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, 2020

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 27, 2020, there were 68,235,739 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:

- expectations with respect to our gene therapy platform, including any potential regulatory submissions and potential approvals, including those related to our gene therapy for the treatment of Aromatic L-Amino Acid Decarboxylase, or AADC deficiency, or PTC-AADC, our manufacturing capabilities and the potential financial impact and benefits of our leased biologics manufacturing facility and the potential achievement of development, regulatory and sales milestones and contingent payments that we may be obligated to make;

- our 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 marketing authorization of Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, in the European Economic Area, or EEA, which is subject to the specific obligation to conduct and submit the results of Study 041 to the European Medicines Agency, or EMA, and annual review and renewal by the European Commission following reassessment of the benefit-risk balance of the authorization by the EMA;

- our ability to enroll, fund, and 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, and by the EMA's deadline;

- the anticipated period of market exclusivity for Emflaza for the treatment of DMD in the United States under the Orphan Drug Act of 1983, or Orphan Drug Act, the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act;

- our ability to complete any dystrophin study necessary in order to resolve the matters set forth in the United States Food and Drug Administration's, or the FDA, denial of our appeal to the Complete Response Letter we received from the FDA in connection with our New Drug Application, or NDA, for Translarna for the treatment of nmDMD, and our ability to perform additional clinical trials, non-clinical studies or CMC assessments or analyses at significant cost;

- the timing and scope of our commercialization of our products and product candidates;

- 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;

- our ability to obtain additional and maintain existing reimbursed named patient and cohort early access programs, or EAP programs, for our products on adequate terms, or at all;

- our expectations with respect to the development, regulatory and 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., which we refer to collectively as Roche, and the Spinal Muscular Atrophy Foundation,

or the SMA 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 Licensing Agreement with Akcea Therapeutics, Inc., or Akcea, 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 contingent payments to Akcea based on the potential achievement of certain regulatory milestones and royalty payments by us to Akcea based on our potential achievement of certain net sales thresholds;

- 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 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 gene therapy, splicing, Bio-e and oncology programs, studies of PTC923 for phenylketonuria, or PKU, and studies of PTC299 for COVID-19 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 product and product candidates through alternative means if pricing and reimbursement negotiations in the applicable territory do not have a positive outcome;

- our ability to complete the FDA post-marketing requirements to the marketing authorization of Emflaza and any other post-marketing requirements for our products;

- 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 maintain the current labeling under the marketing authorization in the EEA or expand the approved product label of Translarna for the treatment of nmDMD;

- the potential receipt of revenues from future sales of our products or product candidates;

- the potential impact that enrollment, funding and completion of Study 041 may have on our revenue growth;

- our sales, marketing and distribution capabilities and strategy, including the ability of our third-party manufacturers to manufacture and deliver our products and product candidates in clinically and commercially sufficient quantities and the ability of distributors to process orders in a timely manner and satisfy their other obligations to us;

- our ability to establish and maintain arrangements for the manufacture of our products and product candidates that are sufficient to meet clinical trial and commercial launch requirements;
- our ability to establish and grow our manufacturing capabilities for our gene therapy platform;
- 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 3.00% convertible senior notes due August 15, 2022 and 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, gene therapy, Bio-e and oncology programs;
- whether we may pursue business development opportunities, including potential collaborations, alliances, and acquisition or licensing of assets and our ability to successfully develop or commercialize any assets to which we may gain rights pursuant to such business development opportunities;
- the potential advantages of our products and any product candidate;
- our intellectual property position;
- the impact of government laws and regulations;
- the impact of litigation that has been or may be brought against us or of litigation that we are pursuing against others; and
- our competitive position.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this Quarterly Report on Form 10-Q, particularly in Part II, Item 1A. Risk Factors as well as in Part I, Item 1A. Risk Factors in our Annual Report on Form 10-K for the year ended December 31, 2019, 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, 2019 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, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 433,970	$ 288,028
Marketable securities	707,049	398,535
Trade receivables, net	56,786	55,538
Inventory, net	19,481	19,285
Prepaid expenses and other current assets	26,888	17,898
Total current assets	1,244,174	779,284
Fixed assets, net	28,141	21,549
Intangible assets, net	712,536	710,500
Goodwill	82,341	82,341
Operating lease ROU assets	85,810	13,693
Deposits and other assets	28,057	16,415
Total assets	$ 2,181,059	$ 1,623,782
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 218,561	$ 159,276
Current portion of long-term debt	—	20,000
Deferred revenue	5,992	8,242
Operating lease liabilities- current	5,859	5,153
Finance lease liabilities- current	1,251	—
Liability for sale of future royalties- current	22,159	—
Deferred consideration payable	—	40,000
Other current liabilities	—	3,186
Total current liabilities	253,822	235,857
Deferred revenue- long term	—	3,415
Long-term debt	302,971	293,859
Contingent consideration payable	234,100	356,300
Deferred tax liability	130,863	130,862
Operating lease liabilities- noncurrent	81,674	9,018
Finance lease liabilities- noncurrent	22,608	—
Liability for sale of future royalties- noncurrent	642,099	—
Other long-term liabilities	1,968	141
Total liabilities	1,670,105	1,029,452
Stockholders' equity:		
Common stock, $0.001 par value. Authorized 125,000,000 shares; issued and outstanding 67,809,560 shares at September 30, 2020. Authorized 125,000,000 shares; issued and outstanding 61,935,870 shares at December 31, 2019.	68	62
Additional paid-in capital	2,101,136	1,795,351
Accumulated other comprehensive income	(35,727)	(10,584)
Accumulated deficit	(1,554,523)	(1,190,499)
Total stockholders' equity	510,954	594,330
Total liabilities and stockholders' equity	$ 2,181,059	$ 1,623,782

See accompanying unaudited notes.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Revenues:				
Net product revenue	$ 82,708	$ 71,369	$ 226,143	$ 209,899
Collaboration and grant revenue	35,000	47	35,063	622
Royalty revenue	696	—	696	—
Total revenues	118,404	71,416	261,902	210,521
Operating expenses:				
Cost of product sales, excluding amortization of acquired intangible assets	4,667	3,006	14,056	8,593
Amortization of acquired intangible assets	9,630	7,025	26,309	19,677
Research and development	92,998	63,076	359,630	175,621
Selling, general and administrative	57,840	49,284	169,708	139,044
Change in the fair value of deferred and contingent consideration	8,400	9,500	16,980	35,960
Settlement of deferred and contingent consideration	—	—	10,613	—
Total operating expenses	173,535	131,891	597,296	378,895
Loss from operations	(55,131)	(60,475)	(335,394)	(168,374)
Interest expense, net	(21,039)	(2,666)	(32,060)	(7,028)
Other income, net	28,766	2,800	26,242	2,509
Loss before income tax expense	(47,404)	(60,341)	(341,212)	(172,893)
Income tax (expense) benefit	(22,288)	344	(22,594)	(1,006)
Net loss attributable to common stockholders	$ (69,692)	$ (59,997)	$ (363,806)	$ (173,899)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	67,641,171	56,463,528	65,068,281	57,798,968
Net loss per share—basic and diluted (in dollars per share)	$ (1.03)	$ (1.06)	$ (5.59)	$ (3.01)

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,	
	2020	2019	2020	2019
Net loss	$ (69,692)	$ (59,997)	$ (363,806)	$ (173,899)
Other comprehensive (loss) income:				
Unrealized (loss) gain on marketable securities, net of tax	(1,016)	(463)	1,639	435
Foreign currency translation loss, net of tax	(24,695)	(843)	(26,782)	(1,281)
Comprehensive loss	$ (95,403)	$ (61,303)	$ (388,949)	$ (174,745)

See accompanying unaudited notes.

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

Three months ended September 30, 2020	Common stock		Additional paid-in capital	Accumulated other comprehensive loss	Accumulated deficit	Total stockholders' equity
	Shares	Amount				
Balance, June 30, 2020	67,240,679	$ 67	$ 2,067,274	$ (10,016)	$ (1,484,831)	$ 572,494
Issuance of common stock related to equity offering	173,956	—	9,141	—	—	9,141
Exercise of options	391,738	1	7,942	—	—	7,943
Restricted stock vesting and issuance, net	3,187	—	—	—	—	—
Share-based compensation expense	—	—	16,779	—	—	16,779
Net loss	—	—	—	—	(69,692)	(69,692)
Comprehensive loss	—	—	—	(25,711)	—	(25,711)
Balance, September 30, 2020	67,809,560	$ 68	$ 2,101,136	$ (35,727)	$ (1,554,523)	$ 510,954

Three months ended September 30, 2019	Common stock		Additional paid-in capital	Accumulated other comprehensive income (loss)	Accumulated deficit	Total stockholders' equity
	Shares	Amount				
Balance, June 30, 2019	58,707,185	$ 58	$ 1,539,530	$ 1,922	$ (1,052,825)	$ 488,685
Issuance of common stock related to equity offering	2,539,174	3	99,208	—	—	99,211
Equity component of the convertible notes issuance, net	—	—	119,489			119,489
Exercise of options	334,433	—	7,088	—	—	7,088
Restricted stock vesting and issuance, net	(1,800)	—	—	—	—	—
Share-based compensation expense	—	—	10,484	—	—	10,484
Net loss	—	—	—	—	(59,997)	(59,997)
Comprehensive loss	—	—	—	(1,306)	—	(1,306)
Balance, September 30, 2019	61,578,992	$ 61	$ 1,775,799	$ 616	$ (1,112,822)	$ 663,654

Nine months ended September 30, 2020	Common stock		Additional paid-in capital	Accumulated other comprehensive loss	Accumulated deficit	Total stockholders' equity
	Shares	Amount				
Balance, December 31, 2019	61,935,870	$ 62	$ 1,795,351	$ (10,584)	$ (1,190,499)	$ 594,330
Issuance of common stock related to equity offering	542,470	1	28,091	—	—	28,092
Issuance of common stock related to acquisition	845,364	1	42,868	—	—	42,869
Issuance of common stock related to rights exchange	2,821,176	3	150,525	—	—	150,528
Exercise of options	1,426,026	1	32,986	—	—	32,987
Restricted stock vesting and issuance, net	179,963	—	—	—	—	—
Issuance of common stock in connection with an employee stock purchase plan	58,691	—	2,406	—	—	2,406
Share-based compensation expense	—	—	48,909	—	—	48,909
Other	—	—	—	—	(218)	(218)
Net loss	—	—	—	—	(363,806)	(363,806)
Comprehensive loss	—	—	—	(25,143)	—	(25,143)
Balance, September 30, 2020	67,809,560	$ 68	$ 2,101,136	$ (35,727)	$ (1,554,523)	$ 510,954

Nine months ended September 30, 2019	Common stock		Additional paid-in capital	Accumulated other comprehensive income (loss)	Accumulated deficit	Total stockholders' equity
	Shares	Amount				
Balance, December 31, 2018	50,606,147	$ 51	$ 1,288,137	$ 1,462	$ (938,923)	$ 350,727
Issuance of common stock related to equity offering	10,102,899	10	323,746	—	—	323,756
Equity component of the convertible notes issuance, net	—	—	119,489	—	—	119,489
Exercise of options	645,435	—	12,195	—	—	12,195
Restricted stock vesting and issuance, net	169,792	—	—	—	—	—
Issuance of common stock in connection with an employee stock purchase plan	54,719	—	1,564	—	—	1,564
Share-based compensation expense	—	—	30,668	—	—	30,668
Net loss	—	—	—	—	(173,899)	(173,899)
Comprehensive loss	—	—	—	(846)	—	(846)
Balance, September 30, 2019	61,578,992	$ 61	$ 1,775,799	$ 616	$ (1,112,822)	$ 663,654

See accompanying unaudited notes.

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

	Nine Months Ended September 30,	
	2020	2019
Cash flows from operating activities		
Net loss	$ (363,806)	$ (173,899)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	31,088	22,903
Non-cash operating lease expense	4,295	2,446
Non-cash finance lease amortization expense	41,382	—
Non-cash royalty revenue related to sale of future royalties	(299)	—
Non-cash interest expense on liability related to sale of future royalties	14,557	—
Change in valuation of deferred and contingent consideration	16,980	35,960
Settlement of deferred and contingent consideration	10,613	—
Non-cash stock consideration, acquisition	42,869	—
Unrealized gain on equity investment	(852)	(2,529)
Unrealized gain on ClearPoint convertible debt security	(633)	—
Non-cash interest expense	16,678	6,583
Loss on disposal of asset	25	—
Amortization of discounts on investments, net	(559)	(1,374)
Amortization of debt issuance costs	766	434
Share-based compensation expense	48,909	30,668
Unrealized foreign currency transaction (gains) losses, net	(27,077)	205
Changes in operating assets and liabilities:		
Inventory, net	390	(1,574)
Prepaid expenses and other current assets	(9,289)	(11,771)
Trade receivables, net	121	5,879
Deposits and other assets	(217)	(1,073)
Accounts payable and accrued expenses	54,390	695
Other liabilities	(4,096)	(3,567)
Deferred revenue	(5,940)	1,581
Net cash used in operating activities	$ (129,705)	$ (88,433)
Cash flows from investing activities		
Purchases of fixed assets	$ (11,491)	$ (9,104)
Purchase of convertible debt security	(10,000)	—
Purchases of marketable securities	(1,006,946)	(239,386)
Sale and redemption of marketable securities	700,627	114,500
Acquisition of product rights and licenses	(25,618)	(20,250)
Purchase of equity investment	—	(4,000)
Net cash provided by (used in) investing activities	$ (353,428)	$ (158,240)
Cash flows from financing activities		
Proceeds from issuance of convertible notes	$ —	$ 279,571
Proceeds from exercise of options	32,987	12,195
Termination and exit fees related to payoff of secured term loan	(597)	—
Net proceeds from public offerings	28,092	324,262
Repayment of senior secured term loan	(28,333)	(6,667)
Payments on deferred consideration obligation	(38,100)	—
Proceeds from employee stock purchase plan	2,406	1,564
Payment of finance lease principal	(17,829)	—
Cash consideration received from Royalty Purchase Agreement	650,000	—
Net cash provided by financing activities	$ 628,626	$ 610,925
Effect of exchange rate changes on cash	449	(2,673)
Net decrease in cash and cash equivalents	145,942	361,579
Cash and cash equivalents, and restricted cash beginning of period	295,528	169,498
Cash and cash equivalents, and restricted cash end of period	$ 441,470	$ 531,077

Supplemental disclosure of cash information		
Cash paid for interest	$ 9,802	$ 7,042
Cash paid for income taxes	1,562	1,931
Supplemental disclosure of non-cash investing and financing activity		
Unrealized gain on marketable securities, net of tax	$ 1,639	$ 435
Right-of-use assets obtained in exchange for operating lease obligations	76,657	16,120
Right-of-use assets obtained in exchange for finance lease obligations	41,382	—
Acquisition of product rights and licenses	13,786	7,432
Issuance of common stock related to rights exchange	150,528	—
Accrued transaction costs related to public offerings	—	506
Accrued transaction costs related to issuance of convertible notes	—	298

See accompanying unaudited notes.

PTC Therapeutics, Inc.
Notes to Consolidated Financial Statements (unaudited)
September 30, 2020
In thousands (except per share data 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. The Company’s ability to globally commercialize products is the foundation that drives its continued investment in a robust diversified pipeline of transformative medicines and its mission to provide access to best-in-class treatments for patients who have an unmet medical need. The Company’s strategy is to bring best-in-class therapies with differentiated clinical benefit to patients affected by rare disorders and to leverage its global commercial infrastructure to maximize value for its patients and other stakeholders.

The Company has 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 (the “EEA”) for the treatment of nonsense mutation Duchenne muscular dystrophy (“nmDMD”), in ambulatory patients aged 2 years and older and in Brazil for the treatment of nmDMD in ambulatory patients aged 5 years and older, subject to annual renewal and other conditions. In July 2020, the European Commission 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 has a pipeline of gene therapy product candidates for rare monogenic diseases that affect the central nervous system (“CNS”) including PTC-AADC for the treatment of Aromatic L-Amino Acid Decarboxylase (“AADC deficiency”), a rare CNS disorder arising from reductions in the enzyme AADC that results from mutations in the dopa decarboxylase gene. The Company is preparing a biologics license application ("BLA") for PTC-AADC for the treatment of AADC deficiency in the United States and it anticipates submitting a BLA to the United States Food and Drug Administration ("FDA") in the first half of 2021. In January 2020, the Company submitted a marketing authorization application ("MAA") to the European Medicines Agency (“EMA”) for PTC-AADC for the treatment of AADC deficiency in the EEA, and the Company expects an opinion from the Committee for Medical Products for Human Use (“CHMP”) in the first half of 2021.

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 Company’s Collaboration and License Agreement with Akcea Therapeutics, Inc. (“Akcea”). 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”). Waylivra has received marketing authorization in the EU for the treatment of familial chylomicronemia syndrome (“FCS”). The Company filed for marketing authorization for Waylivra for the treatment of FCS with ANVISA, the Brazilian health regulatory authority, in June 2020 and, subject to potential delays in the review process related to the COVID-19 pandemic, expects a regulatory decision on approval from ANVISA in 2021.

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 in August 2020 by the FDA for the treatment of SMA in adults and children two months and older. Evrysdi also received marketing authorization for the treatment of SMA in Brazil in October 2020 and the EMA accepted the MAA filed by Roche for Evrysdi for the treatment of SMA in August 2020.

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, (the “Asset Acquisition”) pursuant to an asset purchase agreement by and between the Company and BioElectron, dated October 1, 2019 (the “Asset Acquisition Agreement”). The transaction was

accounted for as an asset acquisition. The two most advanced molecules in the Company's Bio-e platform are vatiquinone and PTC857. The Company initiated a registration-directed Phase 2/3 placebo-controlled trial of vatiquinone in approximately 60 children with mitochondrial disease and associated refractory epilepsy in the third quarter of 2020. The Company expects to initiate a registration-directed Phase 3 trial of vatiquinone in approximately 100 patients with Friedrich ataxia in the fourth quarter of 2020. In the second quarter of 2020, the Company initiated a Phase 1 trial in healthy volunteers to evaluate the safety and pharmacology of PTC857, which the Company believes has the potential to address multiple CNS disorders with glucocerebrosidase Parkinson's disease as its first planned indication. The Company completed dosing within the single ascending dose study and expects to complete dosing within the multiple ascending dose study from the Phase 1 trial by the end of 2020.

In June 2020, the FDA authorized the initiation of a Phase 2/3 clinical trial evaluating PTC299, a dihydroorotate dehydrogenase inhibitor that the Company has also been developing in oncological indications, as a potential treatment for COVID-19. The integrated Phase 2/3 study, which has been initiated and is being conducted in two stages, will evaluate the efficacy and safety of PTC299 in patients hospitalized with SARS-CoV-2 infection. The Company expects Stage 1 of the clinical trial to be completed in the fourth quarter of 2020 and it anticipates reporting top-line results from both stages in the first half of 2021.

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, including rare diseases and oncology.

The Company's marketing authorization for Translarna in the EEA is subject to annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of the authorization, which the Company refers to as the annual EMA reassessment. This marketing authorization is further subject to the specific obligation to conduct and submit the results of a multi-center, randomized, double-blind, 18-month, placebo-controlled trial, followed by an 18-month open-label extension, according to an agreed protocol, in order to confirm the efficacy and safety of Translarna. The final report on the trial and open-label extension is to be submitted by the Company to the EMA by the end of the third quarter of 2022. The Company refers to the trial and open-label extension together as Study 041.

The marketing authorization in the EEA was last renewed in June 2020 and is effective, unless extended, through August 5, 2021. The renewal was based on the Company's commitment to conduct Study 041 and the totality of the clinical data available from its trials and studies of Translarna for the treatment of nmDMD, including the safety and efficacy results of the Phase 2b and Phase 3 clinical trials. The primary efficacy endpoint was not achieved in either trial within the pre-specified level of statistical significance.

Translarna is an investigational new drug in the United States. During the first quarter of 2017, the Company filed a New Drug Application ("NDA"), over protest with the FDA, for which the FDA granted a standard review. 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, the Company 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 the Company's 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. The Company intends to follow the FDA's recommendation and will collect, using newer technologies via procedures and methods that the Company designed, such dystrophin data in a new study, Study 045, which the Company initiated in the fourth quarter of 2018. The final study muscle biopsies for the patients in Study 045 were completed in October 2020 and the Company has begun the process of analyzing the samples according to its protocol. The Company expects to report top-line results from Study 045 in the first quarter of 2021 followed by a potential resubmission of the NDA for Translarna for the treatment of nmDMD thereafter. Additionally, should a re-submission of an NDA receive accelerated approval, the Office of New Drugs stated that Study 041, which is currently enrolling, could serve as the confirmatory post-approval trial required in connection with the accelerated approval framework.

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 "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 "Merger").

Upon the closing of the Merger, the Company paid to Agilis equityholders total upfront consideration comprised of $49.2 million in cash and 3,500,907 shares of the Company's common stock (the "Closing Stock Consideration"). The Closing Stock Consideration was determined by dividing $150.0 million by the volume-weighted average price per share of the Company's common stock on the Nasdaq Global Select Market for the 10 consecutive trading-day period ending on the second trading-day immediately preceding the closing of the Merger. Agilis equityholders are entitled to receive contingent payments from the Company based on the achievement of certain development, regulatory and net sales milestones as well as based upon a percentage of net sales of certain products.

On April 29, 2020, the Company, certain of the former equity holders of Agilis, and, for the limited purposes set forth in the agreement, Shareholder Representative Services LLC, entered into a Rights Exchange Agreement (the "Rights Exchange Agreement"). Pursuant to the Rights Exchange Agreement, the Company issued 2,821,176 shares of its common stock (the "Common Stock Consideration") and paid $36.9 million (the "Cash Consideration"), in the aggregate, to such former equityholders of Agilis (the "Participating Rightholders") in exchange for the cancellation and forfeiture by the Participating Rightholders of their rights to receive certain milestone-based contingent payments under the Merger Agreement, pursuant to which the Company completed the Merger.

Pursuant to the terms of the Rights Exchange Agreement, the Participating Rightholders have canceled and forfeited their rights under the 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 Merger, regardless of whether the milestones were achieved.

The Rights Exchange Agreement has no effect on the 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 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 in the three month period ended September 30, 2020 upon the passing of the second anniversary of the closing of the 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 Merger Agreement. Refer to Note 5 for further details regarding the Rights Exchange Agreement.

On May 29, 2020, the Company completed its acquisition of Censa Pharmaceuticals, Inc. ("Censa") pursuant to an 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 (the "Censa Merger"). The transaction was accounted for as an asset acquisition. In connection with the Censa Merger, the Company acquired PTC923, which is being pursued as a possible treatment for orphan metabolic diseases associated with defects in the tetrahydrobiopterin biochemical pathways, including phenylketonuria ("PKU"). Refer to Note 3 for further details.

As of September 30, 2020, the Company had an accumulated deficit of approximately $1,554.5 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 and in August 2015 of 3.00% convertible senior notes due 2022 (see Note 11), public offerings of common stock in February 2014, October 2014, April 2018, January 2019, and September 2019, "at the market offerings" of its common stock, its initial public offering of common stock in June 2013, proceeds from the Royalty Purchase Agreement

(see Note 2), private placements of its convertible preferred stock, collaborations, bank debt, the Company's credit and security agreement (the "Credit Agreement"), with MidCap Financial Trust, or MidCap Financial, as administrative agent and MidCap Financial and other certain institutions as lenders thereto (see Note 11), 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. Since 2014, 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, and since May 2017, the Company has generated revenue from net sales of Emflaza for the treatment of DMD in the United States. The Company expects that cash flows from the sales of its products, 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, 2019 included in the Company's Annual Report on Form 10-K filed with the Securities and Exchange Commission (the "SEC") on March 2, 2020 (the "2019 Form 10-K"). Additional significant accounting policies adopted during the nine month period ended September 30, 2020 are discussed in further detail below.

Basis of presentation

The accompanying financial information as of September 30, 2020 and for the three and nine months ended September 30, 2020 and 2019 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, 2019 and notes thereto included in the 2019 Form 10-K.

In the opinion of management, the unaudited financial information as of September 30, 2020 and for the three and nine months ended September 30, 2020 and 2019 reflects all adjustments, which are normal recurring adjustments, necessary to present a fair statement of financial position, results of operations, stockholders' equity, and cash flows. The results of operations for the three and nine month periods ended September 30, 2020 are not necessarily indicative of the results to be expected for the year ended December 31, 2020 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 relates to 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 our 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. The amount is classified within deposits and other assets on the consolidated balance sheet due to the long-term nature of the letter of credit.

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:

	Beginning of period- December 31, 2019	End of period- September 30, 2020
Cash and cash equivalents	$ 288,028	$ 433,970
Restricted cash included in deposits and other assets	7,500	7,500
Total Cash, cash equivalents and restricted cash per statement of cash flows	$ 295,528	$ 441,470

Marketable securities

The Company considers 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 month and nine month periods ended September 30, 2020, no allowance was recorded for credit losses.

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, 2020	December 31, 2019
Raw materials	$ 946	$ 874
Work in progress	8,600	9,652
Finished goods	9,935	8,759
Total inventory	$ 19,481	$ 19,285

The Company periodically reviews its inventories for excess amounts or obsolescence and writes down obsolete or otherwise unmarketable inventory to its estimated net realizable value. For the three and nine months ended September 30, 2020, inventory write downs were immaterial. No write downs were recorded for the three and nine month periods ended September 30, 2019. 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 month periods ended September 30, 2020 and 2019, 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 and royalty payments associated with net product sales. Production costs are expensed as cost of product sales when the related products are sold.

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, 2020 and 2019, net product sales outside of the United States were $44.2 million and $48.5 million, respectively, and net product sales in the United States were $38.5 million and $22.9 million, respectively. For the nine months ended September 30, 2020 and 2019, net product sales outside of the United States were $124.0 million and $141.6 million, respectively, and net product sales in the United States were $102.1 million and $68.3 million, 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 ASC Topic 808 “Collaborative Arrangements” to then determine if ASC Topic 606 is applicable by considering whether the collaborator meets the definition of a customer. If the criteria are met, the Company assesses the promises in the arrangement to identify distinct performance obligations.

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

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

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

Allowance for doubtful accounts

The Company maintains an allowance for estimated losses resulting from the inability of its customers to make required payments. The Company estimates uncollectible amounts based upon current customer receivable balances, the age of customer receivable balances, the customer’s financial condition and current economic trends. The allowance for doubtful accounts was $0.1 million as of September 30, 2020 and $0.3 million as of December 31, 2019. Bad debt expense was immaterial for the three and nine month periods ended September 30, 2020 and 2019. For the three and nine month periods ended September 30, 2020, no allowances were recorded for credit losses.

Liability for sale of future royalties

On July 17, 2020, the Company, RPI Intermediate Finance Trust (“RPI”), and, for the limited purposes set forth in the agreement, Royalty Pharma PLC, entered into a Royalty Purchase Agreement (the “Royalty Purchase Agreement”). Pursuant to the Royalty Purchase Agreement, the Company sold to RPI 42.933% (the “Assigned Royalty Payment”) of the Company’s right to receive sales-based royalty payments (the “Royalty”) on worldwide net sales of Evrysdi and any other product developed pursuant to the License and Collaboration Agreement (the “License Agreement”), dated as of November 23, 2011, by and among the Company, Roche and, for the limited purposes set forth therein, the SMA Foundation under the SMA program. In consideration for the sale of the Assigned Royalty Payments, RPI paid the Company $650.0 million in cash consideration. The Company has retained a 57.067% interest in the Royalty and all economic rights to receive the remaining potential regulatory and sales milestone payments under the License Agreement, which milestone payments equal $362.5 million in the aggregate as of September 30, 2020. 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 License Agreement and the date on which RPI has received $1.3 billion in respect of the Assigned Royalty Payments.

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 will be amortized using the effective interest method over the life of the arrangement, in accordance with the respective guidance. The Company will utilize the prospective method to account for subsequent changes in the estimated future payments to be made to RPI. Refer to Note 11 for further details.

Indefinite-lived intangible assets

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

Goodwill

Goodwill represents the amount of consideration paid in excess of the fair value of net assets acquired as a result of the Company’s business acquisitions accounted for using the acquisition method of accounting. Goodwill is not amortized and is subject to impairment testing at a reporting unit level on an annual basis or when a triggering event occurs that may indicate the carrying value of the goodwill is impaired. The Company reassess its reporting units as part of its annual

segment review. An entity is permitted to first assess qualitative factors to determine if a quantitative impairment test is necessary. Further testing is only required if the entity determines, based on the qualitative assessment, that it is more likely than not that the fair value of the reporting unit is less than its carrying amount.

Income Taxes

On March 27, 2020, the United States enacted the Coronavirus Aid, Relief, and Economic Security Act, referred to herein as the CARES Act, as a response to the economic uncertainty resulting from a strain of novel coronavirus, COVID-19. The CARES Act includes modifications for net operating loss carryovers and carrybacks, limitations of business interest expense for tax, immediate refund of alternative minimum tax (AMT) credit carryovers as well as a technical correction to the 2017 Tax Cuts and Jobs Act ("the 2017 Tax Act") for qualified improvement property. As of September 30, 2020, the Company expects that these provisions will not have a material impact. Tax provisions of the CARES Act also include the deferral of certain payroll taxes, relief for retaining employees, and other provisions. The Company is evaluating the impact of the CARES Act and currently expects to benefit from the deferral of certain payroll taxes and retention credit through the end of calendar year 2020. The ultimate impact of the CARES Act may differ from this estimate due to changes in interpretations and guidance that may be issued and actions the Company may take in response to the CARES Act. The Company will continue to assess the impact that various provisions will have on its business.

On December 22, 2017, the U.S. government enacted the 2017 Tax Act, which significantly revises U.S. tax law by, among other provisions, lowering the U.S. federal statutory 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-tax 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, 2020.

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.

The Company recorded a deferred tax liability in conjunction with the Merger of $122.0 million related to the tax basis difference in the IPR&D 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.

The $650.0 million cash consideration received from RPI pursuant to the Royalty Purchase Agreement was treated as taxable income in the current year and resulted in a current tax provision of $22.1 million related to state income taxes after considering the state NOL and tax credits. The Company did not generate a federal income tax liability after considering the federal NOL and tax credits.

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 4 Leases for additional information.

Recently issued accounting standards

In August 2020, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") 2020-06, "Debt—Debt with Conversion and Other Options (Subtopic 470-20) and Derivatives and Hedging—Contracts in Entity's Own Equity (Subtopic 815-40): Accounting for Convertible Instruments and Contracts in an Entity's Own Equity." ASU 2020-06 simplifies the accounting for convertible instruments by removing certain separation models in Subtopic 470- 20, Debt—Debt with Conversion and Other Options, for convertible instruments. Under ASU 2020-06, the embedded conversion features no longer are separated from the host contract for convertible instruments with conversion features that are not required to be accounted for as derivatives under Topic 815, Derivatives and Hedging, or that do not result in substantial premiums accounted for as paid-in capital. Consequently, a convertible debt instrument will be accounted for as a single liability measured at its amortized cost and a convertible preferred stock will be accounted for as a single equity instrument measured at its historical cost, as long as no other features require bifurcation and recognition as derivatives. By removing those separation models, the interest rate of convertible debt instruments typically will be closer to the coupon interest rate when applying the guidance in Topic 835, Interest. The amendments under ASU 2020-06 also include revisions related to the derivatives scope exception for contracts in an entity's own equity and earnings per share. The amendments under ASU 2020-06 are effective for public business entities that meet the definition of a SEC filer, excluding entities eligible to be smaller reporting companies as defined by the SEC, for fiscal years beginning after December 15, 2021, including interim periods within those fiscal years. For all other entities, the amendments are effective for fiscal years beginning after December 15, 2023, including interim periods within those fiscal years. Early adoption is permitted, but no earlier than fiscal years beginning after December 15, 2020, including interim periods within those fiscal years. The FASB specified that an entity should adopt the guidance as of the beginning of its annual fiscal year. The Company expects to adopt this guidance when effective and is currently assessing what effect the adoption of ASU 2020-06 will have on its consolidated financial statements and accompanying notes.

Impact of recently adopted accounting pronouncements

In December 2019, the FASB issued ASU 2019-12,"Income Taxes (Topic 740): Simplifying the Accounting for Income Taxes". ASU 2019-12 simplifies the accounting for income taxes by removing certain exceptions to the general principles in Topic 740. The amendments also improve consistent application of and simplify GAAP for other areas of Topic 740 by clarifying and amending the existing guidance. For public business entities, the guidance is effective for annual periods, and interim periods within those annual periods, beginning after December 15, 2020. For all other entities, it is effective

for annual periods beginning after December 15, 2021 and interim periods in annual periods beginning after December 15, 2022. Early adoption is permitted, including adoption in any interim period. The Company early adopted this guidance January 1, 2020. The adoption of the guidance did not have a material impact on the consolidated financial statements and accompanying notes.

In June 2016, the FASB issued ASU No. 2016-13, “Financial Instruments — Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments”. This standard requires financial assets measured at amortized cost basis to be presented at the net amount expected to be collected. This standard is effective for public companies who are SEC filers for fiscal years beginning after December 15, 2019, including interim periods within those years. In November 2019, the FASB issued ASU 2019-11, Codification Improvements to Topic 326, Financial Instruments - Credit Losses, which expands the scope of the practical expedient that allows entities to exclude the accrued interest component of amortized cost from various disclosures required by ASC 326 to also include certain disclosures required by ASC 320. Entities that elect to apply the practical expedient must disclose the total amount of accrued interest that they exclude from their disclosures of amortized cost. The amendments have the same effective dates as ASU 2016-13 (Topic 326) for entities that have not yet adopted that standard. The Company adopted ASU 2016-13 and ASU 2019-11 effective January 1, 2020. The adoption of the guidance did not have a material impact on the consolidated financial statements. The Company has updated its accounting policy for marketable securities within this footnote as well as its fair value footnote (Note 5) with additional disclosures as required by the standard upon adoption.

In August 2018, the FASB issued ASU 2018-13, "Fair Value Measurement (Topic 820), Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement". This standard eliminates certain disclosure requirements for fair value measurements for all entities, requires public entities to disclose certain new information and modifies some disclosure requirements. The new guidance is effective for all entities for fiscal years beginning after December 15, 2019 and for interim periods within those fiscal years. An entity is permitted to early adopt either the entire standard or only the provisions that eliminate or modify requirements. Entities can elect to early adopt in interim periods, including periods for which they have not yet issued financial statements or made their financial statements available for issuance. The Company adopted this guidance January 1, 2020. The adoption of the guidance did not have a material impact on the consolidated financial statements. The Company has updated its fair value footnote (Note 5) with additional and modified disclosures as required by the standard upon adoption.

In August 2018, the FASB issued ASU 2018-15,"Intangibles - Goodwill and Other - Internal-Use Software (Subtopic 350-40): Customer’s Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement That Is a Service Contract". ASU 2018-15 requires a customer in a cloud computing arrangement that is a service contract to follow the internal-use software guidance in Accounting Standards Codification 350-40 to determine which implementation costs to defer and recognize as an asset. For public business entities, the guidance is effective for annual periods, and interim periods within those annual periods, beginning after December 15, 2019. For all other entities, it is effective for annual periods beginning after December 15, 2020 and interim periods in annual periods beginning after December 15, 2021. Early adoption is permitted, including adoption in any interim period for all entities. The Company adopted this guidance January 1, 2020. The adoption of the guidance did not have a material impact on the consolidated financial statements and accompanying notes.

In November 2018, the FASB issued ASU 2018-18,"Collaborative Arrangements (Topic 808): Clarifying the Interaction between Topic 808 and Topic 606”. ASU 2018-18 provides guidance on whether certain transactions between collaborative arrangement participants should be accounted for with revenue under Topic 606. For public business entities, the guidance is effective for annual periods, and interim periods within those annual periods, beginning after December 15, 2019. For all other entities, it is effective for annual periods beginning after December 15, 2020 and interim periods in annual periods beginning after December 15, 2021. Early adoption is permitted, including adoption in any interim period for all entities. The Company adopted this guidance January 1, 2020. The adoption of the guidance did not have a material impact on the consolidated financial statements and accompanying notes.

3. Acquisition

Censa Acquisition

On May 29, 2020, the Company acquired Censa, pursuant to the Censa Merger Agreement. Upon the closing of the Censa Merger, the Company paid to the Censa securityholders (i) cash consideration of $15.0 million, which consisted of an upfront payment of $10.4 million and an additional $4.6 million for the net assets on Censa's opening balance sheet as of the date of the acquisition, and (ii) 845,364 shares of the Company's common stock, which were valued at $42.9 million based on the closing stock price on the acquisition date. The number of shares issued was determined using a 30-day VWAP pursuant to the Censa Merger Agreement.

The Company determined that substantially all of the fair value is concentrated in PTC923 and accounted for the transaction as an asset acquisition under ASC 805-50. The purchase price consisted of the cash consideration of $15.0 million and $42.9 million in the Company's common stock, in addition to $0.7 million of acquisition costs. As such, the total consideration transferred was determined to be $58.6 million. The opening balance sheet net assets of $4.6 million, which consisted of cash of $3.8 million and other current assets of $0.8 million, were determined to be non-qualifying assets and recorded at their fair values, respectively. The remaining consideration of $54.0 million was allocated to PTC923. As PTC923 is an IPR&D asset, the Company concluded that it did not have any alternative future use, and accordingly, the fair value amount allocated to the IPR&D was expensed. Of the $54.0 million, $53.3 million is included in research and development expense and $0.7 million is included in selling, general, and administrative expense within the Company's statement of operations for the nine month period ended September 30, 2020.

Subject to the terms and conditions of the Censa Merger Agreement, 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 PTC923'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 PTC923, (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 PTC923, on a country-by-country basis, which contingent payment shall equal to a mid-double digit percentage of any such sublicense fees. Pursuant to the Censa Merger Agreement, the Company has the option to pay the initial $30.0 million development milestone, for the completion of enrollment of a Phase 3 clinical trial for PTC923 for PKU, if achieved, in cash or shares of the Company's common stock. The Company will record the milestone and royalty payments when they become payable. Milestone payments prior to FDA approval of PTC923 for PKU (or other indications) will be expensed accordingly and milestone payments that will only occur after PTC923 for PKU (or other indications) is FDA approved, will be capitalized and amortized over their expected useful lives.

4. Leases

The Company leases office space in South Plainfield, New Jersey for its principal office under three noncancelable operating leases through May 2022 and August 2024, in addition to 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 (the "Campus") pursuant to a Lease Agreement (the "Lease") with Hopewell Campus Owner LLC (the "Landlord"). The rental term of the Lease commenced on July 1, 2020 and has an initial term of fifteen years (the "Initial Term"), with two consecutive ten year renewal periods, each at the Company's option.

The aggregate rent for the 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 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 Lease contains customary events of default, representations, warranties and covenants.

Subject to the terms of the Lease, the Company has a right of first refusal to rent certain other space of the Campus, which would be triggered upon the Landlord's issuance of a second round proposal or letter of intent to another tenant for such space. The Company also may seek to build a new separate building on the Campus, which may not contain less than 75,000 square feet (the "New Building"). Upon receipt of notice of the Company's intention to build the New Building, the Landlord may, in its sole discretion, construct and lease the New Building to the Company or enter into a ground lease with the Company permitting the Company to construct the New Building. Rent terms for the New Building would be determined based on the land value, construction and project costs subject to whether the Landlord or Company constructs the New Building.

The Company entered into a lease agreement with COE Bridgewater LLC on March 20, 2020 relating to the lease of office and laboratory space located in Bridgewater, New Jersey. This lease replaced the Company's existing lease on the property beginning on May 1, 2020 and includes additional rental property of approximately 59,000 square feet.

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 0.1 years to 14.8 years and certain of the leases include renewal options to extend the lease for up to 10 years. Rent expense was approximately $5.2 million and $1.7 million for the three month periods ended September 30, 2020 and 2019, respectively. Rent expense was $10.2 million and $3.8 million for the nine month periods ended September 30, 2020 and 2019, respectively.

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 agreement will expire on December 31, 2032 unless the Company terminates it on 24 months prior written notice to MassBio. Pursuant to the terms of the agreement, MassBio agreed to provide the Company with four dedicated rooms for its gene therapy AADC program. The Company concluded that the agreement contains an embedded lease as the Company controls the use of the four dedicated rooms and the equipment therein. As the present value of the facilities exceeds the assessed fair value, the Company determined that it is a finance lease. Given that the embedded finance lease 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 and accordingly, expensed the present value of all guaranteed future cash payments of $41.4 million during the nine month period ending September 30, 2020. Additionally, during the three and nine month periods ending September 30, 2020, the Company recorded finance lease costs of $0.5 million and $0.5 million, respectively.

The components of operating lease expense were as follows:

	Three Months Ended September 30, 2020	Three Months Ended September 30, 2019	Nine Months Ended September 30, 2020	Nine Months Ended September 30, 2019
Operating Lease Cost				
Fixed lease cost	$ 4,097	$ 1,431	$ 7,896	$ 3,078
Variable lease cost	890	184	1,914	488
Short-term lease cost	175	94	340	235
Total operating lease cost	$ 5,162	$ 1,709	$ 10,150	$ 3,801

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

Supplemental balance sheet information related to leases was as follows:

	September 30, 2020	December 31, 2019
Operating lease ROU asset	$ 85,810	$ 13,693
Operating lease liabilities- current	$ 5,859	$ 5,153
Operating lease liabilities- noncurrent	81,674	9,018
Total operating lease liability	$ 87,533	$ 14,171

	September 30, 2020	December 31, 2019
Finance lease liabilities- current	$ 1,251	$ —
Finance lease liabilities- noncurrent	22,608	—
Total finance lease liability	$ 23,859	$ —

The Company's leases in Bridgewater, New Jersey and Hopewell Township, New Jersey are the primary drivers of the increase in total operating lease ROU asset and total operating lease liability from December 31, 2019 to September 30, 2020.

Supplemental lease term and discount rate information related to leases was as follows as of September 30, 2020 and December 31, 2019:

	September 30, 2020	December 31, 2019
Weighted-average remaining lease terms - operating leases (years)	11.66	3.38
Weighted-average discount rate - operating leases	8.64 %	7.33 %
Weighted-average remaining lease terms - finance lease (years)	12.25	—
Weighted-average discount rate - finance lease	7.80 %	—

Supplemental cash flow information related to leases was as follows as of September 30, 2020 and 2019:

	Nine Months Ended September 30, 2020	2019
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows from operating leases	$ 5,611	$ 2,751
Financing cash flows from finance lease	17,829	—
Right-of-use assets obtained in exchange for lease obligations:		
Operating leases	$ 76,657	$ 16,120
Finance lease	41,382	—

Future minimum lease payments under non-cancelable leases as of September 30, 2020 were as follows:

	Operating Leases	Finance Lease
2020 (excludes the nine months ended September 30, 2020)	$ 2,842	$ —
2021	13,472	3,000
2022	13,229	3,000
2023	12,831	3,000
2024 and thereafter	104,142	27,000
Total lease payments	146,516	36,000
Less: Imputed Interest expense	58,983	12,141
Total	$ 87,533	$ 23,859

In conjunction with the Asset Acquisition, the Company acquired BioElectron's lease in Mountainview, California. As substantially all of the fair value of the gross assets acquired was related to vatiquinone, the relative fair value allocated to the right of use asset and corresponding lease liability for the Mountainview lease was determined to be immaterial, and accordingly is not included in the tables above. The future minimum lease payments for the Mountainview lease as of September 30, 2020 are $0.4 million, $1.8 million, and $1.4 million for 2020, 2021 and 2022, respectively.

5. Fair value of financial instruments and marketable securities

The Company follows the fair value measurement rules, which provide guidance on the use of fair value in accounting and disclosure for assets and liabilities when such accounting and disclosure is called for by other accounting literature.

These rules establish a fair value hierarchy for inputs to be used to measure fair value of financial assets and liabilities. This hierarchy prioritizes the inputs to valuation techniques used to measure fair value into three levels: Level 1 (highest priority), Level 2, and Level 3 (lowest priority).

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

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

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

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

In May 2019, the Company purchased $4.0 million of shares of ClearPoint Neuro, Inc.'s ("ClearPoint"), formerly known as MRI Interventions, Inc., common stock, at a purchase price of $3.10 per share, in connection with a securities purchase agreement that the Company entered into with ClearPoint, a publicly traded medical device company. The Company determined that the equity investment represents a financial instrument and therefore, recorded it at fair value, which is readily determinable. The equity investment is a component of deposits and other assets on the consolidated balance sheet. During the three and nine month periods ended September 30, 2020, the Company recorded unrealized gains of $2.5 million and $0.9 million, respectively, which are components of other income, net within the consolidated statement of operations. The fair value of the equity investment was $7.0 million as of September 30, 2020. The Company classifies its equity investment in ClearPoint as a Level 1 asset 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 month periods ended September 30, 2020, the Company recorded unrealized gains of $1.4 million and $0.6 million, respectively, which are components of other income, net within the consolidated statement of operations. The fair value of the convertible debt security was $10.6 million as of September 30, 2020. 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 equity investment and the convertible debt security, no other items included in deposits and other assets on the consolidated balance sheets are fair valued.

Fair value of certain marketable 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 investments, 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, 2020 and December 31, 2019:

	September 30, 2020			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities	$ 707,049	$ —	$ 707,049	$ —
Equity investment in ClearPoint	$ 7,045	$ 7,045	$ —	$ —
ClearPoint convertible debt security	$ 10,633	$ —	$ 10,633	$ —
Contingent consideration payable- development and regulatory milestones	$ 138,200	$ —	$ —	$ 138,200
Contingent consideration payable- net sales milestones and royalties	$ 95,900	$ —	$ —	$ 95,900

	December 31, 2019			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities	$ 398,535	$ —	$ 398,535	$ —
Equity investment in ClearPoint	$ 6,194	$ 6,194	$ —	$ —
Stock appreciation rights liability	$ 3,186	$ —	$ —	$ 3,186
Deferred consideration payable	$ 40,000	$ —	$ 40,000	$ —
Contingent consideration payable- development and regulatory milestones	$ 290,500	$ —	$ —	$ 290,500
Contingent consideration payable- net sales milestones and royalties	$ 65,800	$ —	$ —	$ 65,800

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, 2020 and December 31, 2019.

The following is a summary of marketable securities accounted for as available-for-sale securities at September 30, 2020 and December 31, 2019:

	September 30, 2020			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 260,392	$ 74	$ (16)	$ 260,450
Corporate debt securities	316,198	2,082	(19)	318,261
Asset-backed securities	35,461	232	(2)	35,691
Government obligations	92,602	61	(16)	92,647
Total	$ 704,653	$ 2,449	$ (53)	$ 707,049

	December 31, 2019			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 157,936	$ 162	$ —	$ 158,098
Corporate debt securities	188,778	576	(20)	189,334
Asset-backed securities	51,062	49	(8)	51,103
Total	$ 397,776	$ 787	$ (28)	$ 398,535

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 month and nine month periods ended September 30, 2020, 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 bases, 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 month and nine month periods ended September 30, 2020, 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 month and nine month periods ended September 30, 2020, the Company had $0.3 million and $0.5 million, respectively, realized gains from the sale of marketable securities. Realized gains are reported as a component of interest expense, net in the consolidated statement of operations.

The unrealized losses and fair values of available-for-sale securities that have been in an unrealized loss position for a period of less than and greater than 12 months as of September 30, 2020 are as follows:

	September 30, 2020					
	Securities in an unrealized loss position less than 12 months		Securities in an unrealized loss position greater than 12 months		Total	
	Unrealized losses	Fair Value	Unrealized losses	Fair Value	Unrealized losses	Fair Value
Commercial paper	$ (16)	53,580	—	—	(16)	53,580
Corporate debt securities	(19)	62,811	—	—	(19)	62,811
Asset-backed securities	(2)	2,899	—	—	(2)	2,899
Government obligations	(16)	31,955	—	—	(16)	31,955
Total	$ (53)	$ 151,245	$ —	$ —	$ (53)	$ 151,245

The unrealized losses and fair values of available-for-sale securities that have been in an unrealized loss position for a period of less than and greater than 12 months as of December 31, 2019 are as follows:

	December 31, 2019					
	Securities in an unrealized loss position less than 12 months		Securities in an unrealized loss position greater than 12 months		Total	
	Unrealized losses	Fair Value	Unrealized losses	Fair Value	Unrealized losses	Fair Value
Corporate debt securities	$ (20)	$ 71,779	$ —	$ —	$ (20)	$ 71,779
Asset-backed securities	(8)	24,211	—	—	(8)	24,211
Total	$ (28)	$ 95,990	$ —	$ —	$ (28)	$ 95,990

Marketable securities on the balance sheet at September 30, 2020 and December 31, 2019 mature as follows:

	September 30, 2020	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 260,451	$ —
Corporate debt securities	260,199	58,062
Asset-backed securities	14,375	21,316
Government obligations	39,530	53,116
Total Marketable securities	$ 574,555	$ 132,494

	December 31, 2019	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 158,098	$ —
Corporate debt securities	139,596	49,738
Asset-backed securities	44,724	6,379
Total Marketable securities	$ 342,418	$ 56,117

The Company classifies all of its marketable securities as current as they are all available for sale and are available for current operations.

Convertible senior notes

In August 2015, the Company issued $150.0 million of 3.00% convertible senior notes due August 15, 2022 (the "2022 Convertible Notes"). In September 2019, the Company issued $287.5 million of 1.50% convertible senior notes due September 15, 2026 (the "2026 Convertible Notes," together with the "2022 Convertible Notes," the "Convertible Notes"). The Company separately accounted for the liability and equity components of the Convertible Notes by allocating the proceeds between the liability component and equity component, as further discussed in Note 11. The fair value of the 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 Convertible Notes observed in market trading which are Level 2 inputs. The estimated fair value of the 2022 Convertible Notes at September 30, 2020 and December 31, 2019 was $171.3 million and $171.2 million, respectively. The estimated fair value of the 2026 Convertible Notes at September 30, 2020 and December 31, 2019 was $329.2 million and $335.0 million, respectively.

Deferred and contingent consideration payable

Pursuant to the Merger Agreement, Agilis equityholders were previously entitled to receive contingent consideration payments from the Company based on the achievement of certain development milestones up to an aggregate maximum amount of $60.0 million and 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. The Company was required to pay $40.0 million of development milestone payments upon the passing of the second anniversary of the closing of the Merger, regardless of whether the applicable milestones have been achieved. The $40.0 million of development milestones were classified as deferred consideration on the Company's consolidated balance sheets.

Pursuant to the terms of the Rights Exchange Agreement, in the three month period ended June 30, 2020, the Company issued 2,821,176 shares of its common stock and paid $36.9 million in the aggregate, to Participating Rightholders, who in exchange have canceled and forfeited their rights under the 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, or the deferred consideration, that would have been due upon the passing of the second anniversary of the closing of the Merger. As a result of the Rights Exchange Agreement, the remaining deferred consideration payable was $2.4 million, which was paid out in the three month period ended September 30, 2020 upon the passing of the second anniversary of the closing of the Merger. Accordingly, as of September 30, 2020, the remaining balance of the deferred consideration payable was $0.

As of result of the Rights Exchange Agreement, the Company recognized a gain of $0.7 million on the settlement of the development milestones and a loss of $11.3 million on the settlement of the regulatory milestones. The $0.7 million gain and $11.3 million loss are included in the settlement of deferred and contingent consideration in the Company's statement of operations for the nine month period ended September 30, 2020. Additionally, as of the date of the Rights Exchange Agreement, the Company recognized a gain on the fair value of the contingent consideration of $1.0 million related to the portion of regulatory milestones that were forfeited, which is included in the change in fair value of the deferred and contingent liability within the Company's statement of operations for the nine month period ended September 30, 2020. This non-recurring Level 3 fair value measurement was estimated using the same valuation methodology and unobservable inputs for development and regulatory milestones in the Level 3 valuation section below. In conjunction with the Rights

Exchange Agreement, the Company also incurred $2.0 million of transaction fees, which were included in other expense in the Company's statement of operations for the nine month period ended September 30, 2020.

Level 3 valuation

The stock appreciation rights ("SARs") liability is classified in other liabilities on the Company's consolidated balance sheets. The SARs liability is marked-to-market each reporting period with the change in fair value recorded as compensation expense on the Company's consolidated statements of operations until the SARs vest. The fair value of the SARs liability is determined at each reporting period by utilizing the Black-Scholes option pricing model. The last payment of the SARs liability was made in the three month period ended March 31, 2020, and accordingly, the balance of the SARS liability as of September 30, 2020 was $0.

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 deferred and 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. At September 30, 2020, the weighted average discount rate for the development and regulatory milestones was 4.2% and the weighted average probability of success was 42%. The fair value of the net sales milestones and royalties is determined utilizing an option pricing model with Monte Carlo simulation to simulate a range of possible payment scenarios, and the average of the payments in these scenarios is then discounted to calculate present fair value. At September 30, 2020, the weighted average discount rate for the net sales milestones and royalties was 12.0% and the weighted average probability of success for the net sales milestones was 48%.

The table presented below is a summary of changes in the fair value of the Company's Level 3 valuations for the SARs liability, and the contingent consideration payable for the periods ended September 30, 2020 and September 30, 2019.

	Level 3 liabilities		
	SARs	Contingent consideration payable-development and regulatory milestones	Contingent consideration payable-net sales milestones and royalties
Beginning balance as of December 31, 2019	$ 3,186	$ 290,500	$ 65,800
Additions	—	—	—
Change in fair value	—	(13,120)	30,100
Payments	(3,186)	—	—
Rights Exchange settlement	—	(139,180)	—
Ending balance as of September 30, 2020	$ —	$ 138,200	$ 95,900

	Level 3 liabilities		
	SARs	Contingent consideration payable-development and regulatory milestones	Contingent consideration payable-net sales milestones and royalties
Beginning balance as of December 31, 2018	$ 3,814	$ 257,040	$ 53,200
Additions	—	—	—
Change in fair value	2,236	28,260	6,400
Payments	(3,815)	—	—
Ending balance as of September 30, 2019	$ 2,235	$ 285,300	$ 59,600

The following significant unobservable inputs were used in the valuation of the contingent consideration payable for the periods ended September 30, 2020 and December 31, 2019 and of the SARs liability for the period ended December 31, 2019:

	September 30, 2020			
	Fair Value	Valuation Technique	Unobservable Input	Range
Contingent consideration payable- development and regulatory milestones	$138,200	Probability-adjusted discounted cash flow	Potential development and regulatory milestones Probabilities of success Discount rates Projected years of payments	$0 - $381 million 25% - 94% 3.1% - 5.3% 2021 - 2027
Contingent considerable payable- net sales milestones and royalties	$95,900	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% - 94% 2% - 6% 12.0% 2022 - 2039

	December 31, 2019			
	Fair Value	Valuation Technique	Unobservable Input	Range
SARs	$3,186	Option-pricing model	Volatility Risk free interest rate Strike price Fair value of common stock Expected life	28.93% 0.19% $6.76 - $30.86 $48.03 0.01 years
Contingent consideration payable- development and regulatory milestones	$290,500	Probability-adjusted discounted cash flow	Potential development and regulatory milestones Probabilities of success Discount rates Projected years of payments	$0 - $555 million 25% - 94% 2.2% - 4.7% 2020 - 2026
Contingent considerable payable- net sales milestones and royalties	$65,800	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% - 89% 2% - 6% 14.5% 2021 - 2038

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.

6. Other comprehensive income (loss) and accumulated other comprehensive items

Other comprehensive income (loss) includes changes in equity that are excluded from net income (loss), such as unrealized gains and losses on marketable securities.

The following tables summarize other comprehensive income (loss) and the changes in accumulated other comprehensive items for the three and nine month periods ended September 30, 2020:

	Unrealized Gains/(Losses) On Marketable Securities, net of tax	Foreign Currency Translation	Total Accumulated Other Comprehensive Items
Balance, June 30, 2020	$ 3,410	$ (13,426)	$ (10,016)
Other comprehensive loss before reclassifications	(1,016)	(24,695)	(25,711)
Amounts reclassified from other comprehensive items	—	—	—
Other comprehensive loss before reclassifications	(1,016)	(24,695)	(25,711)
Balance, September 30, 2020	$ 2,394	$ (38,121)	$ (35,727)

	Unrealized Gains On Marketable Securities, net of tax	Foreign Currency Translation	Total Accumulated Other Comprehensive Items
Balance, December 31, 2019	$ 755	$ (11,339)	$ (10,584)
Other comprehensive income (loss) before reclassifications	1,639	(26,782)	(25,143)
Amounts reclassified from other comprehensive items	—	—	—
Other comprehensive income (loss)	1,639	(26,782)	(25,143)
Balance, September 30, 2020	$ 2,394	$ (38,121)	$ (35,727)

7. Accounts payable and accrued expenses

Accounts payable and accrued expenses at September 30, 2020 and December 31, 2019 consist of the following:

	September 30, 2020	December 31, 2019
Employee compensation, benefits, and related accruals	$ 34,485	$ 38,390
Income tax payable	21,475	499
Consulting and contracted research	17,659	12,969
Professional fees	4,590	3,562
Sales allowance and other costs	50,644	41,155
Sales rebates and royalties	68,628	42,997
Accounts payable	15,663	10,324
Other	5,417	9,380
Total	$ 218,561	$ 159,276

8. 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. During the three and nine month periods ending September 30, 2020, the Company issued and sold an aggregate of 173,956 and 542,470 shares of common stock pursuant to the Sales Agreement at a weighted average public offering price of $54.39 and $53.37 per share, respectively. During the three and nine month periods ending September 30, 2020, the Company received net proceeds of $9.1 million and $28.1 million, respectively, after deducting underwriting discounts and commissions and other offering expenses payable by the Company. 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, 2020.

As a result of the Rights Exchange Agreement, during the nine month period ended September 30, 2020, the Company issued 2,821,176 shares of its common stock to Participating Rightholders. The shares had a fair value of $150.5 million upon issuance.

As a result of the Censa Merger, during the nine month period ended September 30, 2020, the Company issued 845,364 shares of the Company's common stock to Censa security holders, which were valued at $42.9 million based on the closing stock price on the acquisition date. The number of shares issued was determined using a 30-day VWAP pursuant to the Censa Merger Agreement.

9. Net loss per share

Basic earnings per share is computed by dividing net loss by the weighted-average number of common shares outstanding. Diluted earnings per share is computed by dividing net loss by the weighted-average number of common shares plus the effect of any dilutive potential common shares outstanding during the period.

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,	
	2020	2019	2020	2019
Numerator				
Net loss	$ (69,692)	$ (59,997)	$ (363,806)	$ (173,899)
Denominator				
Denominator for basic and diluted net loss per share	67,641,171	56,463,528	65,068,281	57,798,968
Net loss per share:				
Basic and diluted	$ (1.03)*	$ (1.06)*	$ (5.59)*	$ (3.01)*

* In the three and nine months ended September 30, 2020 and 2019, 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,	
	2020	2019
Stock Options	11,528,241	10,743,181
Unvested restricted stock awards and units	961,795	650,103
Total	12,490,036	11,393,284

10. Stock award plan

On March 5, 2013, the Company's Board of Directors approved the 2013 Stock Incentive Plan, which provides for the granting of stock option awards, stock appreciation rights, restricted stock, restricted stock units and other stock-based awards in the aggregate of 739,937 shares of common stock. On March 5, 2013, the Company's Board of Directors approved a grant of 735,324 shares of restricted stock and 4,613 stock options. There are no additional shares available for issuance under this plan.

In 2009, the Company's shareholders approved the 2009 Equity and Long-Term Incentive Plan, which provides for the granting of stock option awards, restricted stock awards, and other stock-based and cash-based awards, subject to certain adjustments and annual increases. In May 2013, the Company's Board of Directors and stockholders increased by 2,500,000 the number of shares authorized under the 2009 Equity and Long Term Incentive Plan, which provides for the granting of stock option awards, restricted stock awards, and other stock-based and cash-based awards. There are no additional shares available for issuance under this 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. The 2013 Long Term Incentive Plan provides for the grant of incentive stock options, nonstatutory stock options, restricted stock awards and other stock-based awards. The number of shares of common stock reserved for issuance under the 2013 Long Term Incentive Plan is the sum of (1) 122,296 shares of common stock available for issuance under the Company's 2009 Equity and Long Term Incentive Plan and 2013 Stock Incentive Plan, (2) the number of shares (up to 3,040,444 shares) equal to the sum of the number of shares of common stock subject to outstanding awards under the Company's 1998 Employee, Director and Consultant Stock Option Plan, 2009 Equity and Long Term Incentive Plan and 2013 Stock Incentive Plan that expire, terminate or are otherwise surrendered, canceled, forfeited or repurchased by the Company at their original issuance price pursuant to a contractual repurchase right plus (3) an annual increase, to be added on the first day of each fiscal year until the expiration of the 2013 Long Term Incentive Plan, equal to the lowest of 2,500,000 shares of common stock, 4% of the number of shares of common stock outstanding on the first day of the fiscal year and an amount determined by the Company's Board of Directors. As of September 30, 2020, awards for 935,092 shares of common stock are available for issuance under the 2013 Long Term 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 up to 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. As of September 30, 2020, awards for 291,025 shares of common stock are available for issuance under the 2020 Inducement Stock Incentive Plan.

From January 1, 2020 through September 30, 2020, the Company issued a total of 2,671,660 stock options to various employees. Of those, 667,285 were inducement grants for non-statutory stock options, all of which were made pursuant to the 2020 Inducement Stock Incentive Plan.

A summary of stock option activity is as follows:

	Number of options	Weighted-average exercise price	Weighted-average remaining contractual term	Aggregate intrinsic value(in thousands)
Outstanding at December 31, 2019	11,043,939	$ 31.67		
Granted	2,671,660	$ 50.76		
Exercised	(1,426,026)	$ 23.14		
Forfeited/Cancelled	(761,332)	$ 42.96		
Outstanding at September 30, 2020	11,528,241	$ 36.37	7.32 years	$ 138,286
Vested or Expected to vest at September 30, 2020	5,297,899	$ 40.09	8.70 years	$ 44,050
Exercisable at September 30, 2020	5,576,283	$ 32.19	5.84 years	$ 90,361

The fair value of grants made in the nine months ended September 30, 2020 was contemporaneously estimated on the date of grant using the following assumptions:

	Nine months ended September 30, 2020
Risk-free interest rate	0.34% - 1.45%
Expected volatility	87.50% - 89.31%
Expected term	5.75 years

The Company assumed no expected dividends for all grants. The weighted average grant date fair value of options granted during the nine month period ended September 30, 2020 was $36.76 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.

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
January 1, 2020	642,419	$ 24.50
Granted	625,390	$ 50.76
Vested	(232,263)	$ 23.86
Forfeited	(73,751)	$ 36.28
Unvested at September 30, 2020	961,795	$ 41.05

Stock Appreciation Rights—SARs entitle the holder to receive, upon exercise, an amount of the Company's common stock or cash (or a combination thereof) determined by reference to appreciation, from and after the date of grant, in the fair market value of a share of the Company's common stock over the measurement price based on the exercise date.

In May 2016, a total of 897,290 SARs were granted to non-executive employees (the "2016 SARs"). The 2016 SARs vested annually in equal installments over four years and were settled in cash on each vest date, which required the Company to remeasure the SARs at each reporting period until vesting occurs. For the nine month period ended September 30, 2020, a total of 132,136 SARs vested. The last payment of the SARs liability was made in the three month period ended March 31, 2020, and accordingly, the balance of the SARS liability as of September 30, 2020 was $0.

Employee Stock Purchase Plan—In June 2016, the Company established an Employee Stock Purchase Plan ("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. The total number of shares available for purchase under the Plan is one 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 month periods ended September 30, 2020, the Company recorded $0.6 million and $1.3 million in compensation expense related to the ESPP.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Research and development	$ 9,220	$ 4,988	$ 25,961	$ 15,191
Selling, general and administrative	7,559	5,496	22,948	15,477
Total	$ 16,779	$ 10,484	$ 48,909	$ 30,668

As of September 30, 2020, there was approximately $173.2 million of total unrecognized compensation cost related to unvested share-based compensation arrangements granted under the 2009 Equity and Long Term Incentive Plan, the 2013 Long Term Incentive Plan and equity awards made pursuant to the Nasdaq Listing Rule 5635(c)(4) inducement grant exception for new hires. This cost is expected to be recognized as share-based compensation expense over the weighted average remaining service period of approximately 2.91 years.

11. 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 will be amortized using the effective interest method over the life of the arrangement, in accordance with the respective guidance. The initial annual effective interest rate was determined to be 11.0%. The Company will utilize the prospective method to account for subsequent changes in the estimated future payments to be made to RPI. 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 three and nine month periods ended September 30, 2020:

Liability for sale of future royalties- (current and noncurrent)	Three Months Ended September 30, 2020	Nine Months Ended September 30, 2020
Beginning balance	$ —	$ —
Proceeds from sale of future royalties	650,000	650,000
Less: Non-cash royalty revenue payable to RPI	(299)	(299)
Plus: Non-cash interest expense recognized	14,557	14,557
Ending balance	$ 664,258	$ 664,258
Effective interest rate for the period	11.0 %	11.0 %

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

2017 Credit Facility

In May 2017, the Company entered into the Credit Agreement, which provided for a senior secured term loan facility of $60.0 million, of which $40.0 million was drawn by the Company on May 5, 2017 (the "Credit Facility"). The Company's ability to draw on the remaining $20.0 million under the senior secured term loan facility expired on December 31, 2018. The Company capitalized approximately $0.4 million of debt issuance costs, which were netted against the carrying value of the Credit Facility and were amortized over the term of the Credit Facility.

Borrowings under the Credit Agreement bore interest at a rate per annum equal to the London Interbank Offered Rate, or LIBOR, (with a LIBOR floor rate of 1.00%) plus 6.15%. The Company was obligated to make interest only payments (payable monthly in arrears) through April 30, 2019. Commencing on May 1, 2019 and continuing for the remaining twenty-four months of the facility, the Company was required to make monthly interest payments and monthly principal payments. The principal payments were made based on straight-line amortization of the principal over the twenty-four month period. The maturity date of the Credit Agreement was May 1, 2021, unless terminated earlier.

On July 1, 2020, the Company terminated the Credit Facility. In connection with the termination of the Credit Facility, the Company repaid outstanding principal of $18.3 million, which was classified as the current portion of long term debt on the consolidated balance sheet and accrued interest of $0.1 million, which was classified within accrued liabilities on the consolidated balance sheet, thereunder totaling $18.4 million. The Company paid an additional $0.6 million in termination

and exit fees, which are included as a component of other income, net in the Company's statement of operations for the three and nine month periods ended September 30, 2020. All liens and security interests securing the term loan made pursuant to the Credit Facility were released upon termination.

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. 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 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 is 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 Company accounts for the 2026 Convertible Notes as a liability and equity component where the carrying value of the liability component will be valued based on a similar instrument. In accounting for the issuance of the 2026 Convertible Notes, the Company separated the 2026 Convertible Notes into liability and equity components. The carrying amount of the liability component was calculated by measuring the fair value of a similar liability that does not have an associated convertible feature. The carrying amount of the equity component representing the conversion option was determined by deducting the fair value of the liability component from the par value of the 2026 Convertible Notes as a whole. The excess of the principal amount of the liability component over its carrying amount, referred to as the debt discount, is amortized to interest expense over the seven-year term of the 2026 Convertible Notes. The equity component is not re-measured as long as it continues to meet the conditions for equity classification. The equity component recorded at issuance related to the 2026 Convertible Notes was $123.0 million and was recorded in additional paid-in capital.

In accounting for the transaction costs related to the issuance of the 2026 Convertible Notes, the Company allocated the total costs incurred to the liability and equity components of the 2026 Convertible Notes based on their relative values. Transaction costs attributable to the liability component are amortized to interest expense over the seven-year term of the 2026 Convertible Notes, and transaction costs attributable to the equity component are netted with the equity components in stockholders' equity. Additionally, the Company initially recorded a net deferred tax liability of $25.3 million in connection with the 2026 Convertible Notes.

The 2026 Convertible Notes consist of the following:

Liability component	September 30, 2020	December 31, 2019
Principal	$ 287,500	$ 287,500
Less: Debt issuance costs	(4,192)	(4,567)
Less: Debt discount, net(1)	(109,563)	(119,350)
Net carrying amount	$ 173,745	$ 163,583

(1) Included in the consolidated balance sheets within convertible senior notes (due 2026) and amortized to interest expense over the remaining life of the 2026 Convertible Notes using the effective interest rate method.

As of September 30, 2020, the remaining contractual life of the 2026 Convertible Notes is approximately 6.0 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,	
	2020	2019	2020	2019
Contractual interest expense	$ 1,081	$ 130	$ 3,223	$ 130
Amortization of debt issuance costs	127	15	374	15
Amortization of debt discount	3,313	391	9,786	391
Total	$ 4,521	$ 536	$ 13,383	$ 536
Effective interest rate of the liability component	10.2 %	10.2 %	10.2 %	10.2 %

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 bear cash interest at a rate of 3.00% per year, payable semi-annually on February 15 and August 15 of each year, beginning on February 15, 2016. The 2022 Convertible Notes will mature on August 15, 2022, unless earlier repurchased or converted. The net proceeds to the Company from the offering were $145.4 million after deducting the initial purchasers' discounts and commissions and the offering expenses payable by the Company.

The 2022 Convertible Notes are governed by an indenture (the "2022 Convertible Notes Indenture") with U.S Bank National Association as trustee (the "2022 Convertible Notes Trustee").

Holders may convert their 2022 Convertible Notes at their option at any time prior to the close of business on the business day immediately preceding February 15, 2022 only under the following circumstances:

- during any calendar quarter commencing on or after September 30, 2015 (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 2022 Convertible Notes Indenture) per $1,000 principal amount of 2022 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 February 15, 2022, until the close of business on the business day immediately preceding the maturity date, holders may convert their 2022 Convertible Notes at any time, regardless of the foregoing circumstances. Upon conversion, the Company will pay cash up to the aggregate principal amount of the 2022 Convertible Notes to be converted and deliver shares of its common stock in respect of the remainder, if any, of its conversion obligation in excess of the aggregate principal amount of 2022 Convertible Notes being converted.

The conversion rate for the 2022 Convertible Notes was initially, and remains, 17.7487 shares of the Company's common stock per $1,000 principal amount of the 2022 Convertible Notes, which is equivalent to an initial conversion price of approximately $56.34 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 2022 Convertible Notes prior to August 20, 2018. As of August 20, 2018, the Company may redeem for cash all or any portion of the 2022 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 2022 Convertible Notes to be redeemed, plus accrued and unpaid interest to, but excluding, the redemption date. No sinking fund is provided for the 2022 Convertible Notes, which means that the Company is not required to redeem or retire the 2022 Convertible Notes periodically. There have been no redemptions to date.

If the Company undergoes a “fundamental change” (as defined in the 2022 Convertible Notes Indenture), subject to certain conditions, holders of the 2022 Convertible Notes may require the Company to repurchase for cash all or part of their 2022 Convertible Notes at a repurchase price equal to 100% of the principal amount of the 2022 Convertible Notes to be repurchased, plus accrued and unpaid interest to, but excluding, the fundamental change repurchase date.

The 2022 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 2022 Convertible Notes Indenture contains customary events of default with respect to the 2022 Convertible Notes, including that upon certain events of default (including the Company’s failure to make any payment of principal or interest on the 2022 Convertible Notes when due and payable) occurring and continuing, the 2022 Convertible Notes Trustee by notice to the Company, or the holders of at least 25% in principal amount of the outstanding 2022 Convertible Notes by notice to the Company and the Convertible Notes Trustee, may, and the 2022 Convertible Notes Trustee at the request of such holders (subject to the provisions of the 2022 Convertible Notes Indenture) shall, declare 100% of the principal of and accrued and unpaid interest, if any, on all the 2022 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 2022 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 Company accounts for the 2022 Convertible Notes as a liability and equity component where the carrying value of the liability component will be valued based on a similar instrument. In accounting for the issuance of the 2022 Convertible Notes, the Company separated the 2022 Convertible Notes into liability and equity components. The carrying amount of the liability component was calculated by measuring the fair value of a similar liability that does not have an associated convertible feature. The carrying amount of the equity component representing the conversion option was determined by deducting the fair value of the liability component from the par value of the 2022 Convertible Notes as a whole. The excess of the principal amount of the liability component over its carrying amount, referred to as the debt discount, is amortized to interest expense over the seven-year term of the 2022 Convertible Notes. The equity component is not re-measured as long as it continues to meet the conditions for equity classification. The equity component recorded at issuance related to the 2022 Convertible Notes was $57.5 million and was recorded in additional paid-in capital.

In accounting for the transaction costs related to the issuance of the 2022 Convertible Notes, the Company allocated the total costs incurred to the liability and equity components of the 2022 Convertible Notes based on their relative values. Transaction costs attributable to the liability component are amortized to interest expense over the seven-year term of the 2022 Convertible Notes, and transaction costs attributable to the equity component are netted with the equity components in stockholders’ equity. Additionally, the Company initially recorded a net deferred tax liability of $22.3 million in connection with the 2022 Convertible Notes.

The 2022 Convertible Notes consist of the following:

Liability component	September 30, 2020	December 31, 2019
Principal	$ 150,000	$ 150,000
Less: Debt issuance costs	(986)	(1,329)
Less: Debt discount, net (1)	(19,794)	(26,686)
Net carrying amount	$ 129,220	$ 121,985

(1) Included in the consolidated balance sheets within convertible senior notes (due 2022) and amortized to interest expense over the remaining life of the 2022 Convertible Notes using the effective interest rate method.

As of September 30, 2020, the remaining contractual life of the 2022 Convertible Notes is approximately 1.9 years.

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,	
	2020	2019	2020	2019
Contractual interest expense	$ 1,131	$ 1,134	$ 3,375	$ 3,375
Amortization of debt issuance costs	118	106	343	308
Amortization of debt discount	2,372	2,137	6,892	6,192
Total	$ 3,621	$ 3,377	$ 10,610	$ 9,875
Effective interest rate of the liability component	11.0 %	11.0 %	11.0 %	11.0 %

12. 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's first such milestone payment of $0.8 million payable to Wellcome Trust occurred in the second quarter of 2016. Additional milestone payments of up to an aggregate of $22.4 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 may become 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, if the Company outlicenses rights to a collaboration product, a specified percentage of certain payments the Company receives from its licensee. The Company is not obligated to make such payments unless and until annual sales of a collaboration product exceed a designated threshold. The Company's obligation to make such payments would end upon the Company's payment to the SMA Foundation of a specified amount.

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 has the opportunity to receive a single $50.0 million sales-based milestone.

Pursuant to the Merger Agreement with Agilis, 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 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 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 Merger, regardless of whether the milestones are achieved.

The Rights Exchange Agreement has no effect on the 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 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 in the three month period ended September 30, 2020 upon the passing of the second anniversary of the closing of the 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 Merger Agreement.

Subject to the terms and conditions of the Asset Acquisition 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 Asset Acquisition 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 Censa Merger Agreement, 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 PTC923'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 PTC923, (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 PTC923, on a country-by-country basis, which contingent payment shall equal to a mid-double digit percentage of any such sublicense fees. Pursuant to the Censa Merger Agreement, the Company has the option to pay the initial $30.0 million development milestone, for the completion of enrollment of a Phase 3 clinical trial for PTC923 for PKU, if achieved, in cash or shares of the Company's common stock.

The Company also has a Collaboration and License Agreement with Akcea for the commercialization of Tegsedi and Waylivra, and products containing those compounds in countries in Latin America and the Caribbean (the "Akcea

Collaboration and License Agreement"). Pursuant to the Akcea Collaboration and License Agreement, the Company paid Akcea an upfront licensing fee, which included an initial payment of $12.0 million. In 2019, a $6.0 million milestone was paid upon receipt of regulatory approval of Waylivra from the EMA and a $4.0 million milestone was paid upon regulatory approval of Tegsedi from ANVISA, the Brazilian health regulatory authority, upon receipt of regulatory approval for Waylivra from ANVISA. In addition, Akcea is eligible to receive an additional milestone payment of $4.0 million upon receipt of regulatory approval for Waylivra from ANVISA. Akcea is also entitled to receive royalty payments subject to certain terms set forth in the Akcea Collaboration and License 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 and Emflaza product net sales, 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, including as a result of patients seeking to participate in the Company's clinical trials or otherwise gain access to its product candidates. The Company is not currently aware of any material legal proceedings against it.

13. 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, 2020 and 2019, net product sales in the United States were $38.5 million and $22.9 million, respectively, consisting solely of Emflaza, and net product sales not in the United States were $44.2 million and $48.5 million, respectively, consisting of Translarna, Tegsedi, and Waylivra. For the three months ended September 30, 2020 and 2019, the Company had a total of two and two distributors, respectively, that each accounted for over 10% of the Company's net product sales.

During the nine months ended September 30, 2020 and 2019, net product sales in the United States were $102.1 million and $68.3 million, respectively, consisting solely of Emflaza, and net product sales not in the United States were $124.0 million and $141.6 million, respectively, consisting of Translarna, Tegsedi, and Waylivra. For the nine months ended September 30, 2020 and 2019, the Company had a total of two and two distributors, respectively, that each accounted for over 10% of the Company's net product sales.

The Company's contract liabilities balances as of September 30, 2020 and as of December 31, 2019 were $6.0 million and $11.7 million, respectively. The Company did not have any contract assets as of September 30, 2020 and as of December 31, 2019. During the three and nine month periods ended September 30, 2020, the Company recognized $2.0 million and $6.0 million of revenue, respectively, related to the amounts included in the contract liability balance at the beginning of the period. For the three and nine month periods ended September 30, 2019, the Company recognized $2.3 million and $2.3 million, respectively, relating to the contract liability balance at the beginning of the period. The Company has not made significant changes to the judgments made in applying ASC Topic 606 for the three and nine month periods ending September 30, 2020 and 2019.

Remaining performance obligations

Remaining performance obligations represent the transaction price for goods the Company has yet to provide. As of September 30, 2020 and December 31, 2019, the aggregate amount of the transaction price allocated to the remaining performance obligations relating to Translarna net product revenue was $6.0 million and $11.7 million, respectively. The Company expects to recognize revenue within 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.

Collaboration and Royalty revenue

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

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™ (risdiplam), which was approved in August 2020 by the FDA for the treatment of SMA in adults and children two months and older. The first commercial sale of Evrysdi in the United States was made in August 2020. This event triggered a $20.0 million milestone payment to the Company from Roche. In August 2020, the EMA accepted the MAA filed by Roche for Evrysdi for the treatment of SMA, which triggered a $15.0 million milestone payment to the Company from Roche. As of September 30, 2020, the remaining potential research and development event milestones that can be received is $37.5 million, and the remaining potential sales milestones that can be received is $325.0 million.

For the three months ended September 30, 2020 and 2019, the Company recognized $35.0 million and $0.1 million of revenue, respectively, related to the licensing and collaboration agreement with Roche. For the nine months ended September 30, 2020 and 2019, the Company recognized revenue related to the licensing and collaboration agreement with Roche of $35.1 million and $0.1 million, respectively.

In addition to research and development and sales milestones, the Company is eligible to receive double-digit royalties on worldwide annual net sales of a commercial product under the License Agreement. For the three and nine months ended September 30, 2020, the Company has recognized $0.7 million of royalty revenue related to Evrysdi.

14. Intangible assets and goodwill

Definite-lived intangibles

On April 20, 2017, the Company completed its previously announced acquisition of all rights to Emflaza pursuant to the Asset Purchase Agreement, dated March 15, 2017, and amended on April 20, 2017, by and between the Company and Marathon. The assets acquired by the Company in the transaction include intellectual property rights related to Emflaza, inventories of Emflaza, and certain contractual rights related to Emflaza. In accordance with ASU 2017-01, the Company determined that substantially all of the fair value is concentrated in the Emflaza rights intangible asset and as such accounted for the transaction as an asset acquisition under ASC 805-50 and recorded an intangible asset of $148.4 million, which is being amortized to cost of product sales over its expected useful life of approximately seven years on a straight line basis.

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. As of September 30, 2020 and 2019, milestone payments of $13.8 million and $7.4 million, respectively, were recorded and are included on the balance sheet within accounts payable and accrued expenses. These payments are being amortized over the remaining useful life of the Emflaza rights asset on a straight line basis.

Pursuant to the Akcea Collaboration and License Agreement, in May 2019 the Company made a $6.0 million milestone payment to Akcea upon regulatory approval of Waylivra from the EMA. The payment was recorded as an intangible asset and is being amortized to cost of product sales over its expected useful life of approximately ten years on a straight line basis. Additionally, in December 2019, the Company made a $4.0 million milestone payment to Akcea upon regulatory approval of Tegsedi from ANVISA. The payment was recorded as an intangible asset and is being amortized to cost of product sales over its expected useful life of approximately ten years on a straight line basis.

Akcea is also entitled to receive royalty payments subject to certain terms set forth in the Akcea Collaboration and License Agreement related to sales of Waylivra. In accordance with the guidance for an asset acquisition, the Company will record royalty payments when they become payable to Akcea and increase the cost basis for the Waylivra intangible asset.

For the three months ended September 30, 2020 and 2019, the Company recognized amortization expense of $9.6 million and $7.0 million, respectively, related to the Emflaza rights, Waylivra, and Tegsedi intangible assets. For the nine months ended September 30, 2020 and 2019, the Company recognized amortization expense of $26.3 million and $19.7 million, respectively. The estimated future amortization of the Emflaza rights, Waylivra, and Tegsedi intangible assets is expected to be as follows:

	As of September 30, 2020
2020	$ 9,627
2021	38,506
2022	38,506
2023	38,506
2024 and thereafter	10,891
Total	$ 136,036

The weighted average remaining amortization period of the definite-lived intangibles as of September 30, 2020 is 3.7 years.

Indefinite-lived intangibles

In connection with the acquisition of the Company's gene therapy platform from Agilis, the Company acquired rights to PTC-AADC, 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 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 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 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. The value allocated to the indefinite lived intangible assets was $576.5 million. There have been no changes to the balance of the indefinite-lived intangibles since the Merger.

Goodwill

As a result of the Merger on August 23, 2018, the Company recorded $82.3 million of goodwill. There were no changes to the recorded value of goodwill for the three and nine month periods ended September 30, 2020.

15. Subsequent events

In October 2020, Chugai Pharmaceutical Co., Ltd., a subsidiary of Roche, filed an NDA in Japan for Evrysdi for the treatment of SMA, which triggered a $7.5 million milestone payable to the Company from Roche.

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

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, 2019 included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 2, 2020, or our 2019 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 2019 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 commercialize products is the foundation that drives our continued investment in a robust diversified pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need. Our strategy is to bring best-in-class therapies with differentiated clinical benefit to patients affected by rare disorders and to leverage our global commercial infrastructure to maximize value for our patients and other stakeholders. We have a portfolio pipeline that includes commercial products as well as 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, including rare diseases and oncology.

Corporate Updates

COVID-19 Impact

The global pandemic caused by a strain of novel coronavirus, COVID-19, has impacted and is continuing to impact the timing of certain of our clinical trials and regulatory submissions as well as other aspects of our business operations. In addition to our previous disclosures regarding the impact of the COVID-19 pandemic, such as those set forth in our Quarterly Report on Form 10-Q for the period ended March 31, 2020 and our Quarterly Report on Form 10-Q for the period ended June 30, 2020, the following expectations have been revised as a result of the impact or expected impact of the COVID-19 pandemic:

- As previously disclosed, our expected completion of Study 045 was delayed as certain patients were unable to have the final study muscle biopsies performed at our clinical trial site at the University of California, Los Angeles as a result of the COVID-19 pandemic. The final study muscle biopsies were completed in October 2020 and we have begun the process of analyzing the samples according to our protocol. We expect to report top-line results from Study 045 in the first quarter of 2021 followed by a potential resubmission of the New Drug Application, or NDA, for Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, thereafter.

- As previously disclosed, certain of the third-party development and manufacturing organizations that we contract with for analytical testing have prioritized materials and testing kits to support SARS-CoV-2 testing, diverted employees to support COVID-19 related programs and reduced their workforce to comply with social distancing requirements imposed in connection with the COVID-19 pandemic. As a result of this shift in resources, we experienced a delay in generating analytical data needed to respond to questions sent by the European Medicines Agency, or EMA, regarding our marketing authorization application, or MAA, for PTC-AADC for the treatment of Aromatic L-Amino Acid Decarboxylase, or AADC, deficiency in the European Economic Area, or EEA, and we have been granted a clock stop extension to respond to the EMA's questions. We currently expect an opinion from the Committee for Medicinal Products for Human Use, or CHMP, in the first half of 2021.

- As previously disclosed, in response to discussions with the United States Food and Drug Administration, or the FDA, we intend to provide additional information concerning the use of the commercial cannula for PTC-AADC in young patients. However, due to hospitals generally canceling elective surgeries in response to the COVID-19 pandemic and other administrative delays resulting from the COVID-19 pandemic, we have been delayed in our ability to gather such information. We now anticipate submitting a biologics license application, or BLA, for PTC-AADC for the treatment of Aromatic L-Amino Acid Decarboxylase, or AADC, deficiency in the United States in the first half of 2021.

- As a result of the COVID-19 pandemic, the Brazilian Ministry of Health is continuing to experience significant delays processing centralized group purchase orders. Almost all of our Brazilian product revenue for Translarna is attributable to such purchase orders. These centralized group purchase order delays have caused, and may continue to cause, fluctuations in our ability to generate revenue in Brazil.

- To date, except as otherwise disclosed with respect to Brazil, our ability to generate revenue has not been significantly affected by the COVID-19 pandemic. However, due to travel restrictions, social distancing and the continued global uncertainty resulting from the COVID-19 pandemic, we may have difficulty identifying and accessing new patients, supporting existing patients and meeting with regulatory authorities or other governmental entities, which may negatively affect our future revenue. We continue to remotely connect with our existing patient base and have not encountered any material issues in supplying those patients.

- As previously disclosed, in response to the global uncertainty caused by the COVID-19 pandemic, we are continuing to prioritize our expenses where we deem appropriate and strategically positioning our capital allocation.

The COVID-19 pandemic and responsive measures thereto may result in further negative impacts, including additional delays in our clinical and regulatory activities and further fluctuations in our revenue. 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 are, and may continue to, 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 has disrupted, and may continue to disrupt, our operations and may significantly impact our operating results. In addition, this pandemic has caused substantial disruption in the financial markets and economies, which could result in adverse effects on our business and operations.”

FDA Approval of Evrysdi

In August 2020, the FDA approved Evrysdi for the treatment of spinal muscular atrophy, or SMA, in adults and children two months and older. The first commercial sale of Evrysdi in the United States was made in August 2020. This event triggered a $20.0 million milestone payment to us from F. Hoffman-La Roche Ltd., Hoffman-La Roche Inc., or, together with F. Hoffman-La Roche Ltd, Roche, pursuant to the License and Collaboration Agreement, or the License Agreement, dated as of November 23, 2011, by and among us, Roche and, for the limited purposes set forth therein, the Spinal Muscular Atrophy Foundation, or the SMA Foundation, under our SMA program. In August 2020, the EMA accepted the MAA filed by Roche for Evrysdi for the treatment of SMA, which triggered a $15.0 million milestone payment to the Company from Roche. Additionally, in October 2020, Chugai Pharmaceutical Co., Ltd., or Chugai, a subsidiary of Roche, filed an NDA in Japan for Evrysdi for the treatment of SMA, which triggered a $7.5 million milestone payment to the Company from Roche.

Monetization of a Portion of Risdiplam Royalty Stream

On July 17, 2020, we, RPI 2019 Intermediate Finance Trust, or RPI, and, for the limited purposes set forth in the agreement, Royalty Pharma PLC, entered into a Royalty Purchase Agreement, or the Royalty Purchase Agreement. Pursuant to the Royalty Purchase Agreement, we sold to RPI 42.933%, or the Assigned Royalty Payment, of our right to receive sales-based royalty payments, or Royalty, on worldwide net sales of Evrysdi and any other product developed pursuant to the

License Agreement. In consideration for the sale of the Assigned Royalty Payments, RPI paid us $650.0 million in cash consideration. We have retained a 57.067% interest in the Royalty and all economic rights to receive the remaining potential regulatory and sales milestone payments under the License Agreement, which milestone payments equal to $362.5 million in the aggregate as of September 30, 2020. 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 License Agreement and the date on which RPI has received $1.3 billion in respect of the Assigned Royalty Payments.

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 EEA for the treatment of nmDMD in ambulatory patients aged two years and older and in Brazil for the treatment of nmDMD in ambulatory patients aged five years and older. In July 2020, the European Commission 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, 2020, we recognized $43.4 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, 2020, we recognized $38.5 million in net sales from Emflaza.

Our marketing authorization for Translarna in the EEA is subject to annual review and renewal by the European Commission following reassessment by the EMA of the benefit-risk balance of the authorization, which we refer to as the annual EMA reassessment. In June 2020, the European Commission renewed our marketing authorization, making it effective, unless extended, through August 5, 2021. This 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. The final report on the trial and open-label extension is to be submitted by us to the EMA by the end of the third quarter of 2022.

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 challenges in any particular country and can also be affected by political, economic and regulatory developments in such country.

There is substantial risk that if we are unable to renew our EEA marketing authorization during any annual renewal cycle, or if our product label is materially restricted, or if 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.

Translarna is an investigational new drug in the United States. During the first quarter of 2017, we filed an NDA for Translarna for the treatment of nmDMD over protest with the 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 intend to follow the FDA’s recommendation and will collect, using newer technologies via procedures and methods that we designed, such dystrophin data in a new study, Study 045, which we initiated in the fourth quarter of 2018. The final study muscle biopsies for the patients in Study 045 were completed in October 2020 and we have begun the process of analyzing the samples according

to our protocol. We expect to report top-line results from Study 045 in the first quarter of 2021 followed by a potential resubmission of the NDA for Translarna for the treatment of nmDMD thereafter. Additionally, should a re-submission of an NDA receive accelerated approval, the Office of New Drugs stated that Study 041, which is currently enrolling, could serve as the confirmatory post-approval trial required in connection with the accelerated approval framework.

There is substantial risk that Study 045, or any other studies we may use to collect the dystrophin data, will not provide the necessary data to support a marketing approval for Translarna for the treatment of nmDMD in the U.S.

LATAM Commercialization

We hold 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 our Collaboration and License Agreement with Akcea Therapeutics, Inc., or Akcea. Tegsedi has received marketing authorization in the United States, EU and Brazil for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis, or hATTR amyloidosis. Waylivra has received marketing authorization in the European Union, or EU, for the treatment of familial chylomicronemia syndrome, or FCS. We filed for marketing authorization for Waylivra for the treatment of FCS with ANVISA, the Brazilian health regulatory authority, in June 2020 and, subject to potential delays in the review process related to the COVID-19 pandemic, expect a regulatory decision on approval from ANVISA in 2021.

Diversified Development Pipeline

Gene Therapy Platform

We have a pipeline of gene therapy product candidates for rare monogenic diseases that affect the central nervous system, or CNS, including PTC-AADC for the treatment of AADC deficiency, a rare CNS disorder arising from reductions in the enzyme AADC that result from mutations in the dopa decarboxylase gene. We are preparing a BLA for PTC-AADC for the treatment of AADC deficiency in the United States and we anticipate submitting a BLA to the FDA in the first half of 2021. In January 2020, we submitted an MAA for PTC-AADC for the treatment of AADC deficiency in the EEA to the EMA and we expect an opinion from the CHMP in the first half of 2021.

Splicing Platform

We also have an SMA collaboration with Roche and the Spinal Muscular Atrophy Foundation, or SMA Foundation. The SMA program has one approved product, Evrysdi™ (risdiplam), which was approved in August 2020 by the FDA for the treatment of SMA in adults and children two months and older. Evrysdi also received marketing authorization for the treatment of SMA in Brazil in October 2020 and the EMA accepted the MAA filed by Roche for Evrysdi for the treatment of SMA in August 2020.

Bio-e Platform

In 2019, we acquired substantially all of the assets of BioElectron Technology Corporation, or BioElectron, including certain compounds that we have begun to develop as part of our Bio-e platform, pursuant to an asset purchase agreement by and between us and BioElectron, dated October 1, 2019. The two most advanced molecules in our Bio-e platform are vatiquinone and PTC857. We initiated a registration-directed Phase 2/3 placebo-controlled trial of vatiquinone in approximately 60 children with mitochondrial disease and associated refractory epilepsy in the third quarter of 2020. We expect to initiate a registration-directed Phase 3 trial of vatiquinone in approximately 100 patients with Friedrich ataxia in the fourth quarter of 2020. In the second quarter of 2020, we initiated a Phase 1 trial in healthy volunteers to evaluate the safety and pharmacology of PTC857, which we believe has the potential to address multiple CNS disorders with glucocerebrosidase Parkinson’s disease as our first planned indication. We completed dosing within the single ascending dose study and expect to complete dosing within the multiple ascending dose study from the Phase 1 trial by the end of 2020.

PTC299 for COVID-19

In June 2020, the FDA authorized the initiation of a Phase 2/3 clinical trial evaluating PTC299, a dihydroorotate dehydrogenase inhibitor that we have also been developing in oncological indications, as a potential treatment for COVID-19. The integrated Phase 2/3 study, which has been initiated and is being conducted in two stages, will evaluate the efficacy and safety of PTC299 in patients hospitalized with SARS-CoV-2 infection. We expect Stage 1 of the clinical trial to be completed in the fourth quarter of 2020 and we anticipate reporting top-line results from both stages in the first half of 2021.

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.

We expect to initiate a Phase 3 trial for PTC923, an oral formulation of synthetic sepiapterin, a precursor to intracellular tetrahydrobiopterin, which is a critical enzymatic cofactor involved in metabolism and synthesis of numerous metabolic products, for phenylketonuria, or PKU, in mid-2021.

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.

To date, we have financed our operations primarily through our offering of 3.00% convertible senior notes due August 15, 2022, or the 2022 Convertible Notes, our offering of 1.50% convertible senior notes due September 15, 2026, or the 2026 Convertible Notes, and, together with the 2022 Convertible Notes, the 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 Royalty Purchase Agreement, private placements of our preferred stock, collaborations, bank debt and convertible debt financings, our credit and security agreement, or the Credit Agreement, with MidCap Financial Trust, or MidCap Financial, as administrative agent and MidCap Financial and other certain institutions as lenders thereto, and grants and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease areas addressed by our product candidates. Since 2014, we have also relied on revenue generated from net sales of Translarna for the treatment of nmDMD in territories outside of the United States, and since May 2017, we have generated revenue from net sales of Emflaza for the treatment of DMD in the United States.

The 2022 Convertible Notes consist of $150.0 million in aggregate principal amount of 3.00% convertible senior notes due 2022. The 2022 Convertible Notes bear cash interest payable on February 15 and August 15 of each year, beginning on February 15, 2016. The 2022 Convertible Notes are senior unsecured obligations of ours and will mature on August 15, 2022, unless earlier converted, redeemed or repurchased in accordance with their terms prior to such date. We received net proceeds from the offering of approximately $145.4 million, after deducting the initial purchasers’ discounts and commissions and the estimated 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 twelve month period ending December 31, 2019, we issued and sold an aggregate of 63,926 shares of common stock pursuant to the Sales Agreement at a weighted average public offering price of $46.60 per share. We received net proceeds of $2.6 million after deducting agent discounts and

commissions and other offering expenses payable by us. During the nine month period ending September 30, 2020, we issued and sold an aggregate of 542,470 shares of common stock pursuant to the Sales Agreement at a weighted average public offering price of $53.37 per share. We received net proceeds of $28.1 million after deducting underwriting discounts and commissions and other offering expenses payable by us.

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.

As of September 30, 2020, we had an accumulated deficit of $1,554.5 million. We had a net loss of $363.8 million and $173.9 million for the nine month periods ended September 30, 2020 and 2019, 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, distribution and manufacturing, including expanding our direct manufacturing capabilities at our leased biologics manufacturing facility and administrative and employee-based 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 gene therapy, splicing, Bio-e and oncology programs, our studies of PTC923 for PKU and our studies of PTC299 for COVID-19 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. We continue to seek marketing authorization for Translarna for the treatment of nmDMD in territories that we do not currently have marketing authorization in and we may also seek marketing authorization for Translarna for other indications. We submitted an MAA to the EMA for the treatment of AADC deficiency with PTC-AADC in the EEA. We are also preparing a BLA for PTC-AADC for the treatment of AADC deficiency in the United States and we anticipate submitting a BLA to the FDA in the first half of 2021. We filed for marketing authorization for Waylivra with ANVISA in June 2020. 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.

With respect to our outstanding 2022 Convertible Notes, cash interest payments are payable on a semi-annual basis in arrears, which require total funding of $4.5 million annually. 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. Prior to its termination, we made cash payments on a monthly basis in arrears under our Credit Agreement. On April 29, 2020, pursuant to the Rights Exchange Agreement by and among us, the Rightholders set forth therein, or the Participating Rightholders, and, for the limited purposes set forth therein, Shareholder Representative Services LLC, dated as of April 29, 2020, or the Rights Exchange Agreement, we paid $36.9 million as partial consideration to the Participating Rightholders in exchange for the cancellation and forfeiture by the Participating Rightholders of their rights to receive certain milestone-based contingent payments under the Agreement and Plan of Merger, dated as of July 19, 2018 by and among us, Agility Merger Sub, Inc. and, solely in its capacity as the representative, agent and attorney-in-fact of the equityholders of Agilis Biotherapeutics, Inc., Shareholder Representative Services LLC, or the Merger Agreement. Upon the passing of the second anniversary of the closing of the Agilis acquisition, August 23, 2020, we paid an additional $2.4 million in development milestone payments as set forth in the Merger Agreement. In addition, Akcea is eligible to receive from us an additional milestone payment of $4.0 million upon receipt of regulatory approval for Waylivra from ANVISA, the determination for which we expect to potentially occur, subject to potential delays in the review process related to the COVID-19 pandemic, in 2021. 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, 2020 and 2019, net product sales outside of the United States were $44.2 million and $48.5 million, respectively, and net product sales in the United States were $38.5 million and $22.9 million, respectively. For the nine months ended September 30, 2020 and 2019, net product sales outside of the United States were $124.0 million and $141.6 million, respectively, and net product sales in the United States were $102.1 million and $68.3 million, respectively.

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 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, 2020, we had recognized a total of $97.5 million in milestone payments and $0.7 million royalties on net sales pursuant to the License Agreement. As of September 30, 2020, the remaining potential research and development event milestones that can be received is $37.5 million. The remaining potential sales milestones as of September 30, 2020 is $325.0 million upon achievement of certain sales events. Additionally, in October 2020, Chugai filed an NDA in Japan for Evrysdi for the treatment of SMA, which triggered a $7.5 million milestone payment to us from Roche.

Pursuant to the Royalty Purchase Agreement, we sold to RPI the Assigned Royalty Payment, in consideration for $650.0 million. We have retained a 57.067% interest in the Royalty and all economic rights to receive the remaining potential regulatory and sales milestone payments under the License Agreement. 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 License Agreement and the date on which RPI has received $1.3 billion in respect of the Assigned Royalty Payments.

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 gene therapy, splicing, Bio-e and oncology programs, our studies of PTC923 for PKU, our studies of PTC299 for COVID-19, our studies of Translarna for additional indications, and performance of our FDA post-marketing requirements with respect to Emflaza in the United States. The timing and amount of these expenses will depend upon the outcome of our ongoing clinical trials and the costs associated with our planned clinical trials. The timing and amount of these expenses will also depend on the costs associated with potential future clinical trials of our products or product candidates and the related expansion of our research and development organization, regulatory requirements, advancement of our preclinical programs, and product and product candidate manufacturing costs.

The following tables provide research and development expense for our most advanced principal product development programs, for the three and nine months ended September 30, 2020 and 2019.

	Three Months Ended September 30,	
	2020	2019
	(in thousands)	
Translarna (nmDMD, aniridia and Dravet)	$ 15,189	$ 25,239
PTC923	2,093	—
Gene Therapy	32,975	17,016
Bio-e	9,726	—
Oncology	4,223	3,944
Emflaza	2,902	4,760
Other research and preclinical	25,890	12,117
Total research and development	$ 92,998	$ 63,076

	Nine Months Ended September 30,	
	2020	2019
	(in thousands)	
Translarna (nmDMD, aniridia and Dravet)	$ 73,466	$ 53,354
PTC923	55,937	—
Gene Therapy	138,391	44,418
Bio-e	24,156	—
Oncology	14,943	17,769
Emflaza	12,772	19,253
Other research and preclinical	39,965	40,827
Total research and development	$ 359,630	$ 175,621

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 and to obtain and maintain marketing authorizations we currently have or may receive in the future for our products and product candidates;
- 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, is causing 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 Royalty Purchase Agreement, the Convertible Notes outstanding, and from the 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, 2020, there were no material changes to our critical accounting policies as reported in our 2019 Annual Report on Form 10-K.

Results of operations

Three months ended September 30, 2020 compared to three months ended September 30, 2019

The following table summarizes revenues and selected expense and other income data for the three months ended September 30, 2020 and 2019.

(in thousands)	Three Months Ended September 30, 2020	2019	Change 2020 vs. 2019
Net product revenue	$ 82,708	$ 71,369	$ 11,339
Collaboration and grant revenue	35,000	47	34,953
Royalty revenue	696	—	696
Cost of product sales, excluding amortization of acquired intangible asset	4,667	3,006	1,661
Amortization of acquired intangible asset	9,630	7,025	2,605
Research and development expense	92,998	63,076	29,922
Selling, general and administrative expense	57,840	49,284	8,556
Change in the fair value of deferred and contingent consideration	8,400	9,500	(1,100)
Interest expense, net	(21,039)	(2,666)	(18,373)
Other income, net	28,766	2,800	25,966
Income tax (expense) benefit	(22,288)	344	(22,632)

Net product revenues. Net product revenues were $82.7 million for the three months ended September 30, 2020, an increase of $11.3 million, or 16%, from $71.4 million for the three months ended September 30, 2019. The increase in net product revenue was primarily due to an increase in net product sales of Emflaza due to new patient prescriptions and continued operational improvements and efficiencies in our commercial business. This increase was partially offset by a decrease in net product sales of Translarna primarily related to a delay in the anticipated timing of a group purchase order from Brazil. Due to the impact of the COVID-19 pandemic, there was an administrative delay by the Brazilian Ministry of Health in receiving the centralized Translarna group purchase order. The third quarter of 2019 included a material group purchase order from Brazil.

Collaboration and grant revenues. Collaboration and grant revenues were $35.0 million for the three months ended September 30, 2020, an increase of $34.9 million, or over 100%, from $0.1 million for the three months ended September 30, 2019. The increase is related to two milestones that were triggered from Roche in the three months ended September 30, 2020. In August 2020, the FDA approved Evrysdi for the treatment of SMA in adults and children two months and

older. The first commercial sale of Evrysdi in the United States was made in August 2020. This event triggered a $20.0 million milestone payment to us from Roche. In August 2020, the EMA accepted the MAA filed by Roche for Evrysdi for the treatment of SMA, which triggered a $15.0 million milestone payment to us from Roche. No milestones were triggered in the three months ended September 30, 2019. Revenues in the three months ended September 30, 2019 were related to our ongoing collaboration agreements.

Royalty revenue. Royalty revenue was $0.7 million for the three months ended September 30, 2020, an increase of $0.7 million, or 100%, from $0.0 million for the three months ended September 30, 2019. The increase in royalty revenue was due to the FDA approval of Evrysdi in August 2020. In accordance with our License Agreement with Roche, we are entitled to royalties on worldwide annual net sales of the product.

Cost of product sales, excluding amortization of acquired intangible asset. Cost of product sales, excluding amortization of acquired intangible asset, were $4.7 million for the three months ended September 30, 2020, an increase of $1.7 million, or 55%, from $3.0 million for the three months ended September 30, 2019. Cost of product sales consist primarily of royalty payments associated with Emflaza and Translarna net product sales, excluding contingent payments to Marathon Pharmaceuticals, LLC (now known as Complete Pharma Holdings, LLC), or Marathon, and costs associated with Emflaza and Translarna product sold during the period. The increase in cost of product sales, excluding amortization of acquired intangible asset, is primarily due to the increase in net product revenue.

Amortization of acquired intangible asset. Amortization of our intangible assets was $9.6 million for the three months ended September 30, 2020, an increase of $2.6 million, or 37%, from $7.0 million for the three months ended September 30, 2019. These amounts are related to the acquisition of all rights to Emflaza acquired in May 2017, Marathon contingent payments, and our Waylivra and Tegsedi intangible assets. The increase is primarily related to additional Marathon contingent payments. The amount allocated to the Emflaza intangible asset is amortized on a straight-line basis over its estimated useful life of approximately seven years from the date of the completion of the acquisition of all rights to Emflaza, the period of estimated future cash flows. The Marathon contingent payments are amortized prospectively as incurred, straight-line, over the remaining useful life of the Emflaza intangible asset. The Waylivra and Tegsedi assets are amortized on a straight-line basis over their estimated useful life of approximately ten years, respectively.

Research and development expense. Research and development expense was $93.0 million for the three months ended September 30, 2020, an increase of $29.9 million, or 47%, from $63.1 million for the three months ended September 30, 2019. The increase reflects additional costs associated with advancing the gene therapy and Bio-e platforms and increased investment in research programs as well as advancement of the clinical pipeline.

Selling, general and administrative expense. Selling, general and administrative expense was $57.8 million for the three months ended September 30, 2020, an increase of $8.6 million, or 17%, from $49.3 million for the three months ended September 30, 2019. The increase was primarily due to continued investment to support our commercial activities including our expanding commercial portfolio.

Change in the fair value of deferred and contingent consideration. The change in the fair value of deferred and contingent consideration was $8.4 million for the three months ended September 30, 2020, a decrease of $1.1 million, or 12%, from $9.5 million for the three months ended September 30, 2019. 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 $21.0 million for the three months ended September 30, 2020, an increase of $18.4 million, or over 100%, from $2.7 million for the three months ended September 30, 2019. 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 Royalty Purchase Agreement, interest expense recorded from the 2022 and 2026 Convertible Notes and the Credit Agreement, partially offset by interest income from our investments.

Other income, net. Other income, net was $28.8 million for the three months ended September 30, 2020, an increase of $26.0 million, or over 100%, from other income of $2.8 million for the three months ended September 30, 2019. The

increase in other income, net resulted primarily from an unrealized foreign exchange gain from the remeasurement of our intercompany loan and unrealized gains on our equity investment and convertible debt security in ClearPoint Neuro, Inc. of $2.5 million and $1.4 million, respectively.

Income tax expense. Income tax expense was $22.3 million for the three months ended September 30, 2020, an increase of $22.6 million, or over 100%, compared to income tax benefit of $0.3 million for the three months ended September 30, 2019. We recorded a state income tax provision in the three months ended September 30, 2020, which is attributable to the taxable income from the sale of our right to receive sales-based royalty payments on Roche's worldwide net sales of Evrysdi. We also incurred income tax expense 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, 2020 compared to nine months ended September 30, 2019

The following table summarizes revenues and selected expense and other income data for the nine months ended September 30, 2020 and 2019.

(in thousands)	Nine Months Ended September 30, 2020	2019	Change 2020 vs. 2019
Net product revenue	$ 226,143	$ 209,899	$ 16,244
Collaboration and grant revenue	35,063	622	34,441
Royalty revenue	696	—	696
Cost of product sales, excluding amortization of acquired intangible asset	14,056	8,593	5,463
Amortization of acquired intangible asset	26,309	19,677	6,632
Research and development expense	359,630	175,621	184,009
Selling, general and administrative expense	169,708	139,044	30,664
Change in the fair value of deferred and contingent consideration	16,980	35,960	(18,980)
Settlement of deferred and contingent consideration	10,613	—	10,613
Interest expense, net	(32,060)	(7,028)	(25,032)
Other income, net	26,242	2,509	23,733
Income tax expense	(22,594)	(1,006)	(21,588)

Net product revenues. Net product revenues were $226.1 million for the nine months ended September 30, 2020, an increase of $16.2 million, or 8%, from $209.9 million for the nine months ended September 30, 2019. The increase in net product revenue was primarily due to the increase in net product sales of Emflaza. The increase in net product sales of Emflaza was due to new patient prescriptions and continued operational improvements and efficiencies in our commercial business. This increase was partially offset by a decrease in net product sales of Translarna, primarily related to a delay in the anticipated timing of a group purchase order from Brazil. Due to the impact of the COVID-19 pandemic, there was an administrative delay by the Brazilian Ministry of Health in receiving the centralized Translarna group purchase order. The second and third quarters of 2019 included material group purchase orders from Brazil.

Collaboration and grant revenues. Collaboration and grant revenues were $35.1 million for the nine months ended September 30, 2020, an increase of $34.4 million, or over 100%, from $0.6 million for the nine months ended September 30, 2019. The increase is related to two milestones that were triggered from Roche in the nine months ended September 30, 2020. In August 2020, the FDA approved Evrysdi for the treatment of SMA in adults and children two months and older. The first commercial sale of Evrysdi in the United States was made in August 2020. This event triggered a $20.0 million milestone payment to us from Roche. In August 2020, the EMA accepted the MAA filed by Roche for Evrysdi for the treatment of SMA, which triggered a $15.0 million milestone payment to us from Roche. No milestones were triggered in the nine months ended September 30, 2019. Revenues in the nine months ended September 30, 2019 were related to our ongoing collaboration agreements.

Royalty revenue. Royalty revenue was $0.7 million for the nine months ended September 30, 2020, an increase of $0.7 million, or 100%, from $0.0 million for the nine months ended September 30, 2019. The increase in royalty revenue was

due to the FDA approval of Evrysdi in August 2020. In accordance with our License Agreement with Roche, we are entitled to royalties on worldwide annual net sales of the product.

Cost of product sales, excluding amortization of acquired intangible asset. Cost of product sales, excluding amortization of acquired intangible asset, were $14.1 million for the nine months ended September 30, 2020, an increase of $5.5 million, or 64%, from $8.6 million for the nine months ended September 30, 2019. Cost of product sales consist primarily of royalty payments associated with Emflaza and Translarna net product sales, excluding contingent payments to Marathon and costs associated with Emflaza and Translarna product sold during the period. The increase in cost of product sales, excluding amortization of acquired intangible asset, is primarily due to the increase in net product revenue.

Amortization of acquired intangible asset. Amortization of our intangible assets was $26.3 million for the nine months ended September 30, 2020, an increase of $6.6 million, or 34%, from $19.7 million for the nine months ended September 30, 2019. These amounts are related to the acquisition of all rights to Emflaza acquired in May 2017, Marathon contingent payments, and our Waylivra and Tegsedi intangible assets. The increase is primarily related to additional Marathon contingent payments. The amount allocated to the Emflaza intangible asset is amortized on a straight-line basis over its estimated useful life of approximately seven years from the date of the completion of the acquisition of all rights to Emflaza, the period of estimated future cash flows. The Marathon contingent payments are amortized prospectively as incurred, straight-line, over the remaining useful life of the Emflaza intangible asset. The Waylivra and Tegsedi assets are amortized on a straight-line basis over their estimated useful life of approximately ten years, respectively.

Research and development expense. Research and development expense was $359.6 million for the nine months ended September 30, 2020, an increase of $184.0 million, or 105%, from $175.6 million for the nine months ended September 30, 2019. The increase in research and development expenses includes $53.6 million in acquisition related and other expenses from our acquisition of Censa Pharmaceuticals, Inc., or Censa, pursuant to an Agreement and Plan of Merger, dated as of May 5, 2020, by and among us, Hydro Merger Sub, Inc., our wholly owned, indirect subsidiary, and, solely in its capacity as the representative, agent and attorney-in-fact for the securityholders of Censa, Shareholder Representative Services LLC and $41.4 million related to our commercial manufacturing service agreement with MassBiologics of the University of Massachusetts Medical School, or MassBio related to dedicated manufacturing space for our lead gene therapy program, AADC deficiency. The increase also reflects additional costs associated with advancing the gene therapy and Bio-e platforms and increased investment in research programs as well as advancement of the clinical pipeline.

Selling, general and administrative expense. Selling, general and administrative expense was $169.7 million for the nine months ended September 30, 2020, an increase of $30.7 million, or 22%, from $139.0 million for the nine months ended September 30, 2019. The increase was primarily due to continued investment to support our commercial activities including our expanding commercial portfolio.

Change in the fair value of deferred and contingent consideration. The change in the fair value of deferred and contingent consideration was $17.0 million for the nine months ended September 30, 2020, a decrease of $19.0 million, or 53%, from $36.0 million for the nine months ended September 30, 2019. 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.

Settlement of deferred and contingent consideration. Settlement of deferred and contingent consideration was $10.6 million for nine months ended September 30, 2020. The settlement of deferred and contingent consideration is related to a loss upon the settlement of the deferred and contingent consideration liabilities as a result of the Rights Exchange Agreement with certain former equityholders of Agilis, whereby we exchanged their pro rata share of specific future cash milestone payments in the aggregate amount of $225.0 million for a combination of cash and equity. We paid $36.9 million in cash and issued 2,821,176 shares of common stock in exchange for the cancellation and forfeiture of the participating shareholders' rights 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 Merger, regardless of whether the milestones are achieved.

Interest expense, net. Interest expense, net was $32.1 million for the nine months ended September 30, 2020, an increase of $25.0 million, or over 100% from $7.0 million for the nine months ended September 30, 2019. 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 Royalty Purchase Agreement, interest expense recorded from the 2022 and 2026 Convertible Notes and the Credit Agreement, partially offset by interest income from our investments.

Other income, net. Other income, net was $26.2 million for the nine months ended September 30, 2020, an increase of $23.7 million, or over 100%, from other income, net of $2.5 million for the nine months ended September 30, 2019. The increase in other income, net resulted primarily an unrealized foreign exchange gain from the remeasurement of our intercompany loan and unrealized gains on our equity investment and convertible debt security in ClearPoint Neuro, Inc. of $0.9 million and $0.6 million, respectively. These gains were partially offset by Agilis Rights Exchange transaction fees of $2.0 million.

Income tax expense. Income tax expense was $22.6 million for the nine months ended September 30, 2020, an increase of $21.6 million, or over 100%, compared to income tax expense of $1.0 million for the nine months ended September 30, 2019. We recorded a state income tax provision in the nine months ended September 30, 2020, which is attributable to the taxable income from the sale of our right to receive sales-based royalty payments on Roche's worldwide net sales of Evrysdi. We also incurred income tax expense in various foreign jurisdictions, and our foreign tax liabilities are largely dependent upon the distribution of pre-tax earnings among these different jurisdictions.

Liquidity and capital resources

Sources of liquidity

Since inception, we have incurred significant operating losses.

As a growing commercial-stage biopharmaceutical company, we are engaging in significant commercialization efforts for our products while also devoting a substantial portion of our efforts on research and development related to our products, product candidates and other programs. To date, almost all of our product revenue has been 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 authorization in Brazil 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 European Commission following reassessment by the EMA of the benefit-risk balance of the authorization and is subject to the specific obligation to conduct Study 041. 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 Royalty Purchase Agreement, the private placements of our preferred stock, collaborations, bank debt, convertible debt financings, the Credit Agreement 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 several years. The net losses we incur may fluctuate significantly from quarter to quarter.

In August 2015, we closed a private offering of $150.0 million in aggregate principal amount of 3.00% convertible senior notes due 2022 including the exercise by the initial purchasers of an option to purchase an additional $25.0 million in aggregate principal amount of the 2022 Convertible Notes. The 2022 Convertible Notes bear cash interest payable on February 15 and August 15 of each year, beginning on February 15, 2016. The 2022 Convertible Notes are senior unsecured obligations of ours and will mature on August 15, 2022, unless earlier converted, redeemed or repurchased in accordance with their terms prior to such date. We received net proceeds from the offering of approximately $145.4 million, after deducting the initial purchasers' discounts and commissions and the estimated offering expenses payable by us.

On May 5, 2017, we entered into the Credit Agreement with MidCap Financial, which provided for a senior secured term loan facility of $60.0 million, of which $40.0 million was drawn by us on May 5, 2017. Our ability to draw on the remaining $20.0 million under the senior secured term loan facility expired on December 31, 2018. On July 1, 2020, we terminated the Credit Agreement with MidCap Financial.

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. During the twelve month period ending December 31, 2019, we issued and sold an aggregate of 63,926 shares of common stock pursuant to the Sales Agreement at a weighted average public offering price of $46.60 per share. We received net proceeds of $2.6 million after deducting agent discounts and commissions and other offering expenses payable by us. During the nine month period ending September 30, 2020, we issued and sold an aggregate of 542,470 shares of common stock pursuant to the Sales Agreement at a weighted average public offering price of $53.37 per share. We received net proceeds of $28.1 million after deducting underwriting discounts and commissions and other offering expenses payable by us.

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 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 Royalty Purchase Agreement. Pursuant to the Royalty Purchase Agreement, we sold to RPI the Assigned Royalty Payment in consideration for $650.0 million.

Cash flows

As of September 30, 2020, we had cash, cash equivalents and marketable securities of $1,141.0 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)	**2020**	**2019**
Cash (used in) provided by:		
Operating activities	(129,705)	(88,433)
Investing activities	(353,428)	(158,240)
Financing activities	628,626	610,925

Net cash used in operating activities was $129.7 million for the nine months ended September 30, 2020 and $88.4 million for the nine months ended September 30, 2019. The net cash used in operating activities primarily relates to supporting clinical development and commercial activities.

Net cash used in investing activities was $353.4 million and $158.2 million for the nine months ended September 30, 2020 and 2019, respectively. Cash used in investing activities for the nine months ended September 30, 2020 and 2019 were primarily related to net purchases of marketable securities.

Net cash provided by financing activities was $628.6 million for the nine months ended September 30, 2020. Cash provided by financing activities for the nine months ended September 30, 2020 was primarily attributable to cash consideration received from the Royalty Purchase Agreement, proceeds from our "at the market" offerings of our common stock, and the exercise of options, partially offset by payments on our deferred consideration obligation, our finance lease principal, and our senior secured term loan. Cash provided by financing activities was $610.9 million for the nine months ended September 30, 2019. Cash provided by financing activities for the nine months ended September 30, 2019 was

primarily attributable to our equity offering in January 2019, our 2026 Convertible Notes offering, and the exercise of options.

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, distribution and manufacturing and administrative and employee-based expenses. In addition to the foregoing, we expect to continue to incur significant costs in connection with the research and development of our gene therapy, splicing, Bio-e and oncology programs, our studies of PTC923 for PKU and our studies of PTC299 for COVID-19 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. We continue to seek marketing authorization for Translarna for the treatment of nmDMD in territories that we do not currently have marketing authorization in and we may also seek marketing authorization for Translarna for other indications. We submitted an MAA to the EMA for the treatment of AADC deficiency with PTC-AADC in the EEA. We are preparing a BLA for PTC-AADC for the treatment of AADC deficiency in the United States and we anticipate submitting a BLA to the FDA in the first half of 2021. We filed for marketing authorization for Waylivra with ANVISA in June 2020. 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 we assumed in connection with the Agilis acquisition;
- seek to satisfy contractual and regulatory obligations in conjunction with the Akcea Agreement;
- satisfy contractual and regulatory obligations that we assumed through our other acquisitions and collaborations;
- execute our commercialization strategy for our products and product candidates that may receive marketing authorization;
- 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;
- utilize the office, manufacturing and laboratory space at our facility located in Hopewell Township, New Jersey, or the Hopewell Facility, to begin manufacturing program materials for certain of our gene therapy product candidates;
- initiate or continue the research and development of our gene therapy, splicing, Bio-e and oncology programs, our studies of PTC923 for PKU, our studies of PTC299 for COVID-19 as well as studies in our products for maintaining authorizations, including Study 041, label extensions and additional indications;
- seek to discover and develop additional product candidates;
- seek to expand and diversify our product pipeline through strategic transactions;
- maintain, expand and protect our intellectual property portfolio; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and commercialization efforts.

We believe that our cash flows from product sales, together with existing cash and cash equivalents, our offerings of the Convertible Notes, public offerings of common stock, our “at the market offering” of our common stock, and marketable

securities, will be sufficient to fund our operating expenses and capital expenditure requirements for at least the next twelve months. We have based this estimate on assumptions that may prove to be wrong, and we could use our capital resources sooner than we currently expect.

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

- our ability to commercialize and market our products and product candidates that may receive marketing authorization;
- our ability to negotiate, secure and maintain adequate pricing, coverage and reimbursement terms, on a timely basis, with third-party payors for our products and products candidates;
- our ability to maintain the marketing authorization for our products, including in the EEA for Translarna for the treatment of nmDMD and whether the EMA determines on an annual basis that the benefit-risk balance of Translarna supports renewal of our marketing authorization in the EEA, on the current approved label;
- the costs, timing and outcome of Study 041;
- 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 submission by us and, ultimately, may support approval of Translarna for nmDMD in the United States;
- our ability to maintain orphan exclusivity in the United States for Emflaza and successfully completing all post-marketing requirements with respect to Emflaza and any other products;
- the progress and results of activities under our gene therapy, splicing, Bio-e and oncology programs, our studies of PTC923 for PKU and our studies of PTC299 for COVID-19 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 indications or territories in which we receive authorization to market Translarna;
- the costs, timing and outcome of regulatory review of our gene therapy, splicing, Bio-e and oncology programs, PTC923 for PKU, PTC299 for COVID-19 and Translarna for additional indications and in other territories;
- unexpected decreases in revenue or increase in expenses resulting from the COVID-19 pandemic;
- our ability to utilize the Hopewell Facility to begin manufacturing program materials for certain of our gene therapy product candidates;
- our ability to satisfy our obligations under the indentures governing the Convertible Notes;
- the timing and scope of growth in our employee base;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our other product candidates, including those in our gene therapy, splicing, Bio-e and oncology programs, PTC923 for PKU, PTC299 for COVID-19 and Translarna for additional indications;
- revenue received from commercial sales of our products or any of our product candidates;

- our ability to obtain additional and maintain existing reimbursed named patient and cohort EAP programs for Translarna for the treatment of nmDMD on adequate terms, or at all;

- the ability and willingness of patients and healthcare professionals to access Translarna through alternative means if pricing and reimbursement negotiations in the applicable territory do not have a positive outcome;

- the costs of preparing, filing and prosecuting patent applications, maintaining, and protecting our intellectual property rights and defending against intellectual property-related claims;

- the extent to which we acquire or invest in other businesses, products, product candidates, and technologies, including the success of any acquisition, in-licensing or other strategic transaction we may pursue, and the costs of subsequent development requirements and commercialization efforts, including with respect to our acquisition of Emflaza, our acquisition of Agilis, our licensing of Tegsedi and Waylivra, our acquisition of our Bio-e platform and our acquisition of Censa; 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 2022 Convertible Notes, cash interest payments are payable on a semi-annual basis in arrears, which require total funding of $4.5 million annually. 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. Furthermore, since we are a public company, we have incurred and expect to continue to incur additional costs associated with operating as such, including significant legal, accounting, investor relations and other expenses.

We will need to generate significant revenues to achieve and sustain profitability, and we may never do so. 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 or debt 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.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined under Securities and Exchange Commission rules.

Contractual obligations

During the period ended September 30, 2020, there were no material changes to our contractual obligations and commitments outside the ordinary course of business from those disclosed under the heading "Management's Discussion and Analysis of Financial Condition and Results of Operations-Contractual Obligations" in our 2019 Annual Report on Form 10-K, other than as disclosed below.

(in thousands)	Total	Less than 1 year	1 - 3 years	3 - 5 years	More than 5 years
Operating lease obligations (1)	$ 134,633	$ 9,364	$ 31,093	$ 31,835	$ 62,341
Finance lease obligations (2)	36,000	3,000	9,000	9,000	15,000
Total contractual obligations	$ 170,633	$ 12,364	$ 40,093	$ 40,835	$ 77,341

(1) Obligations stem from our lease agreements for office space in Hopewell Township, New Jersey and Bridgewater, New Jersey. The Hopewell lease includes 220,500 square feet of office, manufacturing and laboratory space at a facility located in Hopewell Township, New Jersey. The rental term of the Hopewell lease commenced on July 1, 2020. The Bridgewater lease replaced our previous Bridgewater lease beginning on May 1, 2020 and includes additional rental property of approximately 59,000 square feet.

(2) Obligations stem from a commercial manufacturing service agreement entered into with MassBio on June 19, 2020, for a term of 12.5 years. Pursuant to the terms of the agreement, MassBio agreed to provide us with four dedicated rooms for our AADC program. We concluded that the agreement contains an embedded lease as we control the use of the four dedicated rooms and the equipment therein. As the present value of the facilities exceed the fair value, we determined that it is a finance lease.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

During the period ended September 30, 2020, 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 2019 Annual Report on Form 10-K.

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, 2020. 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, 2020, 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, 2020 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, 2019, 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 COVID-19 pandemic has heightened, and in some cases manifested, certain of the risks we normally face in operating our business, including those disclosed in our Annual Report on Form 10-K for the year ended December 31, 2019, and the risk factor disclosure in our Annual Report on Form 10-K for the year ended December 31, 2019 is qualified by the information related to COVID-19 that is described in this Quarterly Report on Form 10-Q, including the new risk factors set forth below.

Other than as set forth below there have been no material changes in our risk factors since those published in such Form 10-K for the year ended December 31, 2019.

We face risks related to health epidemics and other widespread outbreaks of contagious disease, which are, and may continue to, 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 has 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.

Significant outbreaks of contagious diseases, and other adverse public health developments, could have a material impact on our business operations and operating results. In December 2019, a strain of novel coronavirus, COVID-19, causing respiratory illness emerged in the city of Wuhan in the Hubei province of China. Since that time, multiple other countries throughout the world, including the United States, have been affected by the spread of the virus. To date, responsive measures such as social distancing, travel bans and quarantines have been put into place in many countries throughout the world, including the United States. These responsive measures have had a significant impact, both direct and indirect, on business and commerce worldwide, as worker shortages have occurred, supply chains have been disrupted and facilities and production have been suspended or curtailed.

The spread of COVID-19 and the responsive measures taken to date have limited our access to our facilities and caused the majority of our employees to work from home. We continue to monitor the global spread and response of international, national and local authorities of COVID-19 and have put in place and will continue to put in place measures as appropriate and necessary for our business and the safety of our employees and our business. While we expect the pandemic to continue to have an adverse effect on our business and operations, and the pandemic may have an adverse effect on our financial condition and results of operations, we are unable to predict the extent or nature of the future progression of the outbreak or its effects on our business, operations, financial condition and results of operations at this time.

Furthermore, we have clinical trial sites located in countries that have been affected by COVID-19 that have been and may continue to be disrupted, including the United States. The disruption of our clinical trial sites is having an adverse impact on our clinical trial plans and timelines. For example, we initiated Study 045 in the fourth quarter of 2018 to evaluate the ability of ataluren to increase dystrophin protein levels in boys with nmDMD. We intend to use the data from Study 045 in support of our potential NDA resubmission for Translarna for the treatment of nmDMD. As a result of the COVID-19 pandemic, our patients were temporarily unable to safely travel to our clinical trial site at the University of California, Los Angeles, which also experienced intermittent discontinuations of certain elective procedures, further complicating our patients' ability to have final study muscle biopsies performed. During the delay, patients remained on drug until the biopsies could be performed. The final study muscle biopsies were completed in October 2020 and we have begun the process of analyzing the sample according to our protocol. We expect to report top-line results from Study 045 in the first

quarter of 2021 followed by a potential resubmission of the NDA for Translarna for the treatment of nmDMD thereafter. Other clinical trial sites as well as significant suppliers and manufacturing located in countries that have been affected by COVID-19 may also be disrupted, which may affect our ability to procure items that are essential for our research and development activities and may cause disruptions. In addition, our business and operations may be disrupted as resources, components and materials that are essential for our research and development and manufacturing activities may be diverted towards the ongoing efforts to rapidly diagnose, find and distribute a treatment or vaccine for COVID-19 and may not be readily available. The response to the COVID-19 pandemic may also redirect resources with respect to regulatory matters in a way that would adversely impact our ability to progress regulatory approval. We may also choose to redirect our own resources in a way that may adversely impact or delay certain of our programs. Furthermore, we may face impediments to regulatory meetings and approvals due to measures intended to limit in-person interactions.

We cannot foresee if and when the outbreak of COVID-19 will be effectively contained, nor can we predict the severity and duration of its impact. If the COVID-19 pandemic is not effectively and timely controlled, we may experience prolonged disruption of our clinical trials, suppliers or contract manufacturers, extended closures of facilities, such as clinical trial sites, suppliers, manufacturers and distributors, including single source suppliers, and further delays with respect to regulatory approvals or the commercialization of any current or future products. Such events may materially and adversely affect our business operations and financial condition. Additionally, the pandemic has caused significant disruptions in the financial markets, and may continue to cause such disruptions, which could impact our ability to raise additional funds and has also impacted, and may continue to impact, the volatility of our stock price and trading in our stock. Moreover, it is possible the pandemic will significantly impact economies worldwide, which could result in adverse effects on our business and 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.

We face risks related to the development of PTC299 as a potential treatment for COVID-19 and we may ultimately be unsuccessful in developing a treatment for the virus in a timely manner or at all. Even if we are able to produce a drug that successfully treats the virus, there is significant competition in the search for a treatment for COVID-19 and our product may not be the only effective treatment.

In June 2020, the FDA authorized the initiation of a Phase2/3 clinical trial evaluating PTC299 as a potential treatment for COVID-19 and this trial is now ongoing. Our clinical trial for PTC299 may reveal unfavorable characteristics, including safety concerns, and may not demonstrate efficacy. We cannot be certain that the planned Phase 2/3 clinical trial will be sufficient to enable us to obtain marketing approval of PTC299 for the treatment of COVID-19, and we may need to conduct additional clinical trials before we are able to apply for marketing approval. Additionally, the FDA and other regulators may not agree with our interpretation of the results of the clinical data from our trial. If we are unable to successfully complete our clinical trial, if the results of this clinical trial are not positive or are only modestly positive, or if there are safety concerns, we may be unable to produce a drug that successfully treats COVID-19 and receives regulatory approval in a timely manner, if at all.

The timing and success of our clinical trial of PTC299 for the treatment of patients with COVID-19 will depend on our ability to enroll patients in the trial. Our inability to enroll a sufficient number of patients could result in significant delays or could require us to abandon the trial and development of PTC299 for the treatment of COVID-19 altogether. Patient enrollment may be affected by the availability of commercially available treatments and other ongoing clinical trials. There is significant competition, including from other companies and governmental organizations, to find a treatment for COVID-19. Many of these entities have substantially greater resources, including capital and personnel, than we do, and these entities may be further ahead in pursuit of a treatment than we are. As a result, even if we are able to sufficiently enroll our clinical trial and produce an effective treatment for COVID-19, there is no guarantee that we will have the only effective treatment for COVID-19 or that we will be able to commercialize our product prior to our competitors.

Item 5. Other Information.

On October 27, 2020, our Compensation Committee adopted resolutions, or the Resolutions, ratifying the issuance of 31 shares of our common stock, or the Issued Stock, issued on August 13, 2020 to an employee in connection with existing stock options pursuant to Section 204 of the General Corporation Law of the State of Delaware, or the Ratification, after it determined that the issuance may not have been duly authorized in accordance with Sections 152 and 157 of the General Corporation Law of the State of Delaware. The issuance of the Issued Stock has been ratified pursuant to the Resolutions. Any claim that the defective corporate acts (including all putative stock) ratified in the Resolutions are void or voidable due to the failure of authorization, or any claim that the Court of Chancery of the State of Delaware should declare in its discretion that the ratifications not be effective or be effective only on certain conditions, must be brought within 120 days from the date of the filing of this Quarterly Report on Form 10-Q.

Item 6. Exhibits.

Exhibit Number	Description of Exhibit
10.1†	Collaborative Research Agreement Amendment 5, dated as of January 17, 2020 by and between National Taiwan University and PTC Therapeutics GT, Inc.
10.2†	Collaborative Research Agreement Amendment 6, dated as of May 30, 2020 by and between National Taiwan University and PTC Therapeutics GT, Inc.
10.3†	Royalty Purchase Agreement, dated as of July 17, 2020, by and among PTC Therapeutics, Inc., RPI 2019 Intermediate Finance Trust, and, solely for the limited purposes set forth therein, Royalty Pharma PLC (incorporated by reference to Exhibit 10.2 to the Quarterly Report on Form 10-Q filed by the Registrant on August 5, 2020)
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.

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

SIGNATURES

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

PTC THERAPEUTICS, INC.

Date: October 29, 2020

By: /s/ Emily Hill
Emily Hill
Chief Financial Officer
(Principal Financial Officer and Duly Authorized Signatory)

Collaborative Research Agreement Amendment 5

This Amendment 5 is made and entered into as of the last date signed below (the "Amendment 5 Effective Date") to that certain Collaborative Research Agreement dated September 30, 2015 (as amended, the "Agreement") by and between National Taiwan University at No. 1, Sec. 4, Roosevelt Road, Taipei, 10617 Taiwan (R.O.C) (hereinafter "NTU") and PTC Therapeutics GT, Inc. (formerly Agilis Biotherapeutics), a Delaware corporation duly organized under law and having an address at 6 Kimball Lane, Suite 320, Lynnfield, Massachusetts, 01940 USA (hereinafter "COMPANY"). Capitalized terms herein shall have the meaning ascribed to them in the Agreement. To the extent of any conflict with the prior amendments to the Agreement, this Amendment 5 supersedes the prior amendments.

WHEREAS, the COMPANY and NTU previously amended the agreement via Amendments 1 through 4. In Amendment 4, the COMPANY and NTU agreed to a three year extension of the Term from October 1, 2017 to September 30, 2020; extension and expansion of the AADC-010 and the 011 Phase IIb protocol studies and related research; and corresponding increases in the Budget.

NOW, THEREFORE, the COMPANY and NTU wish to further amend the Agreement as follows:

1. **No-Cost Extension of the Term and the AADC-010 and 011 Phase IIb protocol studies**:

   The parties hereby agree that the Term and the duration of the AADC-010 and the 011 Phase IIb protocol studies are further extended, from September 30, 2020 to December 31, 2020, at no additional cost.

2. **Additional Research Project and Objectives of Amendment 5:**

   The parties hereby agree that the additional research activities shall be conducted under this Amendment 5:

   [**]

3. **Additional Research Budget for Amendment 5:**

   Per Section 3.1 of the Agreement, it is agreed to and understood by the parties that NTU shall be paid for the Project and Research Cost for Amendment 5, as set forth below:

   Cost [**]: [**] NTD

   - [**] cost: [**] NTD [**]
   - Overhead (OH): [**] NTD ([**]% through collaborator research agreement) OH=[**]

   [**]

   Per Section 3.2 of the Agreement, the Research Costs shall be invoiced by NTU [**] (invoices to be emailed to [**] and [**]) based on screenings completed, and Company shall pay such invoices within [**] of receipt.

IN WITNESS WHEREOF, both NTU and COMPANY have executed this Amendment 5, in duplicate originals, by their respective and duly authorized officers on the day and year written.

[Signature Block to Follow]

PTC THERAPEUTICS GT, INC.

By: /s/ Matthew Klein
Authorized Signature

Matthew Klein, MD, MS, FACS
Global Head Gene and
Mitochondrial Therapies

17-Jan-2020 14:49:05 EST
Date

NATIONAL TAIWAN UNIVERSITY

By: /s/ Chung-Ming Kuan
Authorized Signature

Printed Name & Title

Date

Collaborative Research Agreement Amendment 6

This Amendment 6 is made and entered into as of the last date signed below (the "Amendment 6 Effective Date") to that certain Collaborative Research Agreement dated September 30, 2015 (as amended, the "Agreement") by and between National Taiwan University at No. 1, Sec. 4, Roosevelt Road, Taipei, 10617 Taiwan (R.O.C) (hereinafter "NTU") and PTC Therapeutics GT, Inc. (formerly Agilis Biotherapeutics), a Delaware corporation duly organized under law and having an address at 6 Kimball Lane, Suite 320, Lynnfield, Massachusetts, 01940 USA (hereinafter "COMPANY"). Capitalized terms herein shall have the meaning ascribed to them in the Agreement. To the extent of any conflict with the prior amendments to the Agreement, this Amendment 6 supersedes the prior amendments.

WHEREAS, the COMPANY and NTU previously amended the agreement via Amendments 1 through 5. In Amendment 5, the COMPANY and NTU agreed to a further extension of the Term and duration of the AADC-010 and the 011 Phase IIb protocol studies and related research through December 31, 2020; and an additional research project and corresponding increases in the Budget for [**].

NOW, THEREFORE, the COMPANY and NTU wish to further amend the Agreement as follows:

1. **Enrollment of Additional Patients for the AADC-011 Phase IIb protocol study**:

   The parties hereby agree that additional patients [**] will be enrolled for the AADC-011 Phase IIb protocol study.

2. **Additional Research Budget for Amendment 6:**

   Per Section 2.1 of the Agreement, it is agreed to and understood by the parties that NTU shall be paid for the Project and Research Cost for Amendment 6, as set forth in the attached Supplemental Budget for Amendment 6:

   Per Section 2.2 of the Agreement, the Research Costs shall be invoiced by NTU [**] (invoices to be emailed to [**] and [**]), and Company shall pay such invoices within [**] of receipt.

IN WITNESS WHEREOF, both NTU and COMPANY have executed this Amendment 6, in duplicate originals, electronic mail of PDFs or electronic signatures, by their respective and duly authorized officers on the day and year written.

[Signature Block to Follow]

PTC THERAPEUTICS GT, INC.

By: /s/ Matthew Klein
Authorized Signature

Matthew Klein, MD, MS, FACS
Global Head Gene and
Mitochondrial Therapies

30 May 2020
Date

NATIONAL TAIWAN UNIVERSITY

By: /s/ Chung-Ming Kuan
Authorized Signature

Printed Name & Title

Date

Exhibit 31.1

CERTIFICATIONS

I, Stuart W. Peltz, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: October 29, 2020

By: /s/ STUART W. PELTZ
Stuart W. Peltz
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATIONS

I, Emily Hill, certify that:

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

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

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

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

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

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

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

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

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

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

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

Date: October 29, 2020

By: /s/ EMILY HILL
Emily Hill
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, 2020 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Stuart W. Peltz, Chief Executive Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his knowledge:

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

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

Date: October 29, 2020

By: /s/ STUART W. PELTZ
Stuart W. Peltz
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, 2020 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Emily Hill, Chief Financial Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his knowledge:

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

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

Date: October 29, 2020

By: /s/ EMILY HILL
Emily Hill
Chief Financial Officer
(Principal Financial Officer)