Table of Contents

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2023

or

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

For the transition period from to

Commission file number: 001-35969

PTC Therapeutics, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**04-3416587**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
100 Corporate Court South Plainfield, NJ	**07080**
(Address of principal executive offices)	(Zip Code)

(908) 222-7000

(Registrant's telephone number, including area code)

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

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

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

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

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

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

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

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

As of October 24, 2023, there were 75,463,145 shares of Common Stock, $0.001 par value per share, outstanding.

TABLE OF CONTENTS
PTC Therapeutics, Inc.

FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Quarterly Report on Form 10-Q, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "might," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

The forward-looking statements in this Quarterly Report on Form 10-Q include, among other things, statements about:

- our ability to negotiate, secure and maintain adequate pricing, coverage and reimbursement terms and processes on a timely basis, or at all, with third-party payors for our products or product candidates that we commercialize or may commercialize in the future;
- our ability to maintain our conditional marketing authorization of Translarna™ (ataluren) for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, in the European Economic Area, or EEA, and our marketing authorizations in other jurisdictions in which Translarna has been approved, including whether the European Medicines Agency, or EMA, determines in the re-examination process of the Committee for Medicinal Products for Human Use's negative opinion that the benefit-risk balance of Translarna authorization supports renewal of such authorization;
- our ability to utilize results from Study 041 to support a renewal of the conditional marketing authorization for Translarna for the treatment of nmDMD in the EEA and to support a marketing approval for Translarna for the treatment of nmDMD in the United States;
- expectations with respect to our ability to commercialize Upstaza™ (eladocagene exuparvovec) for the treatment of Aromatic L-Amino Acid Decarboxylase, or AADC deficiency, in the EEA, any potential regulatory submissions and potential approvals for our product candidates, our manufacturing capabilities and the potential financial impact and benefits of our leased biologics manufacturing facility and the potential achievement of development, regulatory and sales milestones and contingent payments that we may be obligated to make;
- the anticipated period 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 expectations with respect to the commercial status of Evrysdi® (risdiplam) and our program directed against spinal muscular atrophy in collaboration with F. Hoffmann La Roche Ltd and Hoffmann La Roche Inc. and the Spinal Muscular Atrophy Foundation and our estimates regarding future revenues from sales-based royalty payments or the achievement of milestones in that program;
- our expectations and the potential financial impact and benefits related to our Collaboration and License Agreement with a subsidiary of Ionis Pharmaceuticals, Inc. including with respect to the timing of regulatory approval of Tegsedi® (inotersen) and Waylivra™ (volanesorsen) in countries in which we are licensed to commercialize them, the commercialization of Tegsedi and Waylivra, and our expectations with respect to royalty payments by us based on our potential achievement of certain net sales thresholds;
- the timing and scope of our commercialization of our products and product candidates;
- our estimates regarding the potential market opportunity for our products or product candidates, including the size of eligible patient populations and our ability to identify such patients;
- our ability to obtain additional and maintain existing reimbursed named patient and cohort early access programs for our products on adequate terms, or at all;

- our estimates regarding expenses, future revenues, third-party discounts and rebates, capital requirements and needs for additional financing, including our ability to maintain the level of our expenses consistent with our internal budgets and forecasts and to secure additional funds on favorable terms or at all;
- the timing and conduct of our ongoing, planned and potential future clinical trials and studies in our splicing, metabolic, Bio-e and oncology programs as well as studies in our products for maintaining authorizations, label extensions and additional indications, including the timing of initiation, enrollment and completion of the trials and the period during which the results of the trials will become available;
- our ability to realize the anticipated benefits of our acquisitions or other strategic transactions, including the possibility that the expected impact of benefits from the acquisitions or strategic transactions will not be realized or will not be realized within the expected time period, significant transaction costs, the integration of operations and employees into our business, our ability to obtain marketing approval of our product candidates we acquired from the acquisitions or other strategic transactions and unknown liabilities;
- the rate and degree of market acceptance and clinical utility of any of our products or product candidates;
- the ability and willingness of patients and healthcare professionals to access our products and product candidates through alternative means if pricing and reimbursement negotiations in the applicable territory do not have a positive outcome;
- the timing of, and our ability to obtain additional marketing authorizations for our products and product candidates;
- the ability of our products and our product candidates to meet existing or future regulatory standards;
- our ability to complete Study 041, a multicenter, randomized, double-blind, 18-month, placebo-controlled clinical trial of Translarna for the treatment of nmDMD followed by an 18-month open-label extension, according to the protocol agreed with the EMA;
- the potential receipt of revenues from future sales of our products or product candidates;
- our sales, marketing and distribution capabilities and strategy, including the ability of our third-party manufacturers to manufacture and deliver our products and product candidates in clinically and commercially sufficient quantities and the ability of distributors to process orders in a timely manner and satisfy their other obligations to us;
- our ability to establish and maintain arrangements for the manufacture of our products and product candidates that are sufficient to meet clinical trial and commercial launch requirements;
- the extent, timing and financial aspects of our strategic pipeline prioritization and reductions in workforce;
- our ability to complete any post-marketing requirements imposed by regulatory agencies with respect to our products;
- our expectations with respect to the potential financial impact and benefits of our leased biologics manufacturing facility and our ability to satisfy our obligations under the terms of the lease agreement for such facility;
- our ability to satisfy our obligations under the indenture governing our 1.50% convertible senior notes due September 15, 2026;
- our regulatory submissions, including with respect to timing and outcome of regulatory review;
- our plans to advance our earlier stage programs and pursue research and development of other product candidates, including our splicing, metabolic, Bio-e and oncology programs;

- our expectations with respect to the COVID-19 pandemic and related response measures and their effects on our business, operations, clinical trials, potential regulatory submissions and approvals, our collaborators, contract research organizations, suppliers and manufacturers;
- whether we may pursue business development opportunities, including potential collaborations, alliances, and acquisition or licensing of assets and our ability to successfully develop or commercialize any assets to which we may gain rights pursuant to such business development opportunities;
- the potential advantages of our products and any product candidate;
- our intellectual property position;
- the impact of government laws and regulations;
- the impact of litigation that has been or may be brought against us or of litigation that we are pursuing against others; and
- our competitive position.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this Quarterly Report on Form 10-Q, particularly in Part II, Item 1A. Risk Factors as well as in Part I, Item 1A. Risk Factors in our Annual Report on Form 10-K for the year ended December 31, 2022, 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, 2022 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, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 165,157	$ 279,834
Marketable securities	129,653	130,871
Trade and royalty receivables, net	165,718	155,614
Inventory, net	35,752	21,808
Prepaid expenses and other current assets	44,082	105,658
Total current assets	540,362	693,785
Fixed assets, net	85,031	72,590
Intangible assets, net	423,342	705,891
Goodwill	82,341	82,341
Operating lease ROU assets	94,072	102,430
Deposits and other assets	34,737	48,582
Total assets	$ 1,259,885	$ 1,705,619
Liabilities and stockholders' deficit		
Current liabilities:		
Accounts payable and accrued expenses	$ 370,731	$ 320,366
Deferred revenue	1,224	1,351
Operating lease liabilities- current	11,703	9,370
Finance lease liabilities- current	2,611	3,000
Liability for sale of future royalties- current	105,849	72,149
Total current liabilities	492,118	406,236
Long-term debt	573,174	571,722
Contingent consideration payable	39,000	164,000
Deferred tax liability	51,927	102,834
Operating lease liabilities- noncurrent	99,682	100,860
Finance lease liabilities- noncurrent	17,184	18,675
Liability for sale of future royalties- noncurrent	657,469	685,737
Other long-term liabilities	141	2,641
Total liabilities	1,930,695	2,052,705
Stockholders' deficit:		
Common stock, $0.001 par value. Authorized 250,000,000 shares; issued and outstanding 75,459,022 shares at September 30, 2023. Authorized 250,000,000 shares; issued and outstanding 73,104,692 shares at December 31, 2022.	75	72
Additional paid-in capital	2,447,292	2,305,020
Accumulated other comprehensive income	9,609	4,796
Accumulated deficit	(3,127,786)	(2,656,974)
Total stockholders' deficit	(670,810)	(347,086)
Total liabilities and stockholders' deficit	$ 1,259,885	$ 1,705,619

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,	
	2023	2022	2023	2022
Revenues:				
Net product revenue	$ 144,038	$ 134,186	$ 506,187	$ 407,720
Collaboration revenue	—	50,017	6	50,024
Royalty revenue	50,173	32,924	117,857	73,645
Manufacturing revenue	2,365	—	6,716	—
Total revenues	196,576	217,127	630,766	531,389
Operating expenses:				
Cost of product sales, excluding amortization of acquired intangible assets	9,493	14,011	36,368	33,785
Amortization of acquired intangible assets	58,649	31,023	145,461	80,790
Research and development	164,212	165,462	545,210	462,802
Selling, general and administrative	80,886	80,118	256,249	233,280
Change in the fair value of contingent consideration	1,500	(5,300)	(125,000)	(32,200)
Intangible asset impairment	—	—	217,800	—
Total operating expenses	314,740	285,314	1,076,088	778,457
Loss from operations	(118,164)	(68,187)	(445,322)	(247,068)
Interest expense, net	(28,160)	(20,880)	(84,905)	(66,371)
Other expense, net	(20,266)	(38,141)	(8,832)	(84,355)
Loss before income tax benefit	(166,590)	(127,208)	(539,059)	(397,794)
Income tax benefit	33,620	17,893	68,247	9,666
Net loss attributable to common stockholders	$ (132,970)	$ (109,315)	$ (470,812)	$ (388,128)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	75,377,997	71,654,671	74,618,611	71,415,849
Net loss per share—basic and diluted (in dollars per share)	$ (1.76)	$ (1.53)	$ (6.31)	$ (5.43)

See accompanying unaudited notes.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Net loss	$ (132,970)	$ (109,315)	$ (470,812)	$ (388,128)
Other comprehensive income (loss):				
Unrealized gain (loss) on marketable securities, net of tax	19	736	470	(2,333)
Foreign currency translation gain, net of tax	11,021	25,218	4,343	62,820
Comprehensive loss	$ (121,930)	$ (83,361)	$ (465,999)	$ (327,641)

See accompanying unaudited notes.

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

Three months ended September 30, 2023	Common stock		Additional paid-in capital	Accumulated other comprehensive (loss) income	Accumulated deficit	Total stockholders' deficit
	Shares	Amount				
Balance, June 30, 2023	75,318,233	$ 75	$ 2,416,904	$ (1,431)	$ (2,994,816)	$ (579,268)
Exercise of options	113,795	—	3,446	—	—	3,446
Restricted stock vesting and issuance, net	26,994	—	—	—	—	—
Share-based compensation expense	—	—	26,942	—	—	26,942
Net loss	—	—	—	—	(132,970)	(132,970)
Comprehensive income	—	—	—	11,040	—	11,040
Balance, September 30, 2023	75,459,022	$ 75	$ 2,447,292	$ 9,609	$ (3,127,786)	$ (670,810)

Three months ended September 30, 2022	Common stock		Additional paid-in capital	Accumulated other comprehensive income	Accumulated deficit	Total stockholders' deficit
	Shares	Amount				
Balance, June 30, 2022	71,505,889	$ 71	$ 2,184,230	$ 10,251	$ (2,376,770)	$ (182,218)
Exercise of options	331,379	—	10,005	—	—	10,005
Restricted stock vesting and issuance, net	17,624	—	—	—	—	—
Share-based compensation expense	—	—	28,670	—	—	28,670
Net loss	—	—	—	—	(109,315)	(109,315)
Comprehensive income	—	—	—	25,954	—	25,954
Balance, September 30, 2022	71,854,892	$ 71	$ 2,222,905	$ 36,205	$ (2,486,085)	$ (226,904)

Nine months ended September 30, 2023	Common stock		Additional paid-in capital	Accumulated other comprehensive income	Accumulated deficit	Total stockholders' deficit
	Shares	Amount				
Balance, December 31, 2022	73,104,692	$ 72	$ 2,305,020	$ 4,796	$ (2,656,974)	$ (347,086)
Exercise of options	806,407	1	23,770	—	—	23,771
Restricted stock vesting and issuance, net	773,157	1	—	—	—	1
Issuance of common stock in connection with an employee stock purchase plan	117,304	—	3,805	—	—	3,805
Issuance of common stock in connection with a milestone payable	657,462	1	29,569	—	—	29,570
Share-based compensation expense	—	—	85,128	—	—	85,128
Net loss	—	—	—	—	(470,812)	(470,812)
Comprehensive income	—	—	—	4,813	—	4,813
Balance, September 30, 2023	75,459,022	$ 75	$ 2,447,292	$ 9,609	$ (3,127,786)	$ (670,810)

Nine months ended September 30, 2022	Common stock		Additional paid-in capital	Accumulated other comprehensive (loss) income	Accumulated deficit	Total stockholders' equity (deficit)
	Shares	Amount				
Balance, December 31, 2021	70,828,226	$ 71	$ 2,123,606	$ (24,282)	$ (2,097,957)	$ 1,438
Exercise of options	456,399	—	13,203	—	—	13,203
Restricted stock vesting and issuance, net	479,004	—	—	—	—	—
Issuance of common stock in connection with an employee stock purchase plan	91,263	—	3,107	—	—	3,107
Share-based compensation expense	—	—	82,989	—	—	82,989
Net loss	—	—	—	—	(388,128)	(388,128)
Comprehensive income	—	—	—	60,487	—	60,487
Balance, September 30, 2022	71,854,892	$ 71	$ 2,222,905	$ 36,205	$ (2,486,085)	$ (226,904)

See accompanying unaudited notes.

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

	Nine Months Ended September 30,	
	2023	2022
Cash flows from operating activities		
Net loss	$ (470,812)	$ (388,128)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	155,885	89,971
Non-cash operating lease expense	8,156	7,505
Non-cash royalty revenue related to sale of future royalties	(50,599)	(31,617)
Non-cash interest expense on liability related to sale of future royalties	56,031	55,778
Intangible asset impairment	217,800	—
Change in valuation of contingent consideration	(125,000)	(32,200)
Unrealized loss on ClearPoint Equity Investments	3,107	1,113
Unrealized loss on ClearPoint convertible debt security	4,594	2,392
Unrealized (gain) loss on marketable securities- equity investments	(5,484)	11,319
Realized loss on the sale of Clearpoint Equity Investment	782	—
Non-cash stock consideration, milestone payment	29,570	—
Disposal of asset	135	81
Deferred income taxes	(50,909)	—
Amortization of (discounts) premiums on investments, net	(89)	1,792
Amortization of debt issuance costs	1,574	1,316
Share-based compensation expense	85,128	82,989
Unrealized foreign currency transaction losses, net	3,394	71,246
Changes in operating assets and liabilities:		
Inventory, net	(14,121)	(768)
Prepaid expenses and other current assets	59,467	(35,615)
Trade and royalty receivables, net	(9,887)	(24,773)
Deposits and other assets	4,597	(3,752)
Accounts payable and accrued expenses	40,434	1,568
Other liabilities	(1,756)	(908)
Deferred revenue	(127)	—
Net cash used in operating activities	$ (58,130)	$ (190,691)
Cash flows from investing activities		
Purchases of fixed assets	$ (22,872)	$ (23,394)
Purchases of marketable securities- available for sale	—	(40,429)
Purchases of marketable securities- equity investments	(26,378)	(22,787)
Sale and redemption of marketable securities- available for sale	21,544	341,990
Sale and redemption of marketable securities- equity investments	12,078	104,431
Sale and redemption of ClearPoint Equity Investments	2,594	—
Acquisition of product rights and licenses	(69,285)	(102,069)
Net cash (used in) provided by investing activities	$ (82,319)	$ 257,742
Cash flows from financing activities		
Proceeds from exercise of options	$ 23,771	$ 13,203
Repayment of Convertible Notes	—	(150,000)
Proceeds from employee stock purchase plan	3,805	3,107
Debt issuance costs related to senior secured term loan	(282)	—
Payment of finance lease principal	(1,379)	(1,276)
Net cash provided by (used in) financing activities	$ 25,915	$ (134,966)
Effect of exchange rate changes on cash	19	(8,792)
Net decrease in cash and cash equivalents	(114,515)	(76,707)
Cash and cash equivalents, and restricted cash beginning of period	295,925	197,218
Cash and cash equivalents, and restricted cash end of period	$ 181,410	$ 120,511
Supplemental disclosure of cash information		
Cash paid for interest	$ 34,020	$ 12,679
Cash paid for income taxes	13,631	4,029
Supplemental disclosure of non-cash investing and financing activity		
Unrealized gain (loss) on marketable securities, net of tax	$ 470	$ (2,333)
Right-of-use assets obtained in exchange for operating lease obligations	—	35,294
Acquisition of product rights and licenses	44,963	27,828
Milestone payable	2,500	50,000

See accompanying unaudited notes.

PTC Therapeutics, Inc.

Notes to Consolidated Financial Statements (unaudited)
September 30, 2023
In thousands (except share and per share amounts unless otherwise noted)

1. The Company

PTC Therapeutics, Inc. (the "Company" or "PTC") is a science-driven global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. PTC's ability to innovate to identify new therapies and to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. PTC's mission is to provide access to best-in-class treatments for patients who have little to no treatment options. PTC's strategy is to leverage its strong scientific and clinical expertise and global commercial infrastructure to bring therapies to patients. PTC believes that this allows it to maximize value for all of its stakeholders.

PTC has a 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, metabolism and oncology.

The Company has two products, Translarna™ (ataluren) and Emflaza® (deflazacort), for the treatment of Duchenne muscular dystrophy ("DMD"), a rare, life threatening disorder. Translarna has marketing authorization in the European Economic Area (the "EEA") for the treatment of nonsense mutation Duchenne muscular dystrophy ("nmDMD") in ambulatory patients aged 2 years and older and in Russia for the treatment of nmDMD in patients aged two years and older. Translarna also has marketing authorization in Brazil for the treatment of nmDMD in ambulatory patients two years and older and for continued treatment of patients that become non-ambulatory. In July 2020, the European Commission ("EC") approved the removal of the statement "efficacy has not been demonstrated in non-ambulatory patients" from the indication statement for Translarna. Emflaza is approved in the United States for the treatment of DMD in patients two years and older.

The Company's marketing authorization for Translarna in the EEA is subject to annual review and renewal by the EC following reassessment by the European Medicines Agency ("EMA") of the benefit-risk balance of the authorization, which the Company refers to as the annual EMA reassessment. In September 2022, the Company submitted a Type II variation to the EMA to support conversion of the conditional marketing authorization for Translarna to a standard marketing authorization, which included a report on the placebo-controlled trial of Study 041 and data from the open-label extension. In February 2023, the Company also submitted an annual marketing authorization renewal request to the EMA. In September 2023, the Committee for Medicinal Products for Human Use ("CHMP'), gave a negative opinion on the conversion of the conditional marketing authorization to full marketing authorization of Translarna for the treatment of nmDMD and a negative opinion on the renewal of the existing conditional marketing authorization of Translarna for the treatment of nmDMD. The Company has requested re-examination of the CHMP opinion regarding the renewal of the existing conditional marketing authorization. In re-examination, the Company intends to address concerns raised during the previous review process on the benefit demonstrated in Translarna clinical trials and regarding the robustness of the Strategic Targeting of Registries and International Database of Excellence, or STRIDE, real world registry. In accordance with EMA guidelines, the Company expects the CHMP opinion following the re-examination process to occur in late January 2024, with EC ratification of the opinion within the following 67 days.

Translarna is an investigational new drug in the United States. Following the Company's announcement of top-line results from the placebo-controlled trial of Study 041 in June 2022, the Company submitted a meeting request to the U.S. Food and Drug Administration ("FDA") to gain clarity on the regulatory pathway for a potential re-submission of a New Drug Application ("NDA") for Translarna. The FDA provided initial written feedback that Study 041 does not provide substantial evidence of effectiveness to support an NDA re-submission. The Company then had an informal meeting with the FDA, during which the Company discussed the potential path to an NDA re-submission for Translarna. Based on the meeting discussion, the Company has scheduled an additional Type C meeting with the FDA to review the totality of data

collected to date, including dystrophin and other mechanistic data as well as additional analyses that could support the benefit of Translarna.

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. The Company is also preparing a biologics license application ("BLA") for Upstaza for the treatment of AADC deficiency in the United States and anticipates submitting the BLA shortly after its pre-BLA meeting that is scheduled for December 2023, pending the outcome of such meeting.

The Company holds the rights for the commercialization of Tegsedi® (inotersen) and Waylivra® (volanesorsen) for the treatment of rare diseases in countries in Latin America and the Caribbean pursuant to the Collaboration and License Agreement (the "Tegsedi-Waylivra Agreement"), dated August 1, 2018, by and between the Company and Akcea Therapeutics, Inc. ("Akcea"), a subsidiary of Ionis Pharmaceuticals, Inc. Tegsedi has received marketing authorization in the United States, the European Union (the "EU") and Brazil for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis ("hATTR amyloidosis"). The Company began to make commercial sales of Tegsedi for the treatment of hATTR amyloidosis in Brazil in the second quarter of 2022 and it continues to make Tegsedi available in certain other countries within Latin America and the Caribbean through early access programs ("EAP Programs"). In August 2021, ANVISA, the Brazilian health regulatory authority, approved Waylivra as the first treatment for familial chylomicronemia syndrome ("FCS") in Brazil and the Company began to make commercial sales of Waylivra in Brazil in the third quarter of 2022 while continuing to make Waylivra available in certain other countries within Latin America and the Caribbean through EAP Programs. In December 2022, ANVISA approved Waylivra for the treatment of familial partial lipodystrophy ("FPL"). Waylivra has also received marketing authorization in the EU for the treatment of FCS.

The Company also has a spinal muscular atrophy ("SMA") collaboration with F. Hoffman-La Roche Ltd and Hoffman-La Roche Inc. (referred to collectively as "Roche") and the Spinal Muscular Atrophy Foundation ("SMA Foundation"). The SMA program has one approved product, Evrysdi® (risdiplam), which was approved by the FDA in August 2020 for the treatment of SMA in adults and children two months and older and by the EC in March 2021 for the treatment of 5q SMA in patients two months and older with a clinical diagnosis of SMA Type 1, Type 2 or Type 3 or with one to four SMN2 copies. Evrysdi also received marketing authorization for the treatment of SMA in Brazil in October 2020 and Japan in June 2021. 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 PTC518, which is being developed for the treatment of Huntington's disease ("HD"). The Company initiated a Phase 2 study of PTC518 for the treatment of HD in the first quarter of 2022, which consists of an initial 12-week placebo-controlled phase focused on safety, pharmacology and pharmacodynamic effects followed by a nine-month placebo-controlled phase focused on PTC518 biomarker effect. In June 2023, the Company announced interim data from the 12-week placebo-controlled phase. The Company expects the next data update from the Phase 2 study of PTC518 for the treatment of HD in the first half of 2024. In the Phase 2 study, enrollment outside of the United States remains active and ongoing while enrollment within the United States is paused as the FDA requested additional data to allow the Phase 2 study to proceed. The Company had a Type A meeting with the FDA to review the clinical safety data needed to enable resumption of enrollment in the United States. At that meeting, the FDA stated that the existing three-month safety data could support 12-week dosing and that six months of clinical safety data could support dosing in the 12-month Phase 2 study.

The most advanced molecule in the Company's metabolic platform is sepiapterin, a precursor to intracellular tetrahydrobiopterin, which is a critical enzymatic cofactor involved in metabolism and synthesis of numerous metabolic products, for orphan diseases. In May 2023, the Company announced that the primary endpoint was achieved in its registration-directed Phase 3 trial for sepiapterin for phenylketonuria ("PKU"). The primary endpoint of the study was the achievement of statistically-significant reduction in blood Phe level. The Company participated in a pre-NDA meeting with the FDA in the third quarter of 2023. At that meeting, the FDA stated that the sepiapterin clinical safety and efficacy

data supported NDA submission for the treatment of pediatric and adult PKU patients. However, the FDA has requested that PTC completes a 26-week nonclinical mouse study to assess sepiapterin carcinogenicity potential prior to NDA submission. PTC expects to submit an NDA to the FDA for sepiapterin for the treatment of PKU by the end of the third quarter of 2024 and PTC intends to discuss with the FDA the potential for an earlier submission if PTC is permitted to submit the 26-week mouse study report during the review process of the NDA. Additionally, PTC expects to submit a marketing authorization application ("MAA") to the EMA for sepiapterin for the treatment of PKU in the EEA in the first half of 2024.

The Company's Bio-e platform consists of small molecule compounds that target oxidoreductase enzymes that regulate oxidative stress and inflammatory pathways central to the pathology of a number of CNS diseases. The two most advanced molecules in the Company's Bio-e platform are vatiquinone and utreloxastat. The Company announced topline results from a registration-directed Phase 3 trial of vatiquinone in children and young adults with Friedreich ataxia in May 2023. While the study did not meet its primary endpoint of statistically significant change, the Company participated in a Type C written response only meeting with the FDA in the third quarter of 2023 to discuss the potential for an NDA submission for vatiquinone for the treatment of Friedreich ataxia. In their written response, the FDA stated that while they see the value of upright stability as a clinically meaningful endpoint, they believed a confirmatory study would likely be needed to support NDA submission. PTC has requested a follow-up live meeting to address the issues raised by the FDA. Additionally, PTC is participating in a scientific advice procedure with the EMA to determine if the data from the registration-directed Phase 3 trial of vatiquinone in children and young adults with Friedreich ataxia could support a conditional MAA in the EEA. The Company initiated a Phase 2 trial of utreloxastat for amyotrophic lateral sclerosis in the first quarter of 2022 and enrollment is ongoing.

Unesbulin is the Company's most advanced oncology agent. The Company completed its Phase 1 trials evaluating unesbulin in leiomyosarcoma ("LMS") and diffuse intrinsic pontine glioma ("DIPG") in the fourth quarter of 2021. The Company initiated a registration-directed Phase 2/3 trial of unesbulin for the treatment of LMS in the first quarter of 2022 and enrollment is ongoing. The Company is evaluating its plans for a potential initiation of a registration-directed Phase 2/3 trial of unesbulin for the treatment of DIPG.

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, 2023, the Company had an accumulated deficit of approximately $3,127.8 million. The Company has financed its operations to date primarily through the private offerings in September 2019 of 1.50% convertible senior notes due 2026 (see Note 9), public offerings of common stock in February 2014, October 2014, April 2018, January 2019, and September 2019, "at the market offering" of its common stock, its initial public offering of common stock in June 2013, proceeds from the Royalty Purchase Agreement dated as of July 17, 2020, by and among the Company, RPI 2019 Intermediate Finance Trust ("RPI"), and, solely for the limited purposes set forth therein, Royalty Pharma PLC (the "Royalty Purchase Agreement") (see Note 2), net proceeds from the Company's' borrowings under its credit agreement with Blackstone (see Note 9), private placements of its convertible preferred stock and common stock, collaborations, bank and institutional lender debt, other convertible debt, grant funding and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease area addressed by the Company's product candidates. The Company has also relied on revenue generated from net sales of Translarna for the treatment of nmDMD in territories outside of the United States since 2014, Emflaza for the treatment of DMD in the United States since 2017, and Upstaza for the treatment of AADC deficiency in the EEA since May 2022. The Company has also relied on revenue associated with milestone and royalty payments from Roche pursuant to the License and Collaboration Agreement (the "SMA License Agreement") dated as of November 23, 2011, by and among the Company, Roche and, for the limited purposes set forth therein, the SMA Foundation, under its SMA program. The Company expects that cash flows from the sales of its products, milestone and royalty payments from Roche, together with the Company's cash, cash equivalents and marketable securities, will be sufficient to fund its operations for at least the next twelve months.

2. Summary of significant accounting policies

The Company's complete listing of significant accounting policies is set forth in Note 2 of the notes to the Company's audited financial statements as of December 31, 2022 included in the Company's Annual Report on Form 10-K filed with the Securities and Exchange Commission (the "SEC") on February 21, 2023 (the "2022 Form 10-K"). Selected significant accounting policies are discussed in further detail below.

Basis of presentation

The accompanying financial information as of September 30, 2023 and for the three and nine months ended September 30, 2023 and 2022 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, 2022 and notes thereto included in the 2022 Form 10-K.

In the opinion of management, the unaudited financial information as of September 30, 2023 and for the three and nine months ended September 30, 2023 and 2022 reflects all adjustments, which are normal recurring adjustments, necessary to present a fair statement of financial position, results of operations, stockholders' (deficit) equity, and cash flows. The results of operations for the three and nine months ended September 30, 2023 are not necessarily indicative of the results to be expected for the year ended December 31, 2023 or for any other interim period or for any other future year.

Use of estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. Significant estimates in these consolidated financial statements have been made in connection with the calculation of net product sales, royalty revenue, certain accruals related to the Company's research and development expenses, valuation procedures for liability for sale of future royalties, valuation procedures for convertible notes, fair value of the contingent consideration, and the provision for or benefit from income taxes. Actual results could differ from those estimates. Changes in estimates are reflected in reported results in the period in which they become known.

Restricted cash

Restricted cash included in deposits and other assets on the consolidated balance sheet contains an unconditional, irrevocable and transferable letter of credit that was entered into during the twelve-month period ended December 31, 2019 in connection with obligations under a facility lease for the Company's leased biologics manufacturing facility in Hopewell Township, New Jersey. The amount of the letter of credit is $7.5 million, is to be maintained for a term of not less than five years and has the potential to be reduced to $3.8 million if after five years the Company is not in default of its lease. Restricted cash also contains an unconditional, irrevocable and transferable letter of credit that was entered into during June 2022 in connection with obligations for the Company's new facility lease in Warren, New Jersey. The amount of the letter of credit is $8.1 million and has the potential to be reduced to $4.1 million if after five years the Company is not in default of its lease. Both amounts are classified within deposits and other assets on the consolidated balance sheet due to the long-term nature of the respective letters of credit. Restricted cash also includes a bank guarantee of $0.6 million denominated in a foreign currency.

The following table provides a reconciliation of cash, cash equivalents, and restricted cash reported within the consolidated balance sheet that sum to the total of the same amounts shown in the statement of cash flows:

	End of period- September 30, 2023	Beginning of period- December 31, 2022
Cash and cash equivalents	$ 165,157	$ 279,834
Restricted cash included in deposits and other assets	16,253	16,091
Total Cash, cash equivalents and restricted cash per statement of cash flows	$ 181,410	$ 295,925

Marketable securities

The Company's marketable securities consists of both debt securities and equity investments. The Company considers its investments in debt securities with original maturities of greater than 90 days to be available for sale securities. Securities under this classification are recorded at fair value and unrealized gains and losses within accumulated other comprehensive income. The estimated fair value of the available for sale securities is determined based on quoted market prices or rates for similar instruments. In addition, the cost of debt securities in this category is adjusted for amortization of premium and accretion of discount to maturity. For available for sale debt securities in an unrealized loss position, the Company assesses whether it intends to sell or if it is more likely than not that the Company will be required to sell the security before recovery of its amortized cost basis. If either of the criteria regarding intent or requirement to sell is met, the security's amortized cost basis is written down to fair value. If the criteria are not met, the Company evaluates whether the decline in fair value has resulted from a credit loss or other factors. In making this assessment, management considers, among other factors, the extent to which fair value is less than amortized cost, any changes to the rating of the security by a rating agency, and adverse conditions specifically related to the security. If this assessment indicates that a credit loss exists, the present value of cash flows expected to be collected from the security are compared to the amortized cost basis of the security. If the present value of the cash flows expected to be collected is less than the amortized cost basis, a credit loss exists and an allowance for credit losses is recorded for the credit loss, limited by the amount that the fair value is less than the amortized costs basis. Any impairment that has not been recorded through an allowance for credit losses is recognized in other comprehensive income. For the three and nine months ended September 30, 2023 and 2022, no allowance was recorded for credit losses.

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

Inventory and cost of product sales

Inventory

Inventories are stated at the lower of cost and net realizable value with cost determined on a first-in, first-out basis by product. The Company capitalizes inventory costs associated with products following regulatory approval when future commercialization is considered probable and the future economic benefit is expected to be realized. Products which may be used in clinical development programs are included in inventory and charged to research and development expense when the product enters the research and development process and no longer can be used for commercial purposes. Inventory used for marketing efforts are charged to selling, general and administrative expense. Amounts related to clinical development programs and marketing efforts are immaterial.

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

	September 30, 2023	December 31, 2022
Raw materials	$ 907	$ 1,078
Work in progress	24,595	14,074
Finished goods	10,250	6,656

Total inventory	$ 35,752	$ 21,808

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, 2023, the Company's inventory write-downs were immaterial. For the nine months ended September 30, 2023, the Company recorded inventory write-downs of $0.4 million primarily related to product approaching expiration. For the three and nine months ended September 30, 2022, the Company recorded inventory write-downs of $0.3 million and $1.2 million, respectively, primarily related to product approaching expiration. Additionally, though the Company's product is subject to strict quality control and monitoring which it performs throughout the manufacturing processes, certain batches or units of product may not meet quality specifications resulting in a charge to cost of product sales. For the three and nine months ended September 30, 2023 and 2022, these amounts were immaterial.

Cost of product sales

Cost of product sales consists of the cost of inventory sold, manufacturing and supply chain costs, storage costs, amortization of the acquired intangible asset, royalty payments associated with net product sales, and royalty payments to collaborative partners associated with royalty revenues and collaboration revenue related to milestones. Production costs are expensed as cost of product sales when the related products are sold or royalty revenues and collaboration revenue milestones are earned.

Revenue recognition

Net product revenue

The Company's net product revenue primarily consists of sales of Translarna in territories outside of the U.S. for the treatment of nmDMD and sales of Emflaza in the U.S. for the treatment of DMD. The Company recognizes revenue when its performance obligations with its customers have been satisfied. The Company's performance obligations are to provide products based on customer orders from distributors, hospitals, specialty pharmacies or retail pharmacies. The performance obligations are satisfied at a point in time when the Company's customer obtains control of the product, which is typically upon delivery. The Company invoices its customers after the products have been delivered and invoice payments are generally due within 30 to 90 days of the invoice date. The Company determines the transaction price based on fixed consideration in its contractual agreements. Contract liabilities arise in certain circumstances when consideration is due for goods the Company has yet to provide. As the Company has identified only one distinct performance obligation, the transaction price is allocated entirely to product sales. In determining the transaction price, a significant financing component does not exist since the timing from when the Company delivers product to when the customers pay for the product is typically less than one year. Customers in certain countries pay in advance of product delivery. In those instances, payment and delivery typically occur in the same month.

The Company records product sales net of any variable consideration, which includes discounts, allowances, rebates related to Medicaid and other government pricing programs, and distribution fees. The Company uses the expected value or most likely amount method when estimating its variable consideration, unless discount or rebate terms are specified within contracts. The identified variable consideration is recorded as a reduction of revenue at the time revenues from product sales are recognized. These estimates for variable consideration are adjusted to reflect known changes in factors and may impact such estimates in the quarter those changes are known. Revenue recognized does not include amounts of variable consideration that are constrained.

For the three months ended September 30, 2023 and 2022, net product sales outside of the United States were $76.6 million and $79.4 million, respectively, consisting of sales of Translarna, Tegsedi, Waylivra, and Upstaza. Translarna net revenues made up $69.0 million and $76.6 million of the net product sales outside of the United States for the three months ended September 30, 2023 and 2022, respectively. For the three months ended September 30, 2023 and 2022, net product sales in the United States were $67.4 million and $54.8 million, respectively, consisting solely of sales of Emflaza. During the three months ended September 30, 2023, one country, the United States, accounted for at least 10% of the Company's net product sales, representing $67.4 million of net product sales. During the three months ended September 30, 2022, two

countries, the United States and Russia, accounted for at least 10% of the Company's net product sales, representing $54.8 million and $28.3 million of net product sales, respectively.

For the nine months ended September 30, 2023 and 2022, net product sales outside of the United States were $318.5 million and $247.6 million, respectively, consisting of Translarna, Tegsedi, Waylivra, and Upstaza. Translarna net revenues made up $280.6 million and $232.9 million of the net product sales outside of the United States for the nine months ended September 30, 2023 and 2022, respectively. For the nine months ended September 30, 2023 and 2022, net product sales in the United States were $187.7 million and $160.1 million, respectively, consisting solely of Emflaza. During the nine months ended September 30, 2023, three countries, the United States, Russia, and Brazil, accounted for at least 10% of the Company's net product sales, representing $187.7 million, $69.0 million, and $52.5 million of net product sales, respectively. During the nine months ended September 30, 2022, two countries, the United States and Russia, accounted for at least 10% of the Company's net product sales, representing $160.1 million and $57.1 million of net product sales, respectively.

In relation to customer contracts, the Company incurs costs to fulfill a contract but does not incur costs to obtain a contract. These costs to fulfill a contract do not meet the criteria for capitalization and are expensed as incurred. The Company considers any shipping and handling costs that are incurred after the customer has obtained control of the product as a cost to fulfill a promise. Shipping and handling costs associated with finished goods delivered to customers are recorded as a selling expense.

Collaboration and royalty revenue

The terms of these agreements typically include payments to the Company of one or more of the following: nonrefundable, upfront license fees; milestone payments; research funding and royalties on future product sales. In addition, the Company generates service revenue through agreements that generally provide for fees for research and development services and may include additional payments upon achievement of specified events.

At the inception of a collaboration arrangement, the Company needs to first evaluate if the arrangement meets the criteria in Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC") Topic 808 "Collaborative Arrangements" to then determine if ASC Topic 606 is applicable by considering whether the collaborator meets the definition of a customer. If the criteria are met, the Company assesses the promises in the arrangement to identify distinct performance obligations.

For licenses of intellectual property, the Company assesses, at contract inception, whether the intellectual property is distinct from other performance obligations identified in the arrangement. If the licensing of intellectual property is determined to be distinct, revenue is recognized for nonrefundable, upfront license fees when the license is transferred to the customer and the customer can use and benefit from the license. If the licensing of intellectual property is determined not to be distinct, then the license will be bundled with other promises in the arrangement into one distinct performance obligation. The Company needs to determine if the bundled performance obligation is satisfied over time or at a point in time. If the Company concludes that the nonrefundable, upfront license fees will be recognized over time, the Company will need to assess the appropriate method of measuring proportional performance.

For milestone payments, the Company assesses, at contract inception, whether the development or sales-based milestones are considered probable of being achieved. If it is probable that a significant revenue reversal will occur, the Company will not record revenue until the uncertainty has been resolved. Milestone payments that are contingent upon regulatory approval are not considered probable of being achieved until the applicable regulatory approvals or other external conditions are obtained as such conditions are not within the Company's control. If it is probable that a significant revenue reversal will not occur, the Company will estimate the milestone payments using the most likely amount method. The Company will re-assess the development and sales-based milestones each reporting period to determine the probability of achievement. The Company recognizes royalties from product sales at the later of when the related sales occur or when the performance obligation to which the royalty has been allocated has been satisfied. If it is probable that a significant revenue reversal will not occur, the Company will estimate the royalty payments using the most likely amount method.

The Company recognizes revenue for reimbursements of research and development costs under collaboration agreements as the services are performed. The Company records these reimbursements as revenue and not as a reduction of research and development expenses as the Company has the risks and rewards as the principal in the research and development activities.

For the three months ended September 30, 2023, the Company did not recognize collaboration revenue related to the SMA License Agreement with Roche. For the three months ended September 30, 2022, the Company recognized $50.0 million of collaboration revenue related to the SMA License Agreement. For the nine months ended September 30, 2023, the amounts recognized for the collaboration revenue related to the SMA License Agreement with Roche were immaterial. For the nine months ended September 30, 2022, the Company recognized $50.0 million of collaboration revenue related to the SMA License Agreement with Roche. In September 2022, the Company recognized a sales milestone of $50.0 million for the achievement of $750.0 million in worldwide annual net sales from Evrysdi.

For the three and nine months ended September 30, 2023, the Company has recognized $50.2 million and $117.9 million of royalty revenue, respectively, related to Evrysdi. For the three and nine months ended September 30, 2022, the Company has recognized $32.9 million and $73.6 million of royalty revenue, respectively, related to Evrysdi.

Manufacturing Revenue

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

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

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

For the three and nine months ended September 30, 2023, the Company recognized $2.4 million and $6.7 million of manufacturing revenue, respectively, related to plasmid DNA and AAV vector production for external customers. No manufacturing revenue was recognized for the three and nine months ended September 30, 2022. As of September 30, 2023, the Company has contract assets of $1.4 million and remaining performance obligations of $1.2 million related to the production of plasmid DNA and AAV vectors for gene therapy applications for external customers. For the period ended December 31, 2022, the Company had remaining performance obligations of $1.4 million and no contracts assets related to plasmid DNA and AAV production for external customers.

Allowance for doubtful accounts

The Company maintains an allowance for estimated losses resulting from the inability of its customers to make required payments. The Company estimates uncollectible amounts based upon current customer receivable balances, the age of customer receivable balances, the customer’s financial condition and current economic trends. The Company also assesses whether an allowance for expected credit losses may be required which includes a review of the Company’s receivables

portfolio, which are pooled on a customer basis or country basis. In making its assessment of whether an allowance for credit losses is required, the Company considers its historical experience with customers, current balances, levels of delinquency, regulatory and legal environments, and other relevant current and future forecasted economic conditions. For the three and nine months ended September 30, 2023 and 2022, no allowance was recorded for credit losses. The allowance for doubtful accounts was $1.0 million as of September 30, 2023 and $0.3 million as of December 31, 2022. For the three and nine months ended September 30, 2023, bad debt expense was $0.4 million and $0.7 million, respectively. For the three and nine months ended September 30, 2022, bad debt expense was immaterial.

Liability for sale of future royalties

On July 17, 2020, the Company, RPI, and, for the limited purposes set forth in the agreement, Royalty Pharma PLC, entered into the Royalty Purchase Agreement. Pursuant to the Royalty Purchase Agreement, the Company sold to RPI 42.933% (the “Assigned Royalty Rights”) of the Company’s right to receive sales-based royalty payments (the “Royalty”) on worldwide net sales of Evrysdi and any other product developed pursuant to the SMA License Agreement. In consideration for the sale of the Assigned Royalty Rights, RPI paid the Company $650.0 million in cash consideration. The Company has retained a 57.067% interest in the Royalty and all economic rights to receive the remaining potential regulatory and sales milestone payments under the SMA License Agreement, which remaining milestone payments equal $250.0 million in the aggregate as of September 30, 2023. The Royalty Purchase Agreement will terminate 60 days following the earlier of the date on which Roche is no longer obligated to make any payments of the Royalty pursuant to the SMA License Agreement and the date on which RPI has received $1.3 billion in respect of the Assigned Royalty Rights.

The cash consideration obtained pursuant to the Royalty Purchase Agreement is classified as debt and is recorded as “liability for sale of future royalties-current” and “liability for sale of future royalties-noncurrent” on the Company’s consolidated balance sheet based on the timing of the expected payments to be made to RPI. The fair value for the liability for sale of future royalties at the time of the transaction was based on the Company’s estimates of future royalties expected to be paid to RPI over the life of the arrangement, which was determined using forecasts from market data sources, which are considered Level 3 inputs. The liability is being amortized using the effective interest method over the life of the arrangement, in accordance with the respective guidance. The Company utilizes the prospective method to account for subsequent changes in the estimated future payments to be made to RPI. Refer to Note 9 for further details.

Indefinite-lived intangible assets

Indefinite-lived intangible assets consist of in process research and development ("IPR&D"). IPR&D acquired directly in a transaction other than a business combination is capitalized if the projects will be further developed or have an alternative future use; otherwise they are expensed. The fair values of IPR&D projects and license agreement assets acquired in business combinations are capitalized. Several methods may be used to determine the estimated fair value of the IPR&D and license agreement asset acquired in a business combination. The Company utilizes the "income method" and uses estimated future net cash flows that are derived from projected sales revenues and estimated costs. These projections are based on factors such as relevant market size, patent protection, and expected pricing and industry trends. The estimated future net cash flows are then discounted to the present value using an appropriate discount rate. These assets are treated as indefinite-lived intangible assets until completion or abandonment of the projects, at which time the assets are amortized over the remaining useful life or written off, as appropriate. Intangible assets with indefinite lives, including IPR&D, are tested for impairment if impairment indicators arise and, at a minimum, annually. However, an entity is permitted to first assess qualitative factors to determine if a quantitative impairment test is necessary. Further testing is only required if the entity determines, based on the qualitative assessment, that it is more likely than not that an indefinite-lived intangible asset’s fair value is less than its carrying amount. Otherwise, no further impairment testing is required. The indefinite-lived intangible asset impairment test consists of a one-step analysis that compares the fair value of the intangible asset with its carrying amount. If the carrying amount of an intangible asset exceeds its fair value, an impairment loss is recognized in an amount equal to that excess. The Company considers many factors in evaluating whether the value of its intangible assets with indefinite lives may not be recoverable, including, but not limited to, expected growth rates, the cost of equity and debt capital, general economic conditions, the Company’s outlook and market performance of the Company’s industry and recent and forecasted financial performance.

In May 2023, as part of the Company's strategic portfolio prioritization, the Company decided to discontinue its preclinical and early research programs in its gene therapy platform, which included Friedreich ataxia and Angelman syndrome. As a result, the Company determined that the Friedreich ataxia and Angelman syndrome indefinite lived intangible assets were fully impaired and recorded impairment expense of $217.8 million during the nine months ended September 30, 2023, which is recorded as intangible asset impairment in the statement of operations. Refer to Note 12 for further information regarding the Company's intangible assets.

Goodwill

Goodwill represents the amount of consideration paid in excess of the fair value of net assets acquired as a result of the Company's business acquisitions accounted for using the acquisition method of accounting. Goodwill is not amortized and is subject to impairment testing at a reporting unit level on an annual basis or when a triggering event occurs that may indicate the carrying value of the goodwill is impaired. The Company reassesses its reporting units as part of its annual segment review. An entity is permitted to first assess qualitative factors to determine if a quantitative impairment test is necessary. Further testing is only required if the entity determines, based on the qualitative assessment, that it is more likely than not that the fair value of the reporting unit is less than its carrying amount.

Income Taxes

The Organization for Economic Co-operation and Development ("OECD"), the European Community ("the EC"), and individual taxing jurisdictions where the Company and its affiliates do business have recently focused on issues related to the taxation of multinational corporations. The OECD has released its comprehensive plan to create an agreed set of international rules for fighting base erosion and profit shifting. In addition, the OECD, the EC and individual taxing jurisdictions are examining changes to how taxing rights should be allocated among countries considering the digital economy. As a result, the tax laws in the U.S. and other countries in which the Company and its affiliates do business could change on a prospective or retroactive basis and any such changes could materially adversely affect the Company's business.

On December 22, 2017, the U.S. government enacted the 2017 Tax Act, which significantly revised U.S. tax law by, among other provisions, lowering the U.S. federal statutory corporate income tax rate to 21%, imposing a mandatory one-time transition tax on previously deferred foreign earnings, and eliminating or reducing certain income tax deductions. The Global Intangible Low-Taxed Income ("GILTI") provisions of the 2017 Tax Act require the Company to include in its U.S. income tax return foreign subsidiary earnings in excess of an allowable return on the foreign subsidiary's tangible assets. The Company has elected to account for GILTI tax in the period in which it is incurred, and therefore has not provided any deferred tax impacts of GILTI in its consolidated financial statements for the period ended September 30, 2023.

Starting in 2022, TCJA amendments to IRC Section 174 no longer permits an immediate deduction for research and development (R&D) expenditures in the tax year that such costs are incurred. Instead, these IRC Section 174 development costs must now be capitalized and amortized over either a five- or 15-year period, depending on the location of the activities performed. The new amortization period begins with the midpoint of any taxable year that IRC Section 174 costs are first incurred, regardless of whether the expenditures were made prior to or after July 1, and runs until the midpoint of year five for activities conducted in the United States or year 15 in the case of development conducted on foreign soil.

Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases and net operating loss and credit carryforwards. Deferred tax assets and liabilities are measured at rates expected to apply to taxable income in the years in which those temporary differences and carryforwards are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in the statement of operations in the period that includes the enactment date. A valuation allowance is recorded when it is not more likely than not that all or a portion of the net deferred tax assets will be realized.

On August 23, 2018, the Company completed its acquisition of Agilis Biotherapeutics, Inc. ("Agilis"), pursuant to an Agreement and Plan of Merger, dated as of July 19, 2018 (the "Agilis Merger Agreement"), by and among the Company,

Agility Merger Sub, Inc., a Delaware corporation and the Company's wholly owned, indirect subsidiary, Agilis and, solely in its capacity as the representative, agent and attorney-in-fact of the equityholders of Agilis, Shareholder Representative Services LLC, (the "Agilis Merger"). The Company recorded a deferred tax liability in conjunction with the Agilis Merger of $122.0 million in 2018, related to the tax basis difference in the IPRD indefinite-lived intangibles acquired. The Company's policy is to record a deferred tax liability related to acquired IPR&D which may eventually be realized either upon amortization of the asset when the research is completed, and a product is successfully launched or the write-off of the asset if it is abandoned or unsuccessful. In July 2022, the Company received EMEA approval for a portion of the IPR&D assets, and thus, began the amortization of the intangible.

In May 2023, the Company announced the discontinuation of its preclinical and early research programs in gene therapy as part of a strategic portfolio prioritization. In conjunction with the announcement, the Company recorded an impairment to its indefinite-lived intangible for IP research and development relating to the Friedreich ataxia and Angelman syndrome gene therapy assets. As a result of the impairment, the Company recorded a deferred tax benefit of $50.9 million.

Leases

The Company determines if an arrangement is a lease at inception. This determination generally depends on whether the arrangement conveys to the Company the right to control the use of an explicitly or implicitly identified fixed asset for a period of time in exchange for consideration. Control of an underlying asset is conveyed to the Company if the Company obtains the rights to direct the use of and to obtain substantially all of the economic benefits from using the underlying asset. The Company has lease agreements which include lease and non-lease components, which the Company accounts for as a single lease component for all leases. Operating and finance leases are classified as right of use ("ROU") assets, short term lease liabilities, and long term lease liabilities. Operating and finance lease ROU assets and lease liabilities are recognized at the commencement date based on the present value of lease payments over the lease term. ROU assets are amortized and lease liabilities accrete to yield straight-line expense over the term of the lease. Lease payments included in the measurement of the lease liability are comprised of fixed payments.

Variable lease payments associated with the Company's leases are recognized when the event, activity, or circumstance in the lease agreement on which those payments are assessed occurs. Variable lease payments are presented in the Company's consolidated statements of operations in the same line item as expense arising from fixed lease payments for operating leases.

Leases with an initial term of 12 months or less are not recorded on the consolidated balance sheet and the Company recognizes lease expense for these leases on a straight-line basis over the lease term. The Company applies this policy to all underlying asset categories.

A lessee is required to discount its unpaid lease payments using the interest rate implicit in the lease or, if that rate cannot be readily determined, its incremental borrowing rate. As most of the Company's leases do not provide an implicit rate, the Company uses its incremental borrowing rate based on the information available at the commencement date in determining the present value of lease payments. The Company gives consideration to its recent debt issuances as well as publicly available data for instruments with similar characteristics when calculating its incremental borrowing rates.

The lease term for all of the Company's leases includes the non-cancellable period of the lease plus any additional periods covered by either a Company option to extend (or not to terminate) the lease that the Company is reasonably certain to exercise, or an option to extend (or not to terminate) the lease controlled by the lessor. Leasehold improvements are capitalized and depreciated over the lesser of useful life or lease term. See Note 3 Leases for additional information.

3. Leases

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

The Company also leases approximately 220,500 square feet of office, manufacturing and laboratory space at a facility located in Hopewell Township, New Jersey pursuant to a Lease Agreement (the “Hopewell Lease”) with Hopewell Campus Owner LLC. The rental term of the Hopewell Lease commenced on July 1, 2020 and has an initial term of fifteen years (the “Hopewell Initial Term”), with two consecutive ten year renewal periods, each at the Company’s option. The aggregate rent for the Hopewell Initial Term will be approximately $111.5 million. The rental rate for the renewal periods will be 95% of the Prevailing Market Rate (as defined in the Hopewell Lease) and determined at the time of the exercise of the renewal. The Company is also responsible for maintaining certain insurance and the payment of proportional taxes, utilities and common area operating expenses. The Hopewell Lease contains customary events of default, representations, warranties and covenants.

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

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

On June 19, 2020, the Company entered into a commercial manufacturing service agreement for a term of 12.5 years with MassBiologics of the University of Massachusetts Medical School ("MassBio"). The Company determined that the agreement was a finance lease, for which the Company recorded a finance lease ROU asset for $41.4 million and corresponding finance lease liability for $41.4 million at the onset of the lease agreement. Given that the leased asset is designed for the production of PTC’s AADC program and would not have an alternate use outside the PTC gene therapy platform without incurring significant costs, the Company determined that the lease should be treated as research and development expense under ASC 730. Accordingly, the full $41.4 million relating to the finance lease ROU asset was written off and expensed to research and development during the year ended December 31, 2020. As of September 30, 2023, the balance of the finance lease liabilities-current and finance lease liabilities-noncurrent are $2.6 million and $17.2 million, respectively, and are directly related to the Company’s MassBio agreement. As of December 31, 2022, the balance of the finance lease liabilities-current and finance lease liabilities-noncurrent were $3.0 million and $18.7 million, respectively. Additionally, the Company recorded finance lease costs of $0.4 million and $1.1 million related to interest on the lease liability during the three and nine months ended September 30, 2023, respectively. The Company recorded finance lease costs of $0.4 million and $1.2 million related to interest on the lease liability during the three and nine months ended September 30, 2022, respectively.

The Company also leases certain vehicles, lab equipment, and office equipment under operating leases. The Company's leases have remaining operating lease terms ranging from 0.4 years to 15.7 years and certain of the leases include renewal options to extend the lease for up to 20 years. Rent expense was $7.1 million and $6.8 million for the three months ended September 30, 2023 and 2022, respectively. Rent expense was $21.5 million and $18.0 million for the nine months ended September 30, 2023 and 2022, respectively.

The components of operating lease expense were as follows:

	Three Months Ended September 30, 2023	Three Months Ended September 30, 2022	Nine Months Ended September 30, 2023	Nine Months Ended September 30, 2022
Operating Lease Cost				
Fixed lease cost	$ 5,490	$ 5,377	$ 16,463	$ 14,267
Variable lease cost	1,376	1,151	4,140	3,152
Short-term lease cost	271	262	866	600
Total operating lease cost	$ 7,137	$ 6,790	$ 21,469	$ 18,019

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

Supplemental lease term and discount rate information related to leases was as follows as September 30, 2023 and December 31, 2022:

	September 30, 2023	December 31, 2022
Weighted-average remaining lease terms - operating leases (years)	11.61	11.61
Weighted-average discount rate - operating leases	8.67 %	8.61 %
Weighted-average remaining lease terms - finance lease (years)	9.26	10.01
Weighted-average discount rate - finance lease	7.80 %	7.80 %

Supplemental cash flow information related to leases was as follows as of September 30, 2023 and 2022:

	Nine Months Ended September 30, 2023	2022
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows from operating leases	$ 11,479	$ 10,954
Financing cash flows from finance lease	1,379	1,276
Operating cash flows from finance lease	1,621	1,724
Right-of-use assets obtained in exchange for lease obligations:		
Operating leases	$ —	$ 35,294

Future minimum lease payments under non-cancelable leases as of September 30, 2023 were as follows:

	Operating Leases	Finance Lease
2023 (excludes the nine months ended September 30, 2023)	$ 5,077	$ —
2024	18,369	3,000
2025	20,438	3,000
2026	19,988	3,000
2027 and thereafter	193,792	18,000
Total lease payments	257,664	27,000
Less: Imputed Interest expense	146,279	7,205
Total	$ 111,385	$ 19,795

4. Fair value of financial instruments and marketable securities

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

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

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

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

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

The Company owns common stock in ClearPoint Neuro, Inc. ("ClearPoint") (formerly MRI Interventions, Inc.), a publicly traded medical device company. The ClearPoint equity investments (collectively, the "ClearPoint Equity Investments") represent financial instruments, and therefore, are recorded at fair value, which is readily determinable. The ClearPoint Equity Investments are components of prepaids and other current assets on the consolidated balance sheet as of September 30, 2023, and deposits and other assets as of December 31, 2022. During three and nine months ended September 30, 2023, the Company recorded unrealized losses of $2.0 million and $3.1 million, respectively. During the three and nine months ended September 30, 2022, the Company recorded unrealized losses of $3.5 million and $1.1 million, respectively. During the three months ended September 30, 2023, the Company did not record any realized gains or losses for the sale of ClearPoint Equity Investments. During the nine months ended September 30, 2023, the Company recorded a realized loss of $0.8 million for the sale of ClearPoint Equity Investments. During the three and nine months ended September 30, 2022, the Company did not record any realized gains or losses for the sale of Clearpoint Equity Investments. These unrealized and realized losses are components of other expense, net within the consolidated statement of operations. The fair value of the ClearPoint Equity Investments was $4.5 million and $11.0 million as of September 30, 2023 and December 31, 2022, respectively. The Company classifies the ClearPoint Equity Investments as Level 1 assets within the fair value hierarchy, as the value is based on a quoted market price in an active market, which is not adjusted.

In January 2020, the Company purchased a $10.0 million convertible note from ClearPoint that the Company can convert into ClearPoint shares at a conversion rate of $6.00 per share at any point throughout the term of the loan, which matures five years from the purchase date. The Company determined that the convertible note represents an available for sale debt security and the Company has elected to record it at fair value under ASC 825. The Company classifies its ClearPoint convertible debt security as a Level 2 asset within the fair value hierarchy, as the value is based on inputs other than quoted prices that are observable. The fair value of the ClearPoint convertible debt security is determined at each reporting period by utilizing a Black-Scholes option pricing model, as well as a present value of expected cash flows from the debt security utilizing the risk free rate and the estimated credit spread as of the valuation date as the discount rate. During the three and nine months ended September 30, 2023, the Company recorded unrealized losses of $3.1 million and $4.6 million, respectively. During the three and nine months ended September 30, 2022, the Company recorded unrealized losses of $4.4 million and $2.4 million, respectively. These unrealized gains and losses are components of other expense, net within the consolidated statement of operations. The fair value of the convertible debt security was $10.6 million and $15.2

million as of September 30, 2023 and December 31, 2022, respectively. The convertible debt security is considered to be long term and is included as a component of deposits and other assets on the consolidated balance sheet. Other than the ClearPoint Equity Investments and the ClearPoint convertible debt security, no other items included in deposits and other assets and prepaids and other current assets on the consolidated balance sheets are fair valued.

The Company has investments in mutual funds, including one that is denominated in a foreign currency. All of these are equity investments and are classified as marketable securities on the Company's consolidated balance sheets. These equity investments are reported at fair value, as they are readily available, and as such are classified as Level 1 assets. Unrealized holding gains and losses for these equity investments are included as components of other expense, net within the consolidated statement of operations. For the three and nine months ended September 30, 2023, the Company had unrealized gains of $1.1 million and of $5.5 million, respectively, relating to the equity investments still held at the reporting date. For the three and nine months ended September 30, 2022, the Company had unrealized gains of $0.1 million and unrealized losses of $11.3 million relating to the equity investments still held at the reporting date, respectively. For the three and nine months ended September 30, 2023, the Company had redemptions of $7.8 million and $12.1 million, respectively, which were primarily due to liquidation of one of the mutual funds. For the three and nine months ended September 30, 2022, the Company had redemptions of $100.8 million and $104.4 million, respectively, which were primarily due to liquidation of one of the mutual funds. For the three months ended September 30, 2023, the Company had foreign currency unrealized losses of $1.4 million relating to these equity investments. For the nine months ended September 30, 2023, the amounts recognized for foreign currency unrealized losses relating to these investments were immaterial. For the three and nine months ended September 30, 2022, the Company had foreign currency unrealized losses of $1.4 million and $1.0 million, respectively, relating to these equity investments.

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, 2023 and December 31, 2022:

	September 30, 2023			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities - available for sale	$ 1,625	$ —	$ 1,625	$ —
Marketable securities - equity investments	$ 128,028	$ 128,028	$ —	$ —
ClearPoint Equity Investments - available for sale	$ 4,482	$ 4,482	$ —	$ —
ClearPoint convertible debt security	$ 10,637	$ —	$ 10,637	$ —
Contingent consideration payable- development and regulatory milestones	$ 26,400	$	$	$ 26,400
Contingent consideration payable- net sales milestones and royalties	$ 12,600	$	$	$ 12,600

	December 31, 2022			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities - available for sale	$ 22,610	$ —	$ 22,610	$ —
Marketable securities - equity investments	$ 108,261	$ 108,261	$ —	$ —
ClearPoint Equity Investments	$ 10,965	$ 10,965	$ —	$ —
ClearPoint convertible debt security	$ 15,231	$ —	$ 15,231	$ —

Contingent consideration payable- development and regulatory milestones	$ 82,500	$ —	$ —	$ 82,500
Contingent consideration payable- net sales milestones and royalties	$ 81,500	$ —	$ —	$ 81,500

No transfers of assets between Level 1, Level 2, or Level 3 of the fair value measurement hierarchy occurred during the periods ended September 30, 2023 and December 31, 2022.

The following is a summary of marketable securities accounted for as available for sale debt securities at September 30, 2023 and December 31, 2022:

	September 30, 2023			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Corporate debt securities	$ 1,649	$ —	$ (24)	$ 1,625
Total	$ 1,649	$ —	$ (24)	$ 1,625

	December 31, 2022			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 12,419	$ 5	$ —	$ 12,424
Corporate debt securities	10,685	—	(499)	10,186
Total	$ 23,104	$ 5	$ (499)	$ 22,610

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, 2023 and 2022, no write downs occurred. The Company does not intend to sell the investments and it is not more likely than not that the Company will be required to sell the investments before recovery of their amortized cost basis, which may be maturity. The Company also reviews its available for sale debt securities in an unrealized loss position and evaluates whether the decline in fair value has resulted from credit losses or other factors. This review is subjective, as it requires management to evaluate whether an event or change in circumstances has occurred in that period that may be related to credit issues. For the three and nine months ended September 30, 2023 and 2022, no allowance was recorded for credit losses. Unrealized gains and losses are reported as a component of accumulated other comprehensive (loss) income in stockholders' equity.

For the three months ended September 30, 2023, the Company did not have any realized gains or losses from the sale of available for sale debt securities. For the nine months ended September 30, 2023, the Company had $0.3 million of realized losses from the sale of available for sale debt securities. For the three and nine months ended September 30, 2022, the Company had $1.3 million and $1.6 million of realized losses from the sale of available for sale debt securities, respectively. 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, 2023 are as follows:

	September 30, 2023					
	Securities in an unrealized loss position less than 12 months		Securities in an unrealized loss position greater than or equal to 12 months		Total	
	Unrealized losses	Fair Value	Unrealized losses	Fair Value	Unrealized losses	Fair Value
Corporate debt securities	$ —	—	(24)	1,625	(24)	$ 1,625
Total	$ —	$ —	$ (24)	$ 1,625	$ (24)	$ 1,625

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, 2022 are as follows:

	December 31, 2022					
	Securities in an unrealized loss position less than 12 months		Securities in an unrealized loss position greater than or equal to 12 months		Total	
	Unrealized losses	Fair Value	Unrealized losses	Fair Value	Unrealized losses	Fair Value
Corporate debt securities	$ —	—	(499)	10,186	(499)	$ 10,186
Total	$ —	$ —	$ (499)	$ 10,186	$ (499)	$ 10,186

Available for sale debt securities at September 30, 2023 and December 31, 2022 mature as follows:

	September 30, 2023	
	Less Than 12 Months	More Than 12 Months
Corporate debt securities	$ 1,625	$ —
Total	$ 1,625	$ —

	December 31, 2022	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 12,424	$ —
Corporate debt securities	—	10,186
Total	$ 12,424	$ 10,186

The Company classifies all of its marketable securities as current as they are all either available for sale debt securities or equity investments and are available for current operations.

Convertible senior notes

In September 2019, the Company issued $287.5 million of 1.50% convertible senior notes due September 15, 2026 (the "2026 Convertible Notes,"). The fair value of the 2026 Convertible Notes, which differs from their carrying values, is influenced by interest rates, the Company's stock price and stock price volatility and is determined by prices for the 2026 Convertible Notes observed in market trading which are Level 2 inputs. The estimated fair value of the 2026 Convertible Notes at September 30, 2023 and December 31, 2022 was $228.8 million and $281.7 million, respectively.

Level 3 valuation

The contingent consideration payable is fair valued each reporting period with the change in fair value recorded as a gain or loss within the change in the fair value of contingent consideration on the consolidated statements of operations. The fair value of the development and regulatory milestones is estimated utilizing a probability adjusted, discounted cash flow approach. The discount rates are estimated utilizing Corporate B rated bonds maturing in the years of expected payments based on the Company's estimated development timelines for the acquired product candidate. The fair value of the net sales milestones is determined utilizing a valuation framework that estimates net sales volatility to simulate a range of possible payment scenarios. The average of the payments in these scenarios is then discounted to calculate present fair value.

In May 2023, as part of the Company's strategic portfolio prioritization, the Company decided to discontinue its preclinical and early research programs in its gene therapy platform, which included Friedreich ataxia and Angelman syndrome. As a result, the Company fully impaired the Friedreich ataxia and Angelman syndrome intangible assets and determined that the fair value for all of the contingent consideration payable related to Friedreich ataxia and Angelman syndrome was $0. The change in fair value for the contingent consideration payable related to Friedreich ataxia and Angelman syndrome for the nine months ended September 30, 2023 was $129.8 million and is included in the change in fair value of the contingent consideration in the statement of operations. The remaining contingent consideration as of September 30, 2023 is $39.0 million, which is solely related to the development and regulatory milestones, and net sales milestones, for Upstaza.

As of September 30, 2023, the weighted average discount rate for the Upstaza development and regulatory milestones was 7.1% and the weighted average probability of success was 90%. As of September 30, 2023, the weighted average discount rate for the Upstaza net sales milestones was 12.0% and the weighted average probability of success for the net sales milestones was 93%.

The table presented below is a summary of changes in the fair value of the Company's Level 3 valuations for the contingent consideration payable for the periods ended September 30, 2023 and September 30, 2022:

	Level 3 liabilities	
	Contingent consideration payable- development and regulatory milestones	Contingent consideration payable- net sales milestones and royalties
Beginning balance as of December 31, 2022	$ 82,500	$ 81,500
Additions	—	—
Change in fair value	(56,100)	(68,900)
Payments	—	—
Ending balance as of September 30, 2023	$ 26,400	$ 12,600

	Level 3 liabilities	
	Contingent consideration payable- development and regulatory milestones	Contingent consideration payable- net sales milestones and royalties
Beginning balance as of December 31, 2021	$ 139,300	$ 100,600
Additions	—	—
Change in fair value	(10,800)	(21,400)
Reclass to accounts payable and accrued expenses	(50,000)	—
Payments	—	—
Ending balance as of September 30, 2022	$ 78,500	$ 79,200

The following significant unobservable inputs were used in the valuation of the contingent consideration payable for the periods ended September 30, 2023 and December 31, 2022:

	September 30, 2023			
	Fair Value	Valuation Technique	Unobservable Input	Range
Contingent consideration payable- development and regulatory milestones	$26,400	Probability-adjusted discounted cash flow	Potential development and regulatory milestones Probabilities of success Discount rates Projected years of payments	$0 - $31 million 85% - 92% 6.9% - 7.6% 2023 - 2026
Contingent considerable payable- net sales milestones and royalties	$12,600	Option-pricing model with Monte Carlo simulation	Potential net sales milestones Probabilities of success Potential percentage of net sales for royalties Discount rate Projected years of payments	$0 - $50 million 85% - 100% 0% 12% 2025 - 2034

	December 31, 2022			
	Fair Value	Valuation Technique	Unobservable Input	Range
Contingent consideration payable- development and regulatory milestones	$82,500	Probability-adjusted discounted cash flow	Potential development and regulatory milestones Probabilities of success Discount rates Projected years of payments	$0 - $331 million 25% - 92% 6.2% - 8.3% 2023 - 2029
Contingent considerable payable- net sales milestones and royalties	$81,500	Option-pricing model with Monte Carlo simulation	Potential net sales milestones Probabilities of success Potential percentage of net sales for royalties Discount rate Projected years of payments	$0 - $150 million 25% - 100% 2% - 6% 11.5% 2025 - 2041

The contingent consideration payables are classified Level 3 liabilities as their valuation requires substantial judgment and estimation of factors that are not currently observable in the market. If different assumptions were used for the various inputs to the valuation approaches, including but not limited to, assumptions involving probability adjusted sales estimates

for the gene therapy platform and estimated discount rates, the estimated fair value could be significantly higher or lower than the fair value determined.

5. Accounts payable and accrued expenses

Accounts payable and accrued expenses at September 30, 2023 and December 31, 2022 consist of the following:

	September 30, 2023	December 31, 2022
Employee compensation, benefits, and related accruals	$ 70,778	$ 62,669
Income tax payable	2,822	4,712
Consulting and contracted research	21,236	38,882
Professional fees	3,067	3,093
Sales allowance	72,202	63,787
Sales rebates	119,971	67,355
Royalties	52,087	40,546
Accounts payable	19,152	27,268
Other	9,416	12,054
Total	$ 370,731	$ 320,366

During the three and nine month periods ended September 30, 2023, the Company incurred $22.6 million and $30.6 million, respectively, of restructuring costs from a reduction in workforce in connection with the Company's strategic pipeline prioritization and discontinuation of its preclinical and early research programs in its gene therapy platform. The costs are included in research and development and selling, general, and administrative expenses on the Company's consolidated statement of operations. As of September 30, 2023, the remaining $23.4 million of accrued restructuring costs are included above within employee compensation, benefits, and related accruals.

6. Capitalization

In August 2019, the Company entered into an At the Market Offering Sales Agreement (the "Sales Agreement") with Cantor Fitzgerald and RBC Capital Markets, LLC (together, the "Sales Agents"), pursuant to which, the Company may offer and sell shares of its common stock, having an aggregate offering price of up to $125.0 million from time to time through the Sales Agents by any method that is deemed to be an "at the market offering" as defined in Rule 415(a)(4) promulgated under the Securities Act of 1933, as amended. No shares were sold during the three and nine months ended September 30, 2023 and 2022. The remaining shares of the Company's common stock available to be issued and sold, under the At the Market Offering, have an aggregate offering price of up to $93.0 million as of September 30, 2023.

7. Net loss per share

Basic and diluted net loss per share is computed by dividing net loss by the weighted-average number of common shares outstanding. Potentially dilutive securities were excluded from the diluted calculation because their effect would be anti-dilutive.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Numerator				
Net loss	$ (132,970)	$ (109,315)	$ (470,812)	$ (388,128)
Denominator				
Denominator for basic and diluted net loss per share	75,377,997	71,654,671	74,618,611	71,415,849
Net loss per share:				
Basic and diluted	$ (1.76)*	$ (1.53)*	$ (6.31)*	$ (5.43)*

* In the three and nine months ended September 30, 2023 and 2022, the Company experienced a net loss and therefore did not report any dilutive share impact.

The following table shows historical dilutive common share equivalents outstanding, which are not included in the above historical calculation, as the effect of their inclusion is anti-dilutive during each period.

	As of September 30,	
	2023	2022
Stock Options	11,436,783	11,478,421
Unvested restricted stock awards and units	3,414,102	2,488,944
Total	14,850,885	13,967,365

8. Stock award plan

In May 2013, the Company's Board of Directors and stockholders approved the 2013 Long-Term Incentive Plan, which became effective upon the closing of the Company's initial public offering. On June 8, 2022 (the "Restatement Effective Date"), the Company's stockholders approved the Amended and Restated 2013 Long-Term Incentive Plan (the "Amended 2013 LTIP"). The Amended 2013 LTIP provides for the grant of incentive stock options, nonstatutory stock options, restricted stock units and other stock-based awards. The number of shares of common stock reserved for issuance under the Amended 2013 LTIP is the sum of (A) the number of shares of the Company's common stock (up to 16,724,212 shares) that is equal to the sum of (1) the number of shares issued under the 2013 Long-Term Incentive Plan prior to the Restatement Effective Date, (2) the number of shares that remain available for issuance under the 2013 Long-Term Incentive Plan immediately prior to the Restatement Effective Date and (3) the number of shares subject to awards granted under the 2013 Long-Term Incentive Plan prior to the Restatement Effective Date that are outstanding as of the Restatement Effective Date, plus (B) from and after the Restatement Effective Date, an additional 8,475,000 shares of Common Stock. As of September 30, 2023, awards for 6,802,210 shares of common stock are available for issuance under the Amended 2013 LTIP.

There are no additional shares of common stock available for issuance under the Company's 1998 Employee, Director and Consultant Stock Option Plan, 2009 Equity and Long Term Incentive Plan or 2013 Stock Incentive Plan.

In January 2020, the Company's Board of Directors approved the 2020 Inducement Stock Incentive Plan. The 2020 Inducement Stock Incentive Plan provides for the grant of incentive stock options, nonstatutory stock options, restricted stock awards and other stock-based awards for, initially, up to at the time, an aggregate of 1,000,000 shares of common stock. Any grants made under the 2020 Inducement Stock Incentive Plan must be made pursuant to the Nasdaq Listing Rule 5635(c)(4) inducement grant exception as a material component of the Company's new hires' employment compensation. In December 2020, the Company's Board of Directors approved an additional 1,000,000 shares of common stock that may be issued under the 2020 Inducement Stock Incentive Plan. In April 2022, the Company's Board of Directors approved a reduction in the total number of shares of common stock that may be issued under the 2020 Inducement Stock Incentive Plan to 1,300,000 shares. In December 2022, the Company's Board of Directors approved an additional 1,700,000 shares of common stock that may be issued under the 2020 Inducement Stock Incentive Plan. As of September 30, 2023, awards for 1,705,941 shares of common stock were available for issuance under the 2020 Inducement Stock Incentive Plan.

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

Inducement stock option awards

From January 1, 2023 through September 30, 2023, the Company issued a total of 1,059,054 stock options to various employees. Of those, 121,460 were inducement grants for non-statutory stock options, all of which were made pursuant to the 2020 Inducement Stock Incentive Plan.

Stock option activity—A summary of stock option activity is as follows:

	Number of options	Weighted-average exercise price	Weighted-average remaining contractual term	Aggregate intrinsic value(in thousands)
Outstanding at December 31, 2022	11,502,417	$ 43.33		
Granted	1,059,054	$ 41.29		
Exercised	(806,407)	$ 29.48		
Forfeited/Cancelled	(318,281)	$ 47.84		
Outstanding at September 30, 2023	11,436,783	$ 43.99	5.87 years	$ 5,084
Vested or Expected to vest at September 30, 2023	2,357,250	$ 46.72	8.23 years	$ —
Exercisable at September 30, 2023	8,533,144	$ 43.18	5.05 years	$ 5,084

The fair value of grants made in the nine months ended September 30, 2023 was contemporaneously estimated on the date of grant using the following assumptions:

	Nine months ended September 30, 2023
Risk-free interest rate	3.54% - 3.91%
Expected volatility	53% - 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, 2023 was $21.83 per share.

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

Restricted Stock Awards and Restricted Stock Units—Restricted stock awards and restricted stock units are granted subject to certain restrictions, including in some cases service or time conditions (restricted stock). The grant-date fair value of restricted stock awards and restricted stock units, which have been determined based upon the market value of the Company's shares on the grant date, are expensed over the vesting period. From January 1, 2023, through September 30, 2023, the Company issued a total of 1,973,973 restricted stock units to various employees. Of those, 83,210 were inducement grants for restricted stock units, all of which were made pursuant to the 2020 Inducement Stock Incentive Plan.

The following table summarizes information on the Company's restricted stock awards and units:

	Restricted Stock Awards and Units	
	Number of Shares	Weighted Average Grant Date Fair Value
Unvested at December 31, 2022	2,516,336	$ 45.67
Granted	1,973,973	40.00
Vested	(778,442)	46.34
Forfeited	(297,765)	42.65
Unvested at September 30, 2023	3,414,102	$ 42.50

Employee Stock Purchase Plan—In June 2016, the Company established an Employee Stock Purchase Plan (as amended, "ESPP" or the "Plan"), for certain eligible employees. The Plan is administered by the Company's Board of Directors or a committee appointed by the Company's Board of Directors. In June 2021, the Plan was amended to increase the total number of shares available for purchase under the Plan from one million shares to two million shares of the Company's common stock. Employees may participate over a six month period through payroll withholdings and may purchase, at the end of the six month period, the Company's common stock at a purchase price of at least 85% of the closing price of a share of the Company's common stock on the first business day of the offering period or the closing price of a share of the Company's common stock on the last business day of the offering period, whichever is lower. No participant will be granted a right to purchase the Company's common stock under the Plan if such participant would own more than 5% of the total combined voting power of the Company or any subsidiary of the Company after such purchase. For the three and nine months ended September 30, 2023, the Company recorded $0.7 million and $2.0 million, respectively, in compensation expense related to the ESPP.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Research and development	$ 13,986	$ 15,063	$ 44,828	$ 41,896
Selling, general and administrative	12,956	13,607	40,300	41,093
Total	$ 26,942	$ 28,670	$ 85,128	$ 82,989

As of September 30, 2023, there was approximately $179.3 million of total unrecognized compensation cost related to unvested share-based compensation arrangements granted under the Company's equity award plans. This cost is expected to be recognized as share-based compensation expense over the weighted average remaining service period of approximately 1.9 years.

9. Debt

Liability for sale of future royalties

In July 2020, the Company entered into the Royalty Purchase Agreement. As RPI's interest is explicitly limited, the $650.0 million cash consideration was classified as debt and is recorded as "liability for sale of future royalties-current" and "liability for sale of future royalties-noncurrent" on the Company's consolidated balance sheet based on the timing of the expected payments to be made to RPI. The fair value for the liability for sale of future royalties at the time of the transaction was based on the Company's estimates of future royalties expected to be paid to RPI over the life of the arrangement, which was determined using forecasts from market data sources, which are considered Level 3 inputs. The liability is being amortized using the effective interest method over the life of the arrangement, in accordance with ASC 470 and ASC 835. The initial annual effective interest rate was determined to be 11.0%. The Company utilizes the prospective method to account for subsequent changes in the estimated future payments to be made to RPI and updates the effective interest rate on a quarterly basis. Issuance costs related to the transaction were determined to be immaterial.

The following table shows the activity within the "liability for sale of future royalties- current" and "liability for sale of future royalties- noncurrent" accounts for the nine months ended September 30, 2023:

Liability for sale of future royalties- (current and noncurrent)	Nine Months Ended September 30, 2023
Beginning balance as of December 31, 2022	$ 757,886
Less: Non-cash royalty revenue payable to RPI	(50,599)
Plus: Non-cash interest expense recognized	56,031
Ending balance	$ 763,318
Effective interest rate as of September 30, 2023	9.3 %

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

Senior Secured Term Loan

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

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

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

All obligations under the Blackstone Credit Agreement are secured, subject to certain exceptions and specified inclusions, by security interests in certain assets of the Loan Parties, including (1) intellectual property and other assets related to Translarna, Emflaza, Upstaza, sepiapterin and, until certain release conditions are met, vatiquinone, in each case, together with any other forms, formulations, or methods of delivery of any such products, and regardless of trade or brand name, (2) future acquired intellectual property (but not internally developed intellectual property unrelated to other intellectual property collateral) and other related assets, and (3) the equity interests held by the Loan Parties in certain of their subsidiaries. The Blackstone Credit Agreement contains certain negative covenants with which the Company must remain in compliance. The Blackstone Credit Agreement also requires that the Company maintains consolidated liquidity of at least $100.0 million as of the last day of each fiscal quarter, which shall be increased to $200.0 million upon the Company consummating acquisitions meeting certain consolidated thresholds described therein. In addition, the Company will be required under conditions specified in the Blackstone Credit Agreement to fund a reserve account up to certain amounts specified therein, including $50.0 million that the Company funded into the reserve account during the quarter ended March 31, 2023 and was released back to the Company during the quarter ended June 30, 2023. The funds in the reserve

account are available to prepay the Loans at any time at the Company's option, and are, if funded, subject to release upon certain further conditions. Upon any such release, such funds are freely available for use by the Company subject to the generally applicable terms and conditions of the Blackstone Credit Agreement. The Blackstone Credit Agreement contains certain customary representations and warranties, affirmative covenants and provisions relating to events of default.

The Blackstone Credit Agreement consists of the following:

	September 30, 2023	December 31, 2022
Principal	$ 300,000	$ 300,000
Less: Debt issuance costs	(10,742)	(11,322)
Net carrying amount	$ 289,258	$ 288,678

As of September 30, 2023, the remaining contractual life of the Blackstone Credit Agreement is approximately 6.1 years.

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

	Three Months Ended September 30, 2023	Nine Months Ended September 30, 2023
Contractual interest expense	$ 9,554	$ 28,087
Amortization of debt issuance costs	280	702
Total	$ 9,834	$ 28,789
Effective interest rate	13.1 %	13.1 %

2026 Convertible Notes

In September 2019, the Company issued, at par value, $287.5 million aggregate principal amount of 1.50% convertible senior notes due 2026, which included an option to purchase up to an additional $37.5 million in aggregate principal amount of the 2026 Convertible Notes, which was exercised in full by the initial purchasers. The 2026 Convertible Notes bear cash interest at a rate of 1.50% per year, payable semi-annually on March 15 and September 15 of each year, beginning on March 15, 2020. The 2026 Convertible Notes will mature on September 15, 2026, unless earlier repurchased or converted. The net proceeds to the Company from the offering were $279.3 million after deducting the initial purchasers' discounts and commissions and the offering expenses payable by the Company.

The 2026 Convertible Notes are governed by an indenture (the "2026 Convertible Notes Indenture") with U.S Bank National Association as trustee (the "2026 Convertible Notes Trustee").

Holders of the 2026 Convertible Notes may convert their 2026 Convertible Notes at their option at any time prior to the close of business on the business day immediately preceding March 15, 2026 only under the following circumstances:

- during any calendar quarter commencing on or after December 31, 2019 (and only during such calendar quarter), if the last reported sale price of the Company's common stock for at least 20 trading days (whether or not consecutive) during a period of 30 consecutive trading days ending on the last trading day of the immediately preceding calendar quarter is greater than or equal to 130% of the conversion price on each applicable trading day;

- during the five business day period after any five consecutive trading day period (the "measurement period") in which the trading price (as defined in the 2026 Convertible Notes Indenture) per $1,000 principal amount of 2026 Convertible Notes for each trading day of the measurement period was less than 98% of the product of the last reported sale price of the Company's common stock and the conversion rate on each such trading day;

- during any period after the Company has issued notice of redemption until the close of business on the scheduled trading day immediately preceding the relevant redemption date; or

- upon the occurrence of specified corporate events.

On or after March 15, 2026, until the close of business on the business day immediately preceding the maturity date, holders may convert their 2026 Convertible Notes at any time, regardless of the foregoing circumstances. Upon conversion, the Company will pay or deliver, as the case may be, cash, shares of the Company's common stock or any combination thereof at the Company's election.

The conversion rate for the 2026 Convertible Notes was initially, and remains, 19.0404 shares of the Company's common stock per $1,000 principal amount of the 2026 Convertible Notes, which is equivalent to an initial conversion price of approximately $52.52 per share of the Company's common stock. The conversion rate may be subject to adjustment in some events but will not be adjusted for any accrued and unpaid interest.

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

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

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

The 2026 Convertible Notes consist of the following:

	September 30, 2023	December 31, 2022
Principal	$ 287,500	$ 287,500
Less: Debt issuance costs	(3,584)	(4,456)
Net carrying amount	$ 283,916	$ 283,044

As of September 30, 2023, the remaining contractual life of the 2026 Convertible Notes is approximately 3.0 years.

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,	
	2023	2022	2023	2022
Contractual interest expense	$ 1,080	$ 1,081	$ 3,215	$ 3,217
Amortization of debt issuance costs	294	289	872	856
Total	$ 1,374	$ 1,370	$ 4,087	$ 4,073
Effective interest rate	1.9 %	1.9 %	1.9 %	1.9 %

In April 2022, under the terms of the 2026 Convertible Notes Indenture, the Company paid additional interest on the 2026 Convertible Notes at a rate equal to 0.5% per annum, for a total interest payment of approximately $2.1 million, for the period beginning September 25, 2020 and ending March 14, 2022. In September 2022, under the terms of the 2026 Convertible Notes Indenture, the Company paid additional interest on the 2026 Convertible Notes at a rate equal to 0.5% per annum, for a total interest payment of approximately $0.1 million, for the period beginning March 15, 2022 and ending April 8, 2022. These amounts are not included in the table above, but were recorded as interest expense, net within the statement of operations for the nine months ended September 30, 2022.

2022 Convertible Notes

In August 2015, the Company issued, at par value, $150.0 million aggregate principal amount of 3.00% convertible senior notes due 2022, (the “2022 Convertible Notes”). On August 15, 2022, the Company repaid the outstanding principal amount and accrued interest, totaling $152.3 million, of the 2022 Convertible Notes that was due upon maturity in accordance with the terms of the notes.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Contractual interest expense	$ —	$ 559	$ —	$ 2,800
Amortization of debt issuance costs	—	92	—	460
Total	$ —	$ 651	$ —	$ 3,260
Effective interest rate	— %	3.5 %	— %	3.5 %

10. Commitments and contingencies

Under various agreements, the Company will be required to pay royalties and milestone payments upon the successful development and commercialization of products. The Company has entered into funding agreements with The Wellcome Trust Limited ("Wellcome Trust") for the research and development of small molecule compounds in connection with the Company’s oncology and antibacterial programs. As the Company has discontinued development under its antibacterial program, it no longer expects that milestone and royalty payments from the Company to Wellcome Trust will apply under that agreement, resulting in a change to the total amount of development and regulatory milestone payments the Company may become obligated to pay for this program. Under the oncology program funding agreement, to the extent that the Company develops and commercializes program intellectual property on a for-profit basis itself or in collaboration with a partner (provided the Company retains overall control of worldwide commercialization), the Company may become obligated to pay to Wellcome Trust development and regulatory milestone payments and single-digit royalties on sales of any research program product. The Company’s obligation to pay such royalties would continue on a country-by-country basis until the longer of the expiration of the last patent in the program intellectual property in such country covering the research program product and the expiration of market exclusivity of such product in such country. The Company made the first development milestone payment of $0.8 million to Wellcome Trust under the oncology platform funding agreement during the second quarter of 2016. During the year ended December 31, 2022, the Company incurred $2.5 million of development milestones in connection with the enrollment of patients in the registration-directed Phase 2/3 trial of unesbulin for the treatment of LMS, which is recorded in accounts payable and accrued expenses on the balance sheet and will be payable upon the earlier to occur of the first dose administered to the last patient enrolled in the study or the

termination of dosing of all patients in the study. Additional milestone payments of up to an aggregate of $14.5 million may become payable by the Company to Wellcome Trust under this agreement.

The Company has also entered into a collaboration agreement with the SMA Foundation. The Company is obligated to pay the SMA Foundation single-digit royalties on worldwide net product sales of any collaboration product that is successfully developed and subsequently commercialized or, with respect to collaboration products the Company outlicenses, including Evrysdi, a specified percentage of certain payments the Company receives from its licensee. Since inception, the SMA Foundation has earned $40.3 million, $35.3 million which was paid and $5.0 million which was accrued as of September 30, 2023. The Company's obligation to make such payments would end upon the Company's payment to the SMA Foundation of an aggregate of $52.5 million.

Pursuant to the asset purchase agreement ("Asset Purchase Agreement") between the Company and Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC) ("Marathon"), Marathon is entitled to receive contingent payments from the Company based on annual net sales of Emflaza up to a specified aggregate maximum amount over the expected commercial life of the asset. In addition, Marathon received a $50.0 million sales-based milestone during the nine months ended September 30, 2022.

Pursuant to the Agilis Merger Agreement, Agilis equityholders were previously entitled to receive contingent consideration payments from the Company based on (i) the achievement of certain development milestones up to an aggregate maximum amount of $60.0 million, (ii) the achievement of certain regulatory approval milestones together with a milestone payment following the receipt of a priority review voucher up to an aggregate maximum amount of $535.0 million, (iii) the achievement of certain net sales milestones up to an aggregate maximum amount of $150.0 million, and (iv) a percentage of annual net sales for Friedreich ataxia and Angelman syndrome during specified terms, ranging from 2%-6%. The Company was required to pay $40.0 million of the development milestone payments upon the passing of the second anniversary of the closing of the Agilis Merger, regardless of whether the applicable milestones have been achieved.

Pursuant to the terms of the Rights Exchange Agreement, the Participating Rightholders canceled and forfeited their rights under the Agilis Merger Agreement to receive (i) $174.0 million, in the aggregate, of potential milestone payments based on the achievement of certain regulatory milestones and (ii) $37.6 million, in the aggregate, of $40.0 million in development milestone payments that would have been due upon the passing of the second anniversary of the closing of the Agilis Merger, regardless of whether the milestones are achieved.

The Rights Exchange Agreement has no effect on the Agilis Merger Agreement other than to provide for the cancellation and forfeiture of the Participating Rightholders' rights to receive $211.6 million, in the aggregate, of the milestone payments described above. As a result, all other rights and obligations under the Agilis Merger Agreement remain in effect pursuant to their terms, including the Company's obligation to pay up to an aggregate maximum amount of $20.0 million upon the achievement of certain development milestones (representing the remaining portion of potential development milestone payments for which rights were not canceled and forfeited pursuant to the Rights Exchange Agreement while excluding the remaining $2.4 million milestone payment that was due and paid upon the passing of the second anniversary of the closing of the Agilis Merger), up to an aggregate maximum amount of $361.0 million upon the achievement of certain regulatory milestones (representing the remaining portion of potential regulatory milestone payments for which rights were not canceled and forfeited pursuant to the Rights Exchange Agreement), up to a maximum aggregate amount of $150.0 million upon the achievement of certain net sales milestones and a percentage of annual net sales for Friedreich ataxia and Angelman syndrome during specified terms, ranging from 2% to 6%, pursuant to the terms of the Agilis Merger Agreement.

In July 2022, the European Commission approved Upstaza for the treatment of AADC deficiency for patients 18 months and older within the EEA. As a result of such approval, the Company paid the former equityholders of Agilis $50.0 million in accordance with the terms of the Agilis Merger Agreement in the year ended December 31, 2022. In May 2023, as part of the Company's strategic portfolio prioritization, the Company decided to discontinue its preclinical and early research programs in its gene therapy platform, which included Friedreich ataxia and Angelman syndrome. As a result, the Company does not expect the milestones related to Friedreich ataxia and Angelman syndrome to be achieved. In addition, the Company does not expect to pay the 2% to 6% royalties on annual net sales related to Friedreich ataxia and Angelman syndrome. As of September 30, 2023, the remaining potential development and regulatory milestones the Company

expects to achieve is $31.1 million, and the remaining potential sales milestones the Company expects to achieve is $50.0 million, both of which relate solely to Upstaza.

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

Subject to the terms and conditions of the BioElectron Asset Purchase Agreement, BioElectron may become entitled to receive contingent milestone payments of up to $200.0 million (in cash or in shares of the Company's common stock, as determined by the Company) from the Company based on the achievement of certain regulatory and net sales milestones. Subject to the terms and conditions of the BioElectron Asset Purchase Agreement, BioElectron may also become entitled to receive contingent payments based on a percentage of net sales of certain products.

Subject to the terms and conditions of the Agreement and Plan of Merger, dated as of May 5, 2020 (the "Censa Merger Agreement") by and among the Company, Hydro Merger Sub, Inc., the Company's wholly owned, indirect subsidiary, and, solely in its capacity as the representative, agent and attorney-in-fact of the securityholders of Censa, Shareholder Representative Services LLC (such merger pursuant thereto, the "Censa Merger"), former Censa securityholders may become entitled to receive contingent payments from the Company based on (i) the achievement of certain development and regulatory milestones up to an aggregate maximum amount of $217.5 million for sepiapterin's two most advanced programs and receipt of a priority review voucher from the FDA as set forth in the Censa Merger Agreement, (ii) $109.0 million in development and regulatory milestones for each additional indication of sepiapterin, (iii) the achievement of certain net sales milestones up to an aggregate maximum amount of $160.0 million, (iv) a percentage of annual net sales during specified terms, ranging from single to low double digits of the applicable net sales threshold amount, and (v) any sublicense fees paid to the Company in consideration of any sublicense of Censa's intellectual property to commercialize sepiapterin, on a country-by-country basis, which contingent payment shall equal to a mid-double digit percentage of any such sublicense fees.

In February 2023, the Company completed enrollment of its Phase 3 placebo-controlled clinical trial for sepiapterin for PKU. In connection with this event and pursuant to the Censa Merger Agreement, the Company paid a $30.0 million development milestone to the former Censa securityholders during the nine months ended September 30, 2023. The Company elected to pay this milestone in the form of shares of its common stock, less certain cash payments in accordance with the Censa Merger Agreement. Pursuant to such election, the Company issued 657,462 shares of its common stock and paid $0.4 million to the former Censa securityholders.

The Company also has the Tegsedi-Waylivra Agreement for the commercialization of Tegsedi and Waylivra, and products containing those compounds in countries in Latin America and the Caribbean. Akcea is entitled to receive royalty payments subject to certain terms set forth in the Tegsedi-Waylivra Agreement.

The Company has employment agreements with certain employees which require the funding of a specific level of payments, if certain events, such as a change in control or termination without cause, occur. Additionally, the Company has royalty payments associated with Translarna, Emflaza, and Upstaza net product revenue, payable quarterly or annually in accordance with the terms of the related agreements.

From time to time in the ordinary course of its business, the Company is subject to claims, legal proceedings and disputes. The Company is not currently aware of any material legal proceedings against it.

11. Revenue recognition

Net product sales

The Company views its operations and manages its business in one operating segment.

During the three months ended September 30, 2023 and 2022, net product sales outside of the United States were $76.6 million and $79.4 million, respectively, consisting of sales of Translarna, Tegsedi, Waylivra, and Upstaza. Translarna net revenues made up $69.0 million and $76.6 million of the net product sales outside of the United States for the three months ended September 30, 2023 and 2022, respectively. During the three months ended September 30, 2023, and 2022, net product sales in the United States were $67.4 million and $54.8 million, respectively, consisting solely of sales of Emflaza. During the three months ended September 30, 2023, one country, the United States, accounted for at least 10% of the Company's net product sales, representing $67.4 million of the net product sales. During the three months ended September 30, 2022, two countries, the United States and Russia, accounted for at least 10% of the Company's net product sales, representing $54.8 million and $28.3 million of the net product sales, respectively. For the three months ended September 30, 2023 and 2022, the Company had a total of three and two distributors, respectively, that each accounted for over 10% of the Company's net product sales.

During the nine months ended September 30, 2023 and 2022, net product sales outside of the United States were $318.5 million and $247.6 million, respectively, consisting of Translarna, Tegsedi, Waylivra, and Upstaza. Translarna net revenues made up $280.6 million and $232.9 million of the net product sales outside of the United States for the nine months ended September 30, 2023 and 2022, respectively. For the nine months ended September 30, 2023 and 2022, net product sales in the United States were $187.7 million and $160.1 million, respectively, consisting solely of Emflaza. During the nine months ended September 30, 2023, three countries, the United States, Russia, and Brazil, accounted for at least 10% of the Company's net product sales, representing $187.7 million, $69.0 million, and $52.5 million of net product sales, respectively. During the nine months ended September 30, 2022, two countries, the United States and Russia, accounted for at least 10% of the Company's net product sales, representing $160.1 million and $57.1 million of net product sales, respectively. For the nine months ended September 30, 2023 and 2022, the Company had a total of two and two distributors, respectively, that each accounted for over 10% of the Company's net product sales.

As of September 30, 2023 and December 31, 2022, the Company does not have a contract liabilities balance related to net product sales, and has not made significant changes to the judgments made in applying ASC Topic 606.

Collaboration and Royalty revenue

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

Under the SMA License Agreement, the Company is eligible to receive additional payments from Roche if specified events are achieved with respect to each licensed product, including up to $135.0 million in research and development event milestones, up to $325.0 million in sales milestones upon achievement of specified sales events, and up to double digit royalties on worldwide annual net sales of a commercial product.

The SMA program currently has one approved product, Evrysdi, which was approved in August 2020 by the FDA for the treatment of SMA in adults and children two months and older. As of September 30, 2023, 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 $250.0 million.

For the three months ended September 30, 2023, the Company did not recognize collaboration revenue related to the SMA License Agreement with Roche. For the three months ended September 30, 2022, the Company recognized $50.0 million of collaboration revenue related to the SMA License Agreement. For the nine months ended September 30, 2023, the amounts recognized for the collaboration revenue related to the SMA License Agreement with Roche were immaterial. For the nine months ended September 30, 2022, the Company recognized $50.0 million of collaboration revenue related

to the SMA License Agreement. In September 2022, the Company recognized a sales milestone of $50.0 million for the achievement of $750.0 million in worldwide annual net sales from Evrysdi.

In addition to research and development and sales milestones, the Company is eligible to receive up to double-digit royalties on worldwide annual net sales of a commercial product under the SMA License Agreement. For the three and nine months ended September 30, 2023, the Company has recognized $50.2 million and $117.9 million of royalty revenue related to Evrysdi, respectively. For the three and nine months ended September 30, 2022, the Company has recognized $32.9 million and $73.6 million of royalty revenue, respectively, related to Evrysdi.

Manufacturing Revenue

For the three and nine months ended September 30, 2023, the Company recognized $2.4 million and $6.7 million of manufacturing revenue, respectively, related to the production of plasmid DNA and AAV vectors for gene therapy applications for external customers. No manufacturing revenue was recognized for the three and nine months ended September 30, 2022. The Company has not made significant changes to the judgments made in applying ASC Topic 606 for the three and nine months ended September 30, 2023 and 2022.

As of September 30, 2023 and December 31, 2022, the Company has a contract liabilities balance of $1.2 million and $1.4 million, respectively, related to the production of plasmid DNA and AAV vectors for gene therapy applications for external customers, which is recorded within deferred revenue on the consolidated balance sheet. For the three-month period ended September 30, 2023, the Company did not recognize any revenue related to the amount included in the contract liability balance at the beginning of the period. For the nine-month period ended September 30, 2023, the Company recognized $1.4 million related to the amounts included in the contract liability balance at the beginning of the period.

As of September 30, 2023, the Company has contract assets of $1.4 million related to plasmid DNA and AAV production for external customers, which is recorded within prepaid expenses and other current assets on the consolidated balance sheet. The Company did not have any contract assets for the period ending December 31, 2022.

Remaining performance obligations

As of September 30, 2023 and December 31, 2022, the Company has remaining performance obligations of $1.2 million and $1.4 million, respectively, related to the production of plasmid DNA and AAV vectors for gene therapy applications for external customers. The Company expects to recognize revenue over the next one year, as the specific timing for satisfying the performance obligations is contingent upon a number of factors, including customers' needs and schedules.

12. Intangible assets and goodwill

Definite-lived intangibles

Definite-lived intangible assets consisted of the following at September 30, 2023 and December 31, 2022:

Definite-lived intangibles assets, gross	Ending Balance at December 31, 2022	Additions	Reclass from Indefinite Lived to Definite Lived	Impairment	Foreign currency translation	Ending Balance at September 30, 2023
Emflaza	$ 420,253	$ 76,454	$ —	$ —	$ —	$ 496,707
Waylivra	9,316	157	—	—	(96)	9,377
Tegsedi	7,109	4,299	—	—	(183)	11,225
Upstaza	89,550	—	—	—	—	89,550
Total definite-lived intangibles, gross	$ 526,228	$ 80,910	$ —	$ —	$ (279)	$ 606,859

Definite-lived intangibles assets, accumulated amortization	Ending Balance at December 31, 2022	Amortization	Foreign currency translation	Ending Balance at September 30, 2023
Emflaza	$ (266,023)	$ (138,038)	$ —	$ (404,061)

Waylivra	(2,751)	(791)	45	(3,497)
Tegsedi	(1,709)	(1,035)	36	(2,708)
Upstaza	(3,420)	(5,597)	—	(9,017)
Total definite-lived intangibles, accumulated amortization	$ (273,903)	$ (145,461)	$ 81	$ (419,283)

Total definite-lived intangibles, net	$ 187,576

Marathon is entitled to receive contingent payments from the Company based on annual net sales of Emflaza beginning in 2018, up to a specified aggregate maximum amount over the expected commercial life of the asset. In accordance with the guidance for an asset acquisition, the Company records the milestone payment when it becomes payable to Marathon and increases the cost basis for the Emflaza rights intangible asset. For the nine months ended September 30, 2023, total milestone payments of $76.5 million were recorded. These payments are being amortized over the remaining useful life of the Emflaza rights asset on a straight line basis. As of September 30, 2023, a milestone payable to Marathon of $43.9 million was recorded on the consolidated balance sheet within accounts payable and accrued expenses.

Akcea is also entitled to receive royalty payments subject to certain terms set forth in the Tegsedi-Waylivra Agreement related to sales of Waylivra and Tegsedi. In accordance with the guidance for an asset acquisition, the Company records royalty payments when they become payable to Akcea and increase the cost basis for the Waylivra and Tegsedi intangible assets. For the nine months ended September 30, 2023, royalty payments of $4.3 million and $0.2 million were recorded for Tegsedi and Waylivra, respectively. As of September 30, 2023, a royalty payable of $0.9 million and $0.2 million for Tegsedi and Waylivra, respectively, was recorded on the consolidated balance sheet within accounts payable and accrued expenses.

For the three months ended September 30, 2023 and 2022, the Company recognized amortization expense of $58.6 million and $31.0 million, respectively, related to the Emflaza rights, Upstaza, Waylivra, and Tegsedi intangible assets. For the nine months ended September 30, 2023 and 2022, the Company recognized amortization expense of $145.5 million and $80.8 million, respectively, related to the Emflaza rights, Upstaza, Waylivra, and Tegsedi intangible assets. The estimated future amortization of the Emflaza rights, Upstaza, Waylivra, and Tegsedi intangible assets is expected to be as follows:

	As of September 30, 2023
2023	$ 58,640
2024	46,557
2025	10,041
2026	10,041
2027 and thereafter	62,297
Total	$ 187,576

The weighted average remaining amortization period of the definite-lived intangibles as of September 30, 2023 is 5.3 years.

Indefinite-lived intangibles

Indefinite-lived intangible assets consisted of the following at September 30, 2023 and December 31, 2022:

Indefinite-lived intangibles assets	**Ending Balance at December 31, 2022**	**Additions**	**Reclass from Indefinite Lived to Definite Lived**	**Impairment**	**Foreign currency translation**	**Ending Balance at September 30, 2023**
Upstaza	$ 235,766	$ —	$ —	$ —	$ —	$ 235,766
PTC-FA	112,500	—	—	(112,500)	—	—
PTC-AS	105,300	—	—	(105,300)	—	—
Total indefinite-lived intangibles	$ 453,566	$ —	$ —	$ (217,800)	$ —	$ 235,766

Total intangible assets, net	$ 423,342

In connection with the acquisition of the Company's gene therapy platform from Agilis, the Company acquired rights to Upstaza, for the treatment of AADC deficiency. AADC deficiency is a rare CNS disorder arising from reductions in the enzyme AADC that result from mutations in the dopa decarboxylase gene. The gene therapy platform also includes PTC-FA, an asset targeting Friedreich ataxia, a rare and life-shortening neurodegenerative disease caused by a single defect in the FXN gene which causes reduced production of the frataxin protein. Additionally, the gene therapy platform includes two other programs targeting CNS disorders, including PTC-AS for Angelman syndrome, a rare, genetic, neurological disorder characterized by severe developmental delays.

In accordance with the acquisition method of accounting, the Company allocated the acquisition cost for the Agilis Merger to the underlying assets acquired and liabilities assumed, based upon the estimated fair values of those assets and liabilities at the date of acquisition. The Company classified the fair value of the acquired IPR&D as indefinite lived intangible assets until the successful completion or abandonment of the associated research and development efforts. As of December 31, 2022, the value allocated to the indefinite lived intangible assets was $453.6 million.

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 PTC-FA and PTC-AS. As a result, the Company determined that the PTC-FA and PTC-AS indefinite-lived intangible assets were fully impaired and recorded impairment expense of $217.8 million during the nine month period ended September 30, 2023, which is recorded as intangible asset impairment in the statement of operations. As of September 30, 2023, the remaining indefinite lived intangible asset balance is $235.8 million, consisting solely of Upstaza, which the Company plans to continue to develop and commercialize.

Goodwill

As a result of the Agilis Merger on August 23, 2018, the Company recorded $82.3 million of goodwill. As of September 30, 2023, 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, 2023 is $82.3 million.

13. Subsequent events

Royalty Purchase Agreement

On October 18, 2023, the Company, Royalty Pharma Investments 2019 ICAV ("Royalty Pharma"), and, for the limited purposes set forth in the agreement, Royalty Pharma plc, entered into an Amended and Restated Royalty Purchase Agreement (the "A&R Royalty Purchase Agreement"), which amends and restates in its entirety the Royalty Purchase Agreement. Pursuant to the A&R Royalty Purchase Agreement, the Company has sold or agreed to sell to Royalty Pharma certain portions of the Company's remaining Royalty on worldwide net sales of Evrysdi and any other product (the "Products") developed pursuant to the SMA License Agreement (all such retained Royalty rights of the Company, the "Retained Royalty Rights," and all such Royalty rights that are sold to Royalty Pharma pursuant to the A&R Royalty Purchase Agreement, the "A&R Assigned Royalty Rights"). At closing, Royalty Pharma paid the Company $1.0 billion in cash consideration for 38.0447% of the Company's Retained Royalty Rights (which is in addition to the 42.9330% assigned to Royalty Pharma in connection with the Royalty Purchase Agreement, for a total of 80.9777% of the total Royalty) until such time as Royalty Pharma has received payments in respect of the A&R Assigned Royalty Rights equal to $1.3 billion in the aggregate, and thereafter 66.6667% of the total Royalty. In addition, the Company may sell to Royalty Pharma the remainder of the Company's Retained Royalty Rights in exchange for an aggregate of $500.0 million in additional cash consideration after the closing of the A&R Royalty Purchase Agreement, less royalties received in respect of the Retained Royalty Rights put to Royalty Pharma, which will be payable by Royalty Pharma pursuant to five put options held by the Company that are exercisable at the Company's option between January 1, 2024 and December 31, 2025. If the Company exercises two or fewer of the put options, Royalty Pharma may exercise a call option during the period from and after January 1, 2026 until and including March 31, 2026 for up to 50% of the remainder of the Company's Retained Royalty Rights less amounts exercised by the Company via its put options at a purchase price that is proportional to the purchase price of the Company's unexercised put options. Royalty Pharma's exercise of the call option would result in Royalty Pharma owning 90.4888% of the total Royalty until such time as Royalty Pharma has received payments in respect of the A&R Assigned Royalty Rights equal to $1.3 billion in the aggregate, and thereafter 83.3333% of the total Royalty. The Company is evaluating the accounting impact of the A&R Royalty Purchase Agreement.

Under the A&R Royalty Purchase Agreement, and in connection with its sale of the A&R Assigned Royalty Rights, the Company has agreed to specified negative and affirmative covenants with respect to the exercise of its rights under the SMA License Agreement, including the Company's right to amend, modify, assign or terminate the SMA License Agreement. Subject to certain customary exceptions, the Company has agreed not to grant a security interest in its interest in the SMA License Agreement, the Products or the patent rights that cover the Products. The A&R Royalty Purchase Agreement also contains representations and warranties, covenants and other provisions, including information rights and confidentiality obligations, customary for transactions of this nature.

The A&R Royalty Purchase Agreement will terminate 60 days following the date on which Roche is no longer obligated to make any payments of the Royalty pursuant to the License Agreement.

Senior Secured Term Loan Termination

On October 19, 2023, the Company terminated the Blackstone Credit Agreement. In connection with the termination of the Blackstone Credit Agreement, the Company repaid outstanding principal and accrued interest thereunder totaling $302.1 million and paid an additional $82.1 million in prepayment premiums, expenses and other exit fees. All liens and security interests securing the loans made pursuant to the Blackstone Credit Agreement were released upon termination.

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, 2022 included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 21, 2023, as amended, or our 2022 Annual Report. This discussion contains forward-looking statements that involve significant risks and uncertainties. As a result of many factors, such as those set forth in Part II, Item 1A. (Risk Factors) of this Quarterly Report on Form 10-Q and Part I, Item 1A. (Risk Factors) of our 2022 Annual Report, our actual results may differ materially from those anticipated in these forward-looking statements.

Our Company

We are a science-driven global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. Our ability to innovate to identify new therapies and to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. Our mission is to provide access to best-in-class treatments for patients who have little to no treatment options. Our strategy is to leverage our strong scientific and clinical expertise and global commercial infrastructure to bring therapies to patients. We believe that this allows us to maximize value for all of our stakeholders. We have a 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, metabolism and oncology.

Corporate Updates

Royalty Purchase Agreement

In October 2023, we entered into an Amended and Restated Royalty Purchase Agreement, or the A&R Royalty Purchase Agreement with Royalty Pharma Investments 2019 ICAV, or Royalty Pharma, and, for the limited purposes set forth in the agreement, Royalty Pharma plc which amends and restates in its entirety that certain Royalty Purchase Agreement dated as of July 17, 2020, or the Original Royalty Purchase Agreement. Pursuant to the A&R Royalty Purchase Agreement, we have sold or agreed to sell to Royalty Pharma certain portions of our remaining retained right, title and interest in and to our right to receive sales-based royalty payments, or the Royalty, on worldwide net sales of Evrysdi and any other product developed pursuant to the License and Collaboration Agreement, or the SMA License Agreement, dated as of November 23, 2011, by and among us, F. Hoffman-La Roche Ltd., Hoffman-La Roche Inc., together with F. Hoffman-La Roche Ltd, Roche, and, for the limited purposes set forth therein, the Spinal Muscular Atrophy Foundation, or the SMA Foundation, under the spinal muscular atrophy, or SMA, program (all such Royalty rights retained by us, are referred to as the Retained Royalty Rights, and all such Royalty rights that are sold to Royalty Pharma pursuant to the A&R Royalty Purchase Agreement, are referred to as the A&R Assigned Royalty Rights). At closing, Royalty Pharma paid us $1.0 billion in cash consideration for 38.0447% of our Retained Royalty Rights (which is in addition to the 42.9330% assigned to Royalty Pharma in connection with the Original Royalty Purchase Agreement, for a total of 80.9777% of the total Royalty) until such time as Royalty Pharma has received payments in respect of the A&R Assigned Royalty Rights equal to $1.3 billion in the aggregate, and thereafter 66.6667% of the total Royalty.

Blackstone Credit Agreement Termination

In October 2023, we terminated the Credit Agreement, dated October 27, 2022, by and among us and certain of our subsidiaries from time to time party thereto, as guarantors, or, collectively with us, the Loan Parties, funds and other affiliated entities advised or managed by Blackstone Life Sciences and Blackstone Credit, or collectively, Blackstone, as lenders, together with their permitted assignees, the Lenders, and Wilmington Trust, National Association, as the administrative agent for the Lenders, or the Blackstone Credit Agreement. In connection with the termination of the Blackstone Credit Agreement, we repaid outstanding principal and accrued interest thereunder totaling $302.1 million and paid an additional $82.1 million in prepayment premiums, expenses and other exit fees. All liens and security interests securing the loans made pursuant to the Blackstone Credit Agreement were released upon termination.

Global Commercial Footprint

Global DMD Franchise

We have two products, Translarna™ (ataluren) and Emflaza® (deflazacort), for the treatment of Duchenne muscular dystrophy, or DMD, a rare, life threatening disorder. Translarna has marketing authorization in the European Economic Area, or EEA, for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, in ambulatory patients aged two years and older and in Russia for the treatment of nmDMD in patients aged two years and older. Translarna also has marketing authorization in Brazil for the treatment of nmDMD in ambulatory patients two years and older and for continued treatment of patients that become non-ambulatory. In July 2020, the European Commission, or the EC, approved the removal of the statement “efficacy has not been demonstrated in non-ambulatory patients” from the indication statement for Translarna. During the quarter ended September 30, 2023, we recognized $69.0 million in net sales from Translarna. We hold worldwide commercialization rights to Translarna for all indications in all territories. Emflaza is approved in the United States for the treatment of DMD in patients two years and older. During the quarter ended September 30, 2023, we recognized $67.4 million in net sales from Emflaza.

Our marketing authorization for Translarna in the EEA is subject to annual review and renewal by the EC following reassessment by the European Medicines Agency, or EMA, of the benefit-risk balance of the authorization, which we refer to as the annual EMA reassessment. In September 2022, we submitted a Type II variation to the EMA to support conversion of the conditional marketing authorization for Translarna to a standard marketing authorization, which included a report on the placebo-controlled trial of Study 041 and data from the open-label extension as further described below. In February 2023, we also submitted an annual marketing authorization renewal request to the EMA. In September 2023, the Committee for Medicinal Products for Human Use, or CHMP, gave a negative opinion on the conversion of the conditional marketing authorization to full marketing authorization of Translarna for the treatment of nmDMD and a negative opinion on the renewal of the existing conditional marketing authorization of Translarna for the treatment of nmDMD. We have requested re-examination of the CHMP opinion regarding the renewal of the existing conditional marketing authorization. In re-examination, we intend to address concerns raised during the previous review process on the benefit demonstrated in Translarna clinical trials and regarding the robustness of the Strategic Targeting of Registries and International Database of Excellence, or STRIDE, real world registry. In accordance with EMA guidelines, we expect the CHMP opinion following the re-examination process to occur in late January 2024, with EC ratification of the opinion within the following 67 days.

The conditional marketing authorization is further subject to a specific obligation to conduct and submit the results of an 18-month, placebo-controlled trial, followed by an 18-month open-label extension, which we refer to together as Study 041. In June 2022, we announced top-line results from the placebo-controlled trial of Study 041. Within the placebo-controlled trial, Translarna showed a statistically significant treatment benefit across the entire intent to treat population as assessed by the 6-minute walk test, assessing ambulation and endurance, and in lower-limb muscle function as assessed by the North Star Ambulatory Assessment, a functional scale designed for boys affected by DMD. Additionally, Translarna showed a statistically significant treatment benefit across the intent to treat population within the 10-meter run/walk and 4-stair stair climb, each assessing ambulation and burst activity, while also showing a positive trend in the 4-stair stair descend although not statistically significant. Within the primary analysis group, Translarna demonstrated a positive trend across all endpoints, however, statistical significance was not achieved. Translarna was also well tolerated.

Each country, including each member state of the EEA, has its own pricing and reimbursement regulations. In order to commence commercial sale of product pursuant to our Translarna marketing authorization in any particular country in the

EEA, we must finalize pricing and reimbursement negotiations with the applicable government body in such country. As a result, our commercial launch will continue to be on a country-by-country basis. We also have made, and expect to continue to make, product available under early access programs, or EAP programs, both in countries in the EEA and other territories. Our ability to negotiate, secure and maintain reimbursement for product under commercial and EAP programs can be subject to challenge in any particular country and can also be affected by political, economic and regulatory developments in such country.

There is substantial risk that if the EC ratifies the CHMP's negative opinion, we are otherwise unable to renew our EEA marketing authorization during any annual renewal cycle, our product label is materially restricted, or Study 041 does not provide the data necessary to maintain our marketing authorization, we would lose all, or a significant portion of, our ability to generate revenue from sales of Translarna in the EEA and other territories. For more information regarding the risks associated with the a potential EC ratification of the CHMP's negative opinion on Translarna's marketing authorization, see Item 1A. Risk Factors, "*We may be unable to continue to commercialize Translarna for nonsense mutation Duchenne muscular dystrophy in the European Economic Area if the Committee for Medicinal Products for Human Use of the European Medicines Agency does not reverse its negative opinion on the renewal of the existing conditional authorization for Translarna.*"

Translarna is an investigational new drug in the United States. During the first quarter of 2017, we filed a New Drug Application, or NDA, for Translarna for the treatment of nmDMD over protest with the United States Food and Drug Administration, or FDA. In October 2017, the Office of Drug Evaluation I of the FDA issued a Complete Response Letter for the NDA, stating that it was unable to approve the application in its current form. In response, we filed a formal dispute resolution request with the Office of New Drugs of the FDA. In February 2018, the Office of New Drugs of the FDA denied our appeal of the Complete Response Letter. In its response, the Office of New Drugs recommended a possible path forward for the ataluren NDA submission based on the accelerated approval pathway. This would involve a re-submission of an NDA containing the current data on effectiveness of ataluren with new data to be generated on dystrophin production in nmDMD patients' muscles. We followed the FDA's recommendation and collected, using newer technologies via procedures and methods that we designed, such dystrophin data in a new study, Study 045, and announced the results of Study 045 in February 2021. Study 045 did not meet its pre-specified primary endpoint. In June 2022, we announced top-line results from the placebo-controlled trial of Study 041. Following this announcement, we submitted a meeting request to the FDA to gain clarity on the regulatory pathway for a potential re-submission of an NDA for Translarna. The FDA provided initial written feedback that Study 041 does not provide substantial evidence of effectiveness to support NDA re-submission. We then had an informal meeting with the FDA, during which we discussed the potential path to an NDA re-submission for Translarna. Based on the meeting discussion, we have scheduled an additional Type C meeting with the FDA to review the totality of data collected to date, including dystrophin and other mechanistic data as well as additional analyses that could support the benefit of Translarna.

Upstaza™ (eladocagene exuparvovec)

We have developed Upstaza, a gene therapy used for the treatment of Aromatic L-Amino Decarboxylase, or AADC, deficiency, a rare central nervous system, or CNS, disorder arising from reductions in the enzyme AADC that results from mutations in the dopa decarboxylase gene. In July 2022, the EC approved Upstaza for the treatment of AADC deficiency for patients 18 months and older within the EEA. In November 2022, the Medicines and Healthcare Products Regulatory Agency approved Upstaza for the treatment of AADC deficiency for patients 18 months and older within the United Kingdom. We are also preparing a biologics license application, or BLA, for Upstaza for the treatment of AADC deficiency in the United States. In October 2022, we held a Type C meeting with the FDA to discuss the details of a potential submission package for Upstaza. At that meeting, the FDA asked for additional bioanalytical data in support of comparability between the drug product used in the clinical studies and the commercial drug product. We completed these analyses and provided the results to the FDA for review. The FDA stated that the data that we provided were still not sufficient. However, the FDA also said that the available data from the ongoing clinical study in the United States assessing the safety of the drug delivery cannula for Upstaza could be used to support a BLA for accelerated approval based on biomarker data demonstrating a treatment-related increase in de novo dopamine production. At the FDA's suggestion, we have scheduled a pre-BLA meeting for December 2023 and, pending the outcome of that meeting, we expect to submit a BLA shortly thereafter.

Tegsedi® (inotersen) and Waylivra™ (volanesorsen)

We hold the rights for the commercialization of Tegsedi and Waylivra for the treatment of rare diseases in countries in Latin America and the Caribbean pursuant to a Collaboration and License Agreement, or the Tegsedi-Waylivra Agreement, dated August 1, 2018, by and between us and Akcea Therapeutics, Inc., or Akcea, a subsidiary of Ionis Pharmaceuticals, Inc. Tegsedi has received marketing authorization in the United States, European Union, or EU, and Brazil for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis, or hATTR amyloidosis. We began to make commercial sales of Tegsedi for the treatment of hATTR amyloidosis in Brazil in the second quarter of 2022 and we continue to make Tegsedi available in certain other countries within Latin America and the Caribbean through EAP programs. In August 2021, ANVISA, the Brazilian health regulatory authority, approved Waylivra as the first treatment for familial chylomicronemia syndrome, or FCS, in Brazil and we began to make commercial sales of Waylivra in Brazil in the third quarter of 2022 while continuing to make Waylivra available in certain other countries within Latin America and the Caribbean through EAP programs. In December 2022, ANVISA approved Waylivra for the treatment of familial partial lipodystrophy, or FPL. Waylivra has also received marketing authorization in the EU for the treatment of FCS.

Evrysdi® (risdiplam)

We also have an SMA collaboration with Roche and the 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 Brazil in October 2020 and Japan in June 2021. 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 PTC518, which is being developed for the treatment of Huntington's disease, or HD. We announced the results from our Phase 1 study of PTC518 in healthy volunteers in September 2021 demonstrating dose-dependent lowering of huntingtin messenger ribonucleic acid and protein levels, that PTC518 efficiently crosses blood brain barrier at significant levels and that PTC518 was well tolerated. We initiated a Phase 2 study of PTC518 for the treatment of HD in the first quarter of 2022, which consists of an initial 12-week placebo-controlled phase focused on safety, pharmacology and pharmacodynamic effects followed by a nine-month placebo-controlled phase focused on PTC518 biomarker effect. In June 2023, we announced interim data from the 12-week placebo-controlled phase. The study demonstrated dose-dependent lowering of Huntingtin, or HTT, protein levels in peripheral blood cells, reaching an approximate mean 30% reduction in mutant HTT levels at the 10mg dose level. In addition, PTC518 exposure in the cerebrospinal fluid was consistent with or higher than plasma unbound drug levels. Furthermore, PTC518 was well tolerated with no treatment-related serious adverse events. We expect the next data update from the Phase 2 study of PTC518 for the treatment of HD in the first half of 2024. In the Phase 2 study, enrollment outside of the United States remains active and ongoing while enrollment within the United States is paused as the FDA requested additional data to allow the Phase 2 study to proceed. We had a Type A meeting with the FDA to review the clinical safety data needed to enable resumption of enrollment in the United States. At that meeting, the FDA stated that the existing three-month safety data could support 12-week dosing and that six months of clinical safety data could support dosing in the 12-month Phase 2 study.

Metabolic Platform

The most advanced molecule in our metabolic platform is sepiapterin, a precursor to intracellular tetrahydrobiopterin, which is a critical enzymatic cofactor involved in metabolism and synthesis of numerous metabolic products, for orphan diseases. In May 2023, we announced that the primary endpoint was achieved in our registration-directed Phase 3 trial for sepiapterin for phenylketonuria, or PKU. The primary endpoint of the study was the achievement of statistically-significant

reduction in blood Phe level. The primary analysis population included those patients who have a greater than 30% reduction in blood Phe levels during the Part 1 run-in phase of the trial. Sepiapterin demonstrated Phe level reduction of approximately 63% in the overall primary analysis population and Phe level reduction of approximately 69% in the subset for classical PKU patients. Additionally, sepiapterin was well tolerated with no serious adverse events. Following the placebo-controlled study, patients were eligible to enroll in a long-term open-label study, which is still ongoing and will evaluate long-term safety, durability and Phe tolerance. We participated in a pre-NDA meeting with the FDA in the third quarter of 2023. At that meeting, the FDA stated that the sepiapterin clinical safety and efficacy data supported NDA submission for the treatment of pediatric and adult PKU patients. However, the FDA has requested that we complete a 26-week nonclinical mouse study to assess sepiapterin carcinogenicity potential prior to NDA submission. This nonclinical study was not initially required when we acquired sepiapterin, as the NDA submission was planned under the Section 505(b)(2) pathway. With PTC's decision to file under the Section 505(b)(1) pathway, the 26-week study is considered a required NDA component needed to inform labeling and is typically completed prior to submission. We expect to submit an NDA to the FDA for sepiapterin for the treatment of PKU by the end of the third quarter of 2024 and we intend to discuss with the FDA the potential for an earlier submission if we are permitted to submit the 26-week mouse study report during the review process of the NDA. Additionally, we expect to submit a marketing authorization application, or MAA, to the EMA for sepiapterin for the treatment of PKU in the EEA in the first half of 2024.

Bio-e Platform

Our Bio-e platform consists of small molecule compounds that target oxidoreductase enzymes that regulate oxidative stress and inflammatory pathways central to the pathology of a number of CNS diseases. The two most advanced molecules in our Bio-e platform are vatiquinone and utreloxastat. We announced topline results from a registration-directed Phase 3 trial of vatiquinone in children and young adults with Friedreich ataxia in May 2023. While the study did not meet its primary endpoint of statistically significant change in modified Friedreich Ataxia Rating Scale, or mFARS, score at 72 weeks in the primary analysis population, vatiquinone treatment did demonstrate significant benefit on key disease subscales and secondary endpoints. In addition, in the population of subjects that completed the study protocol, significance was reached in the mFARS endpoint and several secondary endpoints, including the upright stability subscale. Furthermore, vatiquinone was well tolerated. We participated in a Type C written response only meeting with the FDA in the third quarter of 2023 to discuss the potential for an NDA submission for vatiquinone for the treatment of Friedreich ataxia. In their written response, the FDA stated that while they see the value of upright stability as a clinically meaningful endpoint, they believed a confirmatory study would likely be needed to support NDA submission. We have requested a follow-up live meeting to address the issues raised by the FDA. Additionally, we are participating in a scientific advice procedure with the EMA to determine if the data from the registration-directed Phase 3 trial of vatiquinone in children and young adults with Friedreich ataxia could support a conditional MAA in the EEA. In the third quarter of 2021, we completed a Phase 1 trial in healthy volunteers to evaluate the safety and pharmacology of utreloxastat. Utreloxastat was found to be well-tolerated with no reported serious adverse events while demonstrating predictable pharmacology. We initiated a Phase 2 trial of utreloxastat for amyotrophic lateral sclerosis in the first quarter of 2022 and enrollment is ongoing.

Oncology Platform

Unesbulin is our most advanced oncology agent. We completed our Phase 1 trials evaluating unesbulin in leiomyosarcoma, or LMS, and diffuse intrinsic pontine glioma, or DIPG, in the fourth quarter of 2021. We initiated a registration-directed Phase 2/3 trial of unesbulin for the treatment of LMS in the first quarter of 2022 and enrollment is ongoing. We are evaluating our plans for a potential initiation of a registration-directed Phase 2/3 trial of unesbulin for the treatment of DIPG.

Multi-Platform Discovery

In addition, we have a pipeline of product candidates and discovery programs that are in early clinical, pre-clinical and research and development stages focused on the development of new treatments for multiple therapeutic areas, including rare diseases and oncology.

In September 2023, we announced further strategic prioritization following the continued review of our portfolio that we began in May 2023. In connection with the strategic prioritization, we committed to a reduction in workforce of approximately 25%, which will primarily affect employees in the United States, including those employees involved in early-stage research and gene therapy manufacturing and associated selling, general and administrative functions. We plan to substantially complete the reduction in workforce by January 15, 2024.

COVID 19 Impact

The global pandemic caused by a strain of novel coronavirus, COVID-19, has impacted the timing of certain of our clinical trials and regulatory submissions as well as other aspects of our business operations. We cannot be certain what the overall impact of the COVID-19 pandemic will be on our business and it has the potential to materially adversely affect our business, financial condition, results of operations, and prospects. For additional information, see "Item 1A. Risk Factors - *We face risks related to health epidemics and other widespread outbreaks of contagious disease, which have previously, and may once again, delay our ability to complete our ongoing clinical trials and initiate future clinical trials, disrupt regulatory activities and have other adverse effects on our business and operations, including the novel coronavirus (COVID 19) pandemic, which disrupted, and may continue to disrupt, our operations and may significantly impact our operating results. In addition, the COVID 19 pandemic has caused substantial disruption in the financial markets and economies, which could result in adverse effects on our business and operations.*" in our 2022 Annual Report.

Funding

The success of our products and any other product candidates we may develop, depends largely on obtaining and maintaining reimbursement from governments and third-party insurers. Our revenues are primarily generated from sales of Translarna for the treatment of nmDMD in countries where we were able to obtain acceptable commercial pricing and reimbursement terms and in select countries where we are permitted to distribute Translarna under our EAP programs and from sales of Emflaza for the treatment of DMD in the United States. We have also recognized revenue from sales of Upstaza for the treatment of AADC deficiency in the EEA and have recognized revenue associated with milestone and royalty payments from Roche pursuant to the SMA License Agreement.

To date, we have financed our operations primarily through our offering of the 1.50% convertible senior notes due 2026, or the 2026 Convertible Notes, our public offerings of common stock in February 2014, in October 2014, in April 2018, in January 2019, and in September 2019, the common stock issued in our "at the marketing offering", our initial public offering of common stock in June 2013, proceeds from the A&R Royalty Purchase Agreement, 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 Translarna for the treatment of nmDMD in territories outside of the United States since 2014, Emflaza for the treatment of DMD in the United States since 2017 and Upstaza for the treatment of AADC deficiency in the EEA since 2022. We have also relied on revenue associated with milestone and royalty payments from Roche pursuant to the SMA License Agreement, under our SMA program.

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

90.4888% of the total Royalty until such time as Royalty Pharma has received payments in respect of the A&R Assigned Royalty Rights equal to $1.3 billion in the aggregate, and thereafter 83.3333% of the total Royalty.

In October 2022, we entered into the Blackstone Credit Agreement for fundings of up to $950.0 million consisting of a committed loan facility consisting of a senior secured term loan facility funded on October 27, 2022, or the Closing Date, in the aggregate principal amount of $300.0 million, and a delayed draw term loan facility of up to $150.0 million to be funded at our request within 18 months of the Closing Date subject to specified conditions, and further contemplating the potential for up to $500.0 million of additional financing, to the extent that we request such additional financing and subject to the Lenders' agreement to provide such additional financing and to mutual agreement on terms. In October 2023, we terminated the Blackstone Credit Agreement. In connection with the termination of the Blackstone Credit Agreement, we repaid outstanding principal and accrued interest thereunder totaling $302.1 million and paid an additional $82.1 million in prepayment premiums, expenses and other exit fees. All liens and security interests securing the loans made pursuant to the Blackstone Credit Agreement were released upon termination.

The 2026 Convertible Notes consist of $287.5 million aggregate principal amount of 1.50% convertible senior notes due 2026. The 2026 Convertible Notes bear cash interest at a rate of 1.50% per year, payable semi-annually on March 15 and September 15 of each year, beginning on March 15, 2020. The 2026 Convertible Notes will mature on September 15, 2026, unless earlier repurchased or converted. We received net proceeds of $279.3 million after deducting the initial purchasers' discounts and commissions and the offering expenses payable by us.

In August 2019, we entered into an At the Market Offering Sales Agreement, or the Sales Agreement, with Cantor Fitzgerald and RBC Capital Markets, LLC, or together, the Sales Agents, pursuant to which, we may offer and sell shares of our common stock, having an aggregate offering price of up to $125.0 million from time to time through the Sales Agents by any method that is deemed to be an "at the market offering" as defined in Rule 415(a)(4) promulgated under the Securities Act of 1933, as amended, or the Securities Act. During the three and nine months ended September 30, 2023, 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, 2023.

As of September 30, 2023, we had an accumulated deficit of $3,127.8 million. We had a net loss of $470.8 million and $388.1 million for the nine months ended September 30, 2023 and 2022, respectively.

We anticipate that our expenses will continue to increase in connection with our commercialization efforts in the United States, the EEA, Latin America and other territories, including the expansion of our infrastructure and corresponding sales and marketing, legal and regulatory and distribution and manufacturing expenses. In addition to the foregoing, we expect to continue to incur ongoing research and development expenses for our products and product candidates, including our splicing, metabolic, Bio-e and oncology programs as well as studies in our products for maintaining authorizations, including Study 041, label extensions and additional indications. In addition, we may incur substantial costs in connection with our efforts to advance our regulatory submissions. For example, in response to the CHMP's negative opinion for Translarna for the treatment of nmDMD, we have submitted a request for re-examination to the EMA. We continue to seek marketing authorization for Translarna for the treatment of nmDMD in territories that we do not currently have marketing authorization in. We are preparing and anticipate submitting a BLA to the FDA for Upstaza for the treatment of AADC deficiency in the United States shortly after our pre-BLA meeting that is scheduled for December 2023. We also expect to submit an NDA to the FDA for sepiapterin for the treatment of PKU by the end of the third quarter of 2024 and we expect to submit an MAA to the EMA for sepiapterin for the treatment of PKU in the first half of 2024. These efforts may significantly impact the timing and extent of our commercialization expenses.

We may seek to expand and diversify our product pipeline through opportunistically in-licensing or acquiring the rights to products, product candidates or technologies and we may incur expenses, including with respect to transaction costs, subsequent development costs or any upfront, milestone or other payments or other financial obligations associated with any such transaction, which would increase our future capital requirements.

We expect to pay the former equityholders of Agilis Biotherapeutics, Inc., or Agilis, $20.0 million in regulatory milestone payments upon the acceptance for filing by the FDA of a BLA for Upstaza for the treatment of AADC deficiency pursuant

to the Agreement and Plan of Merger, dated as of July 19, 2018, or the Agilis Merger Agreement, by and among us, Agility Merger Sub, Inc., a Delaware corporation and our wholly owned, indirect subsidiary, Agilis and, solely in its capacity as the representative, agent and attorney-in-fact of the equityholders of Agilis, Shareholder Representative Services LLC. We expect to submit a BLA for Upstaza to the FDA shortly after our pre-BLA meeting that is scheduled for December 2023. We also expect to make payments to the former Censa Pharmaceuticals, Inc., or Censa, securityholders of $65.0 million in the aggregate in cash upon the potential achievement in 2024 of development and regulatory milestones relating to sepiapterin pursuant to the Agreement and Plan of Merger, dated as of May 5, 2020, or the Censa Merger Agreement, by and among us, Hydro Merger Sub, Inc., our wholly owned, indirect subsidiary, Censa and, solely in its capacity as the representative, agent and attorney-in-fact of the securityholders of Censa, Shareholder Representative Services LLC.

We also 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 2022 Annual Report. There were no material changes to these obligations and commitments during the period ended September 30, 2023. Furthermore, since we are a public company, we have incurred and expect to continue to incur additional costs associated with operating as such including significant legal, accounting, investor relations and other expenses.

We have never been profitable and we will need to generate significant revenues to achieve and sustain profitability, and we may never do so. Accordingly, we may need to obtain substantial additional funding in connection with our continuing operations. Adequate additional financing may not be available to us on acceptable terms, or at all. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or our commercialization efforts.

Financial operations overview

Revenues

Net product revenues. To date, our net product revenues have consisted primarily of sales of Translarna for the treatment of nmDMD in territories outside of the United States and sales of Emflaza for the treatment of DMD in the United States. We recognize revenue when performance obligations with customers have been satisfied. Our performance obligations are to provide products based on customer orders from distributors, hospitals, specialty pharmacies or retail pharmacies. The performance obligations are satisfied at a point in time when our customer obtains control of the product, which is typically upon delivery. We invoice customers after the products have been delivered and invoice payments are generally due within 30 to 90 days of invoice date. We determine the transaction price based on fixed consideration in its contractual agreements. Contract liabilities arise in certain circumstances when consideration is due for goods not yet provided. As we have identified only one distinct performance obligation, the transaction price is allocated entirely to the product sale. In determining the transaction price, a significant financing component does not exist since the timing from when we deliver product to when the customers pay for the product is typically less than one year. Customers in certain countries pay in advance of product delivery. In those instances, payment and delivery typically occur in the same month.

We record product sales net of any variable consideration, which includes discounts, allowances, rebates related to Medicaid and other government pricing programs, and distribution fees. We use the expected value or most likely amount method when estimating variable consideration, unless discount or rebate terms are specified within contracts. The identified variable consideration is recorded as a reduction of revenue at the time revenues from product sales are recognized. These estimates for variable consideration are adjusted to reflect known changes in factors and may impact such estimates in the quarter those changes are known. Revenue recognized does not include amounts of variable consideration that are constrained. For the three months ended September 30, 2023 and 2022, net product sales outside of the United States were $76.6 million and $79.4 million, respectively, consisting of sales of Translarna, Tegsedi, Waylivra and Upstaza. Translarna net revenues made up $69.0 million and $76.6 million of the net product sales outside of the United States for the three months ended September 30, 2023 and 2022, respectively. For the three months ended September 30, 2023 and 2022, net product sales in the United States were $67.4 million and $54.8 million, respectively, consisting solely of sales of Emflaza. During the three months ended September 30, 2023, one country, the United States, accounted for at least 10% of our net product sales, representing $67.4 million of the net product sales. During the three months ended September 30, 2022, two countries, the United States and Russia, accounted for at least 10% of our net

product sales, representing $54.8 million and $28.3 million of net product sales, respectively. For the three months ended September 30, 2023 and 2022, we had a total of three and two distributors, respectively, that each accounted for over 10% of our net product sales.

During the nine months ended September 30, 2023 and 2022, net product sales outside of the United States were $318.5 million and $247.6 million, respectively, consisting of sales of Translarna, Tegsedi, Waylivra and Upstaza. Translarna net revenues made up $280.6 million and $232.9 million of the net product sales outside of the United States for the nine months ended September 30, 2023 and 2022, respectively. For the nine months ended September 30, 2023 and 2022, net product sales in the United States were $187.7 million and $160.1 million, respectively, consisting solely of sales of Emflaza. During the nine months ended September 30, 2023, three countries, the United States, Russia, and Brazil, accounted for at least 10% of our net product sales, representing $187.7 million, $69.0 million, and $52.5 million of net product sales, respectively. During the nine months ended September 30, 2022, two countries, the United States and Russia, accounted for at least 10% of our net product sales, representing $160.1 million and $57.1 million of net product sales, respectively. For the nine months ended September 30, 2023 and 2022, we had a total of two and two distributors, respectively, that 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, 2023, we had recognized a total of $210.0 million in milestone payments and $290.8 million royalties on net sales pursuant to the SMA License Agreement. As of September 30, 2023, there are no remaining research and development event milestones that we can receive. The remaining potential sales milestones as of September 30, 2023 are $250.0 million upon achievement of certain sales events.

For the three months ended September 30, 2023, we did not recognize collaboration revenue related to the SMA License Agreement with Roche. For the three months ended September 30, 2022 we recognized $50.0 million of collaboration revenue related to the SMA License Agreement. For the nine months ended September 30, 2023, the amounts recognized for the collaboration revenue related to the SMA License Agreement with Roche were immaterial. For the nine months ended September 30, 2022, we recognized $50.0 million of collaboration revenue related to the SMA License Agreement. There were no milestones triggered from Roche in the three and nine months ended September 30, 2023. In September 2022, we recognized a sales milestone of $50.0 million for the achievement of $750.0 million in worldwide annual net sales from Evrysdi.

For the three and nine months ended September 30, 2023, we recognized $50.2 million and $117.9 million of royalty revenue related to Evrysdi, respectively. For the three and nine months ended September 30, 2022, the Company has recognized $32.9 million and $73.6 million of royalty revenue, respectively, related to Evrysdi.

In July 2020, we entered into the Original Royalty Purchase Agreement. Pursuant to the Original Royalty Purchase Agreement, we sold to Royalty Pharma 42.933%, or the Original Assigned Royalty Rights, of the Royalty for $650.0 million. At that time, we retained a 57.067% interest in the Royalty and all economic rights to receive the remaining potential regulatory and sales milestone payments under the SMA License Agreement.

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

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

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

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

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

For the three and nine months ended September 30, 2023, we recognized $2.4 million and $6.7 million of manufacturing revenue, respectively, related to plasmid DNA and AAV vector production for external customers. No manufacturing revenue was recognized for the three and nine months ended September 30, 2022. As of September 30, 2023, we had contract assets of $1.4 million and remaining performance obligations of $1.2 million related to the production of plasmid DNA and AAV vectors for gene therapy applications for external customers. For the period ended December 31, 2022, we had remaining performance obligations of $1.4 million and no contracts assets related to plasmid DNA and AAV production for external customers.

Research and development expense

Research and development expenses consist of the costs associated with our research activities, as well as the costs associated with our drug discovery efforts, conducting preclinical studies and clinical trials, manufacturing development efforts and activities related to regulatory filings. Our research and development expenses consist of:

- external research and development expenses incurred under agreements with third-party contract research organizations and investigative sites, third-party manufacturing organizations and consultants;

- employee-related expenses, which include salaries and benefits, including share-based compensation, for the personnel involved in our drug discovery and development activities; and

- facilities, depreciation and other allocated expenses, which include direct and allocated expenses for rent and maintenance of facilities, IT, human resources and other support functions, depreciation of leasehold improvements and equipment, and laboratory and other supplies.

We use our employee and infrastructure resources across multiple research projects, including our drug development programs. We track expenses related to our clinical programs and certain preclinical programs on a per project basis.

We expect our research and development expenses to fluctuate in connection with our ongoing activities, particularly in connection with Study 041 and other studies for Translarna for the treatment of nmDMD, our activities under our splicing, metabolic, Bio-e and oncology programs and performance of our post-marketing requirements imposed by regulatory agencies with respect to our products. The timing and amount of these expenses will depend upon the outcome of our ongoing clinical trials and the costs associated with our planned clinical trials. The timing and amount of these expenses will also depend on the costs associated with potential future clinical trials of our products or product candidates and the related expansion of our research and development organization, regulatory requirements, advancement of our preclinical programs, and product and product candidate manufacturing costs.

The following table provides research and development expense for our most advanced principal product development programs, for the three and nine months ended September 30, 2023 and 2022.

	Three Months Ended September 30,	
	2023	2022
	(in thousands)	
Global DMD Franchise	$ 21,367	$ 18,014
Metabolic	26,761	16,009
Gene Therapy	42,764	45,336
Bio-e	17,958	18,420
Oncology	9,929	15,857
Splicing	21,260	19,412
Discovery and other	24,173	32,414
Total research and development	$ 164,212	$ 165,462

	Nine Months Ended September 30,	
	2023	2022
	(in thousands)	
Global DMD Franchise	$ 62,532	$ 52,640
Metabolic	115,433	47,181
Gene Therapy	119,968	136,108
Bio-e	54,653	46,029
Oncology	31,617	30,927
Splicing	68,696	52,975
Discovery and other	92,311	96,942
Total research and development	$ 545,210	$ 462,802

The successful development of our products and product candidates is highly uncertain. This is due to the numerous risks and uncertainties associated with developing drugs, including the uncertainty of:

- the scope, rate of progress and expense of our clinical trials and other research and development activities;
- the potential benefits of our products and product candidates over other therapies;
- our ability to market, commercialize and achieve market acceptance for any of our products or product candidates that we are developing or may develop in the future, including our ability to negotiate pricing and reimbursement terms acceptable to us;
- clinical trial results;
- the terms and timing of regulatory approvals; and

- the expense of filing, prosecuting, defending and enforcing patent claims and other intellectual property rights.

A change in the outcome of any of these variables with respect to the development of our products or product candidates could mean a significant change in the costs and timing associated with the development of that product or product candidate. For example, if the EMA or FDA or other regulatory authority were to require us to conduct clinical trials beyond those which we currently anticipate will be required for the completion of clinical development of any of our products or product candidates or if we experience significant delays in enrollment in any of our clinical trials, we could be required to expend significant additional financial resources and time on the completion of clinical development. In addition, the uncertainty with respect to the duration, nature and extent of negative impacts of the COVID-19 pandemic and responsive measures relating thereto on our ability to successfully enroll our current and future clinical trials, has caused us to experience delays, and may cause us to experience further delays, in our clinical trials and regulatory submissions.

Selling, general and administrative expense

Selling, general and administrative expenses consist primarily of salaries and other related costs for personnel, including share-based compensation expenses, in our executive, legal, business development, commercial, finance, accounting, information technology and human resource functions. Other selling, general and administrative expenses include facility-related costs not otherwise included in research and development expense; advertising and promotional expenses; costs associated with industry and trade shows; and professional fees for legal services, including patent-related expenses, accounting services and miscellaneous selling costs.

We expect that selling, general and administrative expenses will increase in future periods in connection with our continued efforts to commercialize our products, including increased payroll, expanded infrastructure, commercial operations, increased consulting, legal, accounting and investor relations expenses.

Interest expense, net

Interest expense, net consists of interest expense from the liability for the sale of future royalties related to the Original Royalty Purchase Agreement, the 2026 Convertible Notes outstanding, the $150.0 million aggregate principal amount of 3.00% convertible senior notes due 2022 outstanding, the Blackstone Credit Agreement, 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, 2023, there were no material changes to our critical accounting policies as reported in our 2022 Annual Report.

Results of operations

Three months ended September 30, 2023 compared to three months ended September 30, 2022

The following table summarizes revenues and selected expense and other income data for the three months ended September 30, 2023 and 2022.

(in thousands)	Three Months Ended September 30, 2023	2022	Change 2023 vs. 2022
Net product revenue	$ 144,038	$ 134,186	$ 9,852
Collaboration revenue	—	50,017	(50,017)
Royalty revenue	50,173	32,924	17,249
Manufacturing revenue	2,365	—	2,365
Cost of product sales, excluding amortization of acquired intangible asset	9,493	14,011	(4,518)
Amortization of acquired intangible asset	58,649	31,023	27,626
Research and development expense	164,212	165,462	(1,250)
Selling, general and administrative expense	80,886	80,118	768
Change in the fair value of contingent consideration	1,500	(5,300)	6,800
Interest expense, net	(28,160)	(20,880)	(7,280)
Other expense, net	(20,266)	(38,141)	17,875
Income tax benefit	33,620	17,893	15,727

Net product revenues. Net product revenues were $144.0 million for the three months ended September 30, 2023, an increase of $9.9 million, or 7%, from $134.2 million for the three months ended September 30, 2022. The increase in net product revenue was primarily due to an increase in net product sales of Emflaza, offset by a decrease in net product sales of Translarna. Emflaza net product revenues were $67.4 million for the three months ended September 30, 2023, an increase of $12.6 million, or 23%, compared to $54.8 million for the three months ended September 30, 2022. These results reflect new patients and high compliance. Translarna net product revenues were $69.0 million for the three months ended September 30, 2023, a decrease of $7.6 million, or 10%, compared to $76.6 million for the three months ended September 30, 2022. The decrease was due to timing of bulk patient orders, partially offset by new patient starts in existing geographies and continued geographic expansion.

Collaboration revenues. Collaboration revenues were $0.0 million for the three months ended September 30, 2023, a decrease of $50.0 million, or 100%, from $50.0 million for the three months ended September 30, 2022. The decrease is due to a sales milestone of $50.0 million for the achievement of $750.0 million in worldwide annual net sales from Evrysdi in the three months ended September 30, 2022. No milestones were triggered in the three months ended September 30, 2023.

Royalty revenue. Royalty revenue was $50.2 million for the three months ended September 30, 2023, an increase of $17.2 million, or 52%, from $32.9 million for the three months ended September 30, 2022. The increase in royalty revenue was due to higher Evrysdi sales in the three months ended September 30, 2023 as compared to the three months ended September 30, 2022. In accordance with the SMA License Agreement, we are entitled to royalties on worldwide annual net sales of the product.

Manufacturing revenue. Manufacturing revenues were $2.4 million for the three months ended September 30, 2023 an increase of $2.4 million, or 100%, from $0.0 million for the three months ended September 30, 2022. The increase is due to the manufacturing services related to the production of plasmid DNA and AAV vectors for gene therapy applications for external customers.

Cost of product sales, excluding amortization of acquired intangible asset. Cost of product sales, excluding amortization of acquired intangible asset, were $9.5 million for the three months ended September 30, 2023, a decrease of $4.5 million, or 32%, from $14.0 million for the three months ended September 30, 2022. Cost of product sales consist primarily of

royalty payments associated with Emflaza, Translarna, and Upstaza net product sales, excluding contingent payments to Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC), or Marathon, costs associated with Emflaza, Translarna, and Upstaza products sold during the period, and royalty expense related to royalty revenues and collaboration milestone revenues. The decrease in cost of product sales, excluding amortization of acquired intangible asset, is primarily due to a decrease in Translarna net product revenue driven by timing of bulk patient orders, as well as a decrease in the collaboration royalty earned by the SMA Foundation, due to a sales milestone of $50.0 million for the achievement of $750.0 million in worldwide annual net sales from Evrysdi in the three months ended September 30, 2022. No milestones were triggered in the three months ended September 30, 2023.

Amortization of acquired intangible asset. Amortization of our intangible assets was $58.6 million for the three months ended September 30, 2023, an increase of $27.6 million, or 89%, from $31.0 million for the three months ended September 30, 2022. These amounts are related to the Emflaza rights acquisition, as well as the Waylivra, Tegsedi, and Upstaza intangible assets, which are all being amortized on a straight-line basis over their estimated useful lives. The amortization increase is primarily related to additional Marathon contingent payments for Emflaza.

Research and development expense. Research and development expense was $164.2 million for the three months ended September 30, 2023, a decrease of $1.3 million, or 1%, from $165.5 million for the three months ended September 30, 2022. The decrease in research and development expenses reflects our strategic portfolio prioritization as the Company continues to focus its resources on its differentiated, high potential R&D programs.

Selling, general and administrative expense. Selling, general and administrative expense was $80.9 million for the three months ended September 30, 2023, an increase of $0.8 million, or 1%, from $80.1 million for the three months ended September 30, 2022. The increase reflects our continued investment to support our commercial activities including our expanding commercial portfolio. The increase also includes restructuring costs from a reduction in workforce in connection with our strategic pipeline prioritization and discontinuation of our preclinical and early research programs in our gene therapy platform in the three months ended September 30, 2023.

Change in the fair value of contingent consideration. The change in the fair value of contingent consideration was a loss of $1.5 million for the three months ended September 30, 2023, a change of $6.8 million, or over 100%, from a gain of $5.3 million for the three months ended September 30, 2022. The change is related to the fair valuation of the potential future consideration to be paid to former equityholders of Agilis as a result of our merger with Agilis which closed in August 2018. Changes in the fair value were due to the re-calculation of discounted cash flows for the passage of time and changes to certain other estimated assumptions.

Interest expense, net. Interest expense, net was $28.2 million for the three months ended September 30, 2023, an increase of $7.3 million, or 35%, from $20.9 million for the three months ended September 30, 2022. The increase in interest expense, net was primarily due to interest expense recorded from the liability for the sale of future royalties related to the Original Royalty Purchase Agreement and the Blackstone Credit Agreement.

Other expense, net. Other expense, net was $20.3 million for the three months ended September 30, 2023, a decrease of $17.9 million, or 47%, from other expense, net of $38.1 million for the three months ended September 30, 2022. Other expense, net for the three months ended September 30, 2023 was primarily related to realized and unrealized foreign exchange losses of $16.4 million, and unrealized losses on our equity investments in Clearpoint Neuro, Inc. of $2.0 million and unrealized losses on our convertible debt security in ClearPoint Neuro, Inc. of $3.1 million, offset by unrealized gains of $1.1 million on marketable securities – equity investments. Other expense, net for the three months ended September 30, 2022, was primarily related to an unrealized foreign exchange loss from the remeasurement of our intercompany loan, offset by unrealized losses on our equity investments and convertible debt security in ClearPoint Neuro, Inc. of $3.5 million and $4.4 million, respectively.

Income tax benefit. Income tax benefit was $33.6 million for the three months ended September 30, 2023, an increase of $15.7 million, or 88%, compared to income tax benefit of $17.9 million for the three months ended September 30, 2022. The increase in income tax benefit is attributable to the receipt of an outstanding state tax refund received during the three months ended September 30, 2023, and the subsequent release of the associated FIN48 reserve.

We also incur income tax expenses in various foreign jurisdictions, and our foreign tax liabilities are largely dependent upon the distribution of pre-tax earnings among these different jurisdictions.

Nine months ended September 30, 2023 compared to nine months ended September 30, 2022.

The following table summarizes revenues and selected expense and other income data for the nine months ended September 30, 2023 and 2022.

(in thousands)	Nine Months Ended September 30, 2023	2022	Change 2023 vs. 2022
Net product revenue	$ 506,187	$ 407,720	$ 98,467
Collaboration revenue	6	50,024	(50,018)
Royalty revenue	117,857	73,645	44,212
Manufacturing revenue	6,716	—	6,716
Cost of product sales, excluding amortization of acquired intangible assets	36,368	33,785	2,583
Amortization of acquired intangible assets	145,461	80,790	64,671
Research and development expense	545,210	462,802	82,408
Selling, general and administrative expense	256,249	233,280	22,969
Change in the fair value of contingent consideration	(125,000)	(32,200)	(92,800)
Intangible asset impairment	217,800	—	217,800
Interest expense, net	(84,905)	(66,371)	(18,534)
Other expense, net	(8,832)	(84,355)	75,523
Income tax benefit	68,247	9,666	58,581

Net product revenues. Net product revenues were $506.2 million for the nine months ended September 30, 2023, an increase of $98.5 million, or 24%, from $407.7 million for the nine months ended September 30, 2022. The increase in net product revenue was primarily due to an increase in net product sales of Translarna and Emflaza. Translarna net product revenues were $280.6 million for the nine months ended September 30, 2023, an increase of $47.7 million, or 20%, compared to $232.9 million for the nine months ended September 30, 2022. These results were driven by treatment of new patients and continued geographic expansion. Emflaza net product revenues were $187.7 million for the nine months ended September 30, 2023, an increase of $27.6 million, or 17%, compared to $160.1 million for the nine months ended September 30, 2022. These results reflect new patients and high compliance.

Collaboration revenues. Collaboration revenues were $6.0 thousand for the three months ended September 30, 2023, a decrease of $50.0 million, or 100%, from $50.0 million for the three months ended September 30, 2022. The decrease is due to a sales milestone of $50.0 million for the achievement of $750.0 million in worldwide annual net sales from Evrysdi in the nine months ended September 30, 2022.No milestones were triggered in the nine months ended September 30, 2023.

Royalty revenue. Royalty revenue was $117.9 million for the nine months ended September 30, 2023, an increase of $44.2 million, or 60%, from $73.6 million for the nine months ended September 30, 2022. The increase in royalty revenue was due to higher Evrysdi sales in the nine months ended September 30, 2023 as compared to the nine months ended September 30, 2022. In accordance with the SMA License Agreement, we are entitled to royalties on worldwide annual net sales of the product.

Manufacturing revenue. Manufacturing revenues were $6.7 million for the nine months ended September 30, 2023, an increase of $6.7 million, or 100%, from $0.0 million for the nine months ended September 30, 2022. The increase is due to the manufacturing services related to the production of plasmid DNA and AAV vectors for gene therapy applications for external customers.

Cost of product sales, excluding amortization of acquired intangible asset. Cost of product sales, excluding amortization of acquired intangible asset, were $36.4 million for the nine months ended September 30, 2023, an increase of $2.6 million, or 8%, from $33.8 million for the nine months ended September 30, 2022. Cost of product sales consist primarily of royalty

payments associated with Emflaza and Translarna net product sales, excluding contingent payments to Marathon, costs associated with Emflaza and Translarna product sold during the period, and royalty expense related to royalty revenues and collaboration milestone revenues. The increase in cost of product sales, excluding amortization of acquired intangible asset, is primarily due to the increase in net product revenue, royalty revenue, and collaboration milestone revenue.

Amortization of acquired intangible asset. Amortization of our intangible assets was $145.5 million for the nine months ended September 30, 2023, an increase of $64.7 million, or 80%, from $80.8 million for the nine months ended September 30, 2022. These amounts are related to the Emflaza rights acquisition, as well as the Waylivra, Tegsedi, and Upstaza intangible assets, which are all being amortized on a straight-line basis over their estimated useful lives. The amortization increase is primarily related to additional Marathon contingent payments for Emflaza.

Research and development expense. Research and development expense was $545.2 million for the nine months ended September 30, 2023, an increase of $82.4 million, or 18%, from $462.8 million for the nine months ended September 30, 2022. The increase in R&D expenses includes the achievement of a $30.0 million success-based development milestone for the completion of enrollment of a Phase 3 clinical trial for sepiapterin for PKU. The increase also includes restructuring costs from a reduction in workforce in connection with our strategic pipeline prioritization and discontinuation of our preclinical and early research programs in our gene therapy platform in the nine months ended September 30, 2023.

Selling, general and administrative expense. Selling, general and administrative expense was $256.2 million for the nine months ended September 30, 2023, an increase of $23.0 million, or 10%, from $233.3 million for the nine months ended September 30, 2022. The increase reflects our continued investment to support our commercial activities including our expanding commercial portfolio. The increase also includes restructuring costs from a reduction in workforce in connection with our strategic pipeline prioritization and discontinuation of our preclinical and early research programs in our gene therapy platform in the nine months ended September 30, 2023.

Change in the fair value of contingent consideration. The change in the fair value of contingent consideration was a gain of $125.0 million for the nine months ended September 30, 2023, a change of $92.8 million, or over 100%, from a gain of $32.2 million for the nine months ended September 30, 2022. The change is primarily related to our strategic portfolio prioritization and decision to discontinue our preclinical and early research programs in our gene therapy platform, which included Friedreich ataxia and Angelman syndrome, which was announced in May 2023. As a result, we fully impaired the Friedreich ataxia and Angelman syndrome intangible assets and determined that the fair value for all of the contingent consideration payable related to Friedreich ataxia and Angelman syndrome was $0. As a result, we recorded a fair value change of $129.8 million for the contingent consideration related to Friedreich ataxia and Angelman syndrome during the nine months ended September 30, 2023.

Intangible asset impairment. Intangible asset impairment was $217.8 million for the nine months ended September 30, 2023, an increase of $217.8 million, or 100%, from intangible asset impairment of $0.0 million for the nine months ended September 30, 2022. The change is due to our strategic portfolio prioritization and decision to discontinue our preclinical and early research programs in our gene therapy platform, which included Friedreich ataxia and Angelman syndrome, which was announced in May 2023. As a result, we fully impaired the Friedreich ataxia and Angelman syndrome intangible assets and recorded impairment expense of $217.8 million during the nine months ended September 30, 2023.

Interest expense, net. Interest expense, net was $84.9 million for the nine months ended September 30, 2023, an increase of $18.5 million, or 28%, from $66.4 million for the nine months ended September 30, 2022. The increase in interest expense, net was primarily due to interest expense recorded from the liability for the sale of future royalties related to the Original Royalty Purchase Agreement and the Blackstone Credit Agreement.

Other expense, net. Other expense, net was $8.8 million for the nine months ended September 30, 2023, a decrease of $75.5 million, or 90%, from $84.4 million for the nine months ended September 30, 2022. Other expense, net for the nine months ended September 30, 2023 was primarily related to realized and unrealized foreign exchange losses of $5.2 million and unrealized and realized losses, net, on our equity investments in ClearPoint Neuro, Inc. of $3.9 million and unrealized losses on our convertible debt security in ClearPoint Neuro, Inc. of $4.6 million. These expenses were offset by unrealized gains of $5.5 million on marketable securities – equity investments for the nine months ended September 30, 2023. The

change in other expense, net for the nine months ended September 30, 2022, resulted primarily from an unrealized foreign exchange loss from the remeasurement of our intercompany loan and unrealized losses on our equity investments and convertible debt security in ClearPoint Neuro, Inc. of $1.1 million and $2.4 million, respectively.

Income tax benefit. Income tax benefit was $68.2 million for the nine months ended September 30, 2023, an increase of $58.6 million, or over 100%, from $9.7 million for the nine months ended September 30, 2022. The increase in income tax benefit is attributable to the receipt of an outstanding state tax refund received during the nine months ended September 30, 2023, and the subsequent release of the associated FIN48 reserve.

Liquidity and capital resources

Sources of liquidity

Since inception, we have incurred significant operating losses.

As a growing commercial-stage biopharmaceutical company, we are engaging in significant commercialization efforts for our products while also devoting a substantial portion of our efforts on research and development related to our products, product candidates and other programs. To date, our product revenue has been primarily attributable to sales of Translarna for the treatment of nmDMD in territories outside of the United States and from Emflaza for the treatment of DMD in the United States. Our ongoing ability to generate revenue from sales of Translarna for the treatment of nmDMD is dependent upon our ability to maintain our marketing authorizations in Brazil, Russia and in the EEA and secure market access through commercial programs following the conclusion of pricing and reimbursement terms at sustainable levels in the member states of the EEA or through EAP Programs in the EEA and other territories. The marketing authorization requires annual review and renewal by the EC following reassessment by the EMA of the benefit-risk balance of the authorization and is subject to the specific obligation to conduct Study 041. In September 2023, the CHMP gave a negative opinion on the conversion of the conditional marketing authorization to full marketing authorization of Translarna for the treatment of nmDMD and a negative opinion on the renewal of the existing conditional marketing authorization of Translarna for the treatment of nmDMD. We have requested re-examination of the CHMP opinion regarding the renewal of the existing conditional marketing authorization. In accordance with EMA guidelines, we expect the CHMP opinion following the re-examination process to occur in late January 2024, with EC ratification of the opinion within the following 67 days. While we have requested a re-examination of the CHMP opinion, the re-examination process may not result in a change to the negative opinion. Our ability to generate product revenue from Emflaza will largely depend on the coverage and reimbursement levels set by governmental authorities, private health insurers and other third-party payors.

We have historically financed our operations primarily through the issuance and sale of our common stock in public offerings, our “at the market offering” of our common stock, proceeds from the A&R Royalty Purchase Agreement, the private placements of our preferred stock, collaborations, bank and institutional lender debt, convertible debt financings and grants and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease areas addressed by our product candidates. We expect to continue to incur significant expenses and operating losses for at least the next fiscal year. The net losses we incur may fluctuate significantly from quarter to quarter.

In August 2019, we entered into the Sales Agreement, pursuant to which, we may offer and sell shares of our common stock, having an aggregate offering price of up to $125.0 million from time to time through the Sales Agents by any method that is deemed to be an “at the market offering” as defined in Rule 415(a)(4) promulgated under the Securities Act. See “Item 2. Management’s Discussion and Analysis of Financial Condition and Results of Operations—Corporate Updates—Funding” for additional information.

In September 2019, we closed a private offering of $287.5 million aggregate principal amount of 1.50% convertible senior notes due 2026 including the full exercise by the initial purchasers of an option to purchase an additional $37.5 million in aggregate principal amount of the 2026 Convertible Notes. The 2026 Convertible Notes bear cash interest at a rate of 1.50% per year, payable semi-annually on March 15 and September 15 of each year, beginning on March 15, 2020. The 2026 Convertible Notes will mature on September 15, 2026, unless earlier repurchased or converted. We received net proceeds of $279.3 million after deducting the initial purchasers’ discounts and commissions and the offering expenses payable by us.

In July 2020, we entered into the Original Royalty Purchase Agreement. Pursuant to the Original Royalty Purchase Agreement, we sold to Royalty Pharma the Original Assigned Royalty Rights in consideration for $650.0 million.

In October 2022, we entered into the Blackstone Credit Agreement for fundings of up to $950.0 million consisting of a committed loan facility of $450.0 million and further contemplating the potential for up to $500.0 million of additional financing, to the extent that we request such additional financing and subject to the Lenders' agreement to provide such additional financing and to mutual agreement on terms.

The Blackstone Credit Agreement provided for a senior secured term loan facility funded on the Closing Date in the aggregate principal amount of $300.0 million and a committed delayed draw term loan facility of up to $150.0 million to be funded at our request within 18 months of the Closing Date subject to specified conditions. In addition, the Blackstone Credit Agreement contemplated the potential for further financings by Blackstone, by providing for incremental discretionary uncommitted further financings of up to $500.0 million.

In October 2023, we terminated the Blackstone Credit Agreement. In connection with the termination of the Blackstone Credit Agreement, we repaid outstanding principal and accrued interest thereunder totaling $302.1 million and paid an additional $82.1 million in prepayment premiums, expenses and other exit fees. All liens and security interests securing the loans made pursuant to the Blackstone Credit Agreement were released upon termination.

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

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

Cash flows

As of September 30, 2023, we had cash, cash equivalents and marketable securities of $294.8 million.

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

	Nine Months Ended September 30,	
(in thousands)	**2023**	**2022**
Cash (used in) provided by:		
Operating activities	(58,130)	(190,691)
Investing activities	(82,319)	257,742
Financing activities	25,915	(134,966)

Net cash used in operating activities was $58.1 million for the nine months ended September 30, 2023 and $190.7 million for the nine months ended September 30, 2022. The net cash used in operating activities primarily relates to supporting clinical development and commercial activities.

Net cash used in investing activities was $82.3 million for the nine months ended September 30, 2023 and net cash provided by investing activities was $257.7 million for the nine months ended September 30, 2022. Cash used in investing activities for the nine months ended September 30, 2023 was primarily related to the acquisition of product rights, purchases of marketable securities-equity investments, and purchases of fixed assets, partially offset by net sales and redemption of marketable securities. Cash provided by investing activities for the nine months ended September 30, 2022 was primarily related to net sales and redemption of marketable securities, partially offset by purchases of marketable securities, purchases of fixed assets, and the acquisition of product rights.

Net cash provided by financing activities was $25.9 million for the nine months ended September 30, 2023 and net cash used in by financing activities was $135.0 million for the nine months ended September 30, 2022. Cash provided by financing activities for the nine months ended September 30, 2023 was primarily attributable to cash received from the exercise of options, and proceeds from our Employee Stock Purchase Plan, partially offset by payments on our finance lease principal and debt issuance costs related to the senior secured term loan. Cash used in financing activities for the nine months ended September 30, 2022 was primarily attributable to the repayment of the 2022 Convertible Notes and payments on our finance lease principal, partially offset by cash received from the exercise of options and proceeds from our Employee Stock Purchase Plan.

Funding requirements

We anticipate that our expenses will continue to increase in connection with our commercialization efforts in the United States, the EEA, Latin America and other territories, including the expansion of our infrastructure and corresponding sales and marketing, legal and regulatory and distribution and manufacturing expenses. In addition to the foregoing, we expect to continue to incur significant costs in connection with the research and development of our splicing, metabolic, Bio-e and oncology programs as well as studies in our products for maintaining authorizations, including Study 041, label extensions and additional indications. In addition, we may incur substantial costs in connection with our efforts to advance our regulatory submissions. For example, in response to the CHMP's negative opinion for Translarna for the treatment of nmDMD, we have submitted a request for re-examination to the EMA. We continue to seek marketing authorization for Translarna for the treatment of nmDMD in territories that we do not currently have marketing authorization in. We are preparing and anticipate submitting a BLA to the FDA for Upstaza for the treatment of AADC deficiency in the United States shortly after our pre-BLA meeting that is scheduled for December 2023. We also expect to submit an NDA to the FDA for sepiapterin for the treatment of PKU by the end of the third quarter of 2024 and we expect to submit an MAA to the EMA for sepiapterin for the treatment of PKU in the first half of 2024. These efforts may significantly impact the timing and extent of our commercialization expenses.

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

- seek to satisfy contractual and regulatory obligations that we assumed through our acquisitions and collaborations;
- execute our commercialization strategy for our products, including initial commercialization launches of our products, label extensions or entering new markets;
- are required to complete any additional clinical trials, non-clinical studies or Chemistry, Manufacturing and Controls, or CMC, assessments or analyses in order to advance Translarna for the treatment of nmDMD in the United States or elsewhere;
- are required to take other steps, in addition to Study 041, to maintain our current marketing authorization in the EEA, Brazil and Russia for Translarna for the treatment of nmDMD or to obtain further marketing authorizations for Translarna for the treatment of nmDMD or other indications;

- initiate or continue the research and development of our splicing, metabolic, Bio-e and oncology programs as well as studies in our products for maintaining authorizations, including Study 041, label extensions and additional indications;
- continue to utilize the Hopewell Facility to manufacture program materials for third parties;
- seek to discover and develop additional product candidates;
- seek to expand and diversify our product pipeline through strategic transactions;
- maintain, expand and protect our intellectual property portfolio; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and commercialization efforts.

We believe that our cash flows from product sales, together with existing cash and cash equivalents, including our offering of the 2026 Convertible Notes, public offerings and private placements of common stock, our "at the market offering" of our common stock, proceeds from the A&R Royalty Purchase Agreement and marketable securities, will be sufficient to fund our operating expenses and capital expenditure requirements for at least the next twelve months. We have based this estimate on assumptions that may prove to be wrong, and we could use our capital resources sooner than we currently expect.

Our future capital requirements will depend on many factors, including:

- our ability to maintain our marketing authorization for Translarna for the treatment of nmDMD in the EEA following the CHMP's negative opinion on the conversion of the conditional marketing authorization to full marketing authorization and the renewal of the existing conditional authorization;
- our ability to maintain the marketing authorization for Translarna and our other products in territories outside of the EEA;
- our ability to commercialize and market our products and product candidates that may receive marketing authorization;
- our ability to negotiate, secure and maintain adequate pricing, coverage and reimbursement terms, on a timely basis, with third-party payors for our products and products candidates;
- our ability to obtain marketing authorization for sepiapterin for the treatment of PKU in the United States and EEA;
- the costs, timing and outcome of Study 041;
- our ability to obtain marketing authorization for Upstaza for the treatment of AADC deficiency in the United States;
- the costs, timing and outcome of our efforts to advance Translarna for the treatment of nmDMD in the United States, including, whether we will be required to perform additional clinical trials, non-clinical studies or CMC assessments or analyses at significant cost which, if successful, may enable FDA review of an NDA re-submission by us and, ultimately, may support approval of Translarna for nmDMD in the United States;
- unexpected decreases in revenue or increase in expenses resulting from the COVID-19 pandemic or other potential widespread outbreaks of contagious disease;

- our ability to maintain orphan exclusivity in the United States for Emflaza;
- our ability to successfully complete all post-marketing requirements imposed by regulatory agencies with respect to our products;
- the progress and results of activities under our splicing, metabolic, Bio-e and oncology programs as well as studies in our products for maintaining authorizations, label extensions and additional indications;
- the scope, costs and timing of our commercialization activities, including product sales, marketing, legal, regulatory, distribution and manufacturing, for any of our products and for any of our other product candidates that may receive marketing authorization or any additional territories in which we receive authorization to market Translarna;
- the costs, timing and outcome of regulatory review of our splicing, metabolic, Bio-e and oncology programs and Translarna and Upstaza in other territories;
- our ability to satisfy our obligations under the indenture governing the 2026 Convertible Notes;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our other product candidates, including those in our splicing, metabolic, Bio-e and oncology programs;
- revenue received from commercial sales of our products or any of our product candidates;
- our ability to obtain additional and maintain existing reimbursed named patient and cohort EAP programs for Translarna for the treatment of nmDMD on adequate terms, or at all;
- the ability and willingness of patients and healthcare professionals to access Translarna through alternative means if pricing and reimbursement negotiations in the applicable territory do not have a positive outcome;
- our ability to continue to utilize the Hopewell Facility to manufacture program materials for third parties;
- the costs of preparing, filing and prosecuting patent applications, maintaining, and protecting our intellectual property rights and defending against intellectual property-related claims;
- the extent to which we acquire or invest in other businesses, products, product candidates, and technologies, including the success of any acquisition, in-licensing or other strategic transaction we may pursue, and the costs of subsequent development requirements and commercialization efforts, including with respect to our acquisitions of Emflaza, Agilis, our Bio-e platform and Censa and our licensing of Tegsedi and Waylivra; and
- our ability to establish and maintain collaborations, including our collaborations with Roche and the SMA Foundation, and our ability to obtain research funding and achieve milestones under these agreements.

With respect to our outstanding 2026 Convertible Notes, cash interest payments are payable on a semi-annual basis in arrears, which will require total funding of $4.3 million annually. Borrowings under the Blackstone Credit Agreement bore interest at a variable rate equal to, at our option, either an adjusted Term SOFR rate plus seven and a quarter percent (7.25%) or the Base Rate plus six and a quarter percent (6.25%), subject to a floor of one percent (1%) and two percent (2%) with respect to Term SOFR rate and Base Rate (each as defined in the Blackstone Credit Agreement), respectively.

We expect to pay the former equityholders of Agilis $20.0 million in regulatory milestone payments upon the acceptance for filing by the FDA of a BLA for Upstaza for the treatment of AADC deficiency pursuant to the Agilis Merger Agreement. We expect to submit a BLA for Upstaza to the FDA shortly after our pre-BLA meeting that is scheduled for December 2023. We also expect to make payments to the former Censa securityholders of $65.0 million in the aggregate

in cash upon the potential achievement in 2024 of development and regulatory milestones relating to sepiapterin pursuant to Censa Merger Agreement.

We also have certain significant contractual obligations and commercial commitments that require funding and we have disclosed these items under the heading “Management’s Discussion and Analysis of Financial Condition and Results of Operations-Funding Obligations” in our 2022 Annual Report. There were no material changes to these obligations and commitments during the period ended September 30, 2023. Furthermore, since we are a public company, we have incurred and expect to continue to incur additional costs associated with operating as such including significant legal, accounting, investor relations and other expenses.

We have never been profitable and we will need to generate significant revenues to achieve and sustain profitability, and we may never do so. We may need to obtain substantial additional funding in connection with our continuing operations. Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs primarily through a combination of equity offerings, debt financings, collaborations, strategic alliances, grants and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease areas addressed by our product and product candidates and marketing, distribution or licensing arrangements. Adequate additional financing may not be available to us on acceptable terms, or at all. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our shareholders ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our common stockholders. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us.

If we are unable to raise additional funds through equity, debt or other financings when needed or on attractive terms, we may be required to delay, limit, reduce or terminate our product development or commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

During the period ended September 30, 2023, 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 2022 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, 2023. 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, 2023, 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, 2023 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, 2022, 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. The risk factor disclosure in our Annual Report on Form 10-K for the year ended December 31, 2022 is qualified by the additional information that is described in this Quarterly Report on Form 10-Q, including the updated risk factor disclosure set forth below.

We may be unable to continue to commercialize Translarna for nonsense mutation Duchenne muscular dystrophy in the European Economic Area if the Committee for Medicinal Products for Human Use of the European Medicines Agency does not reverse its negative opinion on the renewal of the existing conditional authorization for Translarna.

Our marketing authorization for Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, in ambulatory patients aged two years and older in the European Economic Area, or EEA, is subject to annual review and renewal by the European Commission, or EC, following reassessment by the European Medicines Agency, or EMA, of the benefit-risk balance of the authorization. In September 2022, we submitted a Type II variation to the EMA to support conversion of the conditional marketing authorization for Translarna to a standard marketing authorization, which included a report on the placebo-controlled trial of Study 041 and data from the open-label extension. In February 2023, we also submitted an annual marketing authorization renewal request to the EMA. In September 2023, the Committee for Medicinal Products for Human Use, or CHMP, gave a negative opinion on the conversion of the conditional marketing authorization to full marketing authorization of Translarna for the treatment of nmDMD and a negative opinion on the renewal of the existing conditional marketing authorization of Translarna for the treatment of nmDMD. We have requested re-examination of the CHMP opinion regarding the renewal of the existing conditional marketing authorization. In re-examination, we intend to address concerns raised during the previous review process on the benefit demonstrated in Translarna clinical trials and regarding the robustness of the Strategic Targeting of Registries and International Database of Excellence, or STRIDE, real world registry. In accordance with EMA guidelines, we expect the CHMP opinion following the re-examination process to occur in late January 2024, with European Commission, or EC, ratification of the opinion within the following 67 days.

While we have requested a re-examination of the CHMP opinion, the re-examination process may not result in a change to the negative opinion. If the CHMP does not reverse its negative opinion, the EC is likely to refuse to renew the marketing authorization for Translarna. Alternatively, the CHMP may recommend the imposition of other conditions or restrictions on our current marketing authorization. As such, there is substantial risk to our ability to maintain our conditional marketing authorization in the EEA and our ability to commercialize Translarna for the treatment of nmDMD in the EEA. If we are unable to renew our conditional marketing authorization in the EEA, or if our product label is materially restricted, we would lose all, or a significant portion of, our ability to generate revenue from sales of Translarna in the EEA, which would have a material adverse effect on our business, results of operations and financial condition.

Additionally, the CHMP's negative opinion for Translarna and potential loss of the Translarna marketing authorization in the EEA may influence regulatory entities in other jurisdictions in which Translarna has been approved to reassess such approvals. For example, certain countries reference or depend on the determination by the EMA when considering the grant of a marketing authorization. There is substantial risk that we would be unable to maintain our marketing

authorizations in these countries in the event the EC decides not to renew or otherwise varies, suspends or withdraws our marketing authorization in the EEA. Even in countries where our marketing authorization is maintained, there may be an impact on pricing and reimbursement of Translarna within those countries. Any potential reassessments of our marketing authorizations or impacts to pricing and reimbursement may lead to additional regulatory costs, requirements to complete additional clinical trials, restrictions on our marketing authorizations or loss of a significant portion of our revenue for Translarna in other jurisdictions, which could have a material adverse effect on our business, results of operations and financial condition.

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
Pierre Gravier (Chief Financial Officer)	Adoption (July 13, 2023)	Rule 10b5-1 trading arrangement for sell-to-cover transactions for RSUs granted on July 13, 2023	Sale	Until final settlement of RSUs on or around July 13, 2027	Indeterminable (1)

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

Item 6. Exhibits.

Exhibit Number	Description of Exhibit
10.1+	Employment Agreement between PTC Therapeutics, Inc. and Pierre Gravier (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed by the Registrant on July 17, 2023)
31.1*	Certification of Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2*	Certification of Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1*	Certification of Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
32.2*	Certification of Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.INS*	Inline XBRL Instance Document
101.SCH*	Inline XBRL Taxonomy Extension Schema Document
101.CAL*	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.LAB*	Inline XBRL Taxonomy Extension Label Linkbase Database
101.PRE*	Inline XBRL Taxonomy Extension Presentation Linkbase Document
101.DEF*	Inline XBRL Taxonomy Extension Definition Linkbase Document
104	The cover page from this Quarterly Report on Form 10-Q, formatted in Inline XBRL

* Submitted electronically herewith.

† Portions of this exhibit have been omitted pursuant to Item 601(b)(10)(iv) of Regulation S-K.

\+ Management contract, compensatory plan or arrangement.

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

SIGNATURES

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

PTC THERAPEUTICS, INC.

Date: October 26, 2023

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

Exhibit 31.1

CERTIFICATIONS

I, Matthew B. Klein, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: October 26, 2023

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 26, 2023

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, 2023 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 26, 2023

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, 2023 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 26, 2023

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