing Consideration, the "**Purchase Price**"), plus the assumption of the Assumed Liabilities; provided, however, Buyer reserves the right, in its sole discretion, to increase the Purchase Price (or any component thereof), subject to the Bidding Procedures Order and applicable Law. The Closing Consideration shall be paid as provided in Section 3.02(b)(i). The Milestone Payment shall be paid as provided in Section 2.09.

Section 2.09 Milestone Payment.

(a) Buyer shall notify Seller in writing within fifteen Business Days following the achievement of a Milestone Event. If the Accelerated Milestone Event is achieved, Buyer will make a one-time, non-refundable payment of $80,000,000 to Seller (the "**Accelerated Milestone Payment**"), no later than twenty Business Days after Buyer's notice of the achievement of the Accelerated Milestone Event. If the Full Milestone Event is achieved, Buyer will make a one-time, non-refundable payment of $20,000,000 to Seller (the "**Full Milestone Payment**"), no later than twenty Business Days after Buyer's notice of the achievement of the Full Milestone Event. If the Full Milestone Event is achieved and the Accelerated Milestone Event was not achieved prior to the achievement of the Full Milestone Event, Buyer will make both the Accelerated Milestone Payment and the Full Milestone Payment to Seller no later than twenty Business Days after Buyer's notice of the achievement of the Full Milestone Event. For the avoidance of doubt, the aggregate amount payable by Buyer to Seller pursuant to this Section 2.09(a) shall not exceed $100,000,000.

(b) Seller and Buyer acknowledge and agree that, except as set forth in Section 2.09(c): (i) Buyer shall have the right to own, develop, operate and maintain the Business as Buyer deems appropriate, in its sole discretion, (ii) Buyer shall use commercially reasonable efforts to achieve the Milestone Events, (iii) Buyer does not have any obligation to conduct any additional pre-approval or post-approval clinical trial beyond the STAAR Studies, (iv) Buyer does not owe any duty, as a fiduciary or otherwise, to Seller in connection with its management of the Business following the Closing, (v) there is no assurance Seller will receive any Milestone Payment, (vi) Buyer shall have the right to manage, hire and terminate the employment or engagement of any service provider as Buyer deems appropriate in its sole discretion in the Business, (vii) (A) Buyer has not, prior to, at or after the date hereof, promised any amounts to be received by Seller in respect of any Milestone Payment except upon the achievement of any Milestone Event, and (B) Buyer has not made, and Seller is not relying on or has not relied on, any representation or warranty of any kind or other information, documents or materials (or absence thereof) in respect of any Milestone Payment, including with respect to the management of the Business following the Closing and (viii) the Parties intend the express provisions of this Section 2.09 to govern their contractual relationship with respect to any Milestone Payment and to supersede any standard of effort or implied covenant that might otherwise be imposed by applicable Law, any Governmental Authority or otherwise. The Parties hereby expressly disclaim any extra-contractual covenants, obligations or undertakings with respect to any Milestone Payment (including, for the avoidance of doubt, any claims of fraud) and agree that the sole obligations of the Parties with respect to any Milestone Payment shall be as set forth in this Agreement.

(c) Notwithstanding Section 2.09(b) above, following the achievement of the Accelerated Milestone Event, Buyer shall not delay the submission of an application to the FDA for full approval of a US BLA for the Company Product in bad faith and with the primary purpose of avoiding the Full Milestone Payment.

(d) Any right to receive any Milestone Payment is solely a contractual right and shall not be a security for purposes of any federal or state securities Law. The right to receive any Milestone Payment: (i) does not give any dividend rights, voting rights,

liquidation rights, preemptive rights or other rights to Seller; (ii) shall not be evidenced by a certificate or other instrument; (iii) shall not accrue or pay interest on any portion thereof; and (iv) does not represent any right other than the right to receive the consideration set forth in this Agreement.

(e) Any right to receive any Milestone Payment, together with the associated rights to enforce Buyer's obligations with respect to the Milestone Payments (including as set forth in Section 2.09(b)(ii)), shall be fully and freely assignable and transferrable at the discretion of the Seller or any successor to the Seller.

(f) Seller, any Successor to the Seller and any subsequent assignee or transferee of the right to receive any Milestone Payment shall provide advance written notice to Buyer of any assignment or transfer by such Person of the right to receive any Milestone Payment, with the details thereof to be confirmed to Buyer in writing promptly following the completion of such assignment or transfer. Notwithstanding anything to the contrary in this Agreement, the assignment or transfer of the right to receive any Milestone Payment shall be subject in all respects to the requirements of Section 2.09(d).

Section 2.10 Certain Tax Matters.

(a) Buyer shall be entitled to deduct and withhold from the Closing Consideration and any Milestone Payment all Taxes that Buyer is required to deduct and withhold under any provision of Tax Law. All such withheld amounts remitted to the applicable Taxing Authority shall be treated as delivered to Seller hereunder.

(b) Any sales, use, gross-receipts, excise, value-added, property transfer or gains, real estate or land transfer or gains, documentary, stamp, registration, recording, filing, goods and services or other similar Taxes which may be payable by reason of the sale or purchase of the Purchased Assets or the assumption of the Assumed Liabilities under this Agreement or the transactions contemplated hereby (collectively "**Transfer Taxes**") and that are not exempt under the Bankruptcy Code shall be borne 50% by Seller and 50% by Buyer. Seller shall, at its own expense, timely file any Tax Return or other document required to be filed with respect to any Transfer Tax and timely pay any amount of Transfer Tax (whether or not shown on a Tax Return) required to be paid to any Taxing Authority, and Buyer shall join in the execution of any such Tax Return if required by Law and upon receipt of a written notice from Seller reimburse Seller for the portion of any amount of Transfer Tax for which Buyer is responsible hereunder. If, contrary to the preceding sentence, with respect to any Transfer Tax Buyer is required by Law to file a Tax Return, Buyer shall file such Tax Return and, if any amount of Transfer Tax is due to be paid in respect thereof, notify Seller of the amount of the Transfer Tax shown to be due on such Tax Return for which Seller is responsible hereunder, and Seller shall reimburse Buyer for such amount. Payments pursuant to this Section 2.10(b) shall be made by the paying Party to the payee Party by wire transfer of immediately available funds within ten days of receipt of notice from the payee Party to an account or accounts designated by the payee Party.

(c) All personal property and similar *ad valorem* Taxes, if any, levied with respect to the Purchased Assets for a taxable period which includes (but does not end on)

the Closing Date (collectively, the "**Apportioned Taxes**") shall be apportioned between Seller and Buyer based on the number of days of such taxable period ending on and including the Closing Date (such portion of such taxable period, the "**Pre-Closing Tax Period**") and the number of days of such taxable period after the Closing Date (such portion of such taxable period, the "**Post-Closing Tax Period**"). Seller shall be responsible for the proportionate amount of such Apportioned Taxes that is attributable to the Pre-Closing Tax Period and such amount shall be an Excluded Liability, and Buyer shall be responsible for the proportionate amount of such Apportioned Taxes that is attributable to the Post-Closing Tax Period and such amount shall be an Assumed Liability. Any Apportioned Taxes shall be timely paid, and all applicable Tax Returns shall be timely filed, as provided by applicable Law. The paying party (or parties, as applicable) shall be entitled to reimbursement from the non-paying party (or parties, as applicable) for the non-paying party's (or parties', as applicable) portion of the Apportioned Taxes in accordance with this section. Upon payment of any such Apportioned Taxes, the paying party (or parties, as applicable) shall present a statement to the non-paying party (or parties, as applicable) setting forth the amount of reimbursement to which the paying party (or parties, as applicable) is entitled under this section, together with such supporting evidence as is reasonably necessary to calculate the amount to be reimbursed. The non-paying party (or parties, as applicable) shall make such reimbursement by wire transfer in immediately available funds within ten days of receipt of such statement to an account designated by the paying party (or parties, as applicable).

(d) Buyer and Seller agree to provide each other with such information and assistance as is reasonably necessary and is reasonably requested by the other Party, including access to records, Tax Returns and personnel, for the preparation and filing of any Tax Returns or for the defense of any Tax claim or assessment, whether in connection with a Tax proceeding or otherwise.

Section 2.11 Non-Exclusive License to Seller under certain Intellectual Property Assets. Upon Closing, Buyer shall (and hereby does, effective upon Closing) grant to Seller a non-exclusive, worldwide, perpetual, irrevocable, transferable, sublicensable (through multiple tiers), royalty free and fully paid license under the Intellectual Property Assets set forth in Section 2.11 of the Disclosure Schedules to (a) research, develop, make, have made, use, sell, offer for sale, have sold, import and otherwise commercialize and exploit Seller's gene therapy product candidate for the treatment of hemophilia A known as giroctocogene fitelparvovec; and (b) research, develop, make, have made, use, sell, offer for sale, have sold, import and otherwise commercialize and exploit Seller's proprietary capsids and capsid variants (including the blood-brain barrier-penetrant capsid known as STAC-BBB) for any and all uses and purposes, including the diagnosis, prevention and treatment of any and all diseases in humans or animals.

ARTICLE III
CLOSING

Section 3.01 Closing. Subject to the terms and conditions of this Agreement, the consummation of the transactions contemplated by this Agreement (the "**Closing**") shall take place (i) by electronic exchange of documents and signatures (or their electronic counterparts), on the third Business Day after all of the conditions to Closing set forth in ARTICLE VIII are either

satisfied or waived (other than conditions which, by their nature, are to be satisfied on the Closing Date, but subject to the satisfaction or waiver of such conditions), or (ii) at such other place or time or on such other date as Seller and Buyer may mutually agree in writing. The date on which the Closing occurs is referred to in this Agreement as the "**Closing Date**."

Section 3.02 Closing Deliverables.

(a) At the Closing, Seller shall deliver to Buyer the following:

(i) one or more duly executed bills of sale in form and substance satisfactory to Buyer (each being a "**Bill of Sale**"), transferring the tangible personal property included in the Purchased Assets to Buyer, as applicable;

(ii) one or more duly executed assignment and assumption agreements in form and substance satisfactory to Buyer (each being a "**Assignment and Assumption Agreement**"), effecting the assignment to and assumption by Buyer of the Purchased Assets and the Assumed Liabilities;

(iii) one or more duly executed assignments in form and substance satisfactory to Buyer (each being a "**Intellectual Property Assignment**"), transferring all of Seller's right, title and interest in and to the Intellectual Property Assets to Buyer;

(iv) one or more duly executed powers of attorney in form and substance satisfactory to Buyer;

(v) the Seller's Closing Certificates;

(vi) a properly completed and executed Internal Revenue Service Form W-9 of Seller certifying that Seller is a United States person within the meaning of Section 7701(a)(30) of the Code and not subject to backup withholding;

(vii) the certificates of an authorized officer of Seller required by Section 8.02(j) and Section 8.02(k);

(viii) a copy of the Sale Order as entered by the Bankruptcy Court; and

(ix) a duly executed counterpart of the Transition Services Agreement executed by Seller.

(b) At the Closing, Buyer shall deliver to Seller the following:

(i) the Closing Consideration, which shall be delivered by wire transfer of immediately available funds to the bank account designated by Seller in writing to Buyer (including wire instructions) at least three Business Days prior to the Closing;

(ii) all Assignment and Assumption Agreements duly executed by Buyer;

(iii) the Buyer's Closing Certificate; and

(iv) a duly executed counterpart of the Transition Services Agreement executed by Buyer.

(c) At the Closing, each of Seller and Buyer shall deliver such other customary instruments of transfer, assumption, filings or documents, in form and substance reasonably satisfactory to the other Party, as may be required to give effect to this Agreement.

ARTICLE IV
REPRESENTATIONS AND WARRANTIES OF SELLER

Except as set forth in the correspondingly numbered Section of the Disclosure Schedules, Seller represents and warrants to Buyer that the statements contained in this ARTICLE IV are true and correct as of the date hereof and as of the Closing Date, subject in each case to the effects and limitations imposed by the Bankruptcy Court or the Bankruptcy Code.

Section 4.01 Organization and Qualification of Seller. Seller is a corporation duly organized, validly existing and in good standing under the Laws of the State of Delaware and, subject to any limitations that may be imposed on Seller as a result of filing a petition for relief under the Bankruptcy Code, has full corporate power and authority to own, operate or lease the properties and assets now owned, operated or leased by it and to carry on the Business as currently conducted. Section 4.01 of the Disclosure Schedules sets forth each jurisdiction in which Seller is organized and licensed or qualified to do business, and Seller is duly licensed or qualified to do business and is in good standing in each jurisdiction in which the ownership of the Purchased Assets or the operation of the Business as currently conducted makes such licensing or qualification necessary.

Section 4.02 Authority of Seller. Subject to entry of the Bidding Procedures Order and Sale Order, as applicable, Seller has the requisite corporate power and authority to enter into this Agreement and the Ancillary Documents to which such Seller is a party, to carry out its obligations hereunder and thereunder and to consummate the transactions contemplated hereby and thereby. The execution and delivery by Seller of this Agreement and any Ancillary Document to which Seller is a party, the performance by Seller of its obligations hereunder and thereunder and the consummation by Seller of the transactions contemplated hereby and thereby have been duly authorized by all requisite corporate or similar action on the part of Seller. This Agreement has been duly executed and delivered by Seller, and (assuming due authorization, execution and delivery by Buyer and the entry of the Sale Order) this Agreement constitutes a legal, valid and binding obligation of Seller enforceable against Seller in accordance with its terms. When each Ancillary Document to which Seller is or will be a party has been duly executed and delivered by Seller (assuming due authorization, execution and delivery by each other party thereto and the entry of the Sale Order), such Ancillary Document will constitute a legal and binding obligation of Seller enforceable against it in accordance with its terms.

Section 4.03 No Conflicts; Consents. Subject to the Sale Order having been entered, the execution and delivery by Seller of this Agreement, the performance by Seller of its obligations under this Agreement and the Ancillary Documents and the consummation by Seller of the transactions contemplated by this Agreement and the Ancillary Documents do not and will not (a) contravene, conflict with or result in any violation or breach of any provision of the certificate of incorporation or bylaws or other organizational documents of Seller or any of its Subsidiaries; (b) contravene, conflict with or result in a violation or breach of any provision of any applicable Law or Governmental Order applicable to Seller or any of its Subsidiaries, the Business or the Purchased Assets; (c) except as set out in Section 4.03(c) of the Disclosure Schedules, require any consent or other action by any Person under, constitute a breach or default, or an event that, with or without notice or lapse of time or both, would constitute a breach or default, under, or cause or permit the termination, cancellation, acceleration or other change of any right or obligation or the loss of any benefit under any Contract or Permit to which Seller or any of its Subsidiaries is a party or by which Seller or any of its Subsidiaries or the Business is bound or to which any of the Purchased Assets are subject (including any Assigned Contract); or (d) result in the creation or imposition of any Lien other than Permitted Liens on the Purchased Assets, except in the case of each of clauses (b), (c) and (d), as would not be material to the Business. No consent, approval, Permit, Governmental Order, declaration or filing with, or notice to, any Governmental Authority is required by or with respect to Seller or any of its Subsidiaries in connection with the execution and delivery of this Agreement or any of the Ancillary Documents and the consummation of the transactions contemplated hereby and thereby, except (i) to the extent required if the Sale Order is not entered or as would not be material to the Business and (ii) as may be required to obtain clearance of this Agreement under the HSR Act.

Section 4.04 Title to Purchased Assets. Seller and its Subsidiaries have good and valid title to, or in the case of leased assets, have good and valid leasehold interests in, the Purchased Assets free and clear of all Liens except for Permitted Liens. At the Closing, Buyer will be vested with good and valid title to, or in the case of leased assets, good and valid leasehold interest in, such Purchased Assets, free and clear of all Liens (except for Permitted Liens) and Excluded Liabilities, to the fullest extent permissible under Law, including Section 363(f) of the Bankruptcy Code.

Section 4.05 Assets Necessary to Business. Except as set forth in Section 4.05 of the Disclosure Schedules, the Purchased Assets constitute all of the assets, properties, licenses and Contracts necessary and sufficient to conduct the Business after the Closing in substantially the same manner as the Business has been conducted in the six (6) months prior to the Closing.

Section 4.06 Intellectual Property.

(a) Section 4.06(a) of the Disclosure Schedules contains a true, correct and complete list of (i) all Intellectual Property Registrations, specifying as to each, as applicable: (A) the owner (or the co-owners) thereof, (B) the jurisdiction (foreign and domestic) in which such item is issued or registered or in which any application for issuance or registration has been filed, (C) the respective issuance, registration, or application number of such item, and (D) the date of application and issuance or registration of such item; (ii) all unregistered Trademarks included in the Intellectual Property Assets; and (iii) all proprietary software included in the Intellectual Property

Assets. With respect to each Patent included in the Intellectual Property Assets, Section 4.06(a) of the Disclosure Schedules identifies the complete patent family, all priority claims, all continuations, continuations-in-part, divisionals, reissues, reexaminations, substitutions, extensions, supplementary protection certificates, patent term adjustments and patent term extensions, and the current prosecution, maintenance, opposition, interference, derivation, post-grant review, inter partes review, reexamination or other challenge status thereof.

(b) Section 4.06(b) of the Disclosure Schedules contains a true, correct, and complete list of all Intellectual Property Agreements, including all such agreements necessary or useful for operating the Business as currently conducted, specifying for each the date, title and parties thereto. Seller has provided Buyer with true, correct and complete copies (or in the case of any oral agreements, a true, correct and complete written description) of all such Intellectual Property Agreements, including all modifications, amendments and supplements thereto and waivers thereunder. Each Intellectual Property Agreement is valid and binding on Seller or a Subsidiary thereof in accordance with its terms and is in full force and effect. None of Seller or any of its Subsidiaries or, to Seller's Knowledge, any other party thereto is, or is alleged to be, in breach of or default under, or has provided or received any notice of breach of, default under, or intention to terminate (including by non-renewal), any Intellectual Property Agreement.

(c) Except as set forth in Section 4.06(c)(1) of the Disclosure Schedules, Seller or a Subsidiary thereof is the sole, exclusive, legal, beneficial and record owners of all Intellectual Property Assets and hold all right, title and interest in and to all Intellectual Property Assets, free and clear of any Lien (except for Permitted Liens), covenant not to sue, option, right of first negotiation, right of first refusal, reversionary interest, march-in right, retained right, field-of-use restriction, exclusivity restriction, obligation to assign, or obligation to license. Neither Seller nor any of its Subsidiaries has any royalty obligation or milestone obligation with respect to any Intellectual Property Assets. The Intellectual Property Assets, together with the Intellectual Property Rights licensed to Seller or any of its Subsidiaries under the Intellectual Property Agreements that will be assigned to Buyer at Closing, constitutes all of the Intellectual Property Rights owned, controlled or licensed by Seller or any of its subsidiaries and that are necessary or reasonably useful for or used by Seller or any of its Subsidiaries in the conduct of the Business. There exist no restrictions or limitations, on the ownership, disclosure, use, development, enforcement, license or other exploitation or transfer of the Intellectual Property Assets. The consummation of the transactions contemplated by this Agreement will not (i) alter, encumber, impair or extinguish any rights of Seller or any of its Subsidiaries under any Intellectual Property Assets or any Intellectual Property Agreement, (ii) impair or otherwise adversely affect the right of Buyer to develop, use, sell, license or otherwise exploit, transfer or dispose of, or to bring any Action for the infringement of, any Intellectual Property Assets or, to the extent such rights thereunder are currently held by Seller or a Subsidiary thereof, any Intellectual Property Agreements or (iii) through the operation of any Contracts to which Seller or any of its Subsidiaries is a party or otherwise bound, encumber any of the Intellectual Property Assets.

(d) Neither Seller nor any of its Subsidiaries has granted, and no Person holds, any license, sublicense, covenant not to sue, immunity, release, option or other right or interest in or to any Intellectual Property Assets, with respect to, or authorized the retention of any exclusive rights in, any Intellectual Property Assets; except for non-exclusive licenses granted to service providers and vendors in the ordinary course of business in connection with the Business.

(e) No funding, facilities, equipment, materials, data, know-how, or personnel, resources or other support of any Governmental Authority or any university, college, hospital, academic medical center, research institute, nonprofit institution or other educational or research institution has been or is being used, directly or indirectly to conceive, discover, reduce to practice, develop, test, manufacture, analyze, validate or otherwise create, in whole or in part, any Intellectual Property Assets, except for use of facilities or personnel that (i) does not result in such Governmental Authority or educational institution obtaining ownership of, or use rights to, such Intellectual Property Assets, and (ii) does not require or otherwise obligate Seller or any of its Subsidiaries to grant or offer to any such Governmental Authority or educational institution any license or other right to such Intellectual Property Assets. No current or former Employee or Independent Contractor who contributed to the creation or development of the Intellectual Property Assets has performed services for a Governmental Authority or any university, college, research institute or other educational institution related to the Business as presently conducted during a period of time during which such Employee or Independent Contractor was also performing services for Seller or any of its Subsidiaries. No Intellectual Property Asset is a 'subject invention' under the Bayh-Dole Act or similar applicable Law, and no Governmental Authority, university, college, hospital, academic medical center, research institute, nonprofit institution or other educational or research institution has or may have any ownership right, license, march-in right, manufacturing requirement, preference right, consent right, approval right, royalty right, revenue-sharing right or other right or interest in or to any Intellectual Property Asset. No Intellectual Property Asset, biological material, data, know-how or Trade Secret is subject to any publication, data-sharing, material-sharing, deposit, access, non-commercial-use, research-use, academic-use or similar obligation that would restrict or impair Buyer's ownership, use, development, manufacture, commercialization, enforcement, prosecution, maintenance, transfer or other exploitation thereof.

(f) Neither the execution, delivery or performance of this Agreement, nor the consummation of the transactions contemplated hereunder, will result in the loss, modification, impairment, acceleration, of or payment of any additional amounts with respect to, grant of any rights to any Person in respect of, nor require the consent, waiver, notice or other authorization of any other Person in respect of, Buyer's right to own, transfer or use or otherwise exploit any Intellectual Property Assets or any Intellectual Property Rights subject to any Intellectual Property Agreement.

(g) All of the Intellectual Property Assets are subsisting, and, to Seller's Knowledge, valid and enforceable. Except as set forth in Section 4.06(g)(1) of the Disclosure Schedules, all inventors of each Patent included in the Intellectual Property Assets have been correctly identified in accordance with applicable Law, all assignments

necessary to vest sole and exclusive ownership in Seller or a Subsidiary thereof have been duly executed and recorded with the applicable Governmental Authority, and no Person has asserted or has any basis to assert any inventorship, authorship, ownership or compensation claim with respect to any Intellectual Property Asset. Seller and its Subsidiaries have taken all reasonable steps to maintain and enforce the Intellectual Property Assets and to preserve the confidentiality of all Trade Secrets included in the Intellectual Property Assets, including by requiring all Persons having access thereto to execute binding, written non-disclosure agreements. All necessary registration, maintenance, and renewal fees due in connection with the prosecution and maintenance of the Intellectual Property Registrations have been timely paid, and all Intellectual Property Registrations are otherwise in good standing. Seller has provided Buyer with true, correct and complete copies of file histories, documents, certificates, office actions, correspondence and other materials related to all Intellectual Property Registrations. No Intellectual Property Registration is or has been subject to any interference, derivation proceeding, opposition, reissue, reexamination, post-grant review, inter partes review, nullity proceeding, cancellation proceeding or other Action challenging scope, ownership, inventorship, patentability, validity or enforceability. All necessary documents, recordations, and certificates in connection with the Intellectual Property Registrations have been filed with the relevant Patent, Copyright, Trademark or other authorities in the United States or foreign jurisdictions, as the case may be, for the purposes of maintaining the Intellectual Property Registrations registered in such jurisdictions. Except as set forth on Section 4.06(g)(2) of the Disclosure Schedules, there are no actions that must be taken by Seller or any of its Subsidiaries within 60 days of the Closing Date that, if not taken, will result in the loss of any of the Intellectual Property Registrations, including the payment of any registration, maintenance, or renewal fees or the filing of any responses to U.S. Patent and Trademark Office (or equivalent authority) actions, documents, applications or certificates for the purposes of obtaining, maintaining, perfecting or preserving or renewing any of the Intellectual Property Registrations. There has been no publication, presentation, poster, abstract, clinical trial disclosure, regulatory submission, offer for sale, sale, public use, disclosure, transfer or other act or omission by Seller, its Subsidiaries or, to Seller's Knowledge, any of their respective Representatives that has impaired or would reasonably be expected to impair the patentability, validity, enforceability, scope, ownership or value of any Intellectual Property Asset.

(h) To Seller's Knowledge (and determined taking into consideration any safe harbor, research exemption, government or executive declaration of urgent public health need, or similar right available in law or equity), and, except as set forth in Section 4.06(h) of the Disclosure Schedule, the conduct of the Business as currently and formerly conducted and, as currently contemplated to be conducted after Closing, including the use of the Intellectual Property Assets and the Intellectual Property Rights licensed under the Intellectual Property Agreements in connection with the Business, and the Company Products, processes, and services of the Business have not infringed, contributed to or induced the infringement of, misappropriated or otherwise violated and do not and will not infringe, contribute to or induce the infringement of, misappropriate, or otherwise violate, any Intellectual Property Rights or other rights of any Person in any respect. For clarity, any activity that Seller believed in good faith is permissible under the Hatch-Waxman

exemptions codified at 35 U.S.C. § 271(e)(1) or other applicable defenses or safe harbors, or that Seller reasonably believes in good faith will be permissible thereunder, shall not be considered an infringing activity for purposes of this Section 4.06(h). There is no, and since the Reference Date there has been no, Action pending or, to Seller's Knowledge, threatened against or affecting Seller or any of its Subsidiaries, or affecting the conduct of the Business as presently conducted (including the research, development and manufacture, as applicable, of any Company Product) (i) alleging that the use of any of the Intellectual Property Assets or any services provided, processes used or the Company Products manufactured, used, imported, offered for sale or sold by Seller or any of its Subsidiaries do or may conflict with, misappropriate, infringe, contribute to the infringement of or otherwise violate any Intellectual Property Right of any Person or (ii) alleging that Seller or any of its Subsidiaries have infringed, misappropriated or otherwise violated any Intellectual Property Right of any Person. Neither Seller nor any of its Subsidiaries have received from any Person any offer to license any Intellectual Property Rights in connection with any actual or threatened claim of infringement, misappropriation or other violation of any Intellectual Property Rights.

(i) There is no, and since the Reference Date there has been no Action pending or, to Seller's Knowledge, threatened against or affecting Seller or any of its Subsidiaries, or affecting the conduct of the Businesses by Seller or any of its Subsidiaries as presently conducted (i) based upon, or challenging or seeking to deny or restrict, any right of Seller or any of its Subsidiaries in any of the Intellectual Property Assets, or (ii) alleging that any of the Intellectual Property Assets is invalid, unenforceable or not infringed. To Seller's Knowledge, there is no information, materials, facts, or circumstances that would render any of the Intellectual Property Assets invalid or unenforceable, narrow or limit the scope of any Intellectual Property Asset, adversely affect any pending application for any of the Intellectual Property Asset, or adversely affect the ownership, patentability, registrability, validity, enforceability, scope, priority, term or value of any Intellectual Property Assets. To Seller's Knowledge, there are no facts or circumstances that would reasonably be expected to give rise to any such Action. Neither Seller nor any of its Subsidiaries are subject to any outstanding or prospective Governmental Order (including any motion or petition therefor) that does or would reasonably be expected to restrict or impair the use of any Intellectual Property Assets. To Seller's Knowledge, neither Seller nor any of its Subsidiaries has misrepresented, or failed to disclose, and there are no misrepresentations of or failure to disclose, any fact or circumstance in any application for any Intellectual Property Assets that would constitute fraud, inequitable conduct or a misrepresentation with respect to such application or that would otherwise affect the validity or enforceability of any such Intellectual Property Assets.

(j) None of the Intellectual Property Assets has been adjudged invalid or unenforceable in whole or part, or in the case of pending Patent applications included in the Intellectual Property Assets, have been the subject of a final and non-appealable finding of unpatentability. All issued Patents, registered Trademarks and registered Copyrights included in the Intellectual Property Assets are, to Seller's Knowledge, valid, enforceable, in full force and effect and subsisting in all material respects.

(k) To Seller's Knowledge, none of the Intellectual Property Agreements has been adjudged invalid or unenforceable in whole or part and no party to any Intellectual Property Agreement has asserted that any such Intellectual Property Agreement is invalid, unenforceable, terminated or subject to termination. All issued Patents, registered Trademarks and registered Copyrights included in the Intellectual Property Agreements are, to Seller's Knowledge, valid, enforceable, in full force and effect and subsisting in all material respects, and neither Seller nor any of its Subsidiaries has received any notice or other communication challenging the ownership, validity, enforceability, scope, priority or rights of Seller or any of its Subsidiaries under any such Intellectual Property Rights.

(l) To Seller's Knowledge, no Person has infringed, misappropriated, diluted, conflicted with or otherwise violated any Intellectual Property Asset or any Intellectual Property Right exclusively licensed to Seller or any of its Subsidiaries pursuant to an Intellectual Property Agreement and no facts or circumstances exist that would reasonably be expected to give rise to any such infringement, misappropriation, dilution, conflict or violation.

(m) Seller and its Subsidiaries have taken commercially reasonable actions in accordance with current industry practice to maintain the confidentiality of all Intellectual Property Assets, the value of which to Seller or its Subsidiaries is contingent upon maintaining the confidentiality thereof (including any Trade Secrets and confidential materials owned, used or held for use by Seller and its Subsidiaries), and no such Intellectual Property Assets have been disclosed, and access to such Intellectual Property Assets has not been provided to any Person, other than under written confidentiality agreements that protect such Intellectual Property Assets. To Seller's Knowledge, no Person, including Employee or Independent Contractor or agent of Seller or any of its Subsidiaries, is in default or breach of any confidentiality or non-disclosure agreement, or any confidentiality provisions of any employment agreement or other such agreement, with Seller or any of its Subsidiaries relating to the protection, ownership, development, use or transfer of any Intellectual Property Assets of Seller or any of its Subsidiaries.

(n) To the extent that any Intellectual Property Asset has been conceived, discovered, reduced to practice, developed, or created by a Third Party (including any current or former service provider) for Seller or a Subsidiary thereof, each such Third Party, as the case may be, has a valid, binding and enforceable written agreement with Seller with respect thereto pursuant to which such Third Party has agreed to hold all Intellectual Property Assets in confidence and to not use any such Intellectual Property Asset for any purpose other than for the benefit of Seller, and Seller thereby either (i) has obtained ownership of and is the exclusive owner of, or (ii) has obtained a valid and unrestricted right to exploit, sufficient for the conduct of the Business as currently conducted or as proposed to be conducted, such Intellectual Property Asset. In each case where Seller or a Subsidiary thereof has acquired any Intellectual Property Rights from any Person (including any service provider), Seller has obtained a present, valid, binding and enforceable assignment sufficient to irrevocably transfer all right, title and interest in and to such Intellectual Property Rights to Seller, or ownership in such Intellectual Property Rights are vested in Seller pursuant to applicable Law. No current or former Employee or Independent Contractor or other Person involved in the conception, discovery, reduction to

practice, development, manufacture, testing, analysis, validation or creation of any Intellectual Property Asset was or is subject to any obligation to any prior employer, university, institution, Governmental Authority or other Person that conflicts with or would reasonably be expected to impair the ownership of Seller or any of its Subsidiaries, or Buyer's rights in, any Intellectual Property Asset.

Section 4.07 Assigned Contracts.

(a) Section 4.07(a) of the Disclosure Schedules sets forth a true, complete and correct list of all Contracts of Seller or any of its Subsidiaries related primarily to the Purchased Assets, copies of which have been made available to Buyer.

(b) Each Assigned Contract is in full force and effect and is a valid and binding obligation of Seller or one of its Subsidiaries and, to the Knowledge of Seller, the other parties thereto in accordance with its terms and conditions, except as such validity and enforceability may be limited by (a) bankruptcy, insolvency, or other similar laws affecting the enforcement of creditors' rights generally, (b) equitable principles of general applicability (whether considered in a proceeding at law or in equity), and (c) the obligations to pay Cure Costs hereunder. Neither Seller nor any of its Subsidiaries have received written notice from any Person, and to Seller's Knowledge, no Person has threatened or intends to terminate or adversely modify in any material respect Seller's or any of its Subsidiaries' rights under any Assigned Contract. To the Knowledge of Seller, no event has occurred which, with the passage of time or the giving of notice, or both, would constitute a default under or a violation of any Assigned Contract or would cause the acceleration of any obligation of Seller or any of its Subsidiaries or the creation of a Lien upon any Purchased Asset. Seller has delivered or made available to Buyer true, correct and complete copies of all of the Assigned Contracts, together with all amendments, modifications or supplements thereto.

Section 4.08 Reserved.

Section 4.09 Inventory. The Inventory consists of a material quality and quantity usable in the ordinary course of business. All Inventory is owned by Seller or one of its Subsidiaries free and clear of all Liens (other than Permitted Liens). Section 4.09 of the Disclosure Schedules sets forth a listing of the finished Inventory owned, in the possession or control of, Seller or any of its Subsidiaries, including the address at which such Inventory is located. All of Inventory has been (i) to Seller's Knowledge, manufactured in compliance with Law, (ii) not adulterated or misbranded under the FD&C or other comparable laws, and (iii) to the extent administered to any subject or supplied to any site in clinical study, in material compliance with the applicable specifications with respect thereto in then-current IND(s).

Section 4.10 Compliance With Laws; Permits.

(a) Seller and each of its Subsidiaries are, and since the Reference Date have been, in compliance in all material respects with all applicable Laws, including with respect to all research, development and manufacturing of the Company Products (including the generation, preparation, maintenance, submission, and retention of all Regulatory

Documentation). Since the Reference Date, neither Seller nor any of its Subsidiaries has received any written notice of or, to Seller's Knowledge, is under investigation with respect to, any violation of any applicable Law. There is no judgment, decree, injunction, rule or order of any arbitrator or Governmental Authority outstanding against Seller or any of its Subsidiaries that relates to the Business, Purchased Assets or the transactions contemplated hereby.

(b) None of Seller or any of its Subsidiaries, or any of their respective directors, officers, consultants, agents or other Persons acting for or on its behalf, has taken any action that would result in a violation by such Person of (i) the Foreign Corrupt Practices Act of 1977 (15 U.S.C. §§ 78m(b), 78dd-1, 78dd-2, 78ff) (the "**FCPA**") or any other applicable anti-corruption or anti-bribery Law, (ii) any economic sanctions administered or enforced by the U.S. Department of Treasury's Office of Foreign Assets Control, the U.S. Department of State, His Majesty's Treasury or any applicable prohibited party list maintained by any U.S. Governmental Authority, the European Union or His Majesty's Treasury (collectively, "**Sanctions**") or (iii) any applicable Law relating to export controls. Seller and its Subsidiaries have conducted the Business in compliance with the FCPA (and any state or foreign equivalents) any other anti-corruption applicable Law (including the U.S. federal Anti-Kickback Statute (42 U.S.C. § 1320a-7b), the U.S. False Claims Act (31 U.S.C. § 3729 et seq.), and any state or foreign equivalents), Sanctions and applicable Laws relating to export controls, and, except as set forth on Section 4.10(b) of the Disclosure Schedules, Seller and its Subsidiaries have instituted and maintained policies and procedures designed to cause each such Person to comply with all such laws described in this sentence.

(c) Neither Seller nor any of its Subsidiaries, nor any of its or their respective officers, employees or agents, has been debarred or is subject to debarment pursuant to Section 306 of the FD&C Act or analogous provisions of Law outside the United States or is listed on any excluded list, and neither Seller nor any of its Subsidiaries has, to Seller's Knowledge, used in any capacity, in connection with the activities to be performed under this Agreement, any Person who has been debarred pursuant to Section 306 of the FD&C Act or analogous provisions of Law outside the United States or who is the subject of a conviction described in such Section or analogous provisions of Law outside the United States or is listed on any excluded list.

(d) Neither Seller nor any of its Subsidiaries, nor any of their respective directors, officers, consultants, agents or other Persons acting for or on its behalf, is a Person that is, or is owned or controlled by Persons that are (i) the subject of any Sanctions or (ii) located, organized or resident in a country or region that is the subject of Sanctions.

(e) Seller and its Subsidiaries hold all material Permits necessary to conduct the Business (including any IND) in the places and in such manner in which the Business is currently being conducted, and: (i) such Permits are valid and in full force and effect and are not subject to any pending or, to Seller's Knowledge, threatened Action by any Governmental Authority to suspend, cancel, modify, terminate or revoke any such Permit; (ii) Seller and its Subsidiaries are in compliance in all material respects with the terms and requirements of such Permits; and (iii) neither Seller nor any of its Subsidiaries is in

material default under, and, to Seller's Knowledge, no condition exists that with notice or lapse of time or both would constitute a material default under or would reasonably be expected to result in any suspension, cancellation, material modification, termination or revocation of, any such Permit.

(f) The Business does not (i) produce, design, test, manufacture, fabricate, or develop "critical technologies" as that term is defined in 31 C.F.R. § 800.215; (ii) perform the functions as set forth in column 2 of Appendix A to 31 C.F.R. part 800 with respect to covered investment critical infrastructure; or (iii) maintain or collect, directly or indirectly, "sensitive personal data" as that term is defined in 31 C.F.R. § 800.241; and, therefore, in turn, are not, collectively or individually, a "TID U.S. business" within the meaning of 31 C.F.R. § 800.248.

Section 4.11 Litigation.

(a) There is no Action before or by any Governmental Authority or arbitrator pending or, to the Knowledge of Seller, threatened against or affecting (i) the Business, the Purchased Assets or the Assumed Liabilities; or (ii) that challenge or seek to prevent, enjoin or otherwise delay the transactions contemplated by this Agreement. No event has occurred or circumstances exist that may give rise to, or serve as a basis for, any such Action.

(b) There are no outstanding Governmental Orders and no unsatisfied judgments, penalties or awards against, relating to or affecting the Business, the Purchased Assets or Assumed Liabilities.

Section 4.12 Privacy Matters.

(a) Seller and each of its Subsidiaries (and to Seller's Knowledge, each Third Party acting on behalf of Seller or any of its Subsidiaries) are and have been in material compliance with all Laws relating to privacy, data protection, information or cyber security, or the Processing of Personal Information by such Persons applicable to the operations of the Business ("**Privacy Laws**") and all associated contracts and policies. Seller and each of its Subsidiaries, as applicable, have provided all requisite notices, obtained all required consents, and satisfied all other requirements of Privacy Laws for their Processing of Personal Information that are necessary and in connection with the operation of the Business as of the date hereof and for the consummation of the transactions contemplated hereunder.

(b) Since the Reference Date, no claims, investigations, or alleged violations have been asserted or to Seller's Knowledge, threatened against Seller or any of its Subsidiaries by any Person regarding the Processing of Personal Information by or on behalf of Seller or any of its Subsidiaries or alleging a violation of Privacy Laws.

(c) Seller and each of its Subsidiaries maintain and, since the Reference Date, have maintained reasonable physical, administrative and technical security measures. To Seller's Knowledge, neither Seller nor any of its Subsidiaries has experienced (nor to

Seller's Knowledge, have any Third Party acting on behalf of Seller or any of its Subsidiaries) any actual or alleged Security Incidents. Neither Seller nor any of its Subsidiaries has (nor have any Third Parties acting on behalf of Seller or any of its Subsidiaries) notified, or been required to notify, any Person of any Security Incident.

Section 4.13 Employee Benefit Plans; Labor Matters.

(a) Seller has made available to Buyer a true, correct and complete list that sets forth, as of the date hereof: each Specified Individual, including each such Person's name, employer or engaging entity, job title, hire date, work location, current base salary or wage, commission or fee rate, most recent annual bonus received and current annual bonus or incentive opportunity, whether an employee or independent contractor, whether part-time or full-time, status as exempt or non-exempt under the Fair Labor Standards Act (if applicable), immigration status (for employees), whether such individual is in active service or on leave, if on leave, the nature of such leave and the date of expected return, and whether such person has entered into an Employment Agreement and/or Consulting Agreement. With respect to Specified Individuals who are former employees, such list will reflect the information as of the last day of employment as well as any post-employment (e.g., independent contractor) arrangements. Except as set forth on Section 4.13(a) of the Disclosure Schedules, the employment or engagement of each Specified Individual is terminable at will without compensation or other penalty. Seller has a completed a Form I-9 (Employment Eligibility Verification) for each Specified Individual who is an employee. None of Seller or its Subsidiaries utilize a PEO with respect to the Business. All Specified Individuals who are Independent Contractors are properly classified as non-employees pursuant to the Code and other applicable Law and the Seller and its Subsidiaries have no obligation or liability with respect to any Taxes (or the withholding thereof) or applicable employment law requirements in connection with any such Independent Contractors. No Independent Contractor has or would reasonably be expected to have any claim for benefits provided to employees under an Employee Plan with respect to service as an Independent Contractor.

(b) No Employee Plan is, and neither Seller nor any of its ERISA Affiliates has ever sponsored, maintained, administered, or contributed to (or has had any obligation to contribute to) or has or is reasonably expected to have any direct or indirect Liability with respect to: (i) a "defined benefit plan" (as defined in Section 3(35) of ERISA); (ii) a "multiemployer plan" (as defined in Sections 4001(a)(3) and 3(37)(A) of ERISA): (iii) a pension plan subject to Section 302 or Title IV of ERISA or Section 412 or 4971 of the Code; (iv) a "multiple employer welfare arrangement" within the meaning of Section 3(40) of ERISA or other applicable law; (v) a "voluntary employees' beneficiary association" within the meaning of Section 501(c)(9) of the Code or other funding arrangement for the provision of welfare benefits; or (vi) a welfare plan that offers health, life, or other disability benefits on a less-than-fully insured basis.

(c) Each Employee Plan, has been established, maintained, funded and administered in all material respects in compliance with its terms and with all applicable Law, including ERISA and the Code. No Action (other than routine claims for benefits) is pending against or involves or, to Seller's Knowledge, is threatened against or threatens to

involve, any Employee Plan. Seller and its Subsidiaries have complied in all material respects with applicable Law with respect to each plan, arrangement or policy mandated by applicable Law (including with respect to the payment of social insurance Taxes or similar contributions to a fund of a Governmental Authority with respect to wages of an employee).

(d) Except as would not result in material Liability for the Seller or its Subsidiaries, Seller and its Subsidiaries have paid all salaries, bonuses, commissions, wages, severance, accrued vacation pay, fees and any other amounts that have become due and payable to Employees, other Specified Individuals, and former employees of Seller or its Subsidiaries with respect to the Business, and any such amounts for any period ending on or before the Closing Date for services performed on or prior to the date hereof have been paid or accrued in full. With respect to the Employees, other Specified Individuals and former employee of Seller or its Subsidiaries with respect to the Business each of Seller and its Subsidiaries is and for the past three (3) years has been in material compliance with all applicable law governing the employment of labor, including all applicable law relating to wages, hours, affirmative action, collective bargaining, discrimination, civil rights, safety and health, exempt/non-exempt classification, classification of independent contractors, and workers' compensation.

(e) None of Seller or any of its Subsidiaries has, in the past three (3) years, been a party to or had any obligations under a collective bargaining, works council or similar agreement and no union or other collective bargaining unit or employee organizing entity has been certified or recognized by Seller or any of its Subsidiaries as representing any Employees. In the past three (3) years, none of Seller or any of its Subsidiaries has experienced, nor to Seller's Knowledge, is there now threatened, any union activity, any effort to organize or any other similar occurrence or any attempt to organize or represent the Employees. There are no controversies pending or threatened between Seller and its Subsidiaries, on the one hand, and any Employee or former employee of Seller or its Subsidiaries with respect to the Business, on the other hand. No investigation, review, complaint or proceeding by any Governmental Authority or Employee or former employee with respect to Seller or any of its Subsidiaries in relation to the employment of any such individual involved in the Business is pending or, to Seller's Knowledge, threatened, nor has Seller or any of its Subsidiaries received any notice from any Governmental Authority indicating an intention to conduct the same.

(f) Except as set forth in Section 4.13(f) of the Disclosure Schedules, neither the execution of this Agreement nor the consummation of the transactions contemplated hereby (either alone or together with any other event) will (i) entitle any Employee to any increased, new or accelerated payment or benefit, including any bonus, retention, severance, retirement or job security payment or benefit or (ii) result in the payment of any amount that could, individually or in combination with any other such payment, constitute an "excess parachute payment," as defined in 280G(b)(1) of the Code (without regard to subsection (b)(4) thereof).

(g) To the Seller's Knowledge, in the past three (3) years, (i) no allegations of sexual harassment or other sexual misconduct have been made since the Reference Date

against any Employee or other Specified Individual and (ii) neither Seller nor any of its Subsidiaries has entered into any settlement agreements related to allegations of sexual harassment or sexual misconduct by an Employee or other Specified Individual.

Section 4.14 Taxes.

(a) All material Tax Returns filed by, on behalf or in respect of the Business have been filed in accordance with applicable Tax Laws and are true, correct and complete in all material respects. No Governmental Authority has made a claim in writing that the Business or the Purchased Assets may be subject to Taxes, or that a Tax Return related to the Business or the Purchased Assets may be required to be filed, in a jurisdiction where no such Tax Returns have been filed.

(b) Subject to any obligation of Seller or any of its Subsidiaries under the Bankruptcy Code, all Taxes related to the Business or the Purchased Assets have been paid in full.

(c) Except as set forth on Section 4.14(c) of the Disclosure Schedules, there are no Liens in respect of Taxes with respect to any of the Purchased Assets, except for Liens for Taxes not yet due and payable that arise by operation of Law.

Section 4.15 Key Suppliers.

(a) Section 4.15(a) of the Disclosure Schedules sets forth a true, correct and complete list of the top 10 suppliers relating to the Business, measured by annual spend by Seller and its Subsidiaries on a consolidated basis, during the 12-month period ended as of March 31, 2026 ("**Key Suppliers**").

(b) No Key Supplier has terminated, suspended, cancelled or materially and adversely modified (i) its business relationship with Seller or any of its Subsidiaries or (ii) the terms of a Contract with Seller or any of its Subsidiaries. Neither Seller nor any of its Subsidiaries has received notice of any Key Supplier, and to Seller's Knowledge, no Key Supplier has threatened and Seller has no reason to believe that such Person intends, to terminate, suspend, cancel or materially and adversely modify its business relationship with Seller or any of its Subsidiaries or the terms of a Contract with Seller or any of its Subsidiaries. There are no unresolved claims or disputes pending between Seller or any of its Subsidiaries, on the one hand, and any Key Supplier, on the other hand.

Section 4.16 Insurance. Seller has made available to Buyer a true, correct and complete copy of all material insurance policies and all material self-insurance programs and arrangements and similar arrangements (including bonds) primarily relating to the Business and the Purchased Assets (the "**Insurance Policies**") and Section 4.16 of the Disclosure Schedules sets forth a true, correct and complete list of the Insurance Policies. As of the date of this Agreement: (a) each Insurance Policy is in full force and effect, all premiums thereon have been timely paid or, if not yet due, accrued; (b) there is no material claim pending under the Insurance Policies as to which coverage has been questioned, denied or disputed by the underwriters thereof; and (c) Seller and each of its Subsidiaries are in compliance in all material respects with the terms of the Insurance

Policies. Seller has no Knowledge of any threatened termination of, or material premium increase with respect to, any Insurance Policy. The Insurance Policies are sufficient for compliance in all material respects under applicable Law, all Permits and Contracts to which Seller or any of its Subsidiaries is a party or by which Seller or any of its Subsidiaries or any of their properties or assets are bound.

Section 4.17 Finders' Fees. Except as to Raymond James, whose fees are to be paid by Seller, there is no other investment banker, broker, finder or other intermediary that has been retained by or is authorized to act on behalf of Seller or any of its Subsidiaries who might be entitled to any fee or commission from any of Seller or any of its Subsidiaries in connection with any of the transactions contemplated by this Agreement.

Section 4.18 Certain Information. Seller has made available to Buyer true, correct and complete copies of all material information in its possession relating to each of (i) the STAAR Studies, (ii) any IND for any Company Product and (iii) each of Module 1, Module 2 and Module 3 of the rolling BLA for the Company Product.

Section 4.19 No Other Representations and Warranties. EXCEPT FOR THE REPRESENTATIONS AND WARRANTIES CONTAINED IN ARTICLE IV OF THIS AGREEMENT (AS MODIFIED BY THE DISCLOSURE SCHEDULES), THE SELLER'S CLOSING CERTIFICATE OR ANY ANCILLARY AGREEMENT, SELLER MAKES NO EXPRESS OR IMPLIED REPRESENTATION OR WARRANTY AND SELLER HEREBY DISCLAIMS ANY SUCH REPRESENTATION OR WARRANTY.

ARTICLE V
REPRESENTATIONS AND WARRANTIES OF BUYER

Buyer represents and warrants to Seller that the statements contained in this ARTICLE V are true and correct as of the date hereof and as of the Closing Date.

Section 5.01 Organization of Buyer. Buyer is a corporation duly organized, validly existing and in good standing under the Laws of Delaware.

Section 5.02 Authority of Buyer. Buyer has full corporate power and authority to execute and deliver this Agreement and the Ancillary Documents to which Buyer is a party, to carry out its obligations hereunder and thereunder and to consummate the transactions contemplated hereby and thereby. The execution and delivery by Buyer of this Agreement and any Ancillary Document to which Buyer is a party, the performance by Buyer of its obligations hereunder and thereunder and the consummation by Buyer of the transactions contemplated hereby and thereby have been duly authorized by Buyer and no other corporate proceedings on the part of Buyer are necessary to authorize the execution or delivery by Buyer of this Agreement and any Ancillary Document to which Buyer is a party, the performance by Buyer of its obligations under this Agreement and any Ancillary Document to which Buyer is a party or the consummation by Buyer of the transactions contemplated by this Agreement and any Ancillary Document to which Buyer is a party. This Agreement has been duly executed and delivered by Buyer and, assuming due authorization, execution and delivery by Seller, constitutes a valid and binding obligation of Buyer, enforceable against Buyer in accordance with its terms. When each Ancillary Document to which Buyer is or

will be a party has been duly executed and delivered by Buyer (assuming due authorization, execution and delivery by each other party thereto), such Ancillary Document will constitute a legal and binding obligation of Buyer enforceable against them in accordance with its terms.

Section 5.03 No Conflicts; Consents. The execution and delivery by Buyer of this Agreement and the Ancillary Documents to which it is a party, the performance by Buyer of its obligations under this Agreement and the Ancillary Documents to which it is a party and the consummation by Buyer of the transactions contemplated hereby and thereby, do not and will not (a) contravene, conflict with or result in any violation or breach of any provision of the organizational documents of Buyer, (b) contravene, conflict with or result in a violation or breach of any provision of any applicable Law, (c) require any consent or other action by any Person under, constitute a default, or an event that, with or without notice or lapse of time or both, would constitute a default, under, or cause or permit the termination, cancellation, acceleration or other change of any right or obligation or the loss of any benefit to which Buyer is entitled under any provision of any Contract binding on Buyer or any Permit affecting, or relating in any way to, the assets or business of Buyer or (d) result in the creation or imposition of any Lien on any asset of Buyer, with only such exceptions, in the case of each of clauses (c) and (d), as would not reasonably be expected to have, individually or in the aggregate with all other Effects, a Buyer Material Adverse Effect. No consent, approval, Permit, Governmental Order, declaration or filing with, or notice to, any Governmental Authority is required by or with respect to Buyer in connection with the execution and delivery of this Agreement or any of the Ancillary Documents and the consummation of the transactions contemplated hereby and thereby, except (i) as would not reasonably be expected to have, individually or in the aggregate, a Buyer Material Adverse Effect and (ii) as may be required to obtain clearance of this Agreement under the HSR Act.

Section 5.04 Finders' Fees. There is no investment banker, broker, finder or other intermediary that has been retained by or is authorized to act on behalf of Buyer who might be entitled to any fee or commission from Buyer upon consummation of the transactions contemplated by this Agreement.

Section 5.05 Available Funds. Buyer has, and at the Closing will have, funds immediately available that are sufficient to consummate the transactions contemplated by this Agreement and to perform its obligations under this Agreement. Such funds are not subject to any conditions or contingencies (including any financing condition) that would reasonably be expected to prevent or delay the availability of such funds at or prior to the Closing.

Section 5.06 Litigation. As of the date of this Agreement, there are no proceedings pending or, to the knowledge of Buyer, threatened against Buyer that seeks to enjoin the transactions contemplated by this Agreement, other than any such proceedings that have not had and would not have a Buyer Material Adverse Effect.

Section 5.07 Independent Investigation. Buyer acknowledge that it has conducted its own independent investigation and analysis of the Business, the Purchased Assets and the Assumed Liabilities, and that Buyer and its Representatives have received access to such books and records, facilities, equipment, contracts and other assets of Seller and its Subsidiaries that Buyer and its Representatives have desired or requested to review for such purpose.

Section 5.08 Non-Reliance; Purchased Assets.

(a) Buyer acknowledges for the benefit of Seller that it has conducted its own independent investigation and analysis of the business, operations, assets, liabilities, results of operations, condition (financial or otherwise) and prospects of the Business, the Purchased Assets and the Assumed Liabilities, and that it and its Representatives have received sufficient access to certain of the books and records, facilities, equipment, Contracts and other assets of the Business (including the Purchased Assets) for such purpose. Buyer further acknowledges and agrees for the benefit of Seller that, except for the representations and warranties contained in ARTICLE IV (as modified by the Disclosure Schedules), neither Seller nor any of its financial or other professional advisors, nor its or their respective Representatives, makes or has made any representation or warranty, either express or implied, or other statements or information concerning the Business, the Purchased Assets or the Assumed Liabilities in connection with the transactions contemplated by this Agreement and Seller hereby disclaims any other representations made by Seller or any other Person concerning the Business, the Purchased Assets or the Assumed Liabilities in connection with the transactions contemplated by this Agreement. Seller makes no representations or warranties regarding the probable success or profitability of the Business. Buyer has not relied on any representation, warranty or other statement by any Person on behalf of Seller, other than the representations and warranties of Seller expressly contained in ARTICLE IV (as modified by the Disclosure Schedules).

(b) Buyer agrees, warrants and represents that Buyer is purchasing the Purchased Assets "AS IS" and "WITH ALL FAULTS" based solely on the representations and warranties set forth in ARTICLE IV (as modified by the Disclosure Schedules) and Buyer's own investigation of Seller, the Purchased Assets, the Assumed Liabilities and the Business. Buyer further acknowledges that the consideration for the Purchased Assets specified in this Agreement has been agreed upon by Seller and Buyer after good-faith arms-length negotiation in light of Buyer's agreement to purchase the Purchased Assets "AS IS" and "WITH ALL FAULTS." Buyer agrees, warrants and represents that, except for the express representations and warranties set forth in ARTICLE IV (as modified by the Disclosure Schedules), Buyer has relied, and shall rely, solely upon its own investigation of all such matters, and that Buyer assumes all risks with respect thereto. EXCEPT AS SET FORTH IN ARTICLE IV (AS MODIFIED BY THE DISCLOSURE SCHEDULES), SELLER MAKES NO EXPRESS WARRANTY, NO WARRANTY OF MERCHANTABILITY, NO WARRANTY OF FITNESS FOR A PARTICULAR PURPOSE, AND NO IMPLIED OR STATUTORY WARRANTY WHATSOEVER WITH RESPECT TO ANY REAL OR PERSONAL PROPERTY OR ANY FIXTURES OR THE PURCHASED ASSETS, THE ASSUMED LIABILITIES OR THE BUSINESS.

ARTICLE VI
BANKRUPTCY COURT MATTERS

Section 6.01 Competing Transactions. Seller will not agree to any proposals or offers from any Third Party to acquire, directly or indirectly, all or any substantial portion of the Purchased Assets (a "**Competing Transaction**") other than as expressly permitted by and in

accordance with the Bidding Procedures. For the avoidance of doubt, neither (i) a liquidation or wind-down of Seller's estate nor (ii) Seller engaging in a transaction or transactions to divest all or any portion of an Excluded Business, shall be a Competing Transaction.

Section 6.02 Bankruptcy Court Filings.

(a) On the Petition Date, Seller filed a motion (the "**Sale Motion**") in the Chapter 11 Case requesting that the Bankruptcy Court: (i) (A) approve the Bidding Procedures and enter the Bidding Procedures Order, (B) establish procedures for the assumption and assignment of executory contracts and unexpired leases, (C) schedule a date for the Auction, (D) schedule a hearing on entry of the Sale Order, (E) approve the form and manner of notices related thereto, and (F) grant related relief; and (ii) (A) approve and authorize the sale contemplated in this Agreement free and clear of all Claims, Liens and Liabilities, (B) authorize the assumption and assignment of the Assigned Contracts, and (C) grant related relief. Seller shall take all actions as may be reasonably necessary to cause the Sale Order to be issued, entered and become a Final Order, including furnishing affidavits, declarations or other documents or information for filing with the Bankruptcy Court. Buyer agrees that it will promptly take such actions as are reasonably requested by Seller to assist in obtaining entry of the Sale Order and a finding of adequate assurance of future performance by Buyer, including furnishing affidavits or other documents or information for filing with the Bankruptcy Court for the purposes, among others, of providing necessary assurances of performance by Buyer under this Agreement and demonstrating that Buyer is a "good faith" purchaser under Section 363(m) of the Bankruptcy Code.

(b) Seller has provided appropriate notice of the hearings on the Sale Motion, as is required by the Bankruptcy Code and the Federal Rules of Bankruptcy Procedure, to all Persons entitled to notice, including all Persons that have expressed interest in buying the Purchased Assets, all Persons that have asserted Liens, claims or other interests in the Purchased Assets, all parties to the Assigned Contracts, all applicable state and local tax authorities, including the IRS, each Governmental Authority that is an interested party with respect to the Purchased Assets, and all Tax and environmental authorities in jurisdictions applicable to Seller. Seller shall be responsible for making all appropriate filings relating thereto with the Bankruptcy Court, which filings shall be submitted to Buyer within a reasonable time prior to their filing with the Bankruptcy Court to allow for Buyer's prior review and comments.

(c) Reserved.

(d) On July 14, 2026, the Bankruptcy Court entered the Bidding Procedures Order [Docket No. 122], approving the Bidding Procedures. Pursuant to the Bidding Procedures, on August 10, 2026, the Debtor held the Auction, at the conclusion of which on August 11, 2026, Seller designated Buyer as the Successful Bidder.

(e) Seller shall use its best efforts to obtain entry of the Sale Order on or before 50 days after the conclusion of the Auction.

(f) Seller shall consult with Buyer regarding pleadings and papers that it intends to file with the Bankruptcy Court in connection with, or which might reasonably affect the Bankruptcy Court's approval of the Sale Order, and any other orders of the Bankruptcy Court relating to the transactions contemplated by this Agreement, or the bankruptcy proceedings that may impact the transactions contemplated by this Agreement or the Purchased Assets, including sharing in advance any drafts thereof for Buyer's review and comment. Seller shall promptly provide Buyer and its counsel with copies of all notices, filings and orders of the Bankruptcy Court that Seller has in its possession (or receives) pertaining to the Sale Motion, or any other order related to any of the transactions contemplated in this Agreement.

(g) If the Sale Order, or any other orders of the Bankruptcy Court relating to this Agreement or the transactions contemplated in this Agreement are appealed by any Person (or if any petition for certiorari or motion for reconsideration, amendment, clarification, modification, vacation, stay, rehearing or reargument shall be filed with respect to the Bidding Procedures Order and the Sale Order, or other such order), subject to rights otherwise arising from this Agreement, Seller shall use its best efforts to prosecute or defend, as applicable, such appeal, petition or motion and obtain an expedited resolution of any such appeal, petition or motion.

(h) Notwithstanding anything expressed or implied herein to the contrary, Seller shall not consent or agree to the allowance of any claim to the extent it would constitute an Assumed Liability without the prior written consent of Buyer. Seller shall use its best efforts to cause the Sale Order to provide that Buyer will have standing in the Chapter 11 Case to object to the amount of any claim to the extent it would constitute an Assumed Liability and that the Bankruptcy Court will retain the right to hear and determine such objections.

ARTICLE VII
COVENANTS

Section 7.01 Conduct of Business Prior to the Closing.

(a) From the date hereof until the earlier of the Closing Date or the valid termination of this Agreement, except as otherwise required, authorized or restricted pursuant to this Agreement, the Bankruptcy Code, an order of the Bankruptcy Court (provided that Seller shall not seek or consent to, and shall oppose, any order of the Bankruptcy Court that would reasonably be expected to result in Seller violating its obligations under this Agreement), or as consented to in writing by Buyer, Seller shall (and shall cause its Subsidiaries to) use its commercially reasonable efforts to conduct the Business in the ordinary course of business.

(b) Without limiting the generality of Section 7.01(a), from the date hereof until the earlier of the Closing Date or the valid termination of this Agreement, Seller shall not (and shall cause its Subsidiaries not to):

(i) renew, enter into, amend, waive any right under, modify, assign, transfer or terminate (other than terminations in connection with (A) an expiration or (B) a renewal that becomes automatically effective unless a party thereto provides prior notice of an intention not to renew) any Contract to the extent relating to the Business or the Purchased Assets or Assumed Liabilities;

(ii) waive, release, amend, let lapse or assign any material rights, claims or benefits of Seller or any of its Subsidiaries relating to the Business, Purchased Assets or Assumed Liabilities, in each case, except as otherwise required, authorized or restricted pursuant to this Agreement, the Bankruptcy Code, or an order of the Bankruptcy Court (provided that Seller shall not seek or consent to, and shall oppose, any order of the Bankruptcy Court that would reasonably be expected to result in Seller violating its obligations under this Agreement);

(iii) sell, license, sublicense, grant rights of reference to, abandon, dedicate to the public, permit to lapse, disclose, convey, transfer, assign, divest, or otherwise dispose of any asset or property constituting Purchased Assets other than inventory and obsolete assets sold or otherwise disposed of in the ordinary course of business;

(iv) create or assume any mortgage or pledge, restriction, option, or impose any Lien on, or in any other way encumber, any asset or property constituting Purchased Assets, except for those that arise under any DIP Order that constitute Permitted Liens, or as required by applicable Law;

(v) acquire by merging or consolidating with, or by purchasing a substantial portion of the assets of any Person or otherwise acquire any assets that, as of the Closing, would constitute Purchased Assets, except for the acquisition of assets and inventory in the ordinary course of business;

(vi) take any action, or omit to take any action, that would reasonably be expected to result in a default under or material breach of any Assigned Contract, except as otherwise required, authorized or restricted pursuant to this Agreement, the Bankruptcy Code, an order of the Bankruptcy Court (provided that Seller shall not seek or consent to, and shall oppose, any order of the Bankruptcy Court that would reasonably be expected to result in Seller violating its obligations under this Agreement), or as consented to in writing by Buyer;

(vii) take any action, or omit to take any action, that would reasonably be expected to result in Seller or any of its Subsidiaries or any of its or their respective officers, employees or agents to be debarred or become subject to debarment pursuant to Section 306 of the FD&C Act or analogous provisions of Law outside the United States or is listed on any excluded list;

(viii) use in any capacity, in connection with activities to be performed under this Agreement, any Person who has been debarred pursuant to Section 306 of the FD&C Act or analogous provisions of Law outside the United States or who

is the subject of a conviction described in such Section or analogous provisions of Law outside the United States or is listed on any excluded list;

(ix) liquidate, dissolve, recapitalize or otherwise wind up its operations related to the Purchased Assets;

(x) cancel or alter any insurance coverage with respect to the Purchased Assets;

(xi) incur, commit to or make any material capital expenditure that would be an Assumed Liability;

(xii) incur any material indebtedness for borrowed money, enter into any capital lease or guarantee any such indebtedness, in each case to the extent such indebtedness would constitute an Assumed Liability;

(xiii) make any material loans, advances or capital contributions to, or investments in, any other Person with respect to the Business;

(xiv) commence, settle or propose to settle any material Actions that would reasonably be expected to materially diminish the value of the Purchased Assets or impair title thereto or result in an Assumed Liability, including any opposition, interference, derivation proceeding, inter partes review, post-grant review, reexamination, cancellation, audit, inquiry or proceeding that relates to, affects or would reasonably be expected to affect the ownership, scope, validity, enforceability, infringement, misappropriation, confidentiality, transferability or value of any Intellectual Property Assets;

(xv) abandon, allow to lapse, fail to prosecute, fail to maintain, fail to pay any required fees or annuities for, narrow, disclaim, dedicate to the public, license, sublicense, covenant not to sue under, encumber, disclose any material trade secret relating to, or otherwise impair any Intellectual Property Assets, or take any position in prosecution, enforcement or defense of any Intellectual Property Assets that would reasonably be expected to adversely affect the scope, validity, enforceability, ownership or value thereof;

(xvi) make, authorize or fail to prevent any filing, submission, response, amendment, argument, statement, disclaimer, terminal disclaimer, narrowing amendment, election, restriction, appeal decision, withdrawal, abandonment or other action in the prosecution, maintenance, enforcement, defense or ownership of any Intellectual Property Assets that would reasonably be expected to adversely affect the scope, validity, enforceability, ownership, priority, duration, patentability, registrability, confidentiality, exclusivity or value of such Intellectual Property Assets;

(xvii) make any change in accounting principles materially affecting the reporting of the Purchased Assets or the Business, other than as required by applicable Law or a Governmental Authority; or

(xviii) enter into a binding agreement or agree, whether in writing or otherwise, to do any of the foregoing;

in each case, except as otherwise required, authorized or restricted pursuant to this Agreement, the Bankruptcy Code, an order of the Bankruptcy Court (provided that Seller shall not seek or consent to, and shall oppose, any order of the Bankruptcy Court that would reasonably be expected to result in Seller violating its obligations under this Agreement), any DIP Order, or as consented to in writing by Buyer.

(c) Without limiting the generality of Section 7.01(a), from the date hereof until the earlier of the Closing Date or the valid termination of this Agreement, Seller shall (and shall cause its Subsidiaries to) use its commercially reasonable efforts to:

(i) preserve and maintain all Permits and Regulatory Documentation required for the conduct of the Business as currently conducted or the ownership and use of the Purchased Assets and all applications for Permits relating to the Business or any Purchased Asset;

(ii) pay the debts, Taxes and other obligations of the Business when due, subject to the filing of the Chapter 11 Case and good faith disputes over Taxes;

(iii) preserve and maintain the properties and assets included in the Purchased Assets in the same condition as they were on the date of this Agreement, subject to reasonable wear and tear and maintain and protect the confidentiality and trade secret status of all confidential information included in the Purchased Assets;

(iv) continue in full force and effect without modification all their insurance policies, except as required by applicable Law;

(v) defend and protect the properties and assets included in the Purchased Assets from infringement, misappropriation, misuse, unauthorized access, unauthorized disclosure, loss, corruption, destruction, impairment or usurpation;

(vi) perform all of its obligations under all Assigned Contracts;

(vii) maintain in effect all confidentiality, invention assignment, work-for-hire, data access, material transfer, laboratory, vendor, clinical site, investigator, research, development, manufacturing, consultant and other arrangements necessary to ensure that all Intellectual Property Assets, Company Product data, materials, records and other work product generated for or on behalf of Seller or any of its Subsidiaries with respect to any Company Product, the Business or the Purchased Assets are owned by, or validly assignable or transferable to, Seller and included in the Purchased Assets;

(viii) obtain or maintain valid, enforceable and transferable confidentiality, invention assignment, work-for-hire, data access, material transfer and similar agreements from each employee and Third Party who creates, conceives, reduces to practice, generates, accesses, uses or possesses any Intellectual Property Assets or other work product relating to the Purchased Assets;

(ix) maintain the Books and Records in accordance with past practice;

(x) comply in all material respects with all Laws applicable to the conduct of the Business or the ownership and use of the Purchased Assets; and

(xi) perform all of its obligations or Liabilities arising out of, in respect of or in connection with, any DIP Order;

except if otherwise required, authorized or restricted pursuant to this Agreement, the Bankruptcy Code, an order of the Bankruptcy Court or any DIP Order.

(d) Subject to Section 7.01(a), during the period from the date hereof until the Closing, Seller shall, and shall cause its Subsidiaries to, use its reasonable best efforts to (i) continue the STAAR Studies as currently contemplated, including the monitoring of patients enrolled in any ongoing clinical trials of Seller or any of its Subsidiaries relating to the Company Products; and (ii) perform or continue to perform the actions set forth in Schedule 7.01(d)(ii).

Section 7.02 Access to Information.

(a) From the date hereof until the earlier of the Closing Date and the termination of this Agreement in accordance with its terms, Seller (and shall cause its Subsidiaries to) (i) afford Buyer and its Representatives full and free access during normal business hours to the offices, properties, facilities, assets, books, records, service providers and agents of Seller and its Subsidiaries to the extent relating primarily to the Business, Purchased Assets or Assumed Liabilities; (ii) furnish Buyer and its Representatives with such financial and operating data and other information (including the work papers of Seller's and its Subsidiaries independent accountants upon receipt of any required consents from such accountants and subject to the execution of customary access letters) as such Persons may reasonably request to the extent primarily related to the Business, Purchased Assets or Assumed Liabilities as Buyer or any of their Representatives may reasonably request; and (iii) instruct the service providers and Representatives of Seller and its Subsidiaries to cooperate with Buyer in their investigation of the Business; provided, that any such access shall be afforded and any such information shall be furnished at Buyer's expense; provided, further, that Seller will not be required to provide Buyer or its Representatives with access to or to disclose information (A) that is prohibited from being disclosed pursuant to the terms of a confidentiality agreement with a third party entered into before the date hereof, (B) the disclosure of which would violate applicable Law or Order, or (C) the disclosure of which would cause the loss of any attorney-client privilege, attorney work product privilege or other legal privilege; provided, further, that with respect to the foregoing clauses (A) through (C) of the immediately foregoing proviso, Seller shall use

commercially reasonable efforts to (and use commercially reasonable efforts to cause its Subsidiaries to) (1) obtain the required consent of any third party to provide such disclosure and (2) develop an alternative to providing such information so as to address such matters that is reasonably acceptable to Seller. Any investigation pursuant to this Section 7.02(a) shall be conducted in such manner as not to interfere unreasonably with the conduct of the business of Seller or any of its Subsidiaries.

Section 7.03 Notice of Certain Events; Regulatory Submissions.

(a) From the date hereof until the earlier of the Closing Date and the termination of this Agreement in accordance with its terms, Seller shall promptly notify Buyer in writing of:

(i) any fact, circumstance, event or action the existence, occurrence or taking of which (A) has had, or would reasonably be expected to have, individually or in the aggregate, a Seller Material Adverse Effect, (B) has resulted in, or would reasonably be expected to result in, any representation or warranty made by Seller hereunder not being true and correct that would reasonably be expected to result in the failure of the condition set forth in Section 8.02(c) to be satisfied or (C) has resulted in, or would reasonably be expected to result in, the failure of any of the other conditions set forth in Section 8.02 to be satisfied;

(ii) any notice or other written communication from any Person alleging that the consent of such Person is required in connection with the transactions contemplated by this Agreement or the entry of the approval of any order by the Bankruptcy Court;

(iii) any notice or other written communication from any Governmental Authority in connection with the transactions contemplated by this Agreement;

(iv) any Actions commenced or, to Seller's Knowledge, threatened against, relating to or involving or otherwise affecting the Business, the Purchased Assets or the Assumed Liabilities that, if pending on the date of this Agreement, would have been required to have been disclosed pursuant to Section 4.06 or Section 4.11 or that relate to the consummation of the transactions contemplated by this Agreement; and

(v) any substantive or material communication from any Regulatory Authority regarding the Company Products, including any written or oral communications from the FDA or another Regulatory Authority related to any Regulatory Documentation or any clinical trial of the Company Products.

(b) Buyer's receipt of information pursuant to this Section 7.03 shall not operate as a waiver or otherwise affect any representation, warranty or agreement given or made by Seller in this Agreement and shall not be deemed to amend or supplement the Disclosure Schedules.

(c) Subject to applicable Law, Seller shall permit Buyer a reasonable opportunity to review in advance and comment on any substantive communication or submission by Seller or any of its Subsidiaries to any Regulatory Authority and permit Buyer (if permitted by the Regulatory Authority) the opportunity to attend any meeting or conference calls with or participate in any inspection by a Regulatory Authority related to the Company Products, and Seller shall consider in good faith any comments or other input timely provided by Buyer with respect to any such communication or submission prior to its submission to such Regulatory Authority.

(d) From the date hereof until the earlier of the Closing Date and the termination of this Agreement in accordance with its terms, Seller shall promptly provide Buyer true, correct and complete copies of all material information that comes into its possession relating to each of (i) the STAAR Studies, (ii) any IND for any Company Product and (iii) each of Module 1, Module 2 and Module 3 of the rolling BLA for the Company Product.

(e) The Parties, and their respective Affiliates, will use reasonable best efforts to take, or cause to be taken, any and all actions and to do, or cause to be done, any and all things necessary, proper or advisable under the HSR Act so as to obtain at the earliest practicable date all required consents and approvals under the HSR Act.

(f) Buyer and Seller shall as promptly as practicable and in any event not more than seven (7) Business Days after the date of this Agreement (or such later time as may be agreed to in writing by the Parties), make or cause to be made any filings required of each of them or any of their respective Affiliates under the HSR Act seeking early termination of any waiting period under the HSR Act applicable to the transactions contemplated hereunder. Buyer and Seller shall cooperate with each other in connection with any such filing and in connection with resolving any inquiry of any Governmental Authority under any applicable Laws with respect to any such filing or any such transaction, including making an appropriate response as promptly as practicable to any requests for additional information or documents by a Governmental Authority pursuant to the HSR Act. Each Party will be responsible for the costs and expenses associated with the preparation of its respective filing, and Buyer will be responsible for all filing fees related to the HSR Act. Each Party shall (i) provide the other Party reasonable advance notice of substantive submissions to any Governmental Authority and consider in good faith all reasonable comments or suggestions made by the other Party and (ii) promptly inform the other Party of any substantive oral communication with, and provide copies of substantive written communications with, any Governmental Authority regarding any such filing. Neither Buyer nor Seller shall independently participate in any substantive meeting with any Governmental Authority in respect of any such filing or other inquiry with respect to the transactions contemplated by this Agreement without giving the other prior notice of the meeting and, to the extent permitted by such Governmental Authority, the opportunity to attend and participate. Buyer and Seller will consult and cooperate with one another in connection with any analyses, appearances, presentations, memoranda, briefs, arguments, opinions and proposals made or submitted by or on behalf of any Party relating to proceedings under the HSR Act with respect to the transactions contemplated hereby. The Parties will undertake reasonable best efforts to share information protected from disclosure under the attorney-client privilege, work product doctrine, joint defense

privilege or any other privilege in a manner so as to preserve the applicable privilege. The Parties may reasonably designate any competitively sensitive or confidential business material provided to the other under this Section 7.03(f) as "counsel only" or, as appropriate, as "outside counsel only."

Section 7.04 Employees and Employee Benefits.

(a) Buyer shall, or shall cause one of its Affiliates to, offer to engage for services, either as an employee or independent contractor, at least 80% of the Persons set forth on Schedule 7.04(a) (each, a "**Specified Individual**") who continue to be employed as of immediately prior to Closing, which offers may be subject to customary screening procedures of Buyer (which may include background checks) and standard new hire or engagement agreements and other requirements used by Buyer and its Affiliates in the ordinary course.

(b) Seller and its Subsidiaries shall be solely responsible, and Buyer shall have no obligations whatsoever for, any compensation or other amounts payable to any current or former employee, officer, director, independent contractor or consultant of the Business, including, hourly pay, commission, bonus, salary, accrued vacation, fringe, pension or profit sharing benefits or severance pay for any period relating to the service with Seller and its Subsidiaries at any time on or prior to the Closing Date and, subject to applicable Law, Seller and its Subsidiaries shall pay all such amounts to all entitled persons as promptly as reasonably practicable after the Closing. Seller and its Subsidiaries shall bear any and all obligations and liability in connection with the termination of employment of any employees by Seller and its Subsidiaries, including under the WARN Act.

(c) Seller and its Subsidiaries shall remain solely responsible for the satisfaction of all claims for medical, dental, life insurance, health accident or disability benefits brought by or in respect of current or former employees, officers, directors, independent contractors or consultants of the Business or the spouses, dependents or beneficiaries thereof, which claims relate to events occurring on or prior to the Closing Date and for providing COBRA coverage for each individual who becomes an "M&A qualified beneficiary" (within the meaning of Treas. Reg. § 54.4980B-9) in connection with the transaction contemplated by this Agreement, until the expiration of such M&A qualified beneficiary's continuation rights under COBRA. Seller and its Subsidiaries also shall remain solely responsible for all worker's compensation claims of any current or former employees, officers, directors, independent contractors or consultants of the Business which relate to events occurring on or prior to the Closing Date. Seller and its Subsidiaries shall pay, or cause to be paid, all such amounts to the appropriate persons as and when due.

(d) Nothing herein express or implied by this Agreement shall confer upon any Employee or Specified Individual, or legal representative of any of them, any rights or remedies, including any right to employment or other service relationship or benefits, of any nature or kind whatsoever, under or by reason of this Agreement. The covenants contained in this Section 7.04 are agreements solely between the Seller and the Buyer for each other's benefit and nothing contained in this section shall be deemed to be or

construed as amending any Employee Plan or any other employee benefit plan, program or arrangement.

Section 7.05 Confidentiality. Seller shall, and shall cause its Affiliates to, hold, and shall use their best efforts to cause their respective Representatives to hold, in confidence any and all information, whether written or oral, concerning the Business, except to the extent that Seller can show that such information (a) is generally available to and known by the public through no fault of Seller, any of their Affiliates or their respective Representatives or (b) is lawfully acquired by Seller, any of their Affiliates or their respective Representatives from and after the Closing from sources which are not prohibited from disclosing such information by a legal, contractual or fiduciary obligation. Notwithstanding the foregoing, Seller shall be entitled to disclose (i) any information required to be disclosed by Seller to the Bankruptcy Court, the United States Trustee, parties in interest in the Chapter 11 Case, and other Persons bidding on assets of Seller, and (ii) any information required to be disclosed by Seller pursuant to any applicable Law (including the Bankruptcy Code), legal proceeding or Governmental Authority, provided that in each case, such disclosure shall be limited to the information that is so required to be disclosed and the Person(s) to whom such disclosure is required. Notwithstanding anything herein to the contrary, unless disclosure is required by applicable Law, the confidentiality of any Trade Secrets of the Business shall be maintained for so long as such Trade Secrets continue to be entitled to protection as Trade Secrets of the Business.

Section 7.06 Consents. Subject to the requirements of Section 2.05, Section 2.06, Section 6.02 and Section 7.03, Seller and Buyer shall use commercially reasonable efforts to give all notices to, and obtain all consents and approvals contemplated by this Agreement.

Section 7.07 Books and Records. Seller (or any subsequently appointed bankruptcy estate representative, including a Chapter 7 trustee, Chapter 11 trustee, liquidating trust, liquidating trustee, a plan administrator or any other successor entity pursuant to a Chapter 11 plan confirmed by the Bankruptcy Court) and Buyer agree that each of them shall preserve and keep the books and records held by it related to the pre-Closing Business for a period commencing on the date hereof and ending at such date on which an orderly wind-down of Seller's operations has occurred in the reasonable judgment of Buyer and Seller (or any subsequently appointed bankruptcy estate representative) and shall make such books and records available to the other Party (and permit any such other party to make extracts and copies of such books and records at its own expense) as may reasonably required by such other party in connection with, among other things, any insurance claims by, legal proceedings or Tax proceedings against or governmental investigations of Seller or Buyer or in order to enable Seller or Buyer to comply with their respective obligations under this Agreement and each Ancillary Document. In the event Seller, on the one hand, or Buyer, on the other hand, wish to destroy such records, the relevant party shall first give twenty days' prior written notice to the other party and such other party shall have the right at its option and expense, upon prior written notice given within that twenty day period, to take possession of the records within thirty days after the date of such notice.

Section 7.08 Public Announcements. Neither Seller nor Buyer shall issue any press release or public announcement concerning this Agreement or the transactions contemplated hereby without obtaining the prior written approval of the other Party, unless, disclosure is otherwise required by applicable Law (including federal or state securities Laws) or by the

Bankruptcy Court with respect to filings to be made with the Bankruptcy Court in connection with this Agreement, provided that the party intending to make such release shall use its best efforts consistent with such applicable Law or Bankruptcy Court requirement to consult with the other Party with respect to the text thereof. Notwithstanding the foregoing, Seller shall be permitted (unless otherwise required under Section 6.02(e)), without the prior written consent of Buyer, to make any commercially reasonable public announcement, press release, court filing, notice, or other public communication (each, a "**Bankruptcy Disclosure**") that is required by the Bankruptcy Court, any Order of the Bankruptcy Court (including the Bidding Procedures Order), or any provision of the Bankruptcy Code or the Federal Rules of Bankruptcy Procedure; or (ii) relates to the Auction (if any) to be conducted pursuant to the Bidding Procedures Order, the Bidding Procedures, the auction timeline or schedule, or the Sale Hearing, in each case solely as contemplated by or consistent with the Bidding Procedures Order.

Section 7.09 Further Assurances. Subject to the other provisions of this Agreement, each of Buyer and Seller shall use commercially reasonable efforts to (a) take all actions reasonably necessary or appropriate to consummate the transactions contemplated by this Agreement, (b) provide the other Party with reasonable cooperation and take such actions as such other parties may be reasonably request in connection with the consummation of the transactions contemplated herein, and (c) cause the fulfillment at the earliest practicable date of all of the conditions to its obligations to consummate the transactions contemplated herein. If requested by Buyer, Seller will transfer to Buyer the personnel records of any Employees who accept an offer of post-Closing employment with Buyer, except for such personnel records the disclosure of which would violate applicable Law.

Section 7.10 Wrong Pockets. If following the Closing either Buyer or Seller becomes aware that any of the Purchased Assets have not been transferred to Buyer or that any of the Excluded Assets have been transferred to Buyer, it shall promptly notify the other and the Parties shall, as soon as reasonably practicable thereafter, ensure that such property is transferred, at the expense of the party that is seeking the assets to be transferred to it, to (a) Buyer, in the case of any Purchased Assets which were not transferred to Buyer at or in connection with the Closing, or (b) Seller, in the case of any Excluded Assets which were transferred to Buyer at the Closing.

Section 7.11 No Successor Liability. The Parties intend that, except where expressly prohibited under applicable Law, upon the Closing, Buyer shall not be deemed to: (i) be the successor of Seller or any of its Subsidiaries, (ii) have, de facto, or otherwise, merged with or into Seller or any of its Subsidiaries, (iii) be a mere continuation or substantial continuation of Seller or any of its Subsidiaries or the enterprise(s) of Seller or any of its Subsidiaries, or (iv) be liable for any acts or omissions of Seller or any of its Subsidiaries in the conduct of the Business or arising under or related to the Purchased Assets other than as set forth in this Agreement. Without limiting the generality of the foregoing, and except as otherwise provided in this Agreement, the Parties intend that Buyer shall not be liable for any Lien (other than Assumed Liabilities and Permitted Liens) against Seller or any of its Subsidiaries or any of Seller's or any of its Subsidiaries' predecessors or Affiliates, and Buyer shall have no successor or vicarious liability of any kind or character whether known or unknown as of the Closing Date, whether now existing or hereafter arising, or whether fixed or contingent, with respect to the Business, the Purchased Assets or any Liabilities of Seller or any of its Subsidiaries arising prior to, or relating to any period

occurring prior to, the Closing Date. The Parties agree that the provisions substantially in the form of this section shall be reflected in the Sale Order.

Section 7.12 Transition Services Agreement. Following the date hereof, the Parties agree to negotiate the Transition Services Agreement in good faith consistent with the terms set forth on Exhibit D.

Section 7.13 Transfer Taxes. Buyer and Seller shall cooperate in good faith to minimize, to the extent permissible under applicable Law, the amount of any Transfer Taxes due with respect to the transactions contemplated herein. Seller shall, in good faith, and with the cooperative efforts of Buyer, attempt to obtain in the Sale Order an exemption from Transfer Taxes pursuant to Section 1146(a) of the Bankruptcy Code, provided, however, inclusion of such an exemption in the Sale Order is not a condition to Closing and a failure of the Sale Order to include such an exemption shall not be a breach of this Agreement.

ARTICLE VIII
CONDITIONS TO CLOSING

Section 8.01 Conditions to Obligations of All Parties. The obligations of each Party to consummate the transactions contemplated by this Agreement shall be subject to the fulfillment, at or prior to the Closing, of each of the following conditions:

(a) All waiting periods (and any extensions thereof) under the HSR Act applicable to the transactions contemplated hereunder shall have expired or been terminated.

(b) No Governmental Authority shall have enacted, issued, promulgated, enforced or entered any Governmental Order which is in effect and has the effect of making the transactions contemplated by this Agreement illegal, otherwise restraining or prohibiting consummation of such transactions or causing any of the transactions contemplated hereunder to be rescinded following completion thereof.

(c) The Bankruptcy Court shall have entered the Bidding Procedures Order and the Sale Order and each of such orders shall be a Final Order providing for a sale of the Purchased Assets free and clear of any Liens and this Agreement shall become effective in accordance with the terms of such orders.

Section 8.02 Conditions to Obligations of Buyer. The obligations of Buyer to consummate the transactions contemplated by this Agreement shall be subject to the fulfillment or Buyer's waiver, at or prior to the Closing, of each of the following conditions:

(a) Seller shall have complied with the sale process deadlines set forth in the Bidding Procedures Order and any order amending such Bidding Procedures Order in accordance with this Agreement.

(b) Seller shall have delivered to Buyer all the items set forth in Section 3.02(a).

(c) The representations and warranties made by Seller in this Agreement other than the Fundamental Representations shall be true and correct (disregarding for this purpose all qualifications or exceptions in such representations and warranties relating to materiality or Seller Material Adverse Effect), in each case as of the date hereof and as of the Closing Date, with the same force and effect as though all such representations and warranties had been made as of the Closing Date (other than representations and warranties that by their terms address matters only as of another specified date, which shall be so true and correct in all material respects only as of such other specified date), except where the failure of such representations and warranties to be so true and correct has not had and would not reasonably be expected to have, individually or in the aggregate, a Seller Material Adverse Effect; and the Fundamental Representations shall be true and correct in all material respects, in each case as of the date hereof and as of the Closing Date, with the same force and effect as though all such representations and warranties had been made as of the Closing Date (other than representations and warranties that by their terms address matters only as of another specified date, which shall be so true and correct in all material respects only as of such other specified date).

(d) Seller shall have duly performed and complied in all material respects with all agreements, covenants and conditions required by this Agreement to be performed or complied with by it prior to or on the Closing Date.

(e) Reserved.

(f) There shall not have occurred any Seller Material Adverse Effect.

(g) As of immediately prior to the Closing, at least 80% of the Persons set forth on Schedule 8.02(g) continue to be employed by or acting as a consultant to Seller or any of its Subsidiaries.

(h) All Liens relating to the Purchased Assets shall have been released in full, other than Permitted Liens that will be removed or released upon the Closing by operation of the Sale Order, and Seller shall have delivered to Buyer written evidence, in form satisfactory to Buyer in its sole discretion, of the release of such Liens.

(i) Buyer shall have received a certificate from Seller, dated the Closing Date and signed by a duly authorized officer of Seller, that each of the conditions set forth in Section 8.02(c), Section 8.02(d) and Section 8.02(f) have been satisfied (the "**Seller's Closing Certificate**").

(j) Buyer shall have received a certificate of an authorized officer of Seller certifying that attached thereto are true, correct and complete copies of all resolutions adopted by the board of directors of Seller authorizing the execution, delivery and performance of this Agreement and the Ancillary Documents and the consummation of the transactions contemplated hereby and thereby, and that all such resolutions are in full force and effect and are all the resolutions adopted in connection with the transactions contemplated hereby and thereby.

(k) Buyer shall have received a certificate of an authorized officer of Seller certifying the names and signatures of the officers of Seller authorized to sign this Agreement, the Ancillary Documents and the other documents to be delivered hereunder and thereunder.

(l) The Bankruptcy Court shall have entered an order, which may be the Sale Order, providing for the assumption and assignment of all Assigned Contracts to which Seller and any Essential Counterparty are parties, and such order shall have become a Final Order.

(m) Seller shall have delivered to Buyer a complete and unredacted copy of the Stalking Horse APA Disclosure Schedules.

Section 8.03 Conditions to Obligations of Seller. The obligations of Seller to consummate the transactions contemplated by this Agreement shall be subject to the fulfillment or Seller's waiver, at or prior to the Closing, of each of the following conditions:

(a) The representations and warranties made by Buyer in this Agreement shall be true and correct in all respects, in each case as of the date hereof and as of the Closing Date, with the same force and effect as though all such representations and warranties had been made as of the Closing Date (other than representations and warranties that by their terms address matters only as of another specified date, which shall be so true and correct only as of such other specified date), except where the failure of such representations and warranties to be true and correct would not have a Buyer Material Adverse Effect.

(b) Buyer shall have duly performed and complied in all material respects with all agreements, covenants and conditions required by this Agreement to be performed or complied with by it prior to or on the Closing Date.

(c) Seller shall have received a certificate, dated the Closing Date and signed by a duly authorized officer of Buyer, that each of the conditions set forth in Section 8.03(a) and Section 8.03(b) have been satisfied (the "**Buyer's Closing Certificate**").

(d) Buyer shall have delivered such other documents and deliveries set forth in Section 3.02(b).

ARTICLE IX
TERMINATION

Section 9.01 Termination. This Agreement may be terminated at any time prior to the Closing:

(a) by the mutual written consent of Seller and Buyer;

(b) by Buyer by written notice to Seller if:

(i) Buyer is not then in material breach of any provision of this Agreement and there has been a breach, inaccuracy in or failure to perform any

representation, warranty, covenant or agreement made by Seller pursuant to this Agreement that would give rise to the failure of any of the conditions specified in ARTICLE VIII and such breach, inaccuracy or failure is incapable of being cured by the End Date or if curable by the End Date, has not been cured by Seller within the earlier of (A) ten days of Seller's receipt of written notice of such breach from Buyer and (B) five Business Days prior to the End Date; or

(ii) the Closing has not occurred on or before October 15, 2026 (the "**End Date**"), unless the failure to close by such date shall be due to the failure of Buyer to perform or comply with any of the covenants, agreements or conditions hereof to be performed or complied with by Buyer prior to the Closing;

(c) by Seller by written notice to Buyer if:

(i) Seller is not then in material breach of any provision of this Agreement and there has been a breach, inaccuracy in or failure to perform any representation, warranty, covenant or agreement made by Buyer pursuant to this Agreement that would give rise to the failure of any of the conditions specified in ARTICLE VIII and such breach, inaccuracy or failure is incapable of being cured by the End Date or if curable by the End Date, has not been cured by Buyer within the earlier of (A) ten days of Buyer's receipt of written notice of such breach from Seller and (B) five Business Days prior to the End Date; or

(ii) the Closing has not occurred on or before the End Date, unless such failure shall be due to the failure of Seller to perform or comply with any of the covenants, agreements or conditions hereof to be performed or complied with by it prior to the Closing;

(d) by Buyer or Seller in the event that (i) there shall be any Law that makes consummation of the transactions contemplated by this Agreement illegal or otherwise prohibited or (ii) any Governmental Authority shall have issued a Governmental Order restraining or enjoining the transactions contemplated by this Agreement, and such Governmental Order shall have become final and non-appealable;

(e) by Buyer, if (i) Seller or any Affiliate of Seller seeks or otherwise takes material steps in furtherance of, or does not use commercially reasonable efforts to oppose any other Person in seeking, an order of the Bankruptcy Court dismissing the Chapter 11 Case or converting it to a case under Chapter 7 of the Bankruptcy Code, (ii) Seller or any Affiliate of Seller seeks or otherwise takes material steps in furtherance of, or does not use commercially reasonable efforts to oppose any other Person in seeking, the entry of an order by the Bankruptcy Court appointing a trustee in the Chapter 11 Case or an examiner with enlarged powers relating to the operation of the Business, (iii) the Bankruptcy Court abstains from hearing the Chapter 11 Case (or any subsequent chapter 7 case), (iv) the Bankruptcy Court orders, for any reason, an order of a type identified in clauses (i) through (iii) above, or (v) the Bankruptcy Court enters an order pursuant to Section 362 of the Bankruptcy Code lifting the automatic stay with respect to any material Purchased Assets;

(f) by Buyer, if (i) following entry by the Bankruptcy Court of the Bidding Procedures Order, such order is (A) amended, modified or supplemented in any manner adverse to Buyer without Buyer's prior written consent or (B) voided, reversed or vacated or is subject to a stay such that the Bidding Procedures Order is not in full force and effect as of the date set forth in Section 9.01(g) below or (ii) following entry by the Bankruptcy Court of the Sale Order, the Sale Order is (A) amended, modified or supplemented in any manner adverse to Buyer without Buyer's prior written consent or (B) voided, reversed or vacated or is subject to a stay such that the Sale Order is not in full force and effect as of the date set forth in Section 9.01(g) below;

(g) by Buyer, if (i) the Bankruptcy Court shall not have entered the Bidding Procedures Order on or before the earlier of seven Business Days after the Petition Date, as such date may be extended by mutual agreement of Seller and Buyer or (ii) an order of any court in any jurisdiction shall be entered relating to the Chapter 11 Case of Seller (x) staying for a period in excess of 10 days, vacating or reversing the Bidding Procedures Order or (y) materially amending, supplementing or otherwise modifying the Bidding Procedures Order in a manner that results in the Bidding Procedures Order no longer being reasonably acceptable to Buyer;

(h) by Buyer, if the Bankruptcy Court shall not have entered the Sale Order on or before the date that is 50 days after entry of the Bidding Procedures Order, or following entry thereof such order shall have been voided, reversed, vacated or made subject to a stay;

(i) by Buyer, if the Bidding Procedures Order is entered by the Bankruptcy Court and the Auction is not held on or before the date that is 42 days after entry of the Bidding Procedures Order, unless an Auction is not required to be held pursuant to the terms of the Bidding Procedures;

(j) by Buyer, if Seller withdraws or seeks authority to withdraw the Sale Motion; provided, however that this subsection shall not apply to any amendment of the Sale Motion by Seller in a manner that is not inconsistent with this Agreement;

(k) by Buyer, if Buyer is not selected as Successful Bidder or the Backup Bidder following the conclusion of the Auction;

(l) automatically, upon the consummation of a Competing Transaction;

(m) by Buyer, upon the occurrence of any event of default arising out of, in respect of or in connection with any DIP Order that has not been cured (if susceptible to cure under the terms of any DIP Order) or waived by any DIP lender in accordance with the terms of any such DIP Order;

(n) by Buyer or Seller, if the Bankruptcy Court enters an order denying approval of the Sale Order and such order shall have become a Final Order; or

(o) automatically, upon the consummation of a Plan Sponsor Alternative with respect to the Acquired Assets.

Section 9.02 Effect of Termination. In the event of the termination of this Agreement in accordance with this Article, this Agreement shall forthwith become void and there shall be no liability on the part of any Party except:

(a) as set forth in this ARTICLE IX and ARTICLE X hereof; and

(b) that nothing herein shall relieve any Party from liability for any breach of any provision hereof.

ARTICLE X
MISCELLANEOUS

Section 10.01 Notices. All notices, requests, consents, claims, demands, waivers and other communications hereunder shall be in writing (including electronic mail ("**e-mail**") transmission (to the extent that no "bounce back" or similar message indicating non-delivery is received with respect thereto)) and shall be deemed to have been given (a) when delivered by hand (with written confirmation of receipt); (b) when received by the addressee if sent by a nationally recognized overnight courier (receipt requested); (c) on the date sent by e-mail of a PDF document (with confirmation of transmission) if sent during normal business hours of the recipient, and on the next Business Day if sent after normal business hours of the recipient or (d) on the third day after the date mailed, by certified or registered mail, return receipt requested, postage prepaid. Such communications must be sent to the respective parties at the following addresses (or at such other address for a party as shall be specified in a notice given in accordance with this Section 10.01):

if to Buyer, to:

PTC Therapeutics, Inc.
500 Warren Corporate Center Drive
Warren, NJ 07059
Attention: Chief Legal Officer
E-mail: legal@ptcbio.com

with a copy (which shall not constitute notice) to:

Wilmer Cutler Pickering Hale and Dorr LLP
7 World Trade Center
250 Greenwich Street
New York, New York 10007
Attention: Brian A. Johnson; George W. Shuster, Jr.
E-mail: brian.johnson@wilmerhale.com; george.shuster@wilmerhale.com

if to Seller prior to the Closing Date, to:

Sangamo Therapeutics, Inc.
7000 Marina Blvd

Brisbane, CA 94005
Attention: Chief Legal Officer
E-mail: swilloughby@sangamo.com

with a copy (which shall not constitute notice) to:

Cooley LLP
55 Hudson Yards
New York, NY 10001

Attention: Cullen Speckhart
Lauren Reichardt
Lindsey O'Crump Crow

Email: cspeckhart@cooley.com
lreichardt@cooley.com
locrump@cooley.com

Section 10.02 No Survival of Representations and Warranties. The representations and warranties contained herein and in any certificate or other writing delivered pursuant hereto shall not survive the Closing Date. Each of Seller and Buyer agree that the covenants contained in this Agreement to be performed (i) prior to the Closing shall expire at the Closing and (ii) at or after the Closing will survive the Closing hereunder until the expiration of the applicable statute of limitations or for such shorter period explicitly specified therein, and each party to this Agreement will be liable to the other parties after the Closing for any breach thereof.

Section 10.03 Expenses. Except as otherwise expressly provided herein, all costs and expenses, including, fees and disbursements of counsel, financial advisors and accountants, incurred in connection with this Agreement and the transactions contemplated hereby shall be paid by the party incurring such costs and expenses.

Section 10.04 Entire Agreement. This Agreement and the Ancillary Documents constitute the entire agreement between the parties with respect to the subject matter contained herein and therein and supersede all prior agreements and understandings, both oral and written, between the parties with respect to such subject matter. In the event of any inconsistency between the statements in the body of this Agreement and those in the Ancillary Documents, the Exhibits and Disclosure Schedules (other than an exception expressly set forth as such in the Disclosure Schedules), the statements in the body of this Agreement will control.

Section 10.05 Headings. The headings in this Agreement are for reference only and shall not affect the interpretation of this Agreement.

Section 10.06 Severability. If any term or provision of this Agreement is invalid, illegal or unenforceable in any jurisdiction, such invalidity, illegality or unenforceability shall not affect any other term or provision of this Agreement or invalidate or render unenforceable such term or provision in any other jurisdiction. Upon such determination that any term or other provision is invalid, illegal or unenforceable, the Parties shall negotiate in good faith to modify this Agreement

so as to effect the original intent of the parties as closely as possible in a mutually acceptable manner in order that the transactions contemplated hereby be consummated as originally contemplated to the greatest extent possible.

Section 10.07 Binding Effect; Benefit; Assignment.

(a) The provisions of this Agreement shall be binding on and shall inure to the benefit of the Parties and their respective successors and assigns.

(b) No party may assign, delegate or otherwise transfer any of its rights or obligations under this Agreement without the prior written consent of each other Party, except that Buyer may transfer or assign its respective rights and obligations under this Agreement, in whole or from time to time in part, (i) to one or more of its Affiliates at any time, or (ii) as collateral to any financing sources at any time; provided that such transfer or assignment shall not relieve Buyer of its obligations hereunder or enlarge, alter or change any obligation of any other Party or due to Buyer or Seller (including the Milestone Payment(s)); provided, further, that in no event shall Seller assign any of its rights or obligations under this Agreement unless Seller also assigns all of its rights and obligations under all Ancillary Agreements to which it is party to the same assignee.

Section 10.08 No Third Party Beneficiaries. Nothing in this Agreement, express or implied, is intended to or shall confer upon any Person (other than the Parties) any right, benefit or remedy of any nature whatsoever under or by reason of this Agreement.

Section 10.09 Amendment and Waivers.

(a) Any provision of this Agreement may be amended or waived prior to the Closing Date if, but only if, such amendment or waiver is in writing and is signed, in the case of an amendment, by each party to this Agreement or, in the case of a waiver, by each party against whom the waiver is to be effective.

(b) No failure or delay by any party in exercising any right, power or privilege hereunder shall operate as a waiver thereof nor shall any single or partial exercise thereof preclude any other or further exercise thereof or the exercise of any other right, power or privilege. The rights and remedies herein provided shall be cumulative and not exclusive of any rights or remedies provided by applicable Law.

Section 10.10 Governing Law; Jurisdiction; Waiver of Jury Trial.

(a) THIS AGREEMENT IS TO BE GOVERNED BY AND CONSTRUED IN ACCORDANCE WITH FEDERAL BANKRUPTCY LAW, TO THE EXTENT APPLICABLE, AND WHERE STATE LAW IS IMPLICATED, THE INTERNAL LAWS OF THE STATE OF DELAWARE SHALL GOVERN, WITHOUT REGARD TO THE LAWS OF ANY OTHER JURISDICTION THAT MIGHT BE APPLIED BECAUSE OF CONFLICTS OF LAWS PRINCIPLES OF THE STATE OF DELAWARE.

(b) THE PARTIES AGREE THAT, DURING THE PERIOD FROM THE DATE HEREOF UNTIL THE DATE ON WHICH THE CHAPTER 11 CASE IS CLOSED

OR DISMISSED (THE "**BANKRUPTCY PERIOD**"), ANY SUIT, ACTION OR PROCEEDING SEEKING TO ENFORCE ANY PROVISION OF, OR BASED ON ANY MATTER ARISING OUT OF OR IN CONNECTION WITH, THIS AGREEMENT OR THE TRANSACTIONS CONTEMPLATED HEREBY SHALL BE BROUGHT EXCLUSIVELY IN THE BANKRUPTCY COURT. THE PARTIES FURTHER AGREE THAT, FOLLOWING THE BANKRUPTCY PERIOD, ANY SUIT, ACTION OR PROCEEDING WITH RESPECT TO THIS AGREEMENT OR THE TRANSACTIONS CONTEMPLATED HEREBY SHALL BE BROUGHT AGAINST ANY OF THE PARTIES EXCLUSIVELY IN EITHER THE UNITED STATES DISTRICT COURT FOR THE DISTRICT OF DELAWARE OR ANY STATE COURT OF THE STATE OF DELAWARE AND EACH OF THE PARTIES HEREBY IRREVOCABLY CONSENTS TO THE JURISDICTION OF SUCH COURT (AND OF THE APPROPRIATE APPELLATE COURTS THEREFROM) AND, DURING THE BANKRUPTCY PERIOD, THE BANKRUPTCY COURT (AND OF THE APPROPRIATE APPELLATE COURTS THEREFROM) IN ANY SUCH SUIT, ACTION OR PROCEEDING, AND IRREVOCABLY WAIVES, TO THE FULLEST EXTENT PERMITTED BY LAW, ANY OBJECTION THAT IT MAY NOW OR HEREAFTER HAVE TO THE LAYING OF THE VENUE OF ANY SUCH SUIT, ACTION OR PROCEEDING IN SUCH COURTS OR THAT ANY SUCH SUIT, ACTION OR PROCEEDING WHICH IS BROUGHT IN SUCH COURTS HAS BEEN BROUGHT IN AN INCONVENIENT FORUM. PROCESS IN ANY SUCH SUIT, ACTION OR PROCEEDING MAY BE SERVED ON ANY PARTY ANYWHERE IN THE WORLD, WHETHER WITHIN OR WITHOUT THE JURISDICTION OF THE BANKRUPTCY COURT, THE UNITED STATES DISTRICT COURT FOR THE DISTRICT OF DELAWARE OR ANY STATE COURT OF THE STATE OF DELAWARE. WITHOUT LIMITING THE FOREGOING, EACH PARTY AGREES THAT SERVICE OF PROCESS ON SUCH PARTY IN THE MANNER AS PROVIDED IN SECTION 10.01 FOR NOTICES SHALL BE DEEMED EFFECTIVE SERVICE OF PROCESS ON SUCH PARTY.

(c) EACH OF THE PARTIES HEREBY IRREVOCABLY WAIVES ANY AND ALL RIGHT TO TRIAL BY JURY IN ANY LEGAL PROCEEDING ARISING OUT OF OR RELATED TO THIS AGREEMENT OR THE TRANSACTIONS CONTEMPLATED HEREBY.

Section 10.11 Specific Performance. The Parties agree that irreparable damage would occur if any provision of this Agreement were not performed in accordance with the terms hereof, that money damages or other legal remedies would not be an adequate remedy for any such damage, and that the parties shall be entitled to an injunction or injunctions to prevent breaches of this Agreement or to enforce specifically the performance of the terms and provisions hereof (without proof of damages) in any federal court located in the State of Delaware or any Delaware state court, in addition to any other remedy to which they are entitled at law or in equity. Each party agrees that no other party or Person shall be required to obtain, furnish, post or provide any bond or other security or instrument in connection with any remedy referred to in this Section 10.11, and each party irrevocably waives any right that it may have to require the obtaining, furnishing, posting or provision of any such bond or other security or instrument. If, prior to the End Date, any party brings any action, suit or proceeding in accordance with this Section 10.11 to enforce specifically the performance of the terms and provisions hereof by any other party, the End

Date shall automatically be extended by (i) the amount of time during which such action, suit or proceeding is pending, plus 20 Business Days or (ii) such other time period established by the court presiding over such action, suit or proceeding, as the case may be.

Section 10.12 Counterparts; Effectiveness. This Agreement may be signed manually or by facsimile or other electronic transmission by the parties (including in .pdf, .tiff, .jpg or similar format), in any number of counterparts, each of which shall be an original, with the same effect as if the signatures thereto and hereto were upon the same instrument. This Agreement shall become effective when each Party shall have received a counterpart hereof signed by all of the other Parties. Until and unless each party has received a counterpart hereof signed by the other Party, this Agreement shall have no effect and no party shall have any right or obligation hereunder (whether by virtue of any other oral or written agreement or other communication).

Section 10.13 No Recourse.

(a) Notwithstanding anything in this Agreement or in any other Ancillary Document, the Parties hereby acknowledge and agree that, except to the extent a Person is a named party to this Agreement, and then only to the extent of the specific obligations of such Parties set forth in this Agreement, no Person, including any current, former or future director, officer, employee, incorporator, member, manager, partner, investor, shareholder, agent, Representative, or Affiliate of any (collectively, the "**Non-Recourse Parties**"), shall have any liability hereunder or in connection herewith, and each Party shall have no recourse against, any Non-Recourse Party or any other Person other than the other Party in connection with any liability, claim or cause of action arising out of, or in relation to, this Agreement, any other Ancillary Document or the transactions contemplated hereby and thereby, other than in the case of fraud.

(b) Effective as of the Closing, except for any rights or obligations under this Agreement and the other Ancillary Documents, each of Seller and Buyer (each a "**Releasing Party**"), to the fullest extent permissible under the Law, mutually releases and discharges each other Releasing Party and such Releasing Parties' respective current and former predecessors, successors, Affiliates (regardless of whether such interests are held directly or indirectly), assigns, subsidiaries, direct and indirect equity holders, funds, portfolio companies, management companies, current, and former directors, officers, members, employees, partners, managers, independent contractors, agents, representatives, principals, professionals, consultants, financial advisors, attorneys, accountants, investment bankers, advisory board members, investment advisors, and other professionals, each in their capacity as such (collectively, in such capacity, the "**Released Parties**" and each a "**Released Party**"), from any and all claims, interests, obligations, rights, suits, damages, causes of action, remedies, and liabilities of every kind, nature and description whatsoever, which such Releasing Party ever had, now has or may have on or by reason of any matter, cause or thing, including any derivative claims that such Releasing Party (or someone on its behalf) would have been legally entitled to assert, whether known or unknown, foreseen or unforeseen, existing or hereinafter arising, in law, equity, or otherwise, that such Releasing Party would have been legally entitled to assert in their own right (whether individually or collectively) or on behalf of a holder of any claim against a Releasing Party, based on or relating to, or in any manner arising from, in whole or in part,

Seller, the purchase, sale, or rescission of Seller, the subject matter of, or the transactions or events giving rise to, any claim, Seller's in- or out-of-court restructuring efforts, intercompany transactions, the transactions contemplated hereby, entry into this Agreement, the Chapter 11 Case, the formulation, preparation, dissemination, negotiation, filing, or consummation of the transactions contemplated hereby, this Agreement, or any restructuring transaction, Contract, instrument, release, or other agreement or document created or entered into in connection with the transactions contemplated hereby, this Agreement, the filing of the Chapter 11 Case, the pursuit of the transactions contemplated hereby, or upon any other related act or omission, transaction, agreement, event, or other occurrence taking place on or before the Closing Date related or relating to the foregoing; provided, however, that notwithstanding the foregoing, no claims of Buyer arising under or related to that certain License Agreement between Buyer and Seller dated as of December 18, 2024, as amended from time to time, shall be released or discharged pursuant to this Section 10.13.

(c) By its receipt of the Purchase Price hereunder, Seller shall have received reasonably equivalent value for the Purchased Assets sold or otherwise conveyed to the Buyer under this Agreement.

[SIGNATURE PAGE FOLLOWS]

IN WITNESS WHEREOF, the Parties have caused this Agreement to be executed as of the date first written above by their respective officers thereunto duly authorized.

PTC THERAPEUTICS, INC.

By: /s/ Mark E. Boulding
Name: Mark E. Boulding
Title: Executive Vice President and Chief Legal Officer

IN WITNESS WHEREOF, the Parties have caused this Agreement to be executed as of the date first written above by their respective officers thereunto duly authorized.

SANGAMO THERAPEUTICS, INC.

By: /s/ Dr. Alexander Macrae
Name: Dr. Alexander Macrae, M.B., Ch.B., Ph.D.
Title: Chief Executive Officer

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: October 29, 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: October 29, 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 September 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: October 29, 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 September 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: October 29, 2026

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