UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended June 30, 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 August 3, 2020, there were 67,703,214 shares of Common Stock, $0.001 par value per share, outstanding.

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PTC Therapeutics, Inc.

FORWARD-LOOKING STATEMENTS

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

	June 30, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 158,461	$ 288,028
Marketable securities	340,430	398,535
Trade receivables, net	53,644	55,538
Inventory, net	18,373	19,285
Prepaid expenses and other current assets	34,116	17,898
Total current assets	605,024	779,284
Fixed assets, net	26,806	21,549
Intangible assets, net	708,814	710,500
Goodwill	82,341	82,341
Deposits and other assets	53,652	30,108
Total assets	$ 1,476,637	$ 1,623,782
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 164,376	$ 159,276
Current portion of long-term debt	18,333	20,000
Deferred revenue	7,702	8,242
Finance lease liabilities- current	4,180	—
Other current liabilities	4,868	8,339
Deferred consideration payable	2,384	40,000
Total current liabilities	201,843	235,857
Deferred revenue- long term	—	3,415
Long-term debt	297,029	293,859
Contingent consideration payable	225,700	356,300
Deferred tax liability	130,862	130,862
Finance lease liabilities- noncurrent	22,031	—
Other long-term liabilities	26,678	9,159
Total liabilities	904,143	1,029,452
Stockholders' equity:		
Common stock, $0.001 par value. Authorized 125,000,000 shares; issued and outstanding 67,240,679 shares at June 30, 2020. Authorized 125,000,000 shares; issued and outstanding 61,935,870 shares at December 31, 2019.	67	62
Additional paid-in capital	2,067,274	1,795,351
Accumulated other comprehensive income	(10,016)	(10,584)
Accumulated deficit	(1,484,831)	(1,190,499)
Total stockholders' equity	572,494	594,330
Total liabilities and stockholders' equity	$ 1,476,637	$ 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 June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Revenues:				
Net product revenue	$ 75,239	$ 85,476	$ 143,435	$ 138,530
Collaboration and grant revenue	—	46	63	575
Total revenues	75,239	85,522	143,498	139,105
Operating expenses:				
Cost of product sales, excluding amortization of acquired intangible assets	5,304	3,211	9,389	5,587
Amortization of acquired intangible assets	8,731	6,575	16,679	12,652
Research and development	176,525	59,979	266,632	112,544
Selling, general and administrative	53,659	49,215	111,869	89,760
Change in the fair value of deferred and contingent consideration	7,680	5,300	8,580	26,460
Settlement of deferred and contingent consideration	10,613	—	10,613	—
Total operating expenses	262,512	124,280	423,762	247,003
Loss from operations	(187,273)	(38,758)	(280,264)	(107,898)
Interest expense, net	(5,379)	(2,074)	(11,021)	(4,362)
Other income (expense), net	11,309	(183)	(2,523)	(292)
Loss before income tax expense	(181,343)	(41,015)	(293,808)	(112,552)
Income tax expense	(84)	(774)	(306)	(1,350)
Net loss attributable to common stockholders	$ (181,427)	$ (41,789)	$ (294,114)	$ (113,902)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	65,150,780	55,912,748	63,769,958	57,113,141
Net loss per share—basic and diluted (in dollars per share)	$ (2.78)	$ (0.75)	$ (4.61)	$ (1.99)

See accompanying unaudited notes.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Net loss	$ (181,427)	$ (41,789)	$ (294,114)	$ (113,902)
Other comprehensive (loss) income:				
Unrealized gain on marketable securities, net of tax	2,718	839	2,655	898
Foreign currency translation (loss) gain, net of tax	(10,749)	278	(2,087)	(438)
Comprehensive loss	$ (189,458)	$ (40,672)	$ (293,546)	$ (113,442)

See accompanying unaudited notes.

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

Three months ended June 30, 2020

	Common stock		Additional paid-in capital	Accumulated other comprehensive (loss) income	Accumulated deficit	Total stockholders' equity
	Shares	Amount				
Balance, March 31, 2020	62,758,520	$ 62	$ 1,834,061	$ (1,985)	$ (1,303,404)	$ 528,734
Issuance of common stock related to equity offering	106,309	—	5,447	—	—	5,447
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	654,604	1	15,057	—	—	15,058
Restricted stock vesting and issuance, net	(3,985)	—	—	—	—	—
Issuance of common stock in connection with an employee stock purchase plan	58,691	—	2,406	—	—	2,406
Share-based compensation expense	—	—	16,910	—	—	16,910
Net loss	—	—	—	—	(181,427)	(181,427)
Comprehensive loss	—	—	—	(8,031)	—	(8,031)
Balance, June 30, 2020	67,240,679	$ 67	$ 2,067,274	$ (10,016)	$ (1,484,831)	$ 572,494

Three months ended June 30, 2019

	Common stock		Additional paid-in capital	Accumulated other comprehensive income (loss)	Accumulated deficit	Total stockholders' equity
	Shares	Amount				
Balance, March 31, 2019	58,418,790	$ 58	$ 1,523,115	$ 805	$ (1,011,036)	$ 512,942
Issuance of common stock related to equity offering	—	—	106	—	—	106
Exercise of options	230,176	—	2,847	—	—	2,847
Restricted stock vesting and issuance, net	3,500	—	—	—	—	—
Issuance of common stock in connection with an employee stock purchase plan	54,719	—	1,564	—	—	1,564
Share-based compensation expense	—	—	10,920	—	—	10,920
Receivable from investor	—	—	978	—	—	978
Net loss	—	—	—	—	(41,789)	(41,789)
Comprehensive income	—	—	—	1,117	—	1,117
Balance, June 30, 2019	58,707,185	$ 58	$ 1,539,530	$ 1,922	$ (1,052,825)	$ 488,685

Six months ended June 30, 2020

	Common stock		Additional paid-in capital	Accumulated other comprehensive (loss) income	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	368,514	—	18,950	—	—	18,950
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	1,034,288	1	25,044	—	—	25,045
Restricted stock vesting and issuance, net	176,776	—	—	—	—	—
Issuance of common stock in connection with an employee stock purchase plan	58,691	—	2,406	—	—	2,406
Share-based compensation expense	—	—	32,130	—	—	32,130
Other	—	—	—	—	(218)	(218)
Net loss	—	—	—	—	(294,114)	(294,114)
Comprehensive income	—	—	—	568	—	568
Balance, June 30, 2020	67,240,679	$ 67	$ 2,067,274	$ (10,016)	$ (1,484,831)	$ 572,494

Six months ended June 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	7,563,725	7	224,538	—	—	224,545
Exercise of options	311,002	—	4,129	—	—	4,129
Restricted stock vesting and issuance, net	171,592	—	—	—	—	—
Issuance of common stock in connection with an employee stock purchase plan	54,719	—	1,564	—	—	1,564
Share-based compensation expense	—	—	20,184	—	—	20,184
Receivable from investor	—	—	978	—		978
Net loss	—	—	—		(113,902)	(113,902)
Comprehensive income	—	—	—	460	—	460
Balance, June 30, 2019	58,707,185	$ 58	$ 1,539,530	$ 1,922	$ (1,052,825)	$ 488,685

See accompanying unaudited notes.

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

	Six Months Ended June 30,	
	2020	2019
Cash flows from operating activities		
Net loss	$ (294,114)	$ (113,902)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	19,445	14,760
Non-cash operating lease expense	2,401	1,256
Non-cash finance lease amortization expense	41,212	—
Change in valuation of deferred and contingent consideration	8,580	26,460
Settlement of deferred and contingent consideration	10,613	—
Non-cash stock consideration, acquisition	42,869	—
Unrealized loss on equity investment	1,600	(129)
Unrealized loss on ClearPoint convertible debt security	749	—
Non-cash interest expense	10,993	4,055
Loss on disposal of asset	26	50
Amortization of discounts on investments, net	(635)	(907)
Amortization of debt issuance costs	510	280
Share-based compensation expense	32,130	20,184
Unrealized foreign currency transaction (gains) losses, net	(3,248)	62
Changes in operating assets and liabilities:		
Inventory, net	943	(852)
Prepaid expenses and other current assets	(16,569)	(7,250)
Trade receivables, net	1,860	(9,702)
Deposits and other assets	(175)	(9,656)
Accounts payable and accrued expenses	(7,497)	(7,546)
Other long-term liabilities	(4,132)	7,395
Deferred revenue	(3,925)	2,511
Net cash used in operating activities	$ (156,364)	$ (72,931)
Cash flows from investing activities		
Purchases of fixed assets	$ (8,183)	$ (4,851)
Purchase of convertible debt security	(10,000)	—
Purchases of marketable securities	(367,102)	(226,261)
Sale and redemption of marketable securities	428,490	58,424
Acquisition of product rights and licenses	(2,422)	(11,981)
Purchase of equity investment	—	(4,000)
Net cash provided by (used in) investing activities	$ 40,783	$ (188,669)
Cash flows from financing activities		
Proceeds from exercise of options	$ 25,045	$ 4,129
Net proceeds from public offerings	18,950	224,545
Repayment of senior secured term loan	(10,000)	(1,667)
Payments on deferred consideration obligation	(35,829)	—
Proceeds from employee stock purchase plan	2,406	1,564
Payment of finance lease principal	(15,000)	—
Net cash provided by financing activities	$ (14,428)	$ 228,571
Effect of exchange rate changes on cash	442	(375)
Net decrease in cash and cash equivalents	(129,567)	(33,404)
Cash and cash equivalents, and restricted cash beginning of period	295,528	169,498
Cash and cash equivalents, and restricted cash end of period	$ 165,961	$ 136,094

Supplemental disclosure of cash information				
Cash paid for interest	$	5,286	$	3,111
Cash paid for income taxes		1,140		1,013
Supplemental disclosure of non-cash investing and financing activity				
Unrealized gain on marketable securities, net of tax	$	2,655	$	898
Right-of-use assets obtained in exchange for operating lease obligations		18,117		11,643
Right-of-use assets obtained in exchange for finance lease obligations		41,212		—
Acquisition of product rights and licenses		12,186		8,269
Issuance of common stock related to rights exchange		150,528		—

See accompanying unaudited notes.

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, or 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 June 2020, the Committee for Medicinal Products for Human Use (the "CHMP") of the European Medicines Agency ("EMA") recommended to remove the statement “efficacy has not been demonstrated in non-ambulatory patients” from the product label for Translarna. The CHMP's opinion is subject to final approval by the European Commission, which is typically provided within two months of the CHMP's recommendation. 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, or 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 initiating the BLA submission to the United States Food and Drug Administration ("FDA") in the second half of 2020. In January 2020, the Company submitted a marketing authorization application ("MAA") to the EMA for PTC-AADC for the treatment of AADC deficiency in the EEA, and the Company expects an opinion from the CHMP in the first quarter 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, or hATTR amyloidosis. Waylivra has received marketing authorization in the EU for the treatment of familial chylomicronemia syndrome, or 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 lead compound in the SMA program is risdiplam (RG7916, RO7034067). Roche submitted an NDA for risdiplam to the FDA in the fourth quarter of 2019. In April 2020, the FDA extended the Prescription Drug User Fee Act ("PDUFA") date for a decision from May 24, 2020 to August 24, 2020 as a result of additional data that Roche submitted, including comprehensive data from the Sunfish part 2 study. Risdiplam is expected to be indicated in the United States for SMA type 1, 2 and 3 patients, if approved. Roche anticipates submitting an MAA for risdiplam in the EEA imminently.

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, formerly known as PTC743, and PTC857. The Company expects to initiate a potential registrational Phase 2 placebo-controlled trial of vatiquinone in approximately 60 children with mitochondrial disease and associated refractory epilepsy in the third quarter of 2020. The Company also expects to initiate a potential registrational 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. The Company expects data from the single ascending dose and multiple ascending dose studies from the Phase 1 trial to be available by the end of 2020.

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, or 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. As a result of intermittent discontinuations of certain elective procedures at the Company's clinical trial site in response to the COVID-19 pandemic, the Company's expected completion of Study 45 has been delayed as certain patients still require final study muscle biopsies. Once the clinical trial site is open for the necessary procedures and patients are able to safely travel to the site, the Company expects to be able to complete the final biopsies and the data from Study 045 would be available thereafter, followed by a potential re-submission of an NDA. 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., or 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 equity holders 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 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 $22.4 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 after deducting the $37.6 million for which rights were canceled and forfeited pursuant to the Rights Exchange Agreement from the $40.0 million in development milestone payments that are due 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 (formerly known as CNSA-001), 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 June 30, 2020, the Company had an accumulated deficit of approximately $1,484.8 million. The Company has financed its operations to date primarily through the private offerings in September 2019 of 1.50% convertible senior notes due 2026 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, 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 six month period ended June 30, 2020 are discussed in further detail below.

Basis of presentation

The accompanying financial information as of June 30, 2020 and for the three and six months ended June 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 June 30, 2020 and for the three and six months ended June 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 six month period ended June 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, certain accruals related to the Company's research and development expenses, 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- June 30, 2020
Cash and cash equivalents	$ 288,028	$ 158,461
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	$ 165,961

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 six month periods ended June 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:

	June 30, 2020	December 31, 2019
Raw materials	$ 877	$ 874
Work in progress	8,666	9,652
Finished goods	8,830	8,759
Total inventory	$ 18,373	$ 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 months ended June 30, 2020, inventory write downs were immaterial. For the six months ended June 30, 2020, the Company recorded a $0.2 million inventory write-down, primarily related to product approaching expiration. No write downs were recorded for the three and six month periods ended June 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 six month periods ended June 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 June 30, 2020 and 2019, net product sales outside of the United States were $39.0 million and $57.8 million, respectively, and net product sales in the United States were $36.2 million and $27.6 million, respectively. For the six months ended June 30, 2020 and 2019, net product sales outside of the United States were $79.8 million and $93.0 million, respectively, and net product sales in the United States were $63.6 million and $45.4 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 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 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 $0.2 million as of June 30, 2020 and $0.3 million as of December 31, 2019. Bad debt expense was immaterial for the three and six month periods ended June 30, 2020 and 2019. For the three and six month periods ended June 30, 2020, no allowances were recorded for credit losses.

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"), referred to herein as the U.S. Tax Act, for qualified improvement property. As of June 30, 2020, the Company expects that these provisions will not have a material impact. Tax provisions of the Act also include the deferral of certain payroll taxes, relief for retaining employees, and other provisions. The Company is evaluating the impact of the 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 June 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.

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.

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 principals 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 three and six month periods ended June 30, 2020. In addition, the Company incurred $0.3 million of other expenses related to Censa, which was included in research and development expense for the three and six month periods ended June 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 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 "Initial Milestone"). 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 certain vehicles, lab equipment, and office equipment under operating leases. The Company's leases have remaining operating lease terms ranging from 0.3 years to 6.9 years and certain of the leases include renewal options to extend the lease for up to 10 years. Rent expense was approximately $2.6 million, $5.0 million, $1.1 million and $2.1 million for the three and six month periods ended June 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 $41.2 million during the three and six month periods ending June 30, 2020. Additionally, during the three and six month periods ending June 30, 2020, the Company did not record finance lease costs as these amounts were not material due to timing of entering into the lease. The Company will record finance lease costs in the third quarter of 2020.

The components of operating lease expense were as follows:

	Three Months Ended June 30, 2020	Three Months Ended June 30, 2019	Six Months Ended June 30, 2020	Six Months Ended June 30, 2019
Operating Lease Cost				
Fixed lease cost	$ 1,970	$ 835	$ 4,089	$ 1,647
Variable lease cost	507	161	734	304
Short-term lease cost	88	88	165	141
Total operating lease cost	$ 2,565	$ 1,084	$ 4,988	$ 2,092

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:

	June 30, 2020	December 31, 2019
Operating lease ROU asset	$ 29,475	$ 13,693
Operating lease liabilities- current	$ 4,868	$ 5,153
Operating lease liabilities- noncurrent	25,510	9,018
Total operating lease liability	$ 30,378	$ 14,171

	June 30, 2020	December 31, 2019
Finance lease liabilities- current	$ 4,180	$ —
Finance lease liabilities- noncurrent	22,031	—
Total finance lease liability	$ 26,211	$ —

Operating lease ROU asset is a component of deposits and other assets on the consolidated balance sheets. The current portion of operating lease liabilities is a component of other current liabilities on the consolidated balance sheets. The long term portion of operating lease liabilities is a component of other long term liabilities on the consolidated balance sheets. 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 and is the primary driver of the increase in total operating lease ROU asset and total operating lease liability from December 31, 2029 to June 30, 2020.

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

	June 30, 2020	December 31, 2019
Weighted-average remaining lease terms - operating leases (years)	5.87	3.38
Weighted-average discount rate - operating leases	6.77 %	7.33 %
Weighted-average remaining lease terms - finance lease (years)	12.50	—
Weighted-average discount rate - finance lease	7.80 %	—

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

	Six Month Ended June 30,	
	2020	2019
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows from operating leases	$ 3,414	$ 1,508
Financing cash flows from finance lease	15,000	—
Right-of-use assets obtained in exchange for lease obligations:		
Operating leases	$ 18,117	$ 11,643
Finance lease	41,212	—

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

	Operating Leases	Finance Lease
2020 (excludes the six months ended June 30, 2020)	$ 3,719	$ 3,000
2021	6,667	3,000
2022	6,314	3,000
2023	5,960	3,000
2024 and thereafter	14,844	27,000
Total lease payments	37,504	39,000
Less: Imputed Interest expense	7,126	12,789
Total	$ 30,378	$ 26,211

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 June 30, 2020 are $0.9 million, $1.8 million, and $1.4 million for 2020, 2021 and 2022, respectively.

On August 4, 2019, the Company and Bristol-Myers Squibb Company, (the "Landlord"), entered into a Lease Agreement (the "Lease"), relating to the lease of approximately 185,000 square feet of office, manufacturing and laboratory space at a facility located in Hopewell Township, New Jersey (the "Campus"). On March 25, 2020, the Company entered into an amendment increasing the rented space to approximately 220,500 square feet. As of June 30, 2020, the Lease had not yet commenced, and accordingly, is not reflected in the table above. 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 five 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.

5. Fair value of financial instruments and marketable securities

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

- Level 1—Unadjusted quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the balance sheet date.
- Level 2—Inputs other than quoted prices included within Level 1 that are observable for the asset or liability, either directly or indirectly. Level 2 inputs include quoted prices for similar assets and liabilities in active markets, quoted prices for identical or similar assets or liabilities in markets that are not active, inputs other than quoted prices that are observable for the asset or liability (i.e., interest rates, yield curves, etc.), and inputs that are derived principally from or corroborated by observable market data by correlation or other means (market corroborated inputs).
- Level 3—Inputs are unobservable and reflect the Company’s assumptions as to what market participants would use in pricing the asset or liability. The Company develops these inputs based on the best information available.

Cash equivalents and marketable securities are reflected in the accompanying financial statements at fair value. The carrying amount of receivables and accounts payable and accrued expenses approximates fair value due to the short-term nature of those instruments. The carrying amounts for borrowings under the credit and security agreement with MidCap Financial approximate fair value based on market activity for other debt instruments with similar characteristics and comparable risk.

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 six month periods ended June 30, 2020, the Company recorded unrealized losses of $0.1 million and $1.6 million, respectively, which are components of other expense, net within the consolidated statement of operations. The fair value of the equity investment was $4.6 million as of June 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 six month periods ended June 30, 2020, the Company recorded an unrealized gain of $2.1 million and an unrealized loss of $0.7 million, respectively, which are components of other expense and other income, net within the consolidated statement of operations. The fair value of the convertible debt security was $9.3 million as of June 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 June 30, 2020 and December 31, 2019:

	June 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	$ 340,430	$ —	$ 340,430	$ —
Equity investment in ClearPoint	$ 4,594	$ 4,594	$ —	$ —
ClearPoint convertible debt security	$ 9,251	$ —	$ 9,251	$ —
Deferred consideration payable	$ 2,384	$ —	$ 2,384	$ —
Contingent consideration payable- development and regulatory milestones	$ 136,100	$ —	$ —	$ 136,100
Contingent consideration payable- net sales milestones and royalties	$ 89,600	$ —	$ —	$ 89,600

	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 June 30, 2020 and December 31, 2019.

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

	June 30, 2020			
	Amortized Cost	Gross Unrealized		Fair Value
		Gains	Losses	
Commercial paper	$ 53,638	$ 178	$ —	$ 53,816
Corporate debt securities	221,068	2,882	—	223,950
Asset-backed securities	44,492	332	(1)	44,823
Government obligations	17,823	19	(1)	17,841
Total	$ 337,021	$ 3,411	$ (2)	$ 340,430

	December 31, 2019			
	Amortized Cost	Gross Unrealized		Fair Value
		Gains	Losses	
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 six month periods ended June 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 six month periods ended June 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 six month periods ended June 30, 2020, the Company had $0.2 million 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 June 30, 2020 are as follows:

	June 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
Asset-backed securities	(1)	594	—	—	(1)	594
Government obligations	(1)	8,602	—	—	(1)	8,602
Total	$ (2)	$ 9,196	$ —	$ —	$ (2)	$ 9,196

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 June 30, 2020 and December 31, 2019 mature as follows:

	June 30, 2020	
	Less Than 12 Months	More Than 12 Months
Commercial paper	$ 53,816	$ —
Corporate debt securities	168,250	55,700
Asset-backed securities	26,320	18,503
Government obligations	7,201	10,640
Total Marketable securities	$ 255,587	$ 84,843

	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 June 30, 2020 and December 31, 2019 was $175.2 million and $171.2 million, respectively. The estimated fair value of the 2026 Convertible Notes at June 30, 2020 and December 31, 2019 was $343.8 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 at June 30, 2020 was $2.4 million, which was also determined to be the fair value. The Company did not apply a discount, as the milestones will be paid within one calendar year. Accordingly, as of June 30, 2020, the $2.4 million of the deferred consideration payable was classified as current on the balance sheet.

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 three and six month periods ended June 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 three and six month periods ended June 30, 2020. This non-recurring Level 3 fair value measurement was estimated using the same valuation methoology 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 three and six month periods ended June 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 June 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 June 30, 2020, the weighted average discount rate for the development and regulatory milestones was 5.4% and the weighted average probability of success was 43%. 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 June 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 50%.

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 June 30, 2020 and June 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	—	(15,220)	23,800
Payments	(3,186)	—	—
Rights Exchange settlement	—	(139,180)	—
Ending balance as of June 30, 2020	$ —	$ 136,100	$ 89,600

	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	1,961	20,360	5,600
Payments	(3,815)	—	—
Ending balance as of June 30, 2019	$ 1,960	$ 277,400	$ 58,800

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

	June 30, 2020			
	Fair Value	Valuation Technique	Unobservable Input	Range
Contingent consideration payable- development and regulatory milestones	$136,100	Probability-adjusted discounted cash flow	Potential development and regulatory milestones	$0 - $381 million
			Probabilities of success	25% - 94%
			Discount rates	4.6% - 6.1%
			Projected years of payments	2021 - 2027
Contingent considerable payable- net sales milestones and royalties	$89,600	Option-pricing model with Monte Carlo simulation	Potential net sales milestones	$0 - $150 million
			Probabilities of success	25% -94%
			Potential percentage of net sales for royalties	2% - 6%
			Discount rate	12.0%
			Projected years of payments	2022 - 2039

	December 31, 2019			
	Fair Value	Valuation Technique	Unobservable Input	Range
SARs	$3,186	Option-pricing model	Volatility	28.93%
			Risk free interest rate	0.19%
			Strike price	$6.76 - $30.86
			Fair value of common stock	$48.03
			Expected life	0.01 years
Contingent consideration payable- development and regulatory milestones	$290,500	Probability-adjusted discounted cash flow	Potential development and regulatory milestones	$0- $555 million
			Probabilities of success	25% - 94%
			Discount rates	2.2% - 4.7%
			Projected years of payments	2020 - 2026
Contingent considerable payable- net sales milestones and royalties	$65,800	Option-pricing model with Monte Carlo simulation	Potential net sales milestones	$0 - $150 million
			Probabilities of success	25% - 89%
			Potential percentage of net sales for royalties	2% - 6%
			Discount rate	14.5%
			Projected years of payments	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 six months periods ended June 30, 2020:

	Unrealized Gains/(Losses) On Marketable Securities, net of tax	Foreign Currency Translation	Total Accumulated Other Comprehensive Items
Balance at March 31, 2020	$ 692	$ (2,677)	$ (1,985)
Other comprehensive income (loss) before reclassifications	2,718	(10,749)	(8,031)
Amounts reclassified from other comprehensive items	—	—	—
Other comprehensive income (loss)	2,718	(10,749)	(8,031)
Balance at June 30, 2020	$ 3,410	$ (13,426)	$ (10,016)

	Unrealized Gains/(Losses) On Marketable Securities, net of tax	Foreign Currency Translation	Total Accumulated Other Comprehensive Items
Balance at December 31, 2019	$ 755	$ (11,339)	$ (10,584)
Other comprehensive income (loss) before reclassifications	2,655	(2,087)	568
Amounts reclassified from other comprehensive items	—	—	—
Other comprehensive income (loss)	2,655	(2,087)	568
Balance at June 30, 2020	$ 3,410	$ (13,426)	$ (10,016)

7. Accounts payable and accrued expenses

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

	June 30, 2020	December 31, 2019
Employee compensation, benefits, and related accruals	$ 24,861	$ 38,889
Consulting and contracted research	13,452	12,969
Professional fees	3,295	3,562
Sales allowance and other costs	45,848	41,155
Sales rebates and royalties	56,870	42,997
Accounts payable	15,465	10,324
Other	4,585	9,380
Total	$ 164,376	$ 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 six month periods ending June 30, 2020, the Company issued and sold an aggregate of 106,309 and 368,514 shares of common stock pursuant to the Sales Agreement at a weighted average public offering price of $53.08 and $52.89 per share, respectively. During the three and six month periods ending June 30, 2020, the Company received net proceeds of $5.4 million and $19.0 million, respectively, after deducting underwriting discounts and commissions and other offering expenses payable by the Company.

As a result of the Rights Exchange Agreement, during the three and six month periods ended June 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 three and six month periods ended June 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 June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Numerator				
Net loss	$ (181,427)	$ (41,789)	$ (294,114)	$ (113,902)
Denominator				
Denominator for basic and diluted net loss per share	65,150,780	55,912,748	63,769,958	57,113,141
Net loss per share:				
Basic and diluted	$ (2.78) *	$ (0.75) *	$ (4.61) *	$ (1.99) *

*In the three and six months ended June 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 June 30,	
	2020	2019
Stock Options	11,954,684	10,853,301
Unvested restricted stock awards and units	936,922	665,002
Total	12,891,606	11,518,303

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 June 30, 2020, awards for 914,406 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 June 30, 2020, awards for 442,880 shares of common stock are available for issuance under the 2020 Inducement Stock Incentive Plan.

From January 1, 2020 through June 30, 2020, the Company issued a total of 2,515,200 stock options to various employees. Of those, 542,450 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	2,515,200	$ 50.98		
Exercised	(1,034,288)	$ 24.21		
Forfeited/Cancelled	(570,167)	$ 42.27		
Outstanding at June 30, 2020	11,954,684	$ 35.84	7.48 years	$ 183,488
Vested or Expected to vest at June 30, 2020	5,690,848	$ 39.16	8.86 years	$ 66,986
Exercisable at June 30, 2020	5,512,252	$ 31.61	5.85 years	$ 110,222

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

	Six months ended June 30, 2020
Risk-free interest rate	0.40% - 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 six month period ended June 30, 2020 was $36.95 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	589,070	$ 49.67
Vested	(229,076)	$ 23.53
Forfeited	(65,491)	$ 36.11
Unvested at June 30, 2020	936,922	$ 39.97

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 six month period ended June 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 June 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 six month periods ended June 30, 2020, the Company recorded $0.3 million and $0.7 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 June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Research and development	$ 8,562	$ 5,516	$ 16,741	$ 10,203
Selling, general and administrative	8,348	5,404	15,389	9,981
Total	$ 16,910	$ 10,920	$ 32,130	$ 20,184

As of June 30, 2020, there was approximately $187.2 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 3.08 years.

11. Debt

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. As of June 30, 2020, the Company had made loan repayments of $21.7 million on the Credit Facility. The remaining balance of the Credit Facility as of June 30, 2020 was $18.3 million, which was classified as current portion of long term debt on the consolidated balance sheet.

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.

The Credit Facility is subject to certain financial covenants. As of June 30, 2020, the Company was in compliance with all required covenants. On July 1, 2020, the Company terminated the Credit Facility. Refer to Note 15 for further details.

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	**June 30, 2020**	**December 31, 2019**
Principal	$ 287,500	$ 287,500
Less: Debt issuance costs	(4,319)	(4,567)
Less: Debt discount, net(1)	(112,877)	(119,350)
Net carrying amount	$ 170,304	$ 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 June 30, 2020, the remaining contractual life of the 2026 Convertible Notes is approximately 6.2 years.

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

	Three Months Ended June 30, 2020	**Six Months Ended June 30, 2020**
Contractual interest expense	$ 1,066	$ 2,142
Amortization of debt issuance costs	124	248
Amortization of debt discount	3,239	6,473
Total	$ 4,429	$ 8,863
Effective interest rate of the liability component	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	June 30, 2020	December 31, 2019
Principal	$ 150,000	$ 150,000
Less: Debt issuance costs	(1,104)	(1,329)
Less: Debt discount, net(1)	(22,166)	(26,686)
Net carrying amount	$ 126,730	$ 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 June 30, 2020, the remaining contractual life of the 2022 Convertible Notes is approximately 2.1 years.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Contractual interest expense	$ 1,125	$ 1,131	$ 2,244	$ 2,241
Amortization of debt issuance costs	114	103	225	202
Amortization of debt discount	2,295	2,074	4,520	4,055
Total	$ 3,534	$ 3,308	$ 6,989	$ 6,498
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), or 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 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 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 $22.4 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 after deducting the $37.6 million for which rights were canceled and forfeited pursuant to the Rights Exchange Agreement from the $40.0 million in development milestone payments that are due upon the passing of the second anniversary of the closing of the Acquisition), 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 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 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 June 30, 2020 and 2019, net product sales in the United States were $36.2 million and $27.6 million, respectively, consisting solely of Emflaza, and net product sales not in the United States were $39.0 million and $57.8 million, respectively, consisting of Translarna and Tegsedi. For the three months ended June 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 six months ended June 30, 2020 and 2019, net product sales in the United States were $63.6 million and $45.4 million, respectively, consisting solely of Emflaza, and net product sales not in the United States were $79.8 million and $93.0 million, respectively, consisting of Translarna and Tegsedi. For the six months ended June 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 June 30, 2020 and as of December 31, 2019 were $7.7 million and $11.7 million, respectively. The Company did not have any contract assets as of June 30, 2020 and as of December 31, 2019. During the three and six month periods ended June 30, 2020, the Company recognized $2.0 million and $4.0 million of revenue, respectively, related to the amounts included in the contract liability balance at the beginning of the period. For the three and six month periods ended June 30, 2019, the Company did not recognize any revenues 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 six month periods ending June 30, 2020 and 2019.

Remaining performance obligations

Remaining performance obligations represent the transaction price for goods the Company has yet to provide. As of June 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 $7.7 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 revenue

In November 2011, the Company and the Spinal Muscular Atrophy Foundation (SMA Foundation) entered into a licensing and collaboration agreement with F. Hoffman-La Roche Ltd and Hoffman- La Roche Inc. (collectively, Roche) for a spinal muscular atrophy program. Under the terms of the agreement, Roche acquired an exclusive worldwide license to the Company’s spinal muscular atrophy 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. As of June 30, 2020, the remaining potential research and development event milestones that can be received is $72.5 million. The remaining potential sales milestones as of June 30, 2020 is $325.0 million upon achievement of certain sales events. In addition, the Company is eligible to receive up to double digit royalties on worldwide annual net sales of a commercial product.

For the three months ended June 30, 2020, the Company did not recognize revenue related to the licensing and collaboration agreement with Roche. For the three months ended June 30, 2019, the Company recognized $0.1 million of revenue related to the licensing and collaboration agreement with Roche. For the six months ended June 30, 2020 and 2019, the Company recognized revenue related to the licensing and collaboration agreement with Roche of $0.1 million and $0.1 million, respectively.

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 June 30, 2020 and 2019, milestone payments of $12.2 million and $8.3 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 June 30, 2020 and 2019, the Company recognized amortization expense of $8.7 million and $6.6 million, respectively, related to the Emflaza rights, Waylivra, and Tegsedi intangible assets. For the six months ended June 30, 2020 and 2019, the Company recognized amortization expense of $16.7 million and $12.7 million, respectively. The estimated future amortization of the Emflaza rights, Waylivra, and Tegsedi intangible assets is expected to be as follows:

	As of June 30, 2020
2020	$ 17,460
2021	34,920
2022	34,920
2023	34,920
2024 and thereafter	10,094
Total	$ 132,314

The weighted average remaining amortization period of the definite-lived intangibles as of June 30, 2020 is 4.0 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 six month periods ended June 30, 2020.

15. Subsequent events

MidCap Credit Facility Termination

On July 1, 2020, the Company terminated the Credit Facility. In connection with the termination of the Credit Facility, the Company repaid outstanding principal and accrued interest thereunder totaling $18.4 million and paid an additional $0.6 million in termination and exit fees. All liens and security interests securing the term loan made pursuant to the Credit Facility were released upon termination.

Monetization of a Portion of Risdiplam Royalty Stream

On July 17, 2020, the Company, RPI 2019 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 Roche's risdiplam product and any other product (the "Products") developed pursuant to the License and Collaboration Agreement (the "License Agreement"), dated as of November 23, 2011, by and among the Company, F. Hoffman-La Roche Ltd., Hoffman-La Roche Inc. (together with F. Hoffman-La Roche Ltd, "Roche"), and, for the limited purposes set forth therein, the 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 $397.5 million in the aggregate. 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.

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, Part I, Item 1A. (Risk Factors) of our 2019 Annual Report and Part II, Item 1A. (Risk Factors) of our Quarterly Report on Form 10-Q for the period ended March 31, 2020, 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, the following expectations have been revised as a result of the impact or expected impact of the COVID-19 pandemic:

- As a result of the COVID-19 pandemic, our expected completion of Study 045 has been delayed as certain patients still require final study muscle biopsies. Due to the pandemic, we cannot ensure that our patients are able to safely travel to our clinical trial site at the University of California, Los Angeles, which has also experienced intermittent discontinuations of certain elective procedures, further complicating our patients' ability to have the biopsies performed. During the delay, patients are remaining on drug until the biopsies can be performed. Once the clinical trial site is open for the necessary procedures and patients are able to safely travel to the site, we expect to be able to complete the final biopsies and the data from Study 045 would be available thereafter, 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. We are continuously monitoring the situation to determine when it will be possible to safely obtain the final biopsies and we are exploring all potential options in order to have the data from Study045 be available by the end of 2020.
- 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 EEA and we have been granted a clock stop extension to respond to the EMA’s questions. We now expect an opinion from the Committee for Medicinal Products for Human Use, or CHMP, in the first quarter of 2021.
- The COVID-19 pandemic has impacted the activities enabling the first-in-human studies for our gene therapy programs targeting Friedrich ataxia and Angelman syndrome. We continue to anticipate the filings in support of the first-in-human study for each of these programs to be delayed by at least one quarter.
- As a result of the COVID-19 pandemic, the Brazilian Ministry of Health is experiencing 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.”

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. PTC299 is an

oral investigational drug with a novel dual-mechanism of action that we believe has the potential to address the two critical elements of COVID-19: (i) the high viral replication and (ii) the uncontrolled inflammatory response that ensues after infection. The integrated Phase 2/3 study, which is being completed in two stages, will evaluate the efficacy and safety of PTC299 in patients hospitalized with SARS-CoV-2 infection. The primary objective of the study is to evaluate the clinical efficacy of PTC299 compared with placebo assessed by time to respiratory improvement in adult individuals hospitalized with COVID-19. We expect Stage 1 of the clinical trial to be completed in the second half of 2020 and we anticipate reporting top-line results from both stages in the first half of 2021.

Agilis Rights Exchange Agreement

On April 29, 2020, we, certain of the former equityholders of Agilis Biotherapeutics, Inc., or Agilis, and, for the limited purposes set forth in the agreement, Shareholder Representative Services LLC, entered into a Rights Exchange Agreement, or the Rights Exchange Agreement. Pursuant to the Rights Exchange Agreement, we issued 2,821,176 shares of our common stock, or the Common Stock Consideration, and paid $36.9 million, or the Cash Consideration, in the aggregate, to such former equityholders of Agilis, or 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, or the Merger Agreement, by and among us, Agility Merger Sub, Inc., Agilis, and, solely in its capacity as the representative, agent and attorney-in-fact of the equityholders of Agilis, Shareholder Representative Services LLC, dated as of July 19, 2018, pursuant to which we acquired Agilis in 2018, or 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 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 our obligation to pay up to an aggregate maximum amount of $22.4 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 after deducting the $37.6 million for which rights were canceled and forfeited pursuant to the Rights Exchange Agreement from the $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), 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.

Acquisition of Censa Pharmaceuticals, Inc.

On May 29, 2020, we acquired Censa Pharmaceuticals, Inc., or Censa, pursuant to an Agreement and Plan of Merger, dated as of May 5, 2020, or the Censa Merger Agreement, by and among us, Hydro Merger Sub, Inc., our wholly owned, indirect subsidiary, and, solely in its capacity as the representative, agent and attorney-in-fact of the securityholders of Censa, Shareholder Representative Services LLC, or the Censa Merger.

Upon the closing of the Censa Merger, we 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 our common stock. Subject to the terms and conditions of the Censa Merger Agreement, Censa securityholders may become entitled to receive contingent payments from us based on the achievement of certain development, regulatory and net sales milestones as well as based upon a percentage of net sales of PTC923 and sublicense fees paid to us in consideration of any sublicense of Censa's intellectual property to commercialize PTC923. Pursuant to the Censa Merger Agreement, we have the option to pay the initial $30.0 million development milestone, if achieved, in cash or shares of our common stock.

In connection with the Censa Merger, we acquired 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. PTC923 has been pursued as a possible treatment for orphan metabolic diseases associated with defects in the tetrahydrobiopterin biochemical pathways, including phenylketonuria, or PKU. PKU is an inborn error of metabolism caused predominantly by mutations in the phenylalanine hydroxylase gene resulting in toxic buildup of the amino acid

phenylalanine, or Phe, in the brain, and, if left untreated, severe and irreversible disabilities such as permanent intellectual disability, seizures, delayed development, behavioral problems and possibly psychiatric disorders can occur. In December 2019, Censa announced that the Phase 2 trial for PTC923 as a potential treatment for PKU met is primary and secondary endpoints, achieving statistically-significant and clinically-meaningful reduction in blood Phe levels compared to both baseline and an active control group. We expect to initiate a Phase 3 trial for PTC923 for PKU in 2021.

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 Roche's risdiplam product and any other product developed pursuant to the License and Collaboration Agreement, or the License Agreement, dated as of November 23, 2011, by and among us, F. Hoffman-La Roche Ltd., Hoffman-La Roche Inc., or, together with F. Hoffman-La Roche Ltd, Roche, and, for the limited purposes set forth therein, the SMA Foundation under our SMA program. 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 $397.5 million in the aggregate. 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 one billion three hundred million U.S. dollars 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 June 2020, the CHMP recommended to remove the statement "efficacy has not been demonstrated in non-ambulatory patients" from the product label for Translarna. The CHMP's opinion is subject to final approval by the European Commission, which is typically provided within two months of the CHMP's recommendation. During the quarter ended June 30, 2020, we recognized $38.6 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 June 30, 2020, we recognized $36.2 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. As a result of the COVID-19 pandemic, our expected completion of Study 45 has been delayed as certain patients still require final study muscle biopsies. Once the clinical trial site is open for the necessary procedures and patients are able to safely travel to the site, we expect to be able to complete the final biopsies and the data from Study 045 would be available thereafter, followed by a potential re-submission of an NDA. 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 biologics license application, or BLA, for PTC-AADC for the treatment of AADC deficiency in the United States and we anticipate initiating the BLA submission to the FDA in the second half of 2020. 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 quarter of 2021.

Splicing Platform

We also have a spinal muscular atrophy, or SMA, collaboration with F. Hoffman-La Roche Ltd. and Hoffman-La Roche Inc., which we refer to collectively as Roche, and the Spinal Muscular Atrophy Foundation, or SMA Foundation. The lead compound in the SMA program is risdiplam (RG7916, RO7034067). Roche submitted an NDA for risdiplam to the FDA in the fourth quarter of 2019. In April 2020, we announced that the FDA extended the Prescription Drug User Fee Act, or PDUFA, date for a decision from May 24, 2020 to August 24, 2020. Roche submitted additional data to the FDA including comprehensive data from the SUNFISH part 2 study to help provide access to risdiplam for a broad range of people living with SMA, triggering this extension. Risdiplam is expected to be indicated in the United States for SMA type 1, 2 and 3 patients, if approved. Roche anticipates submitting an MAA for risdiplam for the treatment of SMA in the EEA imminently.

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, formerly known as PTC743, and PTC857. We expect to initiate a potential registrational Phase 2 placebo-controlled trial of vatiquinone in approximately 60 children with mitochondrial disease and associated refractory epilepsy in the third quarter of 2020. We also expect to initiate a potential registrational 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. We expect data from the single ascending dose and multiple ascending dose studies from the Phase 1 trial to be available by the end of 2020.

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.

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.

The Credit Agreement 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. As of June 30, 2020, we made loan repayments of $21.7 million on the Credit Agreement. On July 1, 2020, we terminated the Credit Agreement and repaid outstanding principal and accrued interest thereunder totaling $18.4 million and paid an additional $0.6 million in termination and exit fees. All liens and security interests securing the term loan made pursuant to the Credit Agreement were released upon termination.

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 six month period ending June 30, 2020, we issued and sold an aggregate of 368,514 shares of common stock pursuant to the Sales Agreement at a weighted average public offering price of $52.89 per share. We received net proceeds of $19.0 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 June 30, 2020, we had an accumulated deficit of $1,484.8 million. We had a net loss of $294.1 million and $113.9 million for the six month periods ended June 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 initiating the BLA submission to the FDA in the second half of 2020. We filed for marketing authorization for Waylivra with ANVISA 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. 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 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, 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 Merger Agreement. We are still required to pay an additional $2.4 million in development milestone payments upon the passing of the second anniversary of the closing of the Agilis acquisition, August 23, 2020, regardless of whether the applicable milestones have been achieved. 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 June 30, 2020 and 2019, net product sales outside of the United States were $39.0 million and $57.8 million respectively, and net product sales in the United States were $36.2 million and $27.6 million respectively. For the six months ended June 30, 2020 and 2019, net product sales outside of the United States were $79.8 million and $93.0 million, respectively, and net product sales in the United States were $63.6 million and $45.4 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 June 30, 2020, we had received a total of $62.5 million in milestone payments and no royalties on net sales pursuant to the License Agreement. As of June 30, 2020, the remaining potential research and development event milestones that can be received is $72.5 million. The remaining potential sales milestones as of June 30, 2020 is $325.0 million upon achievement of certain sales events.

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, which milestone payments equal $397.5 million in the aggregate. 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 one billion three hundred million U.S. dollars 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 six months ended June 30, 2020 and 2019.

	Three Months Ended June 30,	
	2020	2019
	(in thousands)	
Translarna (nmDMD, aniridia and Dravet)	$ 15,427	$ 19,021
PTC923	53,587	—
Gene Therapy	68,567	14,305
Bio-e	8,800	—
Oncology	7,030	5,833
Emflaza	4,717	7,381
Other research and preclinical	18,397	13,439
Total research and development	$ 176,525	$ 59,979

	Six Months Ended June 30,	
	2020	2019
	(in thousands)	
Translarna (nmDMD, aniridia and Dravet)	$ 52,735	$ 34,778
PTC923	53,587	—
Gene Therapy	105,349	25,740
Bio-e	14,356	—
Oncology	12,844	13,204
Emflaza	9,918	13,382
Other research and preclinical	17,843	25,440
Total research and development	$ 266,632	$ 112,544

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 income earned on investments and interest expense from the Convertible Notes outstanding and interest expense from the Credit Agreement.

Critical accounting policies and significant judgments and estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our financial statements, which we have prepared in accordance with generally accepted accounting principles in the United States. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported revenues and expenses during the reporting periods. Actual results may differ from these estimates under different assumptions or conditions.

During the three and six months ended June 30, 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 June 30, 2020 compared to three months ended June 30, 2019

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

(in thousands)	Three Months Ended June 30, 2020	2019	Change 2020 vs. 2019
Net product revenue	$ 75,239	$ 85,476	$ (10,237)
Collaboration and grant revenue	—	46	(46)
Cost of product sales, excluding amortization of acquired intangible asset	5,304	3,211	2,093
Amortization of acquired intangible asset	8,731	6,575	2,156
Research and development expense	176,525	59,979	116,546
Selling, general and administrative expense	53,659	49,215	4,444
Change in the fair value of deferred and contingent consideration	7,680	5,300	2,380
Settlement of deferred and contingent consideration	10,613	—	10,613
Interest expense, net	(5,379)	(2,074)	(3,305)
Other income (expense), net	11,309	(183)	11,492
Income tax expense	(84)	(774)	690

Net product revenues. Net product revenues were $75.2 million for the three months ended June 30, 2020, a decrease of $10.2 million, or 12%, from $85.5 million for the three months ended June 30, 2019. The decrease in net product revenue was due to the 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 quarter of 2019 included a material group purchase order from Brazil. This decrease was partially offset by an 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.

Collaboration and grant revenues. Collaboration and grant revenues remained relatively flat at $0.0 million for the three months ended June 30, 2020 and the three months ended June 30, 2019. Collaboration and grant revenues is related to our ongoing collaboration arrangements.

Cost of product sales, excluding amortization of acquired intangible asset. Cost of product sales, excluding amortization of acquired intangible asset, were $5.3 million for the three months ended June 30, 2020, an increase of $2.1 million, or 65%, from $3.2 million for the three months ended June 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.

Amortization of acquired intangible asset. Amortization of our intangible assets was $8.7 million for the three months ended June 30, 2020, an increase of $2.2 million, or 33%, from $6.6 million for the three months ended June 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 $176.5 million for the three months ended June 30, 2020, an increase of $116.5 million, or 194%, from $60.0 million for the three months ended June 30, 2019. The increase in research and development expenses includes $53.6 million in acquisition related and other expenses from the Censa Merger, $41.2 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 $53.7 million for the three months ended June 30, 2020, an increase of $4.4 million, or 9%, from $49.2 million for the three months ended June 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 $7.7 million for the three months ended June 30, 2020, an increase of $2.4 million, or 45%, from $5.3 million for the three months ended June 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 three months ended June 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 mixture 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 $5.4 million for the three months ended June 30, 2020, an increase of $3.3 million, or 159%, from $2.1 million for the three months ended June 30, 2019. The increase in interest expense, net was primarily due to interest expense recorded from the 2022 and 2026 Convertible Notes and the Credit Agreement, partially offset by interest income from our investments.

Other income (expense), net. Other income was $11.3 million for the three months ended June 30, 2020, an increase of $11.5 million, or over 100%, from other expense of $0.2 million for the three months ended June 30, 2019. The increase in other income (expense), net resulted primarily from a foreign exchange gain from the remeasurement of our intercompany loan, and an unrealized gain on our convertible debt security in ClearPoint Neuro, Inc. of $2.1 million.

Income tax expense. Income tax expense was $0.1 million for the three months ended June 30, 2020 compared to income tax expense of $0.8 million for the three months ended June 30, 2019. We 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. We are paying minimum income taxes in the United States because of incurred losses in the various state jurisdictions.

Six months ended June 30, 2020 compared to six months ended June 30, 2019

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

	Six Months Ended June 30,		
(in thousands)	**2020**	**2019**	**Change 2020 vs. 2019**
Net product revenue	$ 143,435	$ 138,530	$ 4,905
Collaboration and grant revenue	63	575	(512)
Cost of product sales, excluding amortization of acquired intangible asset	9,389	5,587	3,802
Amortization of acquired intangible asset	16,679	12,652	4,027
Research and development expense	266,632	112,544	154,088
Selling, general and administrative expense	111,869	89,760	22,109
Change in the fair value of deferred and contingent consideration	8,580	26,460	(17,880)
Settlement of deferred and contingent consideration	10,613	—	10,613
Interest expense, net	(11,021)	(4,362)	(6,659)
Other expense, net	(2,523)	(292)	(2,231)
Income tax expense	(306)	(1,350)	1,044

Net product revenues. Net product revenues were $143.4 million for the six months ended June 30, 2020, an increase of $4.9 million, or 4%, from $138.5 million for the six months ended June 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 quarter of 2019 included a material group purchase order from Brazil.

Collaboration and grant revenues. Collaboration and grant revenues were $0.1 million for the six months ended June 30, 2020 , a decrease of 0.5 million, or 89%, from $0.6 million for the six months ended June 30, 2019. The decrease in collaboration and grant revenues is related to our ongoing collaboration arrangements.

Cost of product sales, excluding amortization of acquired intangible asset. Cost of product sales, excluding amortization of acquired intangible asset, were $9.4 million for the six months ended June 30, 2020, an increase of $3.8 million, or 68%, from $5.6 million for the six months ended June 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.

Amortization of acquired intangible asset. Amortization of our intangible assets was $16.7 million for the six months ended June 30, 2020, an increase of $4.0 million, or 32%, from $12.7 million six months ended June 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 $266.6 million for the six months ended June 30, 2020, an increase of $154.1 million, or 137%, from $112.5 million for the six months ended June 30, 2019. The increase in research and development expenses includes $53.6 million in acquisition related and other expenses from the Censa Merger and $41.2 million related to our commercial manufacturing service agreement with 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 $111.9 million for the six months ended June 30, 2020, an increase of $22.1 million, or 25%, from $89.8 million for the six months ended June 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 $8.6 million for the six months ended June 30, 2020, a decrease of $17.9 million, or 68%, from $26.5 million for the six months ended June 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 six months ended June 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 $11.0 million for the six months ended June 30, 2020 , an increase of $6.7 million, or 153%, from $4.4 million for the six months ended June 30, 2019. The increase in interest expense, net was primarily due to interest expense recorded from the 2022 and 2026 Convertible Notes and the Credit Agreement, partially offset by interest income from our investments.

Other expense net. Other expense, net was $2.5 million for the six months ended June 30, 2020, an increase of $2.2 million, or over 100%, from other expense, net of $0.3 million for the six months ended June 30, 2019. The increase in other expense, net resulted primarily from unrealized losses on our equity investment and convertible debt security in ClearPoint Neuro, Inc. of $1.6 million and $0.7 million, respectively, Agilis Rights Exchange transaction fees of $2.0 million, and a foreign exchange loss from the remeasurement of our intercompany loan.

Income tax expense. Income tax expense was $0.3 million for the six months ended June 30, 2020 compared to income tax expense of $1.4 million for the six months ended June 30, 2019. We 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. We are paying minimum income taxes in the United States because of incurred losses in the various state 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 provides 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. The facility was structured to require only monthly interest payments for the initial two years with principal amortization beginning in years three and four. The facility 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%, as well as additional upfront and administrative fees and expenses. 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 six month period ending June 30, 2020, we issued and sold an aggregate of 368,514 shares of common stock pursuant to the Sales Agreement at a weighted average public offering price of $52.89 per share. We received net proceeds of $19.0 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 June 30, 2020, we had cash, cash equivalents and marketable securities of $498.9 million.

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

	Six Months Ended June 30,	
(in thousands)	**2020**	**2019**
Cash (used in) provided by:		
Operating activities	(156,364)	(72,931)
Investing activities	40,783	(188,669)
Financing activities	(14,428)	228,571

Net cash used in operating activities was $156.4 million for the six months ended June 30, 2020 and $72.9 million for the six months ended June 30, 2019. The net cash used in operating activities primarily relates to supporting clinical development and commercial activities.

Net cash provided by investing activities was $40.8 million for the six months ended June 30, 2020. Cash provided by investing activities for the six months ended June 30, 2020 was primarily related to net sales and redemptions of marketable securities.

Net cash used in investing activities was $188.7 million for the six months ended June 30, 2019 and was primarily related to net purchases of marketable securities.

Net cash used in financing activities was $14.4 million for the six months ended June 30, 2020. Cash used in financing activities for the six months ended June 30, 2020 was primarily attributable to payments on our deferred consideration obligation, our finance lease principal, and our senior secured term loan, partially offset by proceeds from our at the market equity offering and the exercise of options. Cash provided by financing activities was $228.6 million for the six months ended June 30, 2019. Cash provided by financing activities for the six months ended June 30, 2019 was primarily attributable to our equity offering in January 2019 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 initiating the BLA submission to the FDA in the second half of 2020. We filed for marketing authorization for Waylivra with ANVISA 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. 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 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 June 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, not yet commenced (1)	$ 111,527	$ 5,387	$ 13,419	$ 13,961	$ 78,760
Operating lease obligations (2)	25,034	3,361	6,976	7,329	7,368
Finance lease obligations (3)	39,000	6,000	6,000	6,000	21,000
Total contractual obligations	$ 175,561	$ 14,748	$ 26,395	$ 27,290	$ 107,128

(1) Obligations stem from our lease agreement entered into with Bristol-Myers Squibb Company in August 2019 relating to the lease of approximately 185,000 square feet of office, manufacturing and laboratory space at a facility located in Hopewell Township, New Jersey. On March 25, 2020, we entered into an amendment increasing the rented space to approximately 220,500 square feet. The rental term of the lease commenced on July 1, 2020 and is accordingly reflected as not yet commenced as of June 30, 2020.(2) Obligations stem from our lease agreement entered into 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 our existing lease on the property beginning on May 1, 2020 and includes additional rental property of approximately 59,000 square feet.

(3) 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 June 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 June 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 June 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 June 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 factor 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 conditions 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, we cannot ensure that our patients are able to safely travel to our clinical trial site at the University of California, Los Angeles, which has also experienced intermittent discontinuations of certain elective procedures, further complicating our patients' ability to have final study muscle biopsies performed. During the delay, patients are remaining on drug until the biopsies can be performed. Once the clinical trial site is open for the necessary procedures and patients are able to safely travel to the site, we expect to complete the final biopsies and the data from Study 045 would be available thereafter, followed by a potential re-submission of an NDA, however, the current pandemic may cause additional unforeseen delays. 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. The response to the COVID-19 pandemic may 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. In addition, 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. 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 6. Exhibits.

Exhibit Number	Description of Exhibit
2.1†	Agreement and Plan of Merger, dated May 5, 2020, by and among PTC Therapeutics, Inc., Hydro Merger Sub, Inc., Censa Pharmaceuticals Inc. and, solely in its capacity as securityholder representative, Shareholder Representative Services LLC (incorporated by reference to Exhibit 2.1 to the Current Report on Form 8-K filed by the Registrant on May 6, 2020)
10.1†	License Agreement dated as of February 8, 2016, as amended, by and between Shiratori Pharmaceutical Co. Ltd. and Censa Pharmaceuticals Inc.
10.2†	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
10.3+	Employment Agreement, as amended, between the Registrant and Matthew Klein
10.4+	Employment Agreement, as amended, between the Registrant and Eric Pauwels
10.5†	Rights Exchange Agreement, by and among PTC Therapeutics, Inc., the Rightholders set forth therein, and, for the limited purposes set forth therein, Shareholder Representatives Services LLC, dated as of April 29, 2020 (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed by the Registrant on April 30, 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.

+ Management contract, compensatory plan or arrangement.

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

SIGNATURES

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

PTC THERAPEUTICS, INC.

Date: August 5, 2020

By: /s/ Emily Hill

Emily Hill
Chief Financial Officer
(Principal Financial Officer and Duly Authorized Signatory)

EXHIBIT 10.1

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) would likely cause competitive harm to the Company, if publicly disclosed. Double asterisks denote omissions.

LICENSE AGREEMENT

DATED AS OF FEBRUARY 8TH, 2015

BETWEEN

SHIRATORI PHARMACEUTICAL CO., LTD.

AND

CENSA PHARMACEUTICALS INC.

LICENSE AGREEMENT

THIS LICENSE AGREEMENT (the "Agreement"), dated as of February 8th, 2016 (the "Effective Date"), is made by and between Shiratori Pharmaceutical Co., Ltd., a Kabushiki Kaisha (KK) corporation organized and existing under the laws of Japan and having its principal office at 2-3-7 Akanehama, Narashino-shi, Chiba-ken, 275-0024 JAPAN ("Shiratori") and Censa Pharmaceuticals Inc., a corporation organized and existing under the laws of the State of Delaware and having its principal office at 222 Third Street, Suite 2240, Cambridge, Massachusetts 02142 USA ("Censa"). Capitalized terms not otherwise defined in this Agreement shall have the meanings set forth in Article 1.

WITNESSETH:

WHEREAS, Shiratori has developed the Product and is the owner of, or has the right to license to Censa, certain Licensed Technology relating thereto;

WHEREAS, manufacture of the Product involves the practice of patented inventions and relies on know-how developed at the expense of Shiratori;

WHEREAS, Censa is a specialty pharmaceutical company focusing on development and commercialization of innovative pharmaceutical drug products for the treatment of Central Nervous System (CNS) diseases;

WHEREAS, Shiratori wishes to serve as supplier of the Product for clinical and commercial purposes;

WHEREAS, Censa wishes to gain access to the Product for use in connection with and furtherance of its development and commercialization;

WHEREAS, Shiratori *qua* licensor wishes to grant a license to Censa under the Licensed Technology to research, have researched, develop, have developed, make, have made, use, import, market, have marketed, offer for sale, sell and have sold the Product in the Territory; and

WHEREAS, Censa *qua* licensee wishes to obtain such a license from Shiratori, subject to the terms and conditions set forth in this Agreement;

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

ARTICLE 1.
DEFINITIONS

When used in this Agreement, each of the following capitalized terms shall have the meanings set forth in this Article 1.

1.1 "Affiliate" means any corporation, company, partnership or other entity which controls, is controlled by, or is under common control with a specified entity. For purposes of this definition, "control" shall be presumed to exist where one entity owns directly or indirectly more than fifty percent (50%) (or such lesser percentage as may be the maximum percentage allowed to be owned by a foreign corporation under the applicable laws or regulations of a particular jurisdiction outside of the United States) of the equity having the power to vote in the election of directors or to direct the management and policies of another entity.

1.2 "Business Day" means any day except a Saturday, Sunday, or any other day on which a commercial bank in Tokyo, Japan or Boston, Massachusetts is authorized to close. Any reference in this Agreement to "day" whether or not capitalized shall refer to a calendar day, not a Business Day.

1.3 "Commercially Reasonable Efforts" means efforts and resources commonly used by a Party for a product owned by it or to which it has rights at a similar stage in its development or product life and of similar market potential taking into account efficacy, safety, the anticipated Regulatory Authority approved labeling, the competitiveness of alternative products in the marketplace or under development, the patent and other proprietary position of the product, the likelihood of Regulatory Approval, the profitability of the product and other relevant factors.

1.4 "Confidential Information" means all proprietary materials, know-how or other information (whether or not patentable) regarding a Party's technology, products, business information or objectives, which is designated as confidential in writing by the Disclosing Party, whether by letter or by the use of an appropriate stamp or legend, prior to or at the time any such material, know-how or other information is disclosed by the Disclosing Party to the other Party. Notwithstanding anything contained in the foregoing to the contrary, materials, know-how or other information which is orally, electronically or visually disclosed by a Party, or is disclosed in writing without an appropriate letter, stamp or legend, shall constitute Confidential Information of a Party (i) if the Disclosing Party, within [**] days after such disclosure, delivers to the other Party a written document or documents describing the materials, know-how or other information and referencing the place and date of such oral, visual, electronic or written disclosure and the names of the persons to whom such disclosure was made, or (ii) such information is of the type that is customarily considered to be confidential information by persons engaged in activities that are substantially similar to the activities being engaged in by the Parties.

1.5 "Controls" or "Controlled" means with respect to the Licensed Technology, ownership or the possession of a respective license prior to or during the term of this Agreement and the ability to grant licenses or sublicenses without violating applicable laws or the terms of any agreement or other arrangement with, or the rights of, any Third Party.

1.6 "Effective Date" means the date set forth in the preamble to this Agreement.

1.7 "European Union" means, from time to time, those countries that are members of the European Union.

1.8 "Evaluation Period" means the period of [**] months commencing with the Effective Date.

1.9 "FDA" means the United States Food and Drug Administration, or any successor agency thereof.

1.10 “First Commercial Sale” means, with respect to any Product approved for commercial sale, the first arm's length sale by Censa or its Affiliates or sublicensees of such Product to a Third Party.

1.11 “Force Majeure” means any occurrence beyond the reasonable control of a Party that prevents or substantially interferes with the performance by the Party of any of its obligations hereunder, if such occurs by reason of any act of God, flood, fire, explosion, earthquake, strike, lockout, labor dispute, casualty or accident; or war, revolution, civil commotion, acts of public enemies, blockage or embargo; or any injunction, law, order, proclamation, regulation, ordinance, demand or requirement of any government or of any subdivision, authority or representative of any such government; or breakdown of plant, inability to procure or use materials, labor, equipment, transportation, or energy sufficient to meet manufacturing needs without the necessity of allocation; or any other cause whatsoever, whether similar or dissimilar to those above enumerated, beyond the reasonable control of such Party, if and only if the Party affected shall have used reasonable efforts to avoid such occurrence and to remedy it promptly if it shall have occurred.

1.12 “IND” means an Investigational New Drug Application, as defined in the U.S. Federal Food, Drug, and Cosmetic Act, as amended, and the regulations promulgated thereunder, and its foreign equivalent for initiating clinical trials in the United States or any corresponding foreign country.

1.13 “Licensed Know-How” means the following to the extent owned or Controlled by Shiratori and/or its Affiliates (a) as of the Effective Date or (b) arising at any time during the term of this Agreement solely by Shiratori or jointly by Shiratori and Censa: technical, scientific and other know-how, data, materials, information, trade secrets, ideas, formulae, inventions, discoveries, processes, protocols, techniques, manufacturing processes, equipment for use in manufacturing and testing, sources of supply of components and raw materials and results of experimentation and testing as are reasonably necessary or useful for the research, development, registration, packaging, manufacture, use, distribution, or sale of the Product in the Territory, and shall include, without limitation, all data related to pre-clinical data, chemistry manufacturing control (CMC) data, regulatory information, reports, studies, clinical studies, processes and pre-clinical, chemistry manufacturing control (CMC), and other data. Licensed Know-How excludes Licensed Patents.

1.14 “Licensed Patents” means the following patent applications and patents owned or Controlled by Shiratori (a) as of the Effective Date or (b) arising at any time during the term of this Agreement solely by Shiratori or jointly by Shiratori and Censa, and all patents issuing from such patent applications and patents and otherwise arising from any improvement or enhancement of the manufacturing processes for or methods related to Sepiapterin, during the term of this Agreement: (i) [**] (ii) [**] entitled [**]; and (iii) certain other patents owned by Shiratori, as are reasonably necessary or useful for the manufacture, use, and sale of the Product in the Territory, including the patent applications and/or patents listed on Schedule 6.2 (b) attached hereto, and all foreign counterparts of any or to any of the aforesaid patents and/or patent applications, and including, without limitation, as it may relate to any such patent applications and patents all provisional applications, substitutions, continuations, continuations-in-part, divisions, and renewals, all letters patent granted thereon, and

all patents-of-addition, reissues, reexaminations and extensions or restorations by existing or future extension or restoration mechanisms, certificates of invention or applications for certificates of invention, all supplementary protection certificates, substitutions, confirmations, registrations, revalidations, additions, continuations, continuations in-part, and divisions of any or to any of the aforesaid patent applications and patents. Licensed Patents excludes Licensed Know-How.

1.15 "Licensed Technology" means the Licensed Know-How and the Licensed Patents.

1.16 "NDA" means a New Drug Application pursuant to 21 U.S.C. Section 355(b)(1), as amended, and the regulations promulgated thereunder, submitted to the FDA or any successor application or procedure and any foreign counterpart of a U.S. New Drug Application for approval to market, including, where applicable, applications for pricing and reimbursement approval.

1.17 "Net Sales" means the gross amount invoiced by Censa and/or its Affiliates and/or sublicensees on account of sales of the Product to Third Parties (including without limitation Third Party distributors and wholesalers), less the total of:

(a) Trade, cash, quantity or other discounts not already reflected in the invoice price;

(b) Excise, sales and other consumption taxes (including VAT on the sale of the Product) and custom duties to the extent included in the invoice price;

(c) Freight, insurance and other transportation charges to the extent included in the invoice price;

(d) Amounts repaid or credited by reason of rejections, defects, recalls or returns or because of chargebacks, retroactive price reductions, refunds or billing errors; and

(e) Compulsory payments and rebates directly related to sales of the Product, accrued, paid or deducted pursuant to agreements (including, but not limited to, managed care agreements) or governmental regulations.

Use of the Product for promotional or sampling purposes and for use in clinical trials contemplated under this Agreement shall not be considered in determining Net Sales. In the case of any sale of the Product between or among Censa and its Affiliates or sublicensees for resale, Net Sales shall be calculated as above only on the value charged or invoiced on the first arm's length sale thereafter to a Third Party.

1.18 "Party" means Censa or Shiratori each individually as the case may be, and "Parties" means Censa and Shiratori together collectively.

1.19 "Person" means any individual, firm, corporation, partnership, limited liability company, trust, unincorporated organization or other entity or a government agency or political thereto, and shall include any successor (by merger or otherwise) of such Person.

1.20 "Product" means any final pharmaceutical product in finished form containing Sepiapterin as active pharmaceutical ingredient (API) covered by Licensed Patents or using Licensed Know-How.

1.21 "Drug Substance" means Sepiapterin for use in the Product.

1.22 “Regulatory Approval” means the technical, medical and scientific licenses, registrations, authorizations and approvals (including, without limitation, IND's, approvals of NDAs, supplements and amendments, pre- and post- approvals, pricing and Third Party reimbursement approvals, and labeling approvals, together with all supplements thereto) of any national, supra-national, regional, state or local regulatory agency, department, bureau, commission, council or other governmental entity, necessary for the development, manufacture, distribution, marketing, promotion, offer for sale, use, import, export or sale of Product(s) in a regulatory jurisdiction.

1.23 “Regulatory Authority” means any national (*e.g.*, the FDA), supra-national (*e.g.*, the European Commission, the Council of the European Union, or the European Agency for the Evaluation of Medicinal Products), regional, state or local regulatory agency, department, bureau, commission, council or other governmental entity in each country of the Territory involved in the granting of Regulatory Approval.

1.24 “Regulatory Filings” means, collectively, INDs, Biologics License Applications, Drug Master Files, NDAs and any other comparable filings as may be required by Regulatory Authorities to obtain Regulatory Approvals.

1.25 “Territory” means all the countries and territories of the world outside of Japan.

1.26 “Third Party(ies)” means any Person other than Shiratori, Censa, and their respective Affiliates.

1.27 “Valid Claim” means a claim of an issued and unexpired patent or supplementary protection certificate within the Licensed Patents, which claim or supplementary protection certificate has not been held invalid or unenforceable by a court or other government agency of competent jurisdiction from which no appeal can be taken as of right or has been taken and has not been admitted to be invalid, canceled, or unenforceable through re-examination or disclaimer, opposition procedure, nullity suit.

1.28 Additional Definitions. Each of the following terms is defined in the section of this Agreement indicated below:

Term	Section
Breach Notice	9.3
Breaching Party	9.3
Censa Introductory Paragraph	
Censa Indemnified Parties	8.2
Censa Inventions	10.1(a)
Development Plan	3.1
Disclosing Party	7.1
Initial Term	3.8(a)
Insolvent Party	9.5
Inventions	10.1
Joint Inventions	10.1(c)
Joint Manufacturing Committee or JMC	3.5
Manufacturing Cost	3.6
Marks	10.7

[**] 2.4
Non-Breaching Party 9.3
Paragraph IV Notice 10.3(e)
Receiving Party 7.1
Services Agreement 3.7
Shiratori Introductory Paragraph
Shiratori Indemnified Parties 8.1
Shiratori Inventions 10.1(b)
Supply Agreement 3.8
Withholding Taxes 5.6

ARTICLE 2.
LICENSES

2.1 License Grants to Censa. Shiratori hereby grants to Censa the sole and exclusive, even as to Shiratori, worldwide right and license, with the right to sublicense, under the Licensed Technology to research, have researched, develop, have developed, use, import, export, market, have marketed, offer for sale, sell and have sold, and otherwise commercialize the Product in the Territory.

2.2 Sublicense Rights. Censa shall have the right in its sole discretion to grant sublicenses and/or to transfer this Agreement without consent by Shiratori. In the event of sublicense by Censa, Censa promptly shall notify Shiratori in writing of the granting of such sublicense. Each sublicense granted by Censa shall not conflict with the terms of this Agreement and Censa shall be responsible for the performance of each sublicensee of all obligations incumbent on Censa under this Agreement. In addition, Censa may, without Shiratori's consent, appoint distributors and sub-distributors throughout the Territory to assist in the commercialization of the Product if those distributors and sub-distributors do not require a sublicense under the License to commercialize the Product; *provided, however*, that no such appointment may adversely impact payments due from Censa to Shiratori.

2.3 Disclosure of Licensed Know-How. To the extent Shiratori has not already done so, promptly after the Effective Date, Shiratori shall make available to Censa the Licensed Know-How not previously furnished to Censa. During the term of this Agreement, Shiratori shall disclose Licensed Know-How promptly to Censa as soon as such Licensed Know-How becomes available to Shiratori.

2.4 Additional Rights. Upon Censa's written request, Shiratori shall make Commercially Reasonable Efforts to assist Censa in obtaining the exclusive license to certain intellectual property owned or Controlled by [**] related to the clinical use of Sepiapterin, including but not limited to [**] (the "[**]"). In the event that Censa obtains the [**], Censa shall sublicense licensed rights to Shiratori in Japan. The Parties equitably shall share the cost of obtaining the [**] on a pro-rata basis to be mutually determined.

ARTICLE 3.
DEVELOPMENT AND COMMERCIALIZATION

3.1 Development.

(a) During the Evaluation Period, (i) Censa shall conduct initial pre-clinical studies in order to determine the feasibility of the Product as provided for in the "Development Plan" set forth as Appendix A to and incorporated by reference in this Agreement and (ii) Shiratori shall supply to Censa the amount of Drug Substance reasonably necessary, and in any event at least [**] grams of Drug Substance, to conduct such studies.

(b) From and after the Effective Date, Censa shall have full legal and financial responsibility for all costs that are incurred and all activities that are undertaken after the signing of this Agreement, which are related to the development, safety, Regulatory Filings, required periodic reporting to the Regulatory Authorities, Regulatory Approvals, and other activities required by Censa or its sublicensee(s) (or their respective agents or distributors) to obtain appropriate Regulatory Approvals for, and to commercialize, the Product in the Territory. Other than as expressly provided for in this Agreement, Censa shall not assume, nor shall Censa be liable for, any costs or activities (whether scientific, financial or otherwise) relating to the Product that were incurred or undertaken prior to the signing of this Agreement (including without limitation any costs, expenses, damages, losses, fines, penalties or the like that may be awarded or assessed after the signing of this Agreement, but which arise out of the events and activities that occurred prior to the signing of this Agreement)

(c) Provided that the Affiliates, sublicensees and other Third Parties agree to substantially the same terms of confidentiality in Article 7, Censa may appoint such Affiliates, sublicensee(s) and other Third Parties to perform any and all development activities necessary to obtain Regulatory Approvals for the Product in the Territory.

(d) Censa shall, in a manner consistent with the effort Censa devotes to its own products having the same or similar potential value as the Product, exercise its Commercially Reasonable Efforts and diligence in developing and commercializing the Product, and in undertaking those investigations and actions required to obtain appropriate Regulatory Approvals to market the Product in the Territory. All such activity shall be undertaken at Censa's expense. Shiratori shall use reasonable efforts to assist or to provide consultation at Censa's expense in support of the pre-clinical and clinical development of the Product.

3.2 Regulatory Matters. Censa shall have complete control over, and authority and responsibility for, the regulatory strategies relating to the development and commercialization of the Product in the Territory and shall be responsible for all Regulatory Filings and Regulatory Approvals relating to the Product immediately after the Effective Date. Shiratori shall transfer to Censa or take other action necessary to make available to Censa any Regulatory Filings and Regulatory Approvals (and updates thereof) initially filed by Shiratori. Censa shall oversee, monitor and coordinate all regulatory actions, communications and filings with and submissions, including filings and submissions of supplements and amendments thereto, to any Regulatory Authority with respect to the Product. Shiratori shall provide to Censa on a timely basis all necessary information to enable compliance with all regulatory obligations on a global basis, including without limitation filing updates, records related to product manufacturing and processes, pharmacovigilance filings, and investigator notifications. Censa shall be responsible for interfacing, corresponding and meeting with all Regulatory Authorities with respect to the Product. Shiratori shall cooperate with Censa to provide

all reasonable assistance and take all actions reasonably requested by Censa that are necessary or desirable (a) to enable Censa to comply with any law applicable to the Product, including, but not limited to, report adverse drug experience reports (and serious adverse drug experience reports) to Regulatory Authorities and (b) to coordinate with Censa regarding regulatory, development, manufacturing, and related status of the Product, Drug Substance, and its precursors in Japan.

3.3 Commercialization of Product. Censa shall have the sole discretion and exclusive right to commercialize, sell, market, promote and distribute the Product in the Territory.

3.4 Censa Access to Development Information and Shiratori Personnel and Consultants. Shiratori shall make available to Censa all relevant and readily available material and information related to Drug Substance, including but not limited to process development and manufacturing quality assurance (QA) / quality control (QC) documentation, GMP and government inspection reports and certifications, preclinical data, clinical data, and regulatory filings and correspondence, within the reasonable control of Shiratori, as soon as reasonably practicable and in any event within [**] days of execution and delivery of a this Agreement.

3.5 Joint Manufacturing Committee and Joint Concerns. The Parties shall establish a Joint Manufacturing Committee (the "JMC") comprised of an equal number of members from each Party. The JMC shall manage the overall relationship between the Parties, including chemistry manufacturing and control information in support of Regulatory Filings for Regulatory Approval of the Product in the Territory and forecast for supply of Drug Substance. In furtherance of the overall relationship between the Parties in general and Drug Substance supply in particular, whether for development-phase purposes under Section 3.6 below or commercialization-phase purposes under Section 3.8 below, Shiratori promptly and continuously shall inform the JMC of any and all discoveries, improvements, inventions, intellectual property or other proprietary rights, title, and interest that arise (including without limitation invention or similar disclosures) in Japan relevant to the Product, Licensed Patents, or Licensed Know-How. Improvements by Censa to the manufacturing method specifically described in the Licensed Patent set forth in Section 1.14(i) above that are dominated by the claims of such Licensed Patent shall be communicated by Censa to Shiratori and may be used solely by Shiratori without compensation to Censa. Pre-clinical and clinical data developed by either of the Parties in connection with development and use of the Product shall be communicated between the Parties and may be used as the case may be (i) by Censa in the Territory and (ii) by Shiratori in Japan, respectively, without compensation by either Party to the other Party. The JMC shall establish a process for joint evaluation relative to the relationship between the Parties and performance of this Agreement, including without limitation as it may relate to patent filing, prosecution, maintenance, and enforcement pursuant to this Agreement.

3.6 Development Phase Drug Substance Supply.

(a) Shiratori shall be solely responsible for supplying Drug Substance for manufacturing pre-clinical and clinical supplies of Product.

(b) Shiratori shall supply Drug Substance solely to Censa and shall not, either directly or indirectly, market or sell Drug Substance anywhere in the Territory, excluding only the supply of Drug Substance to Censa under this Agreement. Censa shall procure Drug Substance solely from Shiratori during the development phase. For clarity and the avoidance of doubt, (i) Shiratori shall not supply Drug Substance to any Third Party

and (ii) Censa shall not procure Drug Substance from any Third Party during the development phase.

(c) Shiratori may use qualified Third-Party subcontractors to manufacture Drug Substance with the prior written approval of Censa.

(d) Shiratori shall supply Drug Substance to Censa in sufficient quantities for Censa to conduct its Development Plan (as set forth in Appendix A) at a price determined by the lowest of (i) the Manufacturing Cost plus [**] percent ([**]%) or (ii) the following respective amounts: Shiratori's "Manufacturing Cost" for this purpose, whether manufactured directly by Shiratori or through approved subcontractors, shall mean the direct materials cost, direct labor cost, and reasonably allocated manufacturing overhead consistent with generally accepted accounting principles (GAAP) in the contract manufacturing of pharmaceutical products.

Product	Amount of Drug Substance (as denominated in grams or kilograms)	Price to Censa
Non-GLP	[**]g	¥ [**]
GLP	[**]g	¥ [**]
cGMP	[**]g	¥ [**]
cGMP	[**]Kg	¥ [**]
cGMP	[**]Kg	¥ [**]

For clarity and the avoidance of doubt, in no event shall the actual price paid by Censa exceed the applicable amount set forth in the "Price to Censa" column of the table immediately above.

(e) The dates for the actual supply of Drug Substance during the Evaluation Period and the development phase shall be agreed upon between the Parties but in any event Shiratori shall not be obligated to supply Drug Substance earlier than the following dates: (i) For the [**]g of Drug Substance for non-GLP Product, [**] months from the Effective Date, (ii) for the [**]g of Drug Substance for GLP and/or cGMP Product as the case may be, [**] months from the Effective Date, or (iii) For [**]Kg of Drug Substance for cGMP Product, [**] months from the Effective Date.

(f) From and after the Effective Date, Shiratori shall work with Censa in good faith to improve Drug Substance manufacturing processes, manufacturing efficiencies and yield, and to reduce the overall Manufacturing Cost of Drug Substance.

(g) As soon as reasonably practicable, and in any event within [**] months from the Effective Date, Shiratori and Censa mutually shall execute and deliver a quality agreement providing for the quality of Drug Substance being supplied by Shiratori to Censa with related provisions customary for an agreement of that nature.

3.7 Development Assistance Services Agreement. In the event Censa desires to have Shiratori provide advisory assistance or other services not otherwise expressly provided for in thisAgreement, and Shiratori agrees to do so, then the parties will endeavor to negotiate and enter into a definitive agreement on mutually acceptable terms and conditions, providing for the specified services to be performed by Shiratori (the "Services Agreement") to be more fully described in one or more Scopes of Work, each Scope of Work to provide the basis for the payment by Censa to Shiratori for the services to be rendered by Shiratori and estimating the cost to Censa (provisions for determining

the cost shall be negotiated and included in the Services Agreement). Neither Party will have any legal obligation to enter into any Services Agreement. If a Services Agreement is entered into, then neither Party will have any legal obligation to enter into any Scope of Work thereunder.

3.9 Commercialization Phase Drug Substance Supply.

(a) During the Evaluation Period the Parties shall negotiate in good faith the terms and conditions of a commercial-scale Drug Substance supply agreement (the "Supply Agreement"). The conditions to be negotiated shall include customary terms and conditions as may be found in comparable contract manufacturing agreements in the pharmaceutical industry, including customary rights for audit, inspection, quality systems assurance, forecasting and ordering, and governance of the Supply Agreement.

(b) For clarity and the avoidance of doubt, in no event shall any actual price to be paid by Censa under the Supply Agreement exceed the applicable amount set forth in the "Price to Censa" column of the table set forth in Section 3.6(d) above or Manufacturing Cost plus [**] percent ([**]%), whichever amount is less. Notwithstanding the foregoing sentence, if Shiratori notifies Censa in writing during the Evaluation Period that it is not interested in entering into the Supply Agreement, then Censa shall have all rights provided for in Section 3.9 below including without limitation the right to enter into a commercial supply agreement with a Third Party.

3.9 Failure to Supply and Second Source of Supply. The Supply Agreement shall contain failure to supply provisions pursuant to which Censa has the right to manufacture Drug Substance itself and/or to engage a Third Party to manufacture Drug Substance on Censa's behalf if Shiratori fails to supply Drug Substance pursuant to Censa's purchase orders. In the event such failure to supply continues for the period of time mutually set forth in the Supply Agreement, this Agreement shall be amended to include the license granted from Shiratori to Censa under Section 2.1 above the right to make and have made the Product in the Territory. The Supply Agreement also shall contain second source provisions pursuant to which Censa in any event has the right to supply Drug Substance for itself or through a qualified Third Party to meet marketing needs in the Territory.

ARTICLE 4.
INITIAL PAYMENT AND MILESTONE PAYMENTS

4.1 Milestone Payments. Each of the following amounts shall be payable by Censa to Shiratori in U.S. Dollars within [**] days following confirmation by Censa that the specified event has occurred:

(a) US$[**] upon [**]

(b) US$[**] upon [**]

(c) US$[**] upon [**]

(d) US$[**] upon [**]

(e) US$[**] upon [**]

(f) US$[**] upon [**]

For clarity and the avoidance of doubt, the obligation of Censa (or its sublicensee, as the case may be) to pay the foregoing amounts shall apply and shall survive notwithstanding any sublicense by Censa under this Agreement.

4.2 Backup or Alternative Product. For clarity and the avoidance of doubt, it is understood that if, for any reason whatsoever, the development of the Product is discontinued in any indication prior to such Product achieving Regulatory Approval, the selection of a backup of such Product or a different Product for development in that same or a different indication shall not trigger the reimbursement by Shiratori or further payment by Censa of any milestone already paid with respect to the terminated Product.

ARTICLE 5.
ROYALTIES

5.1 Royalty Rates. In further consideration of the licenses granted in this Agreement, Censa shall pay to Shiratori royalties on the Product based on aggregate Net Sales of such Product on a country-by-country basis in the Territory in the amount of [**] percent ([**]%).

5.2 Term of Royalties. Royalties shall be payable for each full calendar year during the term of this Agreement and on an aggregate *pro rata* basis for the first and last year of commercialization during the term of this Agreement if such first and last years are not comprised of a full twelve (12) months, as follows:

(a) With respect to royalties payable for the countries of the European Union, until expiration of the last-to-expire Licensed Patent Controlled by Shiratori covering the European Union if the making, having made, using, offering to sell, selling or importing of such Product by Censa, its Affiliates or its sublicensees (or the distributors of any of them) in the absence of this Agreement, would infringe one or more Valid Claim of the Licensed Patents in the European Union.

(b) With respect to royalties payable for the United States, until the expiration of the last-to-expire Licensed Patent Controlled by Shiratori covering the United States if the making, having made, using, offering to sell, selling or importing of such Product by Censa, its Affiliates or its sublicensees (or the distributors of any of them) in the absence of this Agreement, would infringe one or more Valid Claim of the Licensed Patents in the United States.

(c) With respect to royalties payable for any other country in the Territory, until the expiration of the last-to-expire Licensed Patent Controlled by Shiratori covering the relevant country on a country-by-country basis if the making, having made, using, offering to sell, selling or importing of such Product by Censa, its Affiliates or its sublicensees (or the distributors of any of them) in the absence of this Agreement, would infringe one or more Valid Claim of the Licensed Patents in the relevant country.

5.3 No Obligation. Notwithstanding any other provision in this Agreement to the contrary, Censa shall have no obligation to launch any Product in any country. In addition, Censa may, in its sole discretion, discontinue its commercialization of any Product in any country.

5.4 Offset.

(a) In the event that Censa is required to pay a non-affiliated Third Party royalty or other amounts with respect to a Product under agreements for patent rights or other technologies that Censa in its reasonable judgment determines are necessary or desirable to license or acquire with respect to the Product or Licensed Technology (including without limitation amounts paid in connection with the [**]), and only if Censa obtains a prior written consent from Shiratori (which consent shall not be unreasonably, conditioned, delayed, or withheld by Shiratori) to pay such royalty or other amounts, then amounts owed by Censa to Shiratori under this Agreement will be reduced by the amounts paid by Censa to non-affiliated Third Parties; *provided, however*, that the maximum reduction shall be [**] percent ([**]%) of the amounts otherwise owed by Censa to Shiratori.

5.5 Reports and Payments.

(a) Cumulative Royalties. The obligation to pay royalties under this Article 5 shall be imposed only once (i) with respect to any sale of the same unit of Product and (ii) with respect to a single unit of Product regardless of how many Valid Claims or other Licensed Patents included in the Licensed Technology would, but for this Agreement, be infringed by the making, using or selling of such Product.

(b) Statements and Payments. Censa shall deliver to Shiratori within [**] days after the end of each calendar quarter a report setting forth for such calendar quarter the following information on a Product-by-Product basis and a country-by country basis: (i) Net Sales of such Product and (ii) the royalty due hereunder for the sale of such Product. The total royalty due for the sale of the Product during such calendar quarter shall be remitted within [**] business days from the time such report is made if and to the extent Censa actually then has received corresponding proceeds from applicable Net Sales, or, if and to extent Censa then has not received such proceeds, as soon as reasonably practicable after receiving such proceeds.

5.6 Taxes and Withholding. Any payments made by Censa to Shiratori under this Agreement shall be reduced by the amount that Censa is required to pay or withhold pursuant to any applicable law, including, but not limited to, foreign and United States federal, state or local tax law ("Withholding Taxes"). Any such Withholding Taxes required by law to be paid or withheld shall be an expense of, and borne solely by, Shiratori. Censa shall submit to Shiratori reasonable proof of payment of the Withholding Taxes within a reasonable period of time after such Withholding Taxes are remitted to the proper authority. The Parties shall reasonably cooperate in completing and filing documents required under the provisions of any applicable tax laws or under any other applicable law in connection with the making of any required tax payment or withholding payment, or in connection with any claim to a refund of or credit for any such payment. The Parties shall charge each other value-added taxes in accordance with the applicable law in addition to any consideration or payment agreed upon.

5.7 Currency Exchange. With respect to Net Sales invoiced in U.S. dollars, the Net Sales and the amounts due to Shiratori hereunder shall be expressed in U.S. dollars. With respect to Net Sales invoiced in a currency other than U.S. dollars, the Net Sales shall be expressed in the domestic currency of the entity making the sale, together with the U.S. dollar equivalent, calculated using the arithmetic average of the spot rates on the last Business Day of each month of the calendar

quarter in which the Net Sales were made. The "closing mid-point rates" found in the "Dollar spot forward against the Dollar" table published by *The Financial Times* or any other publication as agreed to by the Parties shall be used as the source of spot rates to calculate the average as defined in the preceding sentence. All payments shall be made in United States dollars. If at any time legal restrictions in any country in the Territory prevent the prompt remittance of any payments with respect to sales in that country, Censa shall have the right and option to make such payments by depositing the amount thereof in local currency to Shiratori's account in a bank or depository in such country.

5.8 Maintenance of Records; Audit. For a period of [**] years, Censa shall maintain and shall cause its Affiliates and sublicensees to maintain complete and accurate books and records in connection with the sale of the Product, as necessary to allow the accurate calculation of royalties due hereunder including any records required to calculate any royalty adjustments hereunder. [**] per calendar year, Shiratori shall have the right to engage an independent accounting firm acceptable to Censa, at Shiratori's expense, which shall have the right to examine in confidence the relevant Censa records as may be reasonably necessary to determine and/or verify the amount of royalty payments due hereunder. Such examination shall be conducted during Censa's normal business hours, after at least [**] days' prior written notice to Censa and shall take place at Censa's facility(ies) where such records are maintained. Each such examination shall be limited to pertinent books and records for any year ending not more than [**] months prior to the date of request. Before permitting such independent accounting firm to have access to such books and records, Censa may require such independent accounting firm and its personnel involved in such audit, to sign a confidentiality agreement (in form and substance reasonably acceptable to Censa) as to any of the confidential information of Censa, its Affiliates or sublicensees which is to be provided to such accounting firm or to which such accounting firm shall have access, while conducting the audit under this Section. Shiratori's independent accounting firm shall prepare and provide to both Shiratori and Censa a written report disclosing only whether the royalty reports submitted and royalties paid by Censa are correct or incorrect and the specific details concerning any discrepancies. No other information shall be provided to Shiratori. In the event there was an underpayment by Censa hereunder, Censa shall promptly (but in no event later than [**] days after Censa's receipt of the independent auditor's report so correctly concluding) make payment to Shiratori of any shortfall. In the event that there was an overpayment by Censa hereunder, Shiratori shall promptly (but in no event later than [**] days after Shiratori's receipt of the independent auditor's report so correctly concluding) refund to Censa the excess amount. In the event any payment by Censa shall prove to have been incorrect by more than [**] percent ([**]%) to Shiratori's detriment, Censa shall pay the reasonable fees and costs of Shiratori's independent auditor for conducting such audit.

ARTICLE 6.
REPRESENTATIONS, WARRANTIES, AND COVENANTS

6.1 Mutual Representations and Warranties. Each Party respectively hereby represents and warrants to the other Party that:

(a) such Party is a corporation duly organized, validly existing and in good standing under the laws of the state or other jurisdiction of incorporation or formation and has full corporate power and authority to enter into this Agreement and to carry out the provisions hereof;

(b) such Party is duly authorized, by all requisite corporate action, to execute and deliver this Agreement and the execution, delivery and performance of this Agreement by such Party does not require any shareholder action or approval, and the Person executing this Agreement on behalf of such Party is duly authorized to do so by all requisite corporate action;

(c) no consent, approval, order or authorization of, or registration, qualification, designation, declaration or filing with, any federal, state or local governmental authority is required on the part of such Party in connection with the valid execution, delivery and performance of this Agreement, except where the failure to obtain any of the foregoing would not have a material adverse impact on the ability of such Party to meet its obligations hereunder;

(d) this Agreement is a legal and valid obligation binding upon such Party and enforceable in accordance with its terms except as (i) enforceability may be limited by bankruptcy, insolvency, reorganization, moratorium or similar laws affecting the enforcement of creditors' rights and (ii) equitable principles of general applicability; and

(e) the execution, delivery and performance by it of this Agreement and its compliance with the terms and provisions of this Agreement does not and shall not conflict with or result in a breach of any of the terms or provisions of (i) any other contractual or other obligations of such Party, (ii) the provisions of its charter, operating documents or bylaws, or (iii) any order, writ, injunction or decree of any court or governmental authority entered against it or by which it or any of its property is bound except where such breach or conflict would not materially impact the Party's ability to meet its obligations hereunder.

6.2 Additional Shiratori Representations, Warranties, and Covenants. Shiratori represents, warrants, and covenants to Censa that:

(a) it has the full right, power and authority to grant the licenses granted to Censa under Article 2 hereof;

(b) all Licensed Patents included within the Licensed Technology which are existing as of the Effective Date are listed on Schedule 6.2(b) hereto and, as of the Effective Date, such Licensed Patents are existing and, to its knowledge, are not invalid or unenforceable, in whole or in part;

(c) except as previously disclosed to Censa in writing (i) it is the sole and exclusive owner or the exclusive Censa of the rights, title, and interest in and to the Licensed Technology, free and clear of any liens, charges or encumbrances, including, without limitation, all Licensed Patents included in the Licensed Technology, and (ii) no Third Party has any right, title or interest in or to the Licensed Technology;

(d) except as disclosed to Censa in writing (i) all inventors (who are known as of the Effective Date) of any inventions included within the Licensed Technology have assigned (or are deemed to have assigned by applicable law) their entire right, title and interest in and to such inventions and the corresponding Licensed Patents to Shiratori and (ii) no Person, other than those Persons named as inventors on any patent or patent

application included within the Licensed Technology, is an inventor of the invention(s) claimed in such patent or patent application;

(e) as of the Effective Date, there are no claims, judgments or settlements against, or owed by, Shiratori or, to its knowledge, pending or threatened claims or litigation relating to the Licensed Technology and during the term of this Agreement Shiratori shall promptly notify Censa in writing upon learning of any such actual or threatened claim, judgment or settlement;

(f) during the term of this Agreement, Shiratori shall use Commercially Reasonable Efforts not to diminish the rights under the Licensed Technology;

(g) except as previously disclosed to Censa in writing, as of the Effective Date it is not aware of any patent, patent application or other intellectual property right of any Third Party which could materially adversely affect the ability of either Party to carry out its respective obligations hereunder or the ability of Censa to exercise or exploit any of the rights or licenses granted to it under this Agreement;

(h) except as previously disclosed to Censa in writing, it has no knowledge of any material information which would negatively affect the ability of Censa to use the Licensed Technology;

(i) during the term of this Agreement, Shiratori shall not grant any right to any Third Party relating to any of the Licensed Technology which would conflict with, limit or adversely affect the rights granted to Censa hereunder;

(j) Shiratori shall fulfill its covenants in relation to the JMC as provided for in Section 3.5 above; and

(k) Drug Substance sold by Shiratori to Censa for use in preclinical development and clinical trials of the Product shall meet applicable specifications at the time of delivery, and shall have been manufactured in compliance with law applicable in the country of manufacture, the United States, and the European Union.

ARTICLE 7.
CONFIDENTIALITY; PUBLIC ANNOUNCEMENTS

7.1 Confidentiality. Except to the extent expressly authorized by this Agreement or otherwise agreed in writing, the Parties agree that, until [**] years after the expiration or termination of this Agreement, each Party (the "Receiving Party") receiving hereunder any Confidential Information of the other Party (the "Disclosing Party") or information of the other Party marked "Confidential" shall keep such information confidential and shall not publish or otherwise disclose or use for any purpose other than as provided for in this Agreement, except to the extent that it can be established by the Receiving Party that the Confidential Information:

(a) was already known to the Receiving Party, or its Affiliates (other than under an obligation of confidentiality) at the time of disclosure by the Disclosing Party, and such Receiving Party can so demonstrate by documentary evidence to that effect;

(b) was generally available to the public or otherwise part of the public domain at the time of its disclosure to the Receiving Party;

(c) became generally available to the public or otherwise part of the public domain after its disclosure and other than through any act or omission in breach of a confidentiality obligation;

(d) was disclosed to the Receiving Party or its Affiliates other than under an obligation of confidentiality by a Third Party who had no obligation to the Disclosing Party or its Affiliate not to disclose such information to others; or

(e) was independently discovered or developed by the Receiving Party or its Affiliates without the use of the Confidential Information belonging to the Disclosing Party or its Affiliates and the Receiving Party can so demonstrate by documentary evidence to that effect.

Each Party's Affiliates may receive, use and provide Confidential Information in the performance of the rights and obligations described in this Agreement.

7.2 Authorized Disclosure. Each Party may disclose Confidential Information belonging to the other Party to the extent such disclosure is reasonably necessary to:

(a) file or prosecute patent applications claiming inventions included within the Licensed Technology;

(b) prosecute or defend litigation;

(c) exercise rights hereunder provided such disclosure is covered by terms of confidentiality similar to those set forth in this Agreement; and

(d) comply with applicable governmental laws and regulations.

In the event a Party shall deem it necessary to disclose, pursuant to this Section 7.2, Confidential Information belonging to the other Party, such Party shall, to the extent possible, give reasonable advance notice of such disclosure to the other Party and take reasonable measures to ensure confidential treatment of such information. For clarity and the avoidance of doubt, Censa in any event may disclose (i) the existence of its license and related Product rights under this Agreement, (ii) under appropriate confidentiality protection the terms and conditions of this Agreement to current and potential investors, investment bankers and other financial advisors, and strategic partners, and (iii) as required by government and regulatory authorities including without limitation the United States Securities and Exchange Commission.

7.3 SEC Filings. The Parties shall agree in advance with each other on the terms of this Agreement to be redacted in any Securities and Exchange Commission filings.

7.4 Public Announcements. The Parties agree on the importance of coordinating their public announcements respecting this Agreement and the subject matter thereof. Neither Party shall make any public release, public statement or other disclosure concerning this Agreement or its terms, without the prior written consent and approval of the other Party, which approval shall not be unreasonably conditioned, delayed, or withheld. Notwithstanding the preceding sentence, each Party may make any disclosures, statements or releases regarding this Agreement or its terms as are required by applicable law; *provided, however*, that the Party seeking to make such disclosure gives the other Party at least [**] days prior written notice of such disclosure (unless a shorter time period for such

disclosure is required by applicable law) and provides such Party in good faith the right to review and comment upon such disclosure. In addition, Censa may disclose the existence and terms of this Agreement to its accountants, attorneys, and other professional advisors under a duty of confidentiality and to a Third Party under a duty of confidentiality in connection with any proposed or consummated financing or a proposed or consummated sale of all or substantially all of Censa's business to which this Agreement relates.

7.5 Survival. The provisions of this Article 7 shall survive termination or expiration of this Agreement.

ARTICLE 8.
INDEMNIFICATION

8.1 Indemnification by Censa. Censa agrees to defend Shiratori and its Affiliates at Censa's cost and expense, and shall indemnify and hold Shiratori and its Affiliates and their respective directors, officers, employees and agents (the "Shiratori Indemnified Parties") harmless from and against any losses, costs, damages, fees or expenses arising out of any Third Party claim relating to (i) any material breach by Censa of any of its representations, warranties or obligations pursuant to this Agreement, (ii) the negligence or willful misconduct of Censa in the performance of any of its obligations pursuant to this Agreement, or (iii) Censa's use of the Drug Substance or Product if and to the extent Drug Substance or Product meets or exceeds applicable written specifications. In the event of any such claim against the Shiratori Indemnified Parties by any Third Party, Shiratori shall promptly notify Censa in writing of the claim and Censa shall manage and control, at its sole expense, the defense of the claim and its settlement; provided that Censa shall not agree to any settlement that would admit liability on the part of Shiratori or involve relief other than the payment of money, without the approval of Shiratori, such approval not to be unreasonably conditioned, delayed, or withheld. The Shiratori Indemnified Parties shall cooperate with Censa and may, at their option and expense, be represented in any such action or proceeding. Censa shall not be liable for any litigation costs or expenses incurred by the Shiratori Indemnified Parties without Censa's prior written authorization. In addition, Censa shall not be responsible for the indemnification or defense of any Shiratori Indemnified Party arising from any negligent or intentional acts by any Shiratori Indemnified Party or the breach by Shiratori of any representation, warranty or obligation under this Agreement, or any claims compromised or settled without its prior written consent.

8.2 Indemnification by Shiratori. Shiratori agrees to defend Censa and its Affiliates at Shiratori's cost, and shall indemnify and hold Censa and its Affiliates and their respective directors, officers, employees and agents (the "Censa Indemnified Parties") harmless from and against any losses, costs, damages, fees or expenses arising out of any Third Party claim relating to (i) any material breach by Shiratori of any of its representations, warranties or obligations pursuant to this Agreement or (ii) the negligence or willful misconduct of Shiratori in the performance of any of its obligations pursuant to this Agreement. In the event of any claim against the Censa Indemnified Parties by any Third Party, Censa shall promptly notify Shiratori in writing of the claim and Shiratori shall manage and control, at its sole expense, the defense of the claim and its settlement; provided that Shiratori shall not agree to any settlement that would admit liability on the part of Censa or involve relief other than the payment of money, without the approval of Censa, not to be unreasonably conditioned, delayed, or withheld. The Censa Indemnified Parties shall cooperate with Shiratori and may, at their option and expense, be represented in any such action or proceeding. Shiratori shall not be liable for

any litigation costs or expenses incurred by the Censa Indemnified Parties without Shiratori's prior written authorization. In addition, Shiratori shall not be responsible for the indemnification or defense of any Censa Indemnified Party arising from any negligent or intentional acts by any Censa Indemnified Party, or the breach by Censa of any representation, warranty or obligation under this Agreement, or any claims compromised or settled without its prior written consent.

8.3 Insurance Proceeds. Any indemnification hereunder shall be made net of any insurance proceeds recovered by the indemnified Party; *provided, however*, that if, following the payment to the indemnified Party of any amount under this Article 8, such indemnified Party recovers any insurance proceeds in respect of the claim for which such indemnification payment was made, the indemnified Party shall promptly pay an amount equal to the amount of such proceeds (but not exceeding the amount of such indemnification payment) to the indemnifying Party.

8.4 Survival. The indemnification obligations set forth in this Article 8 shall survive the termination or expiration of this Agreement.

8.5 Insurance. Each Party further agrees to use its Commercially Reasonable Efforts to obtain and maintain commercial general liability insurance, including products liability insurance in timely fashion with respect to clinical-trial commencement, with reputable and financially secure insurance carriers or self-insurance, in such amounts and subject to such deductibles as the Parties may agree based upon standards prevailing in the industry at the time.

ARTICLE 9.
TERM AND TERMINATION

9.1 Term.

(a) Unless earlier terminated by mutual agreement of the Parties or pursuant to the provisions of this Article 9, this Agreement shall continue in full force and effect on a country-by-country and Product-by-Product basis until the obligation to pay royalties with respect to the sale of such Product in such country expires or this Agreement is earlier terminated in accordance with the terms hereof.

(b) On a country-by-country basis and on a Product-by-Product basis, upon the scheduled expiration (as contemplated in Section 5.2) of the obligation to pay Royalties with respect to the sale of such Product in such country, the licenses granted hereunder shall become non-exclusive, fully paid up, royalty-free, perpetual and irrevocable, notwithstanding subsequent expiration or termination of this Agreement.

9.2 Voluntary Termination.

(a) By Censa. At any time during the term of this Agreement, Censa may elect to terminate this Agreement upon sixty (60) days' prior written notice to Shiratori. Censa shall, as of the effectiveness of such termination, be relieved of any and all further obligations to make payments to Shiratori under this Agreement to the extent not accrued prior to such termination. Censa also shall be relieved of any and all further obligations with respect to patents and patent applications in the Territory

(b) By Shiratori. Unless otherwise agreed between the Parties in writing, Shiratori may elect to terminate this Agreement upon sixty (60) days' prior written notice to Censa in the event that Censa fails (i) to achieve Regulatory Approval in either the United States

or European Union for at least one indication within six (6) years or (ii) to launch the Product in the United States or European Union within seven (7) years from the Effective Date. Censa shall, as of the effectiveness of such termination, be relieved of any and all further obligations to make payments to Shiratori under this Agreement to the extent not accrued prior to such termination. Censa also shall be relieved of any and all further obligations with respect to patents and patent applications in the Territory.

9.3 Termination for Material Breach.

(a) Upon a material breach of this Agreement by Censa or Shiratori (in such capacity, the "Breaching Party"), the other Party (in such capacity, the "Non-Breaching Party") may provide written notice (a "Breach Notice") to the Breaching Party specifying the material breach. If the Breaching Party fails to cure such material breach during the [**] day period (or, if applicable, such longer period, but not to exceed [**] days, as would be reasonably necessary for a diligent party to cure such material breach, provided the Breaching Party has commenced and continues its diligent efforts to cure during the initial [**] day period following the date on which the Breach Notice is provided) following the date on which the Breach Notice is provided, then the other Party may terminate this Agreement immediately upon the expiration of the [**] day (or other longer) period upon written notice to the Breaching Party.

(b) Notwithstanding any other provisions of this Agreement to the contrary, the consequences of termination for material breach set forth above shall not become effective until after final and unappealable adjudication of the occurrence and failure to timely cure any such material breach.

9.4 Consequences of Termination or Material Breach.

(a) Voluntary Termination by Censa. In the event this Agreement is voluntarily terminated by Censa pursuant to Section 9.2(a) above, all licenses granted by Shiratori to Censa pursuant to Section 2.1 above shall terminate

(b) Voluntary Termination by Shiratori. In the event this Agreement is voluntarily terminated by Shiratori pursuant to Section 9.2(b) above, all licenses granted by Shiratori to Censa pursuant to Section 2.1 above shall terminate.

(c) Material Breach by Censa. In the event that Censa commits a material breach of this Agreement and such material breach is not cured in accordance with the provisions of Section 9.3 above, then at Shiratori's election to terminate this Agreement, all licenses granted by Shiratori to Censa pursuant to Section 2.1 shall terminate.

(d) Material Breach by Shiratori. In the event that Shiratori commits a material breach of this Agreement and such material breach is not cured in accordance with the provisions of Section 9.3 above, then Censa may in its discretion, terminate this Agreement, in which event all licenses granted by Shiratori to Censa pursuant to Section 2.1 above shall terminate, but Censa (in addition to its indemnification rights under Section 8.2 above) shall have the right to compensation from Shiratori for any and all losses, costs, damages, fees or expenses Censa and its Affiliates and sublicensees may incur as a result of such material breach. In the event Censa decides

not to terminate this Agreement for material breach by Shiratori, Censa shall have the right to continue to develop and to commercialize the Product provided that the royalties otherwise payable by Censa to Shiratori pursuant to Article 5 shall be reduced by [**] Percent ([**]%), and Censa shall also have the right to offset any costs it may incur as a result of such material breach against the amounts payable to Shiratori hereunder.

(e) Additional Remedies. The rights of the Non-Breaching Party set forth in paragraphs (b) or (c) of this Section 9.4 above shall be in addition to any other remedies to which the Non-Breaching Party may otherwise be entitled at law or equity for such breaches.

(f) Rights and Duties Upon Termination. Upon early termination of this Agreement, in its entirety or with respect to any country or Product, Censa shall notify Shiratori of the amount of Product that Censa, its Affiliates and sublicensees then have on hand for sale in such country(ies), the sale of which would, but for the termination, be subject to royalty. Censa, its Affiliates and sublicensees shall thereupon be permitted to sell that amount of Product, provided that Censa shall pay the royalty thereon to Shiratori at the time provided for in this Agreement.

9.5 Bankruptcy. Each Party may, in addition to any other remedies available to it by law or in equity, exercise the rights set forth below by written notice to the other Party (the "Insolvent Party"), in the event the Insolvent Party shall have become insolvent or bankrupt, or shall have made an assignment for the benefit of its creditors, or there shall have been appointed a trustee or receiver of the Insolvent Party or for all or a substantial part of its property, or any case or proceeding shall have been commenced or other action taken by or against the Insolvent Party in bankruptcy or seeking reorganization, liquidation, dissolution, winding-up arrangement, composition or readjustment of its debts or any other relief under any bankruptcy, insolvency, reorganization or other similar act or law of any jurisdiction now or hereafter in effect, or there shall have been issued a warrant of attachment, execution, restraint or similar process against any substantial part of the property of the Insolvent Party, and any such event shall have continued for [**] days undismissed, unbonded, or undischarged. All rights and licenses granted under or pursuant to this Agreement by Shiratori are, and shall otherwise be deemed to be, for purposes of Section 365 (n) of the U.S. Bankruptcy Code, licenses of rights to "intellectual property" as defined under Section 101 of the U.S. Bankruptcy Code. The Parties agree that Censa as licensee of such rights under this Agreement shall retain and may fully exercise all of its rights and elections under the U.S. Bankruptcy Code. The Parties further agree that, in the event of the commencement of a bankruptcy proceeding by or against Shiratori under the U.S. Bankruptcy Code, Censa shall be entitled to a complete duplicate of (or complete access to, as appropriate) any such intellectual property and all embodiments of such intellectual property, and same, if not already in the its possession, shall be promptly delivered to it (i) upon any such commencement of a bankruptcy proceeding upon its written request therefor, unless Shiratori elects to continue to perform all of its obligations under this Agreement or (ii) if not delivered under (i) above, upon the rejection of this Agreement by or on behalf of Shiratori upon written request therefor by Censa.

9.6 Liabilities. Expiration or termination of this Agreement shall not release either Party from any obligation or liability which shall have accrued at the time of termination, or preclude

either Party from pursuing all rights at law and in equity with respect to any breach under this Agreement. Notwithstanding the foregoing, except as may be the case under Article 8, neither Party shall be liable for punitive, exemplary or consequential damages incurred by the other Party arising out of any default under this Agreement.

ARTICLE 10.
INTELLECTUAL PROPERTY

10.1 Ownership of Intellectual Property. It is anticipated that during the course and as a result of the Parties' performance under this Agreement discoveries, improvements, inventions, writings, and other technology, whether or not patentable or copyrightable (collectively, "Inventions") may be conceived, made, or reduced to practice by the respective personnel of Censa and/or Shiratori. Ownership of Inventions shall be determined as follows:

(a) Any Inventions conceived, made, or reduced to practice solely by employees of or Third Parties acting on behalf of Censa or a Censa Affiliate or sublicensee ("Censa Inventions") shall be owned by Censa and recorded in a conception register and shall be submitted to Censa patent counsel. Censa promptly shall disclose to Shiratori the conception, making, or reduction to practice of Censa Inventions.

(b) Any Inventions conceived, made, or reduced to practice solely by employees of or Third Parties acting on behalf of Shiratori or a Shiratori Affiliate ("Shiratori Inventions") shall be owned by Shiratori and recorded in a conception register and shall be submitted to Shiratori patent counsel. Shiratori promptly shall disclose to Censa the conception, making, or reduction to practice of Shiratori Inventions.

(c) All Inventions that are conceived, made, or reduced to practice jointly by employees of Censa or a Censa Affiliate or sublicensee or Third Parties acting on their behalf, on the one hand, and Shiratori or a Shiratori Affiliate or Third Parties acting on their behalf, on the other hand, and which are not Censa Inventions or Shiratori Inventions, are "Joint Inventions" and shall be jointly owned by the Parties (with patent-related responsibilities allocated at a ratio agreed upon by the Parties in good faith as provided for in Section 10.2(b) below) and recorded in a conception register and shall be submitted to both Censa and Shiratori patent counsel. Each Party promptly shall disclose to the other Party the conception, making, or reduction to practice of Joint Inventions.

(d) Inventorship of Inventions will be determined in accordance with principles of U.S. patent law. In the case of trade secrets or know-how not intended for patent application, inventorship will be determined under such principles by treating such Inventions as if they were patentable.

10.2 Patent Prosecution

(a) Patent Prosecution of Solely Owned Inventions. As to Censa Inventions or Shiratori Inventions, respectively, the Party owing such Inventions shall be solely responsible for the filing, prosecution (including oppositions), and maintenance of all patent applications of such Inventions, and such Party shall have sole responsibility for all fees with respect to such patent applications and all fees necessary for maintenance and enforcement, both in the Territory and in Japan.

(b) Patent Prosecution of Joint Inventions. In the Territory, as between the Parties, Censa shall be responsible for the filing, prosecution (including oppositions), and maintenance of all patent applications of Joint Inventions, and in Japan, Shiratori shall be responsible for filing, prosecution (including oppositions), and maintenance of all patent applications of Joint Inventions. The Parties shall share pro-rata based on each Party's respective ownership share in the applicable patent the obligation and responsibility for all fees with respect to such patent applications and all fees necessary for maintenance and enforcement equitably. In Japan, as between the Parties, Shiratori be responsible for, and shall keep Censa currently apprised of, all steps taken or to be taken in the prosecution of the Licensed Patents and any improvements and shall promptly furnish Censa with copies of all patent applications and material correspondence with all patent offices in Japan.

(c) Patent Applications Issued. In the event any patent applications having Valid Claims issue during the term of this Agreement, Shiratori shall notify Censa within [**] Business Days thereof and shall assist Censa in listing the patents with the appropriate Regulatory Authority in the Territory within [**] days of issuance thereof. If the regulatory law changes in the Territory, Shiratori agrees to assist Censa with any patent listing with a Regulatory Authority within the appropriate period of time.

(d) Patent Extensions and Supplementary Protection Certificates. Shiratori shall file all applications and take all actions necessary to obtain patent extensions pursuant to 35 USC 156 or like foreign statutes for Licensed Patents licensed to Censa hereunder.

(e) Licensed Patents and Related Technology Generally. Shiratori and its patent counsel or other legally authorized or agents shall consult with Censa in all aspects of the filing, prosecution (including oppositions), and maintenance of the Licensed Patents and other Licensed Technology and shall provide Censa sufficient opportunity to comment on any related document that Shiratori intends to file or to cause to be filed with the relevant governmental authority in advance of such filing. Any actions recommended by Censa for such purpose shall not be unreasonably denied or delayed by Shiratori.

10.3 Infringement Actions Against Third Parties.

(a) Shiratori and Censa shall promptly notify each other of any infringement or unauthorized use of any Licensed Technology that may come to their attention. Shiratori shall promptly undertake, at Shiratori's expense, reasonable efforts to obtain a discontinuance of the aforesaid infringement or unauthorized use and, if not successful, Shiratori shall bring suit against such infringer or unauthorized user. Censa, within a reasonable period of time and at its option and expense, shall have the right to participate in any such suit. Shiratori shall not enter into any settlement agreement with respect to such suit without Censa's consent.

(b) If Shiratori fails to obtain a discontinuance of said infringement or unauthorized use and/or fails to promptly bring suit against such Third Party, then in any such event Shiratori shall give notice in writing to Censa of the circumstance of such infringement or unauthorized use, including such evidence of infringement as Shiratori may possess and the numbers of the patents and patent applications so infringed. Censa may, in its

discretion (but is not required to) (i) obtain a discontinuance of the alleged infringing operation or unauthorized use or (ii) bring suit against such Third Party. Any suit by Censa shall be either in the name of Censa, or in the name of Shiratori, or jointly by Censa and Shiratori, as may be required by the law of the forum. For this purpose, Shiratori shall execute such legal papers necessary for the prosecution of such suit as may be reasonably requested by Censa.

(c) It is understood and agreed that the Party to this Agreement that institutes the suit or action shall bear solely all costs and expenses associated therewith (excepted as otherwise provided in this Agreement) and shall be entitled to retain and keep any and all sums received, obtained, collected or recovered whether by judgment, settlement or otherwise, as a result of such suit. In addition, with respect to any suit for infringement or unauthorized use of the Licensed Know-How and/or Licensed Patents, the Party that did not institute suit, shall render all reasonable assistance to the Party that did institute suit at the assisting Party's cost and expense, including executing all documents as may be reasonably requested by the Party that did institute suit.

(d) Shiratori and Censa shall keep each other fully informed of all efforts to obtain discontinuation of unauthorized uses and infringements.

(e) Infringements also shall include notices received by Shiratori or Censa under 21 USC §355(b)(3) or §355(j)(2)(3) (a "Paragraph IV Notice"). When a Paragraph IV Notice is received by Shiratori or Censa, each shall notify the other in writing within [**] Business Days after receipt thereof. If Shiratori has not brought suit within [**] days of the date of the Paragraph IV Notice, Censa, at its sole discretion, may bring suit.

10.4 Infringement of Third Party Intellectual Property Rights. Each Party hereto shall notify the other Party promptly in the event of the receipt of notice of any action, suit or claim alleging infringement by Drug Substance or Product of any Third Party patent, trademark, know-how or intellectual property in the Territory. Shiratori shall indemnify and hold Censa harmless in the event that the use, promotion, or sale of Drug Substance infringe or violate any Third Party's patent or know-how, provided such infringement or unauthorized use does not arise or result from the negligence, misconduct or omission of Censa. In such event, Shiratori shall immediately and thoroughly inform Censa of such Third Party's claim or action, and Shiratori shall take all provisional measures so as to protect and preserve Censa's interests hereunder.

10.5 Offset. In the event that it is necessary, in Censa's reasonable judgment, for Censa to make royalty or other payments to a Third Party in order for Censa to exercise or continue to exercise the rights granted to Censa pursuant to the terms of this Agreement in relation to the Product, or Licensed Technology, and only if Censa obtains a prior written consent from Shiratori (which consent shall not be unreasonably conditioned, delayed, or withheld by Shiratori) to pay such royalty or other payments, Censa shall be entitled to offset any amounts so paid to any Third Party against amounts due or which may become due to Shiratori under this Agreement; provided, however, that the maximum reduction shall be [**] percent ([**]%) of the amounts otherwise owed by Censa to Shiratori. Shiratori shall exercise its maximum efforts to protect the rights or interests of Censa obtained hereunder.

10.6 Compulsory Licenses.

(a) In the event that during the term of this Agreement a Regulatory Authority in the Territory grants or compels Shiratori to grant a license to any Third Party(ies) for the Product in any country in the Territory, Censa shall have the benefit of any lower royalty rates granted to such Third Party(ies), but only to the extent that such royalty rates to such Third Party(ies) are more favorable than those granted Censa pursuant to this Agreement, and only during the period such Third Party(ies) sell Product in those countries of the Territory where compulsory license(s) exist and have achieved for a period of at least [**] consecutive months a combined total sales volume of at least [**] percent ([**]%) of Censa's, its Affiliate's and sublicensee's sales of Product in such country(ies).

(b) If a Regulatory Authority in a country in the Territory imposes a maximum royalty rate, such that lower royalty rates than would otherwise apply under this Agreement are mandated in such country, then the royalty rates provided for in this Agreement shall be reduced to equal such lower rates for sales of the Product in such country for the period such lower royalty rate is required by any Regulatory Authority and shall cease when Censa's royalty payment obligations to Shiratori cease under this Agreement.

10.7 Marks for the Product. Censa shall own all trademarks, service marks, trade names, brand names, slogans, logos, copyrights, trade dress, know-how (except as otherwise provided in this Agreement) and goodwill developed by Censa and associated with commercializing a Product (collectively, "Marks"). Censa shall also own any domain names including any Marks. Shiratori shall not have any right, title, interest or other license in or to any of the Marks, and all uses of such Marks shall inure solely to the benefit of Censa.

10.8 No Trademark Rights.

(a) Except as otherwise provided in this Agreement, no right, express or implied, is granted by this Agreement to use in any manner the name "Shiratori Pharmaceutical," "Censa Pharmaceuticals," or any other trade name or trademark of the other Party or its Affiliates in connection with the performance of this Agreement.

(b) Notwithstanding the foregoing, Censa shall be entitled, at its option, to use Shiratori's brand name on its packaging and/or promotional materials; *provided*, *however*, that the use by Censa of Shiratori's brand name, Shiratori's corporate name, or any phrase incorporating such name, shall be prohibited unless and until Shiratori has agreed in advance in writing to the use, the language employed and its location on any packaging and/or promotional materials.

10.9 Invention Dates. In order to protect the Licensed Patents under United States law, Shiratori agrees to maintain a policy which requires its employees to record and maintain all data and information relating to the Licensed Patents is such a manner as to enable the Parties to use such records to establish the earliest date of invention and/or diligence to reduction to practice. At a minimum, the policy shall require such individuals to record all inventions generated by them in standard laboratory notebooks which are dated and corroborated by non-inventors on a regular, contemporaneous basis.

10.9 No Other Rights to Censa Intellectual Property. Nothing in this Agreement shall be construed as granting to Shiratori any ownership interest, license, express or implied, or other right or

title, in or to any technology or intellectual property of Censa, including but not limited to, know-how, patents, patent applications, trade secrets, products, formulations, delivery devices and chemical or biological materials.

ARTICLE 11.
DISPUTES, GOVERNING LAW

11.1 Disputes. If the Parties are unable to resolve a dispute within [**] days after a matter is first considered, Censa or Shiratori, by written notice to the other, may have such dispute referred to its respective executive officer designated by each Party for attempted resolution by good faith negotiations. Any such dispute shall be submitted to such executive officers no later than [**] days following such request by either Censa or Shiratori. Such designated executive officers shall attempt in good faith to resolve any such dispute within [**] days after submission of the dispute.

11.2 Governing Law. This Agreement shall be construed and the respective rights of the Parties determined according to the substantive laws of the State of Delaware in the United States, notwithstanding the provisions governing conflict of laws under Delaware law to the contrary. Each Party hereby irrevocably submits to the jurisdiction of any federal or state court in the State of Delaware to resolve any disputes arising out of or in any way relating to this Agreement. The UNCITRAL Convention for the International Sale of Goods, as well as any other unified law relating to the conclusion and implementation of contracts for the international sale of goods, shall not apply.

ARTICLE 12.

MISCELLANEOUS

12.1 Assignment. Either Party hereto may assign this Agreement without the other Party's consent to an Affiliate or to the successor or assignee of all or substantially all of such Party's business or assets related to the subject matter of this Agreement. Other than by Censa, this Agreement shall not otherwise be assignable by either Party without the prior written consent of the other Party, which consent shall not be unreasonably conditioned, delayed, or withheld. This Agreement shall be binding upon and inure to the benefit of the Parties' permitted successors and assigns. Notwithstanding any of the foregoing provisions of this Agreement, in the event such an assignment would trigger Withholding Taxes on amounts payable by the assigning Party to the other Party, the assigning Party shall be responsible for the payment of all such Withholding Taxes; *provided*, *however*, that if such Party uses a foreign tax credit received as a result of this payment of Withholding Taxes by the assigning Party and thereby reduces the amount of income tax that such Party otherwise would have paid, such Party shall refund to the assigning Party the amount of such reduction with respect to such foreign tax credit.

12.2 Further Actions. Each Party agrees to execute, acknowledge and deliver such further instruments, and to do all such other acts, as may be necessary or appropriate in order to carry out the purposes and intent of this Agreement.

12.3 Force Majeure. No Party shall be liable to the other Party for loss or damages or shall have any right to terminate this Agreement for any default or delay attributable to any Force Majeure, if the Party affected shall give prompt notice of any such cause to the other Party. The Party giving such notice shall thereupon be excused from such of its obligations hereunder as it is thereby disabled from performing for so long as it is so disabled, *provided, however*, that such affected Party commences and continues to use its Commercially Reasonable Efforts to cure such cause.

12.4 Notices.

Notices to Shiratori shall be addressed to:

Shiratori Pharmaceutical Co., Ltd.
2-3-7 Akanehama, Narashino-shi, Chiba-ken
275-0024 JAPAN
Facsimile No.: [**]

Notices to Censa shall be addressed to:

Censa Pharmaceuticals Inc.
222 Third Street, Suite 2240
Cambridge, Massachusetts 02142 USA
Attention: Jonathan Reis, M.D., MBA, Director
Facsimile No.: +1 (617) 225-7780

Either Party may change its address to which notices shall be sent by giving notice to the other Party in the manner provided in this Agreement. Any notice required or provided for by the terms of this Agreement shall be in writing and shall be sent by (a) registered or certified mail, return receipt requested, postage prepaid, (b) a reputable overnight courier service, (c) first class mail, postage prepaid, (d) personal delivery, or (e) facsimile transmission, in each case properly addressed in accordance with the paragraph above. The effective date of notice shall be the actual date of receipt by the Party receiving the same.

12.5 Amendment. No amendment, modification or supplement of any provision of this Agreement shall be valid or effective unless made in writing and signed by a duly authorized officer of each Party.

12.6 Waiver. No provision of this Agreement shall be waived by any act, omission or knowledge of a Party or its agents or employees except by an instrument in writing expressly waiving such provision and signed by a duly authorized officer of the waiving Party.

12.7 Counterparts. This Agreement may be executed in counterparts and such counterparts taken together shall constitute one and the same agreement.

12.8 Descriptive Headings. The descriptive headings of this Agreement are for convenience only, and shall be of no force or effect in construing or interpreting any of the provisions of this Agreement.

12.9 Severability. If any provision hereof is held invalid, illegal or unenforceable in any respect in any jurisdiction, the Parties hereto shall substitute, by mutual consent, valid provisions for such invalid, illegal or unenforceable provisions which valid provisions in their economic effect are sufficiently similar to the invalid, illegal or unenforceable provisions that it can be reasonably assumed that the Parties would have entered into this Agreement with such valid provisions. In case such valid provisions cannot be agreed upon, the invalid, illegal or unenforceable of one or several provisions of this Agreement shall not affect the validity of this Agreement as a whole, unless the invalid, illegal or unenforceable provisions are of such essential importance to this Agreement that it is to be reasonably

assumed that the Parties would not have entered into this Agreement without the invalid, illegal or unenforceable provisions.

12.10 Entire Agreement of the Parties. This Agreement, together with the CDA, and any schedules or exhibits hereto, constitutes and contains the complete, final and exclusive understanding and agreement of the Parties and cancels and supersedes any and all prior negotiations, correspondence, understandings and agreements whether oral or written, among the Parties respecting the subject matter hereof and thereof.

12.11 Independent Contractors. The relationship between Censa and Shiratori created by this Agreement is one of independent contractors and neither Party shall have the power or authority to bind or obligate the other except as may be expressly set forth in this Agreement.

12.12 Accrued Rights; Surviving Obligations. Unless explicitly provided otherwise in this Agreement, termination, relinquishment or expiration of the Agreement for any reason shall be without prejudice to any rights which shall have accrued to the benefit to any Party prior to such termination, relinquishment or expiration, including damages arising from any breach hereunder. Such termination, relinquishment or expiration shall not relieve any Party from obligations which are expressly indicated to survive termination or expiration of the Agreement, including, without limitation, those obligations set forth in Articles 6, 7, 8, and 9, respectively.

12.13 Compliance with Export Regulations. Neither Party shall export any technology licensed to it by the other Party under this Agreement except in compliance with U.S. export laws and regulations.

12.14 No Third Party Beneficiaries. No person or entity other than Censa, Shiratori and their respective Affiliates and permitted assignees hereunder shall be deemed an intended beneficiary hereunder or have any right to enforce any obligation of this Agreement.

12.15 No Strict Construction. This Agreement has been prepared jointly and shall not be strictly construed against either Party.

12.16 Language. All documents and correspondence relating to this Agreement shall be in the English language.

IN WITNESS WHEREOF, duly authorized representatives of the Parties have duly executed and delivered this Agreement as of the Effective Date.

[THE REMAINDER OF THIS PAGE IS INTENTIONALLY LEFT BLANK;
THE SIGNATURE PAGE IMMEDIATELY FOLLOWS]

SHIRATORI PHARMACEUTICAL CO., LTD.

By: /s/ Yutaka Shiratori

Name: Yutaka Shiratori

Title: President

CENSA PHARMACEUTICALS INC.

By: /s/ Jonathan Reis

Name: Jonathan Reis

Title: Chief Executive Officer

Amendment No. 1 to License Agreement

Amendment No. 1 to
License Agreement

This Amendment No. 1 to License Agreement ("Amendment") dated July 31st, 2017 (the "Effective Date") amends that certain License Agreement dated as of February 8, 2015 by and between Shiratori Pharmaceutical Co., Ltd. having a principal place of business at 2-3-7 Akenahma, Narashina-shi, Chiba-ken, 275-0024 JAPAN ("Shiratori") and Censa Pharmaceuticals Inc., having a principal place of business at 222 Third Street, Suite 2240, Cambridge, MA 02142 ("Censa").

WHEREAS, the parties desire to modify the Agreement to reflect the understandings they have reached with respect to their relationship; and

WHEREAS, capitalized terms used in this Amendment but not otherwise defined shall have the meaning ascribed to such terms in the Agreement.

NOW, THEREFORE, for good and valuable consideration, the receipt of which is hereby acknowledged, the parties hereto agree as follows:

Amendments. The Agreement is hereby amended as follows:

1. Amended to Section 4.1. The parties hereby acknowledge that the milestone payments set forth in Section 4.1(a) and 4.2(b) have been made in accordance with the Agreement. The remaining milestone payments due under Sections 4.1(c)-(f) ("Milestones") are hereby replaced with the following:

   (c) USD[**]- upon [**] This milestone shall be subject to the payment terms set forth in Section 2 and 3 below.

   (d) USD[**] upon [**]

   (e) USD[**] upon [**]

   (f) USD[**] upon [**]

   (g) USD[**] upon [**]

   (h)USD[**] upon [**]
   (i) USD[**] upon [**]

   [**]

2. Manufacturing and Supply of [**]kg of Drug Substance. By [**], Shiratori shall manufacture and deliver to Censa [**]kg of Drug Substance manufactured in

accordance with cGMP and the terms and conditions of the Agreement ("[**]kg Batch").

3. Payment for the [**]Kg Batch and Milestone 4.1(c). Payment for the [**]kg Batch and Milestone 4.1(c) shall be made by Censa in either cash or equity, at Censa's option and in accordance with Section 4 below, within [**] of receipt and acceptance by Censa of the [**]kg Batch. Censa shall pay interest on the said amounts due at an interest rate of [**] percent ([**]%) per annum.

4. Payment in Equity. At any time on or before the due date of the amounts set forth in Section 3 (the "Conversion Date"), Censa shall have the option to convert the unpaid amounts due, plus any accrued interest thereon through the Conversion Date, into common shares at a price equal to [**]% less than the purchase price per share of paid by the investors for securities in the Qualified Financing or in absence of a Qualified Financing at a purchase price equal to [**]% less than the price paid by investors in the 2016 Series A financing. For the purposes of this Agreement, "Qualified Financing" shall mean an investment in preferred or common stock of Censa in which Censa receives gross proceeds of at least $[**]. Upon payment in equity, Shiratori shall execute Censa's stockholders' agreement.

5. Inventorship of [**]. Shiratori's personnel will be named as co-inventors, as identified on Exhibit A, for patent claims directed to the [**] as set forth in the provisional patent application entitled [**].

6. Assignment to Censa: Shiratori shall assign to Censa all of Shiratori's right, title, and interest in the [**] as detailed in Exhibit B. Shiratori shall be solely responsible for any remuneration due to its personnel named as co-inventors in accordance with Section 5, including any remuneration required by the Japanese Patent Act or other applicable law.

7. Ownership of [**]: Upon the filing of non-provisional applications from the [**], Censa shall assign to Shiratori any Japanese national phase patent application of the Patent Property as detailed in Exhibit C, which discloses various [**], as detailed in Exhibit C. Shiratori shall fully own such patent application in Japan and shall be responsible for any prosecution, maintenance and expenses (including the patent application filing expenses) thereof.

8. License to [**]: Censa hereby grants to Shiratori a non-exclusive license to make, use, and sell products solely in Japan which would otherwise infringe claims directed to [**]. In consideration for the license grant by Censa, Shiratori shall reimburse Censa for [**]% of the cost associated with filing, prosecution and maintenance of claims directed to [**]. The non-exclusive license by Censa to Shiratori shall terminate immediately upon any Shiratori breach or termination of the License Agreement and/or this Amendment.

9. Technology Transfer to Commercial Manufacturer. Shiratori will transfer all the technology and know-how necessary for Censa to manufacture Drug Substance as defined in the License Agreement. Upon Censa's request, Shiratori shall disclose the Licensed Technology as defined in the License Agreement to Censa's designated third party manufacturer. Shiratori shall (a) designate qualified technical liaisons for communications with Censa's and its manufacturer's technical staff, and (b) provide Censa's and its manufacturer's technical staff with technical assistance, including answering questions concerning the manufacture of the Product as Licensee reasonably requests. Censa agrees to pay for reasonable and actual time spent by Shiratori for such technology transfer and support at a rate of $[**] per hour and in accordance with Section 3.7 of the Agreement and as agreed in advance by the Parties.

10. Miscellaneous. Except as modified and amended by this Amendment, the Agreement shall remain in full force and effect and in all other respects is ratified and confirmed by the parties.

IN WITNESS WHEREOF, the parties have caused this Amendment to be executed by their duly authorized representatives as of the date set forth in the introductory paragraph of this Amendment.

CENSA PHARMACEUTICALS INC.	SHIRATORI PHARMACEUTICAL CO., LTD
By: /s/ Jonathan Reis	By: /s/ Satoshi Shiratori
Jonathan Reis, CEO	Satoshi Shiratori

Amendment No. 2 to License Agreement

This Amendment No. 2 ("2nd Amendment") dated May 28th, 2019 (the "Effective Date") amends the License Agreement dated February 8, 2016 and Amendment No. 1 to the License Agreement dated July 31, 2017 (collectively the "Agreement") between Shiratori Pharmaceutical Co., Ltd. having a principal place of business at 28F WBG Marive east 2-6-1 Nakase, Mihama-ku, Chiba-shi, Chiba 261-7090 JAPAN ("Shiratori") and Censa Pharmaceuticals Inc., having a principal place of business at 65 Willian Street, Suite 200, Wellesley, MA 02481 ("Censa").

WHEREAS, the parties desire to modify the Agreement to reflect the understandings they have reached with respect to their relationship; and

WHEREAS, capitalized terms used in this 2nd Amendment but not otherwise defined shall have the meaning ascribed to such terms in the Agreement.

NOW, THEREFORE, for good and valuable consideration, the receipt of which is hereby acknowledged, the parties hereto agree as follows:

Amendments. The Agreement is hereby amended as follows:

Sepiapterin [**]:

1. **Inventorship of the [**].** Shiratori's personnel, as identified in Exhibit A, will be named as co-inventors for patent claims solely directed to the [**].

2. **Assignment to Censa.** Shiratori shall obtain assignments from Shiratori personnel, as identified in Exhibit A, and shall then assign to Censa of all of Shiratori's right, title, and interest in the inventions of, or applications claiming priority to, [**]. Shiratori shall be solely responsible for any remuneration due to its personnel named as co-inventors in accordance with Section 1, including any remuneration required by the Japanese Patent Act or other applicable law.

3. **Section 7 of Amendment No. 1 to the License Agreement dated July 31, 2017 is hereby replaced in its entirety with the following:** Censa shall file one or more Japanese national stage applications of, and/or Japanese divisional applications claiming priority to, [**]. Within [**] days after grant of each Japanese each, Censa will assign all right, title, and interest in the Japanese granted patent to Shiratori subject to Shiratori reimbursing Censa for the prosecution expenses (including the Japanese patent application filing expenses) thereof.

4. **Section 8 of Amendment No. 1 to the License Agreement dated July 31, 2017 is replaced in its entirety with the following:**
Censa hereby grants to Shiratori a non-exclusive license to make, use, and sell products solely in Japan which would otherwise infringe claims directed to [**] in Japanese national stage applications of, and/or Japanese divisional applications claiming priority to [**]. In consideration for the license grant by Censa, Shiratori shall reimburse Censa for [**]% of the cost associated with filing, prosecution, and maintenance of claims directed to [**]. The non-exclusive license by Censa to Shiratori shall terminate immediately upon any Shiratori breach or termination of the License Agreement as amended.

[**]:

5. **Section 1.14 of the License Agreement is hereby replaced in its entirety with the following:** "Licensed Patents" means the following patent applications and patents owned or Controlled by Shiratori (a) as of the Effective Date or (b) arising at any time during the term of this Agreement solely by Shiratori or jointly by Shiratori and Censa, and all patents issuing from such patent applications and patents and otherwise arising from any improvement or enhancement of the manufacturing processes for or methods related to Sepiapterin, during the term of this Agreement: (i) [**] (ii) [**]; (iii) [**]; and (iv) certain other patents owned by Shiratori, as are reasonably necessary or useful for the manufacture, use, and sale of the Product in the Territory, including the patent applications and/or patents listed on Schedule 6.2(b) as amended, and all foreign counterparts of any or to any of the aforesaid patents and/or patent applications, and including, without limitation, as it may relate to any such patent applications and patents all provisional applications, substitutions, continuations, continuations-in-part, divisions, and renewals, all letters patent granted thereon, and all patents-of-addition, reissues, reexaminations and extensions or restorations by existing or future extension or restoration mechanisms, certificates, substitutions, confirmations, registrations, revalidations, additions, continuations, continuations-in-part, and divisions of any or to any of the aforesaid patent applications and patents. Licensed Patents excludes Licensed Know-How.

6. **Schedule 6.2(b) of the License Agreement is hereby replaced with the following:**
Licensed Patents

[**]

Granted Patent No. or Application No.

[**]

[**]

7. **Assignment of [**]% Interest in [**].** Subject to consent from [**], Censa shall assign a [**]% interest in the inventions of [**] ("the Inventions") to Shiratori such that Censa will own a [**]% interest in the Inventions and Shiratori will own a [**]% interest in the Inventions. Censa will control prosecution of any applications based on the Inventions and pay all associated costs with such prosecution, and shall by itself register its [**]% interest in the Inventions and Shiratori's [**]% interest in the Inventions at the United States Patent and Trademark Office within [**] months after the conclusion of this 2nd Amendment. Shiratori shall provide any necessary documents and take any necessary actions requested by Censa for such registration.

   In the event that Censa Discontinues (as defined herein), then at the written request of Shiratori, Censa shall terminate the [**]. "Discontinue(s)" shall mean when Censa or its assignee meets all of the following criteria at the same time: 1) it does not have any active pre-clinical or clinical studies of sepiapterin for a period of [**] months after the last report or presentation on pre-clinical or clinical studies provided to Shiratori, 2) it is not marketing or selling sepiapterin, and 3) it does not have any regulatory submissions (e.g., IND, NDA, IMPD, or sNDA) relating to sepiapterin pending with either FDA,EMA or other regulatory authority.

   In the event of the termination of the [**], Censa shall provide any necessary documents and take any reasonable action requested by Shiratori in order that Shiratori will register its interests in the Inventions.

   In the event that Shiratori discontinues operations or files for bankruptcy, then Shiratori shall assign their [**]% interest in the inventions of [**] to Censa, its successor, or designated assignee.

8. **Effect of the termination of the Agreement.** Notwithstanding any articles of the Agreement, the provision of section 1,2,3,4 and 7 of this 2nd Amendment shall survive the termination of the Agreement.

9. **Miscellaneous.** Except as modified and amended by this 2nd Amendment, the Agreement shall remain in full force and effect and in all other respects is ratified and confirmed by the parties.

IN WITNESS THEREOF, the parties have caused this 2nd Amendment to be executed by their duly authorized representatives as of the date set forth in the introductory paragraph of this 2nd Amendment.

CENSA PHARMACEUTICALS INC.	SHIRATORI PHARMACEUTICAL CO., LTD
/s/Jonathan Reis	/s/ Satoshi Shiratori
Jonathan Reis	Satoshi Shiratori
President & CEO	President

Amendment No. 3 to License Agreement

This Amendment No. 3 ("3rd Amendment") dated January 8th, 2020 (the "Effective Date") amends the License Agreement dated February 8, 2016 and Amendment No. 1 to the License Agreement dated July 31, 2017 and Amendment No. 2 to the License Agreement dated May 28th, 2019 (collectively the "Agreement") between Shiratori Pharmaceutical Co., Ltd. having a principal place of business at 28F WBG Marive east 2-6-1 Nakase, Mihama-ku, Chiba-shi, Chiba 261-7090 JAPAN ("Shiratori") and Censa Pharmaceuticals Inc., having a principal place of business at 65 Willian Street, Suite 200, Wellesley, MA 02481 ("Censa").

WHEREAS, the parties desire to modify the Agreement to reflect the understandings they have reached related to target dates for certain regulatory and commercial milestones; and

WHEREAS, capitalized terms used in this 3rd Amendment but not otherwise defined shall have the meaning ascribed to such terms in the Agreement.

NOW, THEREFORE, for good and valuable consideration, the receipt of which is hereby acknowledged, the parties hereto agree as follows:

Amendments. The Agreement is hereby amended as follows:

1. **Section 9.2b of the License Agreement is hereby replaced in its entirety with the following:**

By Shiratori. Unless otherwise agreed between the Parties in writing, Shiratori may elect to terminate this Agreement upon sixty (60) days' prior written notice to Censa in the event that Censa fails (i) to achieve Regulatory Approval in either the United States or European Union for at least one indication within eight (8) years from the Effective Date or (ii) to launch the Product in the United States or European Union within nine (9) years from the Effective Date. Censa shall, as of the effectiveness of such termination, be relieved of any and all further obligations to make payments to Shiratori under this Agreement to the extent not accrued prior to such termination. Censa also shall be relieved of any and all further obligations with respect to patents and patent applications in the Territory.

2. **Section 5.1 of the License Agreement is hereby replaced in its entirety with the following:**

Royalty Rates. In further consideration of the licenses granted in this Agreement, Censa shall pay to Shiratori royalties on the Product based on aggregate Net Sales of such Product on a country-by-country basis in the Territory in the amount of [**] percent ([**]%) "Royalty Rate" or [**] percent ([**]%) "Reduced Royalty Rate".

3. Section 5.2 of the License Agreement is hereby replaced in its entirety with the following:

Term of Royalties. Royalties under 5.1 shall be payable for each full calendar year during the term of this Agreement and on an aggregate *pro rata* basis for the first and last year of commercialization during the term of this Agreement if such first and last years are not comprised of a full twelve (12) months, as follows:

Royalty Rate shall be payable for the countries of the European Union, until expiration of the last-to-expire Licensed Patent Controlled by Shiratori covering the European Union if the making, having made, using, offering to sell, selling or importing of such Product by Censa, its Affiliates or its sublicensees (or the distributors of any of them) in the absence of this Agreement, would infringe one or more Valid Claim of the Licensed Patents in the European Union.

Royalty Rate shall be payable for the United States, until the expiration of the last-to-expire Licensed Patent Controlled by Shiratori covering the United States if the making, having made, using, offering to sell, selling or importing of such Product by Censa, its Affiliates or its sublicensees (or the distributors of any of them) in the absence of this Agreement, would infringe one or more Valid Claim of the Licensed Patents in the United States.

Royalty Rate shall be payable for any other country in the Territory, until the expiration of the last-to-expire Licensed Patent Controlled by Shiratori covering the relevant country on a country-by-country basis if the making, having made, using, offering to sell, selling or importing of such Product by Censa, its Affiliates or its sublicensees (or the distributors of any of them) in the absence of this Agreement, would infringe one or more Valid Claim of the Licensed Patents in the relevant country.

(d) Reduced Royalty Rate shall be payable for any country under Section 5.2 (a), 5.2 (b) or 5.2(c) for [**] years after the expiration of the last-to-expire Licensed Patent Controlled by Shiratori covering the relevant country on a country-by-country basis.

2. **Miscellaneous.** Except as modified and amended by this 3rd Amendment, the Agreement shall remain in full force and effect and in all other respects is ratified and confirmed by the parties.

IN WITNESS THEREOF, the parties have caused this 3rd Amendment to be executed by their duly authorized representatives as of the date set forth in the introductory paragraph of this 3rd Amendment.

CENSA PHARMACEUTICALS INC.	SHIRATORI PHARMACEUTICAL CO., LTD
/s/ Jonathan Reis	/s/ Satoshi Shiratori
Jonathan Reis	Satoshi Shiratori
President & CEO	President

Amendment No. 4 to License Agreement

This Amendment No. 4 (the "4th Amendment") dated April 9th, 2020 amends the License Agreement dated February 8, 2016 (the "Original Agreement"), as amended by Amendment No. 1 to the License Agreement dated July 31, 2017 (the "1st Amendment"), as further amended by Amendment No. 2 to the License Agreement dated May 28th, 2019 (the 2nd Amendment") and as further amended by Amendment No. 3 to the License Agreement dated January 8th, 2020 (the "3rd Amendment", together with the Original Agreement, 1st Amendment, and 2nd Amendment, the "Agreement") by and between Shiratori Pharmaceutical Co., Ltd. having a principal place of business at 28F WBG Marive east 2-6-1 Nakase, Mihama-ku, Chiba-shi, Chiba 261-7090 JAPAN ("Shiratori") and Censa Pharmaceuticals Inc., having a principal place of business at 65 Willian Street, Suite 200, Wellesley, MA 02481 ("Censa").

WHEREAS, the parties desire to modify the Agreement to reflect the understandings they have reached related to certain termination rights, royalty payments, and milestone payments; and

WHEREAS, capitalized terms used in this 4th Amendment not otherwise defined shall have the meaning ascribed to such terms in the Agreement

NOW, THEREFORE, for good and valuable consideration, the receipt of which is hereby acknowledged, the parties hereto agree as follows:

1. Section 9.2(b) of the Agreement, previously amended by the 3rd Amendment, is hereby deleted in its entirety and replaced with the following:

(b) By Shiratori. Unless otherwise agreed between the Parties in writing, Shiratori may elect to terminate this Agreement upon sixty (60) days' prior written notice to Censa in the event that Censa fails (i) to achieve Regulatory Approval in either the United States or European Union for at least one indication within ten (10) years from the Effective Date or (ii) to launch the Product in the United States or European Union within eleven (11) years from the Effective Date. Censa shall, as of the effectiveness of such termination, be relieved of any and all further obligations to make payments to Shiratori under this Agreement to the extent not accrued prior to such termination. Censa also shall be relieved of any and all further obligations with respect to patents and patent applications in the Territory.

2. Section 5.2 (d) of the Agreement, previously amended by the 3rd Amendment, is hereby deleted it its entirety and replaced with the following:

(d) Reduced Royalty Rate shall be payable for any country under Section 5.2 (a), 5.2 (b) or 5.2(c) for [**] years after the expiration of the last-to-expire Licensed Patent Controlled by Shiratori covering the relevant country on a country-by-country basis.

3. Section 4.1 of the Agreement, previously amended by 1st Amendment, is hereby amended as follows:

The parties hereby acknowledge that the milestone payments set forth in Section 4.1(a), 4.1(b) and 4.1 (c) have been already made in accordance with the Agreement. The remaining milestone payments set forth in Sections 4.1(d)-(i) ("Milestones") are hereby deleted in their entirety and replaced with the following:

(d) USD[**] – [**] upon [**].

(e) USD[**] – [**] upon [**].

(f) USD[**] – [**] upon [**].

(g) USD[**] - [**] upon [**].

(h) USD[**] - [**] upon [**].

(i) USD[**] - [**] upon [**].

4. Miscellaneous: Except as modified and amended by this 4th Amendment, the Agreement shall remain in full force and effect and in all other respects is ratified and confirmed by the parties.

IN WITNESS THEREOF, the parties have caused this 4th Amendment to be executed by their duly authorized representatives as of the date set forth in the introductory paragraph of this 4th Amendment.

CENSA PHARMACEUTICALS INC. SHIRATORI PHARMACEUTICAL CO., LTD

/s/ Jonathan Reis /s/ Satoshi Shiratori

Jonathan Reis Satoshi Shiratori

President & CEO President

EXHIBIT 10.2
Execution Version

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) would likely cause competitive harm to the Company, if publicly disclosed. Double asterisks denote omissions.

Royalty Purchase Agreement

By and Among

PTC Therapeutics, Inc.,

RPI 2019 Intermediate Finance Trust

and

Solely for the Purposes of Section 5.15 Hereof,

Royalty Pharma plc

Dated as of July 17, 2020

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ROYALTY PURCHASE AGREEMENT

This ROYALTY PURCHASE AGREEMENT, dated as of July 17, 2020 (this "Agreement"), is made and entered into by and among PTC Therapeutics, Inc., a Delaware corporation (the "Seller"), RPI 2019 Intermediate Finance Trust, a Delaware statutory trust (the "Buyer"), and, solely for the purposes of Section 5.15 hereof, Royalty Pharma plc, a limited company organized under the laws of England and Wales.

W I T N E S S E T H:

WHEREAS, pursuant to the License Agreement, the Seller and Licensee granted to each other certain licenses and other development and collaboration rights, and the Seller granted Licensee the exclusive right to (among other activities) develop and commercialize the Products, and Licensee, in partial consideration thereof, agreed to pay the Royalty to the Seller;

WHEREAS, the Seller and the Spinal Muscular Atrophy Foundation (the "Foundation") are party to that certain Sponsored Research Agreement, dated June 1, 2016, as amended on October 12, 2007, May 1, 2009, January 1, 2011 and November 22, 2011 (the "Sponsored Research Agreement"), pursuant to which the Seller is obligated to pay the Foundation single-digit royalties on worldwide net product sales of the Products; and

WHEREAS, the Buyer desires to purchase the Assigned Royalty Payments from the Seller, and the Seller desires to sell the Assigned Royalty Payments to the Buyer.

NOW THEREFORE, in consideration of the representations, warranties, covenants and agreements set forth herein and for good and valuable consideration, the receipt and adequacy of which are hereby acknowledged, the Seller and the Buyer hereby agree as follows:

ARTICLE 1.

DEFINED TERMS AND RULES OF CONSTRUCTION

Section 1.1 Definitions. As used in this Agreement, the following terms shall have the following meanings:

"Affiliate" (a) means, with respect to the Buyer, any individual, corporation, association or other business entity that directly or indirectly controls, is controlled by, or is under common control with the party in question. As used in this definition of "Affiliate," the term "control" shall mean the direct or indirect ownership of more than fifty percent (>50%) of the stock having the right to vote for directors thereof or the ability to otherwise control the management of the corporation or other business entity whether through the ownership of voting securities, by contract, resolution, regulation or otherwise and (b) with respect to the Seller or the Licensee, shall have the meaning ascribed thereto in Section 1.1. of the License Agreement.

"Agreement" is defined in the preamble.

"Alliance Director" shall refer to those Alliance Directors described in Section 7.10 of the License Agreement.

"Applicable Listed Patents" is defined in Section 5.10(c).

"Assigned Royalty Cap" is defined within the definition of "Assigned Royalty Payments."

"Assigned Royalty Payments" means 42.933% (the "Buyer Royalty Percentage") of the amounts due, payable, owed or owing, accrued or otherwise to be paid in respect of the Royalty for all Calendar Year Net Sales arising on or after the Closing Date for any Calendar Year until such Buyer Royalty Percentage of such amounts equals $1.3 billion (the "Assigned Royalty Cap").

"Bankruptcy Laws" means, collectively, bankruptcy, insolvency, reorganization, moratorium, fraudulent conveyance, fraudulent transfer or other similar laws affecting the enforcement of creditors' rights generally.

"Bill of Sale" is defined in Section 3.3.

"Business Day" means any day other than (i) a Saturday or Sunday or (ii) a day on which banking institutions located in New York are permitted or required by applicable law or regulation to remain closed.

"Buyer" is defined in the preamble.

"Buyer Negotiated Arrangement" is defined in Section 5.8(b)(iii).

"Buyer Royalty Percentage" is defined within the definition of "Assigned Royalty Payments."

"Calendar Quarter" shall have the meaning ascribed thereto in Section 1.7 of the License Agreement.

"Calendar Year" shall have the meaning ascribed thereto in Section 1.8 of the License Agreement.

"Change of Control" shall have the meaning ascribed thereto in Section 1.9 of the License Agreement.

"Closing" is defined in Section 3.1.

"Closing Date" means the date on which the Closing occurs.

"Commercialization Reports" means those reports deliverable by Licensee pursuant to Section 10 of the License Agreement.

"Compound" shall have the meaning ascribed thereto in Section 1.13 of the License Agreement.

"Confidential Information" is defined in Section 6.1.

"Confidentiality Breach" is defined in Section 5.4(d).

"Consensus Value" means the net present value (applying a [**]% discount rate) of the projected Net Sales of the Products using projections of Wall Street sell-side analysts then covering the Licensee and the Seller, as determined in accordance with Exhibit A.

"Contracts" is defined in Section 4.1(h)(i).

"Control Party" means (a) the Seller, (i) from the Closing until a Control Shift occurs, (ii) after a Control Shift occurs, from the occurrence of a Revaluation Event in favor of the Seller until another Control Shift or a Revaluation Event in favor of the Buyer occurs; and (b) the Buyer (i) after a Control Shift occurs and until a Revaluation Event in favor of the Seller occurs, and (ii) after a Revaluation Event in favor of the Seller occurs, from the occurrence of another Control Shift or a Revaluation Event in favor of the Buyer until another Revaluation Event in favor of the Seller occurs.

"Control Shift" means any assignment, conveyance, monetization, or imposition of a Lien by the Seller on, following the Closing, any percentage of the Royalty, with the result that the Seller and its Affiliates no longer own, free and clear of all Liens (other than Permitted Liens) the right to receive a majority of the then remaining Royalty based on the Consensus Value.

"Credit Event" means any insolvency, bankruptcy, receivership, assignment for the benefit of creditors, or similar proceeding, following or as a result of which the Licensee fails to pay amounts owing under the License Agreement in respect of the Royalty as a result of the Licensee's financial distress, creditworthiness, or insolvency.

"Data Room" is defined in Section 3.6.

"Deciding Valuation Firm" has the meaning set forth in Exhibit A.

"Disclosing Party" is defined in Section 6.1.

"Disclosure Schedule" means the Disclosure Schedule, dated as of the date hereof, delivered to the Buyer by the Seller concurrently with the execution of this Agreement.

"Escrow Account" means the account controlled by the Escrow Agent pursuant to which Licensee has been instructed to direct all amounts payable by it under the License Agreement.

"Escrow Agent" means U.S. Bank National Association, or the escrow agent under the Escrow Agreement or any permitted successor thereof under the Escrow Agreement.

"Escrow Agreement" means the Escrow Agreement that may be entered into among the Seller, the Buyer, and the Escrow Agent in accordance with Section 5.2(a)(i), substantially in the form attached hereto as Exhibit B.

"[**]" means the product that is the subject of New Drug Application number 213535 that contains risdiplam as the active ingredient.

"Field" shall have the meaning ascribed thereto in Section 1.25 of the License Agreement.

"Final Counter Proposed Consensus Value" has the meaning set forth in Exhibit A.

"Final Proposed Consensus Value" has the meaning set forth in Exhibit A.

"First Commercial Sale" shall have the meaning ascribed thereto in Section 1.28 of the License Agreement.

"Foundation" is defined in the recitals.

"Governmental Entity" means any: (i) nation, principality, republic, state, commonwealth, province, territory, county, municipality, district or other jurisdiction of any nature; (ii) federal, state, local, municipal, foreign or other government; (iii) governmental or quasi-governmental authority of any nature (including any governmental division, subdivision, department, agency, bureau, branch, office, commission, council, board, instrumentality, officer, official, representative, organization, unit, body or other entity and any court, arbitrator or other tribunal); (iv) multi-national organization or body; or (v) individual, body or other entity exercising, or entitled to exercise, any executive, legislative, judicial, administrative, regulatory, police, military or taxing authority or power of any nature.

"Handle" or "Handling" has the meaning ascribed thereto in Section 1.35 of the License Agreement.

"JIPT" shall have the meaning ascribed thereto in Section 7.4 of the License Agreement.

"Joint Invention" shall have the meaning ascribed thereto in Section 1.42 of the License Agreement.

"Joint Patent Rights" shall have the meaning ascribed thereto in Section 1.44 of the License Agreement.

"JOT" shall have the meaning ascribed thereto in Section 7.4 of the License Agreement.

"JSC" shall have the meaning ascribed thereto in Section 7.1 of the License Agreement.

"Judgment" means any judgment, order, writ, injunction, citation, award or decree of any nature.

"Knowledge of the Seller" means the actual knowledge of [**] after due inquiry.

"License Agreement" means that certain License and Collaboration Agreement, dated November 23, 2011, by and among F. Hoffman-La Roche Ltd., Hoffman-La Roche Inc., the

Seller and, solely with respect to the Foundation Provisions (as defined therein), the Foundation, as amended by such parties by means of that First Amendment to License and Collaboration Agreement on April 18, 2013.

"Licensed IP" means, collectively, the Licensed PTC IP and the Licensed Roche IP.

"Licensed Patents" is defined in Section 4.1(j)(i).

"Licensed PTC IP" means the PTC Patent Rights, PTC Know-How and the Seller's interest in the Joint Inventions.

"Licensed Roche IP" means the Roche Patent Rights, the Roche Know-How and Licensee's interest in the Joint Inventions.

"Licensee" means F. Hoffman-La Roche Ltd and Hoffman-La Roche Inc., collectively.

"Licensee Instruction Letter" is defined in Section 5.2(a)(i).

"Lien" means any mortgage, lien, pledge, charge, adverse claim, security interest, encumbrance or restriction of any kind, including any restriction on use, transfer or exercise of any other attribute of ownership of any kind; provided, that, for the avoidance of doubt, Lien shall not include the license of any rights.

"Loss" means any and all Judgments, damages, losses, claims, costs, liabilities and expenses, including reasonable fees and out-of-pocket expenses of counsel.

"Net Sales" shall have the meaning ascribed thereto in Section 1.50 of the License Agreement.

"New Product" shall have the meaning ascribed thereto in Section 1.53 of the License Agreement.

"New Product Development Plan" shall have the meaning ascribed thereto in Section 1.54 of the License Agreement.

"Marketed Product" is defined in Section 5.8(b)(iii).

"Ordinary Course Licenses or Sublicenses" means licenses or sublicenses entered into in the ordinary course of business with respect to the (i) development or manufacture of Products, (ii) distribution, third party logistics, warehousing, packaging, labeling or other commercialization activities that are ancillary to marketing, promotion or selling of Products or (iii) commercial sale of Products outside of [**].

"Original Counter Proposed Consensus Value" has the meaning set forth in Exhibit A.

"Original Proposed Consensus Value" has the meaning set forth in Exhibit A.

"Patent Office" means the applicable patent office, including the United States Patent and Trademark Office and any comparable foreign patent office, for the registration of any patents and patent applications.

"Patent Rights" shall have the meaning ascribed thereto in Section 1.56 of the License Agreement.

"Permitted Liens" means any (i) mechanic's, materialmen's, and similar liens for amounts not yet due and payable, (ii) statutory liens for taxes not yet due and payable or for taxes that the taxpayer is contesting in good faith, (iii) Liens in favor of the Buyer, and (iv) other liens and encumbrances not incurred in connection with the borrowing of money that do not materially and adversely affect the use or value of the affected assets provided that, in each case, such liens are automatically released upon the sale or other transfer of the affected assets (it being understood that any obligations secured by such "Permitted Liens" shall remain the obligations of the Seller).

"Permitted Royalty Reduction" means a reduction to the Royalty pursuant to Sections 11.5.3, 11.5.4, 11.5.5 or 14.2 of the License Agreement or a Permitted Withholding Tax Royalty Reduction, except, in each case, to the extent that such reduction was the direct result of a material breach or default by the Seller of the License Agreement or this Agreement. For the avoidance of doubt, no amounts paid or owed to Foundation, including under the Sponsored Research Agreement, shall be considered a Permitted Royalty Reduction.

"Permitted Withholding Tax Royalty Reduction" means a reduction to a Royalty payment pursuant to Section 13 of the License Agreement, provided that reductions to a Royalty payment under Section 13 of the License Agreement shall not constitute Permitted Withholding Tax Royalty Reductions if such reductions result from changes to the Seller (or its Affiliate's) jurisdiction or corporate structure, or the taking of any action by the Seller which has the effect of subjecting such Person (or payments made by or on their behalf) to additional Taxes or Tax laws.

"Person" means any individual, firm, corporation, company, partnership, limited liability company, trust, joint venture, association, estate, trust, Governmental Entity or other entity, enterprise, association or organization.

"Prime Rate" means the prime rate published by the Wall Street Journal, from time to time, as the prime rate.

"Pro Rata Portion" means (a) with respect to the Buyer, 42.933%, and (b) with respect to the Seller, 57.067%.

"Proceeds" means any amounts actually recovered by the Seller (or its designee) as a result of any settlement or resolution of any actions, suits, proceedings, claims or disputes related to the License Agreement, the Sponsored Research Agreement or otherwise related to or involving the Royalty or any Product.

"Product" shall have the meaning ascribed thereto in Section 1.61 of the License Agreement.

"Product Information" means (a) all notices between, to or from the Seller and Licensee under the License Agreement and (b) all other information the Seller receives or furnishes under the License Agreement with respect to matters that would reasonably be expected to result in a Product MAE.

"Product MAE" means (i) a material adverse effect on the legality, validity or enforceability of any provision of this Agreement, (ii) a material adverse effect on the ability of the Seller to perform any of its obligations hereunder, (iii) a material adverse effect on the rights or remedies of the Buyer hereunder, (iv) an adverse effect on the rights of the Seller under the License Agreement or the Sponsored Research Agreement, other than as a result of Credit Event, that has a material adverse effect on the Royalty, (v) an adverse effect on the performance of the parties under the License Agreement or the Sponsored Research Agreement, including related to the approval or commercialization of the Products, other than as a result of a Credit Event, that has a material adverse effect on the Royalty, or (vi) any other adverse effect in any material respect on the timing, amount or duration of the payments to be made to the Buyer in respect of any portion of the Royalty or the right of the Buyer to receive such payments, in each case other than as a result of a Credit Event or Permitted Royalty Reduction; provided, however, that a delay in obtaining or failure to obtain regulatory approval for any Product shall not in and of itself constitute a Product MAE, it being understood that the underlying facts and circumstances giving rise to such delay or failure may constitute, and may be taken into account in determining whether there has been, a Product MAE as described in the foregoing clauses (i)-(vi).

"PTC Know-How" shall have the meaning ascribed thereto in Section 1.64 of the License Agreement.

"PTC Patent Rights" shall have the meaning ascribed thereto in Section 1.65 of the License Agreement, only to the extent that such PTC Patent Rights relate to risdiplam or a Marketed Product.

"Purchase Price" means $650,000,000.

"Receiving Party" is defined in Section 6.1.

"Representative" means, with respect to any Person, any manager, director, officer, employee, agent, advisor or other representative (including attorneys, accountants, consultants, bankers, financial advisors and actual and potential lenders and investors) of such Person.

"Research Plan" shall have the meaning ascribed thereto in Section 1.69 of the License Agreement.

"Revaluation Event" has the meaning set forth in Exhibit A.

"Revaluation Notice" has the meaning set forth in Exhibit A.

"Reversionary Rights" is defined in Section 5.8(b)(ii).

"Risdiplam" means the compound with the chemical structure set forth in Schedule 1.1 hereto, regardless of whether such term is capitalized herein.

"Roche Know-How" shall have the meaning set forth in Section 1.74 of the License Agreement.

"Roche Patent Rights" shall have the meaning set forth in Section 1.75 of the License Agreement.

"Royalty" means (i) all payments payable to the Seller under Section 11.5 of the License Agreement, (ii) any payments to the Seller under the License Agreement in lieu of such payments of the foregoing clause (i), (iii) amounts payable to the Seller under Section 14.2 of the License Agreement in respect of payments described in the foregoing clauses (i) and (ii) (other than reimbursed audit costs under such section), (iv) subject to Section 5.10(f), amounts payable to the Seller under Section 15.8 of the License Agreement (other than reimbursed expenses as provided in Section 5.10(f)) and (v) any interest payments to the Seller under Section 12.2 of the License Agreement assessed on any payments described in the foregoing clauses (i), (ii), (iii) or (iv). For the avoidance of doubt, the Royalty shall not include any payments or amounts payable to the Seller under Sections 11.1, 11.2, 11.3, or 11.4 of the License Agreement, including any amounts payable to the Seller under Sections 12.2 or 14.2 of the License Agreement in respect of such payments.

"Royalty Reduction" is defined in Section 4.1(h)(xii).

"Royalty Report Certification" is defined in Section 5.4(b).

"Royalty Reports" means the quarterly reports deliverable by Licensee pursuant to Section 12.5 of the License Agreement.

"Royalty Term" shall have the meaning ascribed thereto in Section 1.77 of the License Agreement.

"Seller" is defined in the preamble.

"Seller Business Combination" is defined in Section 5.15.

"Seller Closing Certificate" is defined in Section 3.2(a).

"Seller Direct Undertaking" is defined in Section 5.8(b)(i).

"Seller New Arrangement" is defined in Section 5.8(b)(i).

"Seller New Licensee" is defined in Section 5.8(b)(i).

"SMAF Clinical Trials Advisory Committee" shall have the meaning ascribed thereto in Section 5.1.1 of the License Agreement

"Sponsored Research Agreement" is defined in the recitals.

"SRA Compound" shall have the meaning ascribed thereto in Section 1.82 of the License Agreement.

"SRA Development Plan" shall have the meaning ascribed thereto in Section 1.81 of the License Agreement.

"SRA Product" shall have the meaning ascribed thereto in Section 1.81 of the License Agreement.

"Standstill Termination" is defined in Section 5.15.

"Tax" or "Taxes" means any federal, state, local or non-U.S. income, gross receipts, license, payroll, employment, excise, severance, occupation, premium, windfall profits, environmental, customs duties, capital stock, franchise, profits, withholding, social security, unemployment, disability, real property, personal property, abandoned property, value added, alternative or add-on minimum, estimated or other tax of any kind whatsoever, including any interest, penalty or addition thereto, whether disputed or not.

"Territory" shall have the meaning ascribed thereto in Section 1.85 of the License Agreement.

"Third Party" means any Person other than the Buyer, the Seller or any of their respective Affiliates.

"Transaction Documents" means this Agreement, the Bill of Sale, the Disclosure Schedule and the Licensee Instruction Letter.

"Triggering Termination" is defined in Section 5.8(b)(ii).

"UCC" means Article 9 of the New York Uniform Commercial Code, as in effect from time to time.

Section 1.2 Certain Interpretations. Except where expressly stated otherwise in this Agreement, the following rules of interpretation apply to this Agreement:

(i)"either" and "or" are not exclusive and "include," "includes" and "including" are not limiting and shall be deemed to be followed by the words "without limitation;"

(ii)"extent" in the phrase "to the extent" means the degree to which a subject or other thing extends, and such phrase does not mean simply "if;"

(iii)“hereof,” “hereto,” “herein” and “hereunder” and words of similar import when used in this Agreement refer to this Agreement as a whole and not to any particular provision of this Agreement;

(iv)references to a Person are also to its permitted successors and assigns;

(v)definitions are applicable to the singular as well as the plural forms of such terms;

(vi)unless otherwise indicated, references to an “Article”, “Section” or “Exhibit” refer to an Article or Section of, or an Exhibit to, this Agreement, and references to a “Schedule” refer to the corresponding part of the Disclosure Schedule;

(vii)references to “$” or otherwise to dollar amounts refer to the lawful currency of the United States; and

(viii)references to a law include any amendment or modification to such law and any rules and regulations issued thereunder, whether such amendment or modification is made, or issuance of such rules and regulations occurs, before or after the date of this Agreement.

Section 1.3 Headings. The table of contents and the descriptive headings of the several Articles and Sections of this Agreement and the Exhibits and Schedules are for convenience only, do not constitute a part of this Agreement and shall not control or affect, in any way, the meaning or interpretation of this Agreement.

ARTICLE 2.

PURCHASE, SALE AND ASSIGNMENT OF THE ROYALTY

Section 2.1 Purchase and Sale. Upon the terms and subject to the conditions of this Agreement, at the Closing, the Seller shall sell, transfer, assign and convey to the Buyer, and the Buyer shall purchase, acquire and accept from the Seller, free and clear of all Liens, all of the Seller’s right, title and interest in and to the Assigned Royalty Payments.

Section 2.2 Purchase Price. In full consideration for the sale, assignment, transfer and conveyance of the Assigned Royalty Payments, and subject to the terms and conditions set forth herein, at the Closing, the Buyer shall pay (or cause to be paid) to the Seller, or the Seller’s designee, the Purchase Price, to be paid in immediately available funds by wire transfer to one or more accounts specified by the Seller on Exhibit C.

Section 2.3 No Assumed Obligations, Etc. Notwithstanding any provision in this Agreement to the contrary, the Buyer is purchasing, acquiring and accepting only the Assigned Royalty Payments, and is not assuming any liability or obligation of the Seller of whatever nature, whether presently in existence or arising or asserted hereafter, under the License Agreement, the Sponsored Research Agreement or otherwise, including any payments due to the Foundation under the Sponsored Research Agreement. Except as specifically set forth herein in respect of the Assigned Royalty Payments purchased, acquired and accepted hereunder, the

Buyer does not, by such purchase, acquisition and acceptance, acquire any other contract rights of the Seller under the License Agreement, the Sponsored Research Agreement or any other assets of the Seller.

Section 2.4 True Sale. It is the intention of the parties hereto that, as between the parties hereto, the sale, transfer, assignment and conveyance contemplated by this Agreement constitute a sale of the Assigned Royalty Payments from the Seller to the Buyer and not a financing transaction, borrowing or loan. Following the Closing, the Buyer will be the owner of the Assigned Royalty Payments, the Buyer will have no right to return the Assigned Royalty Payments to the Seller, and the Seller will have no right to repurchase the Assigned Royalty Payments from the Buyer. The sole recourse of the Buyer against the Seller in respect of the Assigned Royalty Payments will be (a) for Royalty Reductions, only to the extent permitted under Section 5.3 hereof, and (b) claims by the Buyer for breach of the representations, warranties, and covenants of the Seller set forth herein. Accordingly, the Seller shall treat the sale, transfer, assignment and conveyance of the Assigned Royalty Payments as a sale of an "account" or a "payment intangible" (as appropriate) in accordance with the UCC for legal purposes, and the Seller hereby authorizes the Buyer to file financing statements (and continuation statements with respect to such financing statements when applicable) naming the Seller as the debtor and the Buyer as the secured party in respect of the Assigned Royalty Payments. Not in derogation of the foregoing statement of the intent of the parties hereto in this regard, and for the purposes of providing additional assurance to the Buyer in the event that, despite the intent of the parties hereto, the sale, transfer, assignment and conveyance contemplated hereby is hereafter held not to be a sale, the Seller does hereby grant to the Buyer, as security for the obligations of the Seller hereunder, a first priority security interest in and to all right, title and interest of the Seller, in, to and under the Assigned Royalty Payments and any "proceeds" (as such term is defined in the UCC) thereof, and the Seller does hereby authorize the Buyer, from and after the Closing, to file such financing statements (and continuation statements with respect to such financing statements when applicable) as are necessary to perfect such security interest. Nothing herein shall mandate or limit the tax or accounting treatment of the transactions contemplated hereby by either party hereto.

ARTICLE 3.

CLOSING

Section 3.1 Closing; Payment of Purchase Price. The purchase and sale of the Assigned Royalty Payments shall take place on the date hereof, or at such other place, time and date as the parties hereto may mutually agree (the "Closing").

Section 3.2 Closing Certificates.

(a) Seller's Closing Certificate. At the Closing, the Seller shall deliver to the Buyer a certificate of the Secretary of the Seller, dated as of the Closing Date, certifying (i) as to the incumbency of the officer of the Seller executing this Agreement and (ii) as to the attached copies of Seller's certificate of incorporation, bylaws and resolutions adopted by the Seller's board of directors authorizing the execution and delivery by the Seller of this Agreement

and the consummation by the Seller of the transactions contemplated hereby (the "Seller Closing Certificate").

(b) Buyer's Incumbency Certificate. At the Closing, the Buyer shall deliver to the Seller a certificate of an authorized person of the owner trustee of the Buyer certifying as to the incumbency of the officers executing this Agreement on behalf of the Buyer (the "Buyer Incumbency Certificate").

Section 3.3 Bill of Sale. At the Closing, upon confirmation of the receipt of the Purchase Price, the Seller shall deliver to the Buyer a duly executed bill of sale evidencing the sale, transfer, assignment and conveyance of the Assigned Royalty Payments, substantially in the form attached hereto as Exhibit D (the "Bill of Sale").

Section 3.4 Form W-9. At the Closing, the Seller shall deliver to the Buyer a valid, properly executed IRS Form W-9 certifying that the Seller is exempt from U.S. federal withholding tax and "backup" withholding tax.

Section 3.5 Form W-8BEN-E. At the Closing, the Buyer shall deliver to the Seller a valid, properly executed IRS Form W-8BEN-E certifying that the Buyer is exempt from U.S. federal withholding tax with respect to any and all payments of in respect of the Assigned Royalty Payments.

Section 3.6 Data Room. At the Closing, the Seller shall deliver to the Buyer an electronic copy of all of the information and documents posted as of the date hereof to the virtual data room established by the Seller and made available to the Buyer [**] (the "Data Room") for archival purposes only.

ARTICLE 4.

REPRESENTATIONS AND WARRANTIES

Section 4.1 Seller's Representations and Warranties. Except as set forth in the Disclosure Schedule, the Seller represents and warrants to the Buyer that as of the date hereof:

(a) Existence; Good Standing. The Seller is a corporation duly incorporated, validly existing and in good standing under the laws of Delaware. The Seller is duly licensed or qualified to do business and is in corporate good standing in each jurisdiction in which the nature of the business conducted by it or the character or location of the properties and assets owned, leased or operated by it makes such licensing or qualification necessary, except where the failure to be so licensed or qualified and in corporate good standing has not and would not reasonably be expected to result in, either individually or in the aggregate, a Product MAE.

(b) Authorization. The Seller has all requisite corporate power and authority to execute, deliver and perform its obligations under this Agreement. The execution, delivery and performance of this Agreement, and the consummation of the transactions

contemplated hereby, have been duly authorized by all necessary corporate action on the part of the Seller.

(c) Enforceability. This Agreement has been duly executed and delivered and constitutes a valid and binding obligation of the Seller enforceable against the Seller in accordance with its terms, except as such enforceability may be limited by applicable Bankruptcy Laws or by other equitable principles of general application.

(d) No Conflicts. The execution, delivery and performance by the Seller of this Agreement and the consummation of the transactions contemplated hereby do not (i) contravene or conflict with the organizational documents of the Seller, (ii) contravene or conflict with or constitute a material default under any law or Judgment binding upon or applicable to the Seller, (iii) contravene or conflict with or constitute a default under the License Agreement or the Sponsored Research Agreement or (iv) except as would not reasonably be expected to result in a Product MAE, contravene or conflict with or constitute a default under any other contract or agreement binding upon or applicable to the Seller.

(e) Consents. Except for filings required by the federal securities laws or stock exchange rules, no consent, approval, license, order, authorization, registration, declaration or filing with or of any Governmental Entity or other Person is required to be done or obtained by the Seller in connection with (i) the execution and delivery by the Seller of this Agreement, (ii) the performance by the Seller of its obligations under this Agreement or (iii) the consummation by the Seller of any of the transactions contemplated by this Agreement.

(f) No Litigation. There is no action, suit, investigation or proceeding pending before any Governmental Entity or, to the Knowledge of the Seller, threatened to which the Seller is a party that, individually or in the aggregate would, if determined adversely, reasonably be expected to result in a Product MAE.

(g) Compliance with Laws. The Seller is not in violation of and, to the Knowledge of the Seller, the Seller is not under investigation with respect to, nor has the Seller been threatened to be charged with or given notice of any violation of, any law or Judgment applicable to the Seller, which violation would reasonably be expected to result in a Product MAE.

(h) License Agreement; Sponsored Research Agreement. Attached hereto as Exhibits E and F are true, correct and complete copies of, respectively, the License Agreement and the Sponsored Research Agreement, including any amendments, modifications, consents, assignments, ancillary agreements, licenses, sublicenses, transfers or waivers thereto or thereof as of the date hereof. To the Knowledge of the Seller, the Seller has delivered to the Buyer, or has provided the Buyer access to, true, correct and complete copies of [**].

(i) No Other Agreements. Exhibits E and F include all material agreements, instruments, arrangements, waivers or understandings (collectively, "Contracts") between, by or among the Seller (or any predecessor or Affiliate thereof), Licensee (or any predecessor or Affiliate

thereof) and/or the Foundation (or any predecessor or Affiliate thereof), relating to the subject matter thereof, and there are no other Contracts between the Seller (or any predecessor or any Affiliate thereof), on the one hand, and Licensee (or any predecessor or Affiliate thereof) and/or the Foundation (or any predecessor or Affiliate thereof), on the other hand, that relate to the Royalty or that are primarily related to any Product (including the development or commercialization thereof), or that (with or without the giving of notice or passage of time, or both) would reasonably be expected to result in a Product MAE. The Seller has not proposed, or received any proposal, to amend or waive any provision of the License Agreement or the Sponsored Research Agreement in any manner that would reasonably be expected (with or without the giving of notice or the passage of time, or both) to result in a Product MAE. None of the executed Contracts included in Exhibits E and F, contain any provision, term or condition that would reasonably be expected to result in a Product MAE.

(ii) Licenses/Sublicenses. To the Knowledge of the Seller, other than Ordinary Course Licenses or Sublicenses or licenses or sublicenses among Licensee and its Affiliates (and its and their predecessors), (A) there are no licenses or sublicenses entered into by Licensee or any other Person (or any predecessor or Affiliate thereof) in respect of Licensee's rights and obligations under the License Agreement (including any Licensed IP) and (B) there are no licenses or sublicenses entered into by the Foundation or any other Person (or any predecessor or Affiliate thereof) in respect of the Foundation's rights and obligations under the Sponsored Research Agreement (including any Licensed IP). The Seller has not received any notice from Licensee pursuant to Section 3.3 or Section 11.5.4 of the License Agreement, nor has the Seller been requested by or given consent to, nor is the Seller in negotiation with, Licensee, pursuant to such provisions.

(iii) Validity and Enforceability of License Agreement and the Sponsored Research Agreement. Each of the License Agreement and the Sponsored Research Agreement is legal, valid, binding, enforceable, and in full force and effect except as may be limited by applicable Bankruptcy Laws or by general principles of equity (whether considered in a proceeding in equity or at law) or by any Credit Event. Each of the License Agreement and the Sponsored Research Agreement will not cease to be legal, valid, binding, enforceable, and in full force and effect on identical terms, immediately following the consummation of the transactions contemplated by this Agreement, as a result of the consummation of the transactions contemplated by this Agreement. The Seller is not, and, to the Knowledge of the Seller, neither the Licensee nor the Foundation is, in breach of the License Agreement or the Sponsored Research Agreement, as applicable, or in default thereunder, and no event has occurred that with notice or lapse of time would (i) permit termination of the License Agreement or the Sponsored Research

Agreement or (ii) constitute a breach, default, modification or trigger acceleration under or to the License Agreement or the Sponsored Research Agreement that would reasonably be expected (with or without the giving of notice or the passage of time, or both) to result in a Product MAE. No party to the License Agreement or the Sponsored Research Agreement has repudiated any provision of the License Agreement or the Sponsored Research Agreement, as applicable, and the Seller has not received any notice in connection with the License Agreement or the Sponsored Research Agreement challenging the validity, enforceability or interpretation of any provision of such agreement or any obligation to pay any portion of the Royalty without set-off, rescission, counterclaim, reduction, credit or other deduction of any kind.

(iv) No Event Triggering Reversionary Rights. The Seller has not received any notice of and is not aware of any acts or omissions, including those of Licensee or its Affiliates, that would reasonably be expected to result in a Reversionary Right.

(v) Products. Risdiplam and any products containing risdiplam as the active pharmaceutical ingredient that are owned or controlled by Seller or Licensee, including [**], constitute SRA Products under the License Agreement. As of the date hereof, [**] (risdiplam) is the only Product that is currently being developed or commercialized under the License Agreement.

(vi) No Liens or Assignments by the Seller. Except for Permitted Liens and as contemplated hereby, the Seller has not conveyed, assigned or in any other way transferred all or any portion of its right, title and interest in and to the Royalty or any Product. There are no Liens, other than Permitted Liens, on all or any portion of the Seller's right, title and interest in and to the Royalty or any Product.

(vii) No Waivers or Releases. Except as provided in Exhibits E and F, the Seller has not granted any waiver under the License Agreement or the Sponsored Research Agreement, nor has the Seller released, in whole or in part, any counterparty from any of such counterparty's obligations under the License Agreement or the Sponsored Research Agreement, in each case in any manner that is primarily related to any Product or that (with or without the giving of notice or passage of time, or both) would reasonably be expected to result in a Product MAE.

(viii) No Termination. The Seller has not (A) given Licensee any notice of termination of the License Agreement (whether in whole or in part) or any notice expressing any intention to terminate the License Agreement or (B) received any notice of termination of the License Agreement (whether in whole or in part) or any notice expressing any intention to terminate either the License Agreement. To the Knowledge of the

Seller, no event has occurred that would give rise to the expiration or termination of the License Agreement. The Seller has not (A) given the Foundation any notice of termination of the Sponsored Research Agreement (whether in whole or in part) or any notice expressing any intention to terminate the Sponsored Research Agreement or (B) received any notice of termination of the Sponsored Research Agreement (whether in whole or in part) or any notice expressing any intention to terminate either the Sponsored Research Agreement. To the Knowledge of the Seller, no event has occurred that would give rise to the expiration or termination of the Sponsored Research Agreement.

(ix) Payments Made. The Seller has timely received from Licensee the full amount of the payments due and payable under the License Agreement in accordance with the terms thereof. The Seller has timely paid all amounts due under the Sponsored Research Agreement in accordance with the terms thereof.

(x) No Assignments by Licensee. The Seller has not consented to any assignment, delegation or other transfer by Licensee or the Foundation or any of their respective predecessors of any of their respective rights or obligations related, directly or indirectly, to the Royalty or that primarily relate to any Product or that (with or without the giving of notice or passage of time, or both) would reasonably be expected to result in a Product MAE, under the License Agreement or the Sponsored Research Agreement, as applicable. To the Knowledge of the Seller, neither Licensee nor the Foundation has assigned or otherwise transferred or granted any Liens with respect to any of its respective rights or obligations related, directly or indirectly, to the Royalty or that primarily relate to any Product or that (with or without the giving of notice or passage of time, or both) would reasonably be expected to result in a Product MAE.

(xi) No Indemnification Claims. The Seller has not notified Licensee or the Foundation or any other Person of, or otherwise made, any claims for indemnification under the License Agreement or the Sponsored Research Agreement, nor has the Seller received any claims for indemnification under the License Agreement or the Sponsored Research Agreement, whether pursuant to Section 5.1.4 or Article 17 of the License Agreement or Article 8 of the Sponsored Research Agreement, or otherwise.

(xii) No Royalty Reductions. The amount of the Royalty due and payable under Section 11.5 of the License Agreement is not, as of the date hereof, subject to any claim against the Seller pursuant to any right of set-off, rescission, counterclaim, reduction, credit, deduction or defense by contract or otherwise (any reduction on account of any such claim, a "Royalty Reduction"), including under Sections 11.5.3, 11.5.4, 11.5.5, 13 or 14.2 of the

License Agreement. To the Knowledge of the Seller, other than with respect to any Royalty Reductions under Section 11.5.3 of the License Agreement, no event or condition exists that, upon notice or passage of time or both, would reasonably be expected to permit Licensee to claim, or have the right to claim, a Royalty Reduction.

(xiii) No Notice of Infringement. The Seller has not received any notice from, or given any notice to, Licensee or the Foundation (or any of their respective predecessors or Affiliates) alleging any actual, potential, suspected or threatened infringement, misappropriation or other violation by a Third Party of any intellectual property rights related to any Product.

(xiv) Audits. None of the Seller, the Licensee nor the Foundation has initiated, pursuant to Section 14 of the License Agreement or otherwise, any inspection or audit of books of accounts or other records pertaining to any Product, Net Sales, the calculation of royalties or other amounts payable to the Seller under the License Agreement.

(xiv) Adverse Events. None of the Seller, Licensee or the Foundation has informed the other about serious adverse events occurring or having occurred in connection with the use of any Product under Section 9.2.1 of the License Agreement that has had, or would reasonably be expected to result in, a Product MAE, and no pharmacovigilance agreement under Section 9.2.3 of the License Agreement is or has been required.

(xvi) Committee Membership. As of the date hereof, the parties to the License Agreement have established the JSC, the SMAF Clinical Trials Advisory Committee, the JIPT, and the JOTs identified on Schedule 4.1(i)(xvi) of the Disclosure Schedule. Schedule 4.1(i)(xvi) includes a list of the Alliance Directors and members of JSC, the SMAF Clinical Trials Advisory Committee, the JIPT, and the JOTs as of the date hereof. All such committees have the authority under, and have been operating under the objectives, responsibilities and in the manner provided in, the License Agreement, and no material disputes have arisen in connection with any matters subject to the oversight of such committees. The Seller has not exercised its rights pursuant to Section 7.14 of the License Agreement to determine not to appoint to, or withdraw members of, the JSC and any JOC and not to appoint or remove an Alliance Director, or withdraw from participation in the JSC and any JOT or other committee under the License Agreement.

(i) Title to Royalty. The Seller has good and marketable title to the Royalty free and clear of all Liens (other than Permitted Liens). Upon payment of the Purchase Price by the Buyer, the Buyer will acquire, subject to the terms and conditions set forth in this Agreement and the License Agreement, good and marketable title to the Assigned Royalty Payments, free and clear of all Liens (other than any Permitted Lien).

(j) Intellectual Property.

(i) Schedule 4.1(j)(i) of the Disclosure Schedule lists all Joint Patent Rights, PTC Patent Rights and, to the Knowledge of the Seller, Roche Patent Rights (collectively, the "Licensed Patents"). The Seller is the sole owner of, and has the sole interest in, all of the PTC Patent Rights. To the Knowledge of the Seller, Licensee is the sole owner of, and has the sole interest in, all of the Roche Patent Rights. The Seller and Licensee collectively are the sole owners of, and collectively have the sole interest in, the Joint Patent Rights, and the Seller is the sole owner of, and has the sole interest in, its undivided half interest in each of the Joint Patent Rights. Schedule 4.1(j)(i) of the Disclosure Schedule specifies as to each of the (x) PTC Patent Rights and Joint Patent Rights Handled by the Seller and (y) to the extent the following information has been provided by Licensee to the Seller with respect to the Roche Patent Rights, as applicable, the jurisdictions by or in which each such patent has issued as a patent or such patent application has been filed, including the respective patent numbers and application numbers and issue and filing dates.

(ii) There are no pending or, to the Knowledge of the Seller, threatened, litigations, interferences, reexamination, re-issue, *inter partes* reviews, post-grant-reviews, oppositions or like procedures involving any PTC Patent Rights or Joint Patent Rights. To the Knowledge of the Seller, there are no pending or threatened litigations, interferences, reexamination, re-issue, inter partes reviews, post-grant-reviews, oppositions or the like procedures involving any Roche Patent Right.

(iii) All of the issued PTC Patent Rights and the Joint Patent Rights Handled by the Seller are in full force and effect and have not lapsed, expired or otherwise terminated, and, to the Knowledge of the Seller, are valid and enforceable. The Seller has not received any written notice relating to the lapse, expiration or other termination of any of the PTC Patent Rights or the Joint Patent Rights Handled by the Seller, or any written legal opinion that alleges that any of the issued PTC Patent Rights or the Joint Patent Rights Handled by the Seller is invalid or unenforceable. To the Knowledge of the Seller, all of the issued Roche Patent Rights are in full force and effect and have not lapsed, expired or otherwise terminated, and are valid and enforceable.

(iv) Each individual associated with the prosecution of the PTC Patent Rights, the Joint Patent Rights Handled by the Seller and, to the Knowledge of the Seller, all other Joint Patent Rights, including the named inventors, has complied in all material respects with all applicable duties of candor and good faith in dealing with any Patent Office, including any duty to disclose to any Patent Office all information known by such inventors to be

material to the patentability of each of the PTC Patent Rights and Joint Patent Rights (including any relevant prior art), in each case, in those jurisdictions where such duties exist. There is no Person who is or claims to be an inventor under any of the PTC Patent Rights or the Joint Patent Rights Handled by the Seller or, to the Knowledge of the Seller, any other Joint Patent Rights, who is not a named inventor thereof.

(v) The Seller has not, and, to the Knowledge of the Seller, Licensee has not, received any written notice of any claim by any Person challenging the inventorship or ownership of, the rights of the Seller or Licensee, as applicable, in and to, or the patentability, validity or enforceability of, any Licensed Patent, or asserting that the development, manufacture, importation, sale, offer for sale or use of the Product infringes any Patent Rights or other intellectual property rights of such Person.

(vi) To the Knowledge of the Seller, the development, manufacture, use, marketing, sale, offer for sale, importation or distribution of the Product has not infringed, misappropriated or otherwise violated any Patent Rights or other intellectual property rights owned by any other Person. Neither the Seller nor, to the Knowledge of the Seller, Licensee, has in-licensed any Patent Rights or other intellectual property rights covering the manufacture, use, marketing, sale, offer for sale, importation or distribution of the Product.

(vii) To the Knowledge of the Seller, no Third Party has infringed, misappropriated or otherwise violated, or is infringing, misappropriating or otherwise violating, any of the Licensed Patents or any other Patent Rights claiming the composition of matter of, or the method of making or using, the Product, except as would not reasonably be expected to result in a Product MAE.

(viii) All required maintenance fees, annuities and like payments with respect to the PTC Patent Rights and Joint Patent Rights Handled by the Seller in accordance with Section 15.3 or 15.5 of the License Agreement, and to the Knowledge of the Seller, with respect to all other Licensed Patents, have been paid timely.

(k) UCC Representation and Warranties. The Seller's exact legal name is, and for the immediately preceding ten (10) years has been, "PTC Therapeutics, Inc.". The Seller is, and for the prior ten (10) years has been, incorporated in Delaware.

(l) Brokers' Fees. There is no investment banker, broker, finder, financial advisor or other intermediary who has been retained by or is authorized to act on behalf of the Seller who might be entitled to any fee or commission in connection with the transactions contemplated by this Agreement.

Section 4.2 The Buyer's Representations and Warranties. The Buyer represents and warrants to the Seller that as of the date hereof:

(a) Existence; Good Standing. The Buyer is a statutory trust duly organized, validly existing and in good standing under the laws of the State of Delaware.

(b) Authorization. The Buyer has the requisite trust right, power and authority to execute, deliver and perform its obligations under this Agreement. The execution, delivery and performance of this Agreement, and the consummation of the transactions contemplated hereby, have been duly authorized by all necessary action on the part of the Buyer.

(c) Enforceability. This Agreement has been duly executed and delivered by an authorized person of the owner trustee of the Buyer and constitutes the valid and binding obligation of the Buyer, enforceable against the Buyer in accordance with its terms, except as may be limited by applicable Bankruptcy Laws or by general principles of equity (whether considered in a proceeding in equity or at law).

(d) No Conflicts. The execution, delivery and performance by the Buyer of this Agreement do not and shall not (i) contravene or conflict with the organizational documents of the Buyer, (ii) contravene or conflict with or constitute a default under any material provision of any law binding upon or applicable to the Buyer or (iii) contravene or conflict with or constitute a default under any material contract or other material agreement or Judgment binding upon or applicable to the Buyer.

(e) Consents. No consent, approval, license, order, authorization, registration, declaration or filing with or of any Governmental Entity or other Person is required to be done or obtained by the Buyer in connection with (i) the execution and delivery by the Buyer of this Agreement, (ii) the performance by the Buyer of its obligations under this Agreement, other than the filing of financing statement(s) in accordance with Section 2.4, or (iii) the consummation by the Buyer of any of the transactions contemplated by this Agreement.

(f) No Litigation. There is no action, suit, investigation or proceeding pending or, to the knowledge of the Buyer, threatened before any Governmental Entity to which the Buyer is a party that would, if determined adversely, reasonably be expected to prevent or materially and adversely affect the ability of the Buyer to perform its obligations under this Agreement.

(g) Financing. The Buyer has sufficient cash on hand to pay the entire Purchase Price. The Buyer acknowledges that its obligations under this Agreement are not contingent on obtaining financing.

(h) Brokers' Fees. There is no investment banker, broker, finder, financial advisor or other intermediary who has been retained by or is authorized to act on behalf of the Buyer who might be entitled to any fee or commission in connection with the transactions contemplated by this Agreement.

Section 4.3 No Implied Representations and Warranties. EXCEPT AS EXPRESSLY SET FORTH IN SECTION 4.1, THE SELLER MAKES NO REPRESENTATION OR WARRANTY, EXPRESS OR IMPLIED, AT LAW OR IN EQUITY, INCLUDING WITH RESPECT TO MERCHANTABILITY, FITNESS FOR ANY PARTICULAR PURPOSE OR NONINFRINGEMENT, AND ANY SUCH REPRESENTATIONS OR WARRANTIES ARE HEREBY EXPRESSLY DISCLAIMED. THE BUYER ACKNOWLEDGES THAT, EXCEPT AS SPECIFICALLY PROVIDED IN THIS ARTICLE 4, THE SELLER HAS ASSUMED NO RESPONSIBILITIES OF ANY KIND WITH RESPECT TO ANY ACT OR OMISSION OF LICENSEE WITH RESPECT TO THE DESIGN, DEVELOPMENT, MANUFACTURE, USE, SALE, DISTRIBUTION, MARKETING OR OTHER ACTIVITIES OF LICENSEE WITH RESPECT TO THE PRODUCT.

ARTICLE 5.

COVENANTS

Section 5.1 Disclosures. Except for a press release previously approved in form and substance by the Seller and the Buyer, or any other public announcement using substantially the same text as such press release, neither the Buyer nor the Seller shall, and each party hereto shall cause its respective Representatives, Affiliates and Affiliates' Representatives not to, issue a press release or other public announcement or otherwise make any public disclosure with respect to this Agreement or the subject matter hereof without the prior written consent of the other party hereto (which consent shall not be unreasonably withheld, conditioned or delayed), except as may be required by applicable law or stock exchange rule (in which case the party hereto required to make the press release or other public announcement or disclosure shall use commercially reasonably efforts to allow the other party hereto reasonable time to comment on such press release or other public announcement or disclosure in advance of such issuance).

Section 5.2 Payments.

(a) Payments.

(i) Promptly, and in any event, within [**] following the Closing, the Seller shall deliver to the Licensee (x) an instruction letter, in substantially the form attached hereto as Exhibit G (the "Licensee Instruction Letter"), duly executed by the Buyer and the Seller, requesting that the Licensee pay the Assigned Royalty Payments to an account designated by the Buyer and deliver Royalty Reports directly to the Buyer simultaneously with delivery of Royalty Reports to the Seller, and (y) an IRS Form W-8BEN-E provided by Buyer pursuant to Section 3.5 hereof. If, prior to the first Commercial Sale of Product, the Licensee does not agree to make payment of the Assigned Royalty Payments to an account designated by the Buyer, then (x) the parties shall execute and deliver to the Escrow Agent within [**] after the First Commercial Sale of a Product the Escrow Agreement and (y) the Seller shall instruct the Licensee to make all payments of the Royalty to the

Escrow Account until such time as the Assigned Royalty Cap is met. In the event that the Seller sells, transfers, assigns or otherwise conveys any portion of its retained rights in the Royalty to an Affiliate or Third Party, the Buyer agrees not to object to amending, and to reasonably cooperate to amend, the Escrow Agreement to add any such Affiliate or Third Party for the purposes of directing to such Third Party any portion of the Royalty to which such Third Party may be entitled as a result of such transaction. In no event shall the Escrow Agreement permit the Escrow Agent to distribute payments of the Royalty to the parties other than in accordance with each party's applicable Pro Rata Portion, or to withhold any distribution to the parties (whether as a result of any claims between the parties or otherwise), in each case without the prior written consent of both parties.

(ii) If, notwithstanding the terms of this Agreement, the Licensee Instruction Letter and the Escrow Agreement, as applicable, the Licensee, or any other Person, makes any payment in respect of the Assigned Royalty Payments to the Seller (or to any of the Seller's Affiliates or designees) instead of to the Buyer or the Escrow Account, as applicable, then: (A) the Seller shall hold (or shall cause such Affiliate or designee to hold) such payment in trust for the sole benefit of the Buyer, (B) the Seller (or such Affiliate or designee) shall have no right, title or interest whatsoever in such payment and shall not create or suffer to exist any Lien thereon, and (C) the Seller (or such Affiliate or designee) promptly, and in any event no later than [**] following the receipt by the Seller (or such Affiliate or designee) of such payment, shall remit, or cause to be remitted, an amount equal to such payment to an account designated in writing by the Buyer or to the Escrow Account to be distributed pursuant to the Escrow Agreement, as applicable.

(iii) If, notwithstanding the terms of this Agreement, the Licensee Instruction Letter and the Escrow Agreement, as applicable, the Licensee, or any other Person, makes any payment due under the License Agreement that does not constitute an Assigned Royalty Payment, to the Buyer (or to any of the Buyer's Affiliates or designees) instead of to the Seller or the Escrow Account, as applicable, or as otherwise instructed by the Seller, then: (A) the Buyer shall hold (or shall cause such Affiliate or designee to hold) such payment in trust for the sole benefit of the Seller, (B) the Buyer (or such Affiliate or designee) shall have no right, title or interest whatsoever in such payment and shall not create or suffer to exist any Lien thereon, and (C) the Buyer (or such Affiliate or designee) promptly, and in any event no later than [**] following the receipt by the Buyer (or such Affiliate or designee) of such payment, shall remit, or cause to be remitted, an amount equal to such payment to an account designated in writing by the Seller or to the Escrow Account to be distributed pursuant to the Escrow Agreement, as applicable.

(b) If either the Seller or the Buyer fails to timely comply with their respective obligations under Section 5.2(a), then all amounts not timely paid by the due date provided therein shall accrue interest from and including the date such amount was due through but excluding the date such payment in full (together with all interest thereon) is made to the applicable party, at a rate, calculated on a 365-day or 366-day basis, as applicable, equal to the then-current Prime Rate plus [**] percent ([**]%), compounded annually, not to exceed the maximum interest that may be charged under applicable law.

(c) Except as set forth in Section 5.2(a)(i), the Seller shall not revoke, amend, modify, supplement, restate, waive, cancel or terminate the Licensee Instruction Letter or any instruction to the Licensee to make payments in respect of the Royalty to the Escrow Agent without the prior written consent of Buyer.

Section 5.3 Royalty Reductions. If Licensee exercises any Royalty Reduction against any payment of the Royalty that is not (a) a Permitted Royalty Reduction, (b) the result of any Credit Event or (c) a breach by Licensee of its payment obligations under the License Agreement, such Royalty Reduction shall not reduce the amount of any Assigned Royalty Payments to which the Buyer is entitled, and if such Royalty Reduction reduces any payment of the Assigned Royalty Payments to less than the full amount of the Assigned Royalty Payments, then the Seller shall promptly (and in any event within [**] following the payment of the Assigned Royalty Payments affected by such Royalty Reduction) make a true-up payment to the Buyer such that the Buyer receives the full amount of such Assigned Royalty Payments that would have been payable to the Buyer had such Royalty Reduction not occurred. The Seller agrees to notify the Buyer in writing as promptly as possible (and in any event within [**]) of becoming aware of any actual or potential Royalty Reductions, including Permitted Royalty Reductions. For the avoidance of doubt, any nonpayment by Licensee as a result of a Credit Event or a breach by Licensee of its payment obligations under the License Agreement shall not constitute a Royalty Reduction for purposes of this Section 5.3 and shall not obligate the Seller to make any payment under this Section 5.3 (unless and until such nonpayment is cured). For all purposes hereunder, any true-up payment made pursuant to this Section 5.3 will be treated as paid with respect to the Royalty for U.S. federal income tax purposes to the fullest extent permitted by applicable law.

Section 5.4 Royalty Reports; Other Product Information.

(a) Until such time as the Licensee has agreed to provide copies of Royalty Reports directly to the Buyer pursuant to the Instruction Letter, as promptly as possible (and in any event within [**]) following the receipt by the Seller of any Royalty Report, the Seller shall deliver to the Buyer a report in the form attached hereto as Exhibit H prepared by the Seller and certified by a duly authorized official of the Seller as to the authenticity of such Royalty Report (a "Royalty Report Certification"). From and after such time as the Licensee has agreed to provide copies of Royalty Reports directly to the Buyer pursuant to the Instruction Letter, the Seller shall as promptly as possible (and in any event within [**]) deliver to the Buyer copies of any Royalty Reports not provided to the Buyer directly, and any supporting

documentation, information, or other reports (if any) received by the Seller from Licensee relating to the Royalty for such Calendar Quarter to the extent not provided to the Buyer directly.

(b) The Seller agrees to request that Roche deliver Product Information (other than a Royalty Report as provided in Section 5.4(a) above) to the Buyer directly as more fully described in, and in accordance with the terms of, Exhibit I. Until such time as the Licensee has agreed to deliver Product Information (other than Royalty Reports) directly to the Buyer, the Seller agrees to deliver to the Buyer, at least on a [**] basis, new or updated Product Information that the Seller receives from the Licensee. On an at least [**] basis, the Seller shall deliver to the Buyer a reasonably detailed summary of new or updated information of which it has Knowledge about any decision, order, dispute or challenge, of or related to the validity, patentability, scope, priority, construction, non-infringement, inventorship, ownership or enforceability of any of the PTC Patent Rights, the Joint Patent Rights Patent or Roche Patent Rights or any claim thereof, or opposition of the grant of any patent that falls within the PTC Patent Rights, Joint Patent Rights or Roche Patent Rights, in any legal or administrative proceedings, including in a court of law, before the United States Patent and Trademark Office or other agency or tribunal in any jurisdiction, or in arbitration, including by reexamination, *inter partes* review, opposition, interference, post-grant review, nullity proceeding, preissuance submission, third party submission, derivation proceeding or declaratory judgment action.

(c) The Seller agrees to notify the Buyer within [**] after the Seller's receipt or Knowledge of a Third Party Paragraph IV certification with respect to the PTC Patent Rights, the Joint Patent Rights or the Roche Patent Rights. The Seller shall provide the Buyer [**] with a list, substantially in the form of Schedule 4.1(j)(i) of the Disclosure Schedule, of PTC Patent Rights and Joint Patent Rights Handled by the Seller or of which the Seller then has Knowledge covering the Product.

(d) Notwithstanding the foregoing, until such time as the Licensee has agreed to deliver Product Information directly to the Buyer, if the Seller is advised by its counsel that providing the Buyer with a true, correct and complete copy of Product Information or other documents or information pursuant to this Agreement (whether pursuant to this Section 5.4 or otherwise) would be reasonably expected to constitute a material breach by the Seller of its confidentiality obligations under the License Agreement (a "Confidentiality Breach"), then the Seller shall, in lieu of delivery of such Product Information, provide a written summary thereof to the Buyer describing in as much detail as would not result in a Confidentiality Breach, the substance of such Product Information or other documents or information.

Section 5.5 Actions under the License Agreement while the Seller is the Control Party. The following provisions shall only apply while the Seller is the Control Party:

(a) Compliance; No Modifications to Reduce Assigned Royalty Payments. The Seller shall comply in all material respects with its obligations under the License Agreement and the Sponsored Research Agreement. The Seller shall not, without the prior written consent of the Buyer, make, propose, effect, deliver, execute or grant any amendment, modification, consent, notice or waiver, or take (or fail to take) any other action, under the License Agreement or the Sponsored Research Agreement that (with or without the giving of notice or passage of

time, or both) would reasonably be expected to result in a Product MAE. Promptly (and in any event within [**]) after the Seller's receipt of any written notice from Licensee of an alleged breach or default under the License Agreement or the Sponsored Research Agreement by the Seller, the Seller shall give written notice thereof to the Buyer, including delivering to the Buyer a copy of any such written notice. The Seller shall use commercially reasonable efforts to cure any material breach or default by the Seller under the License Agreement or the Sponsored Research Agreement, as applicable, and shall give written notice to the Buyer upon curing any such breach or default. The Seller shall pay all of its costs and expenses (including of counsel) in connection with any such breach or default. Promptly (and in any event within [**]) after the Seller becomes aware of, or comes to believe in good faith that there has been, a breach of the License Agreement or the Sponsored Research Agreement by Licensee or the Foundation, the Seller shall provide notice of such breach to the Buyer. If such breach (with or without the giving of notice or passage of time, or both) would reasonably be expected to result in a Product MAE, the Seller shall consult with the Buyer regarding the timing, manner and conduct of any enforcement of Licensee's obligations under the License Agreement and shall consider in good faith all of Buyer's timely comments and suggestions regarding such enforcement, provided that (i) nothing in this Section 5.5(a) shall obligate the Seller to take or refrain from taking any action in respect of any such enforcement and (ii) the Buyer shall pay its Pro Rata Portion of the reasonable and documented costs and expenses of any such enforcement (excluding any costs and expenses of or reimbursable by Licensee).

(b) Notices, Correspondence and Other Information Delivered by the Seller. The Seller shall send a copy of any Product Information that the Seller delivers to Licensee, the Foundation, any Governmental Entity or other governing authority, as the case may be, concurrently to the Buyer; provided, however, that the Seller shall (a) deliver to the Buyer any notices it intends to provide to Licensee pursuant to Section 7.15 of the License Agreement to the Buyer at least [**] prior to providing such notice to Licensee and shall consult with the Buyer regarding such notice.

Section 5.6 Actions under the License Agreement while the Buyer is the Control Party. The following provisions shall only apply while the Buyer is the Control Party:

(a) Notices and Other Information to the Licensee. The Seller shall not send, without the prior written consent of the Buyer (or refrain from sending if instructed to do so by the Buyer), any written notice or correspondence to Licensee, the Foundation, any Governmental Entity or any other Person if such notice or correspondence relates solely to the Assigned Royalty Payment or if such notice or correspondence would reasonably be expected (with or without the giving of notice or passage of time, or both) to result in a Product MAE. Notwithstanding the foregoing, no prior consent of the Buyer shall be required for the Seller to deliver notice pursuant to Sections 7.15 or 20.2 of the License Agreement, provided, that the Seller shall deliver to the Buyer any notices it delivers to Licensee pursuant to Section 7.15 of the License Agreement substantially concurrently with delivery of such notice to Licensee. The Seller shall deliver to the Buyer true, correct and complete copies of any notices it actually provides pursuant to the License Agreement concurrently with the Seller's delivery thereof to the Licensee, Foundation or any other Person, as applicable.

(b) Amendment of License Agreement. The Seller shall not, except with the prior written consent of the Buyer (such consent not to be unreasonably withheld, conditioned or delayed if such amendment would not reasonably be expected, with or without the giving of notice or the passage of time, or both, to result in a Product MAE), amend, modify, waive, consent to or supplement or restate (or consent to any amendment, modification, waiver, consent, supplement or restatement of or to) any provision of the License Agreement or the Sponsored Research Agreement, including any plans or budgets thereunder. Subject to the foregoing, promptly, and in any event within [**], following receipt by the Seller of any final amendment, modification, waiver, consent, supplement or restatement of the License Agreement, or the Sponsored Research Agreement, the Seller shall furnish a copy of the same to the Buyer.

(c) Maintenance of License Agreement.

(i) The Seller shall comply in all material respects with its obligations under the License Agreement and the Sponsored Research Agreement and shall not take any action or forego any action that would reasonably be expected to constitute a material breach or default thereof. Promptly, and in any event within [**], after receipt of any (written or oral) notice from Licensee of an alleged breach or default under the License Agreement or the Sponsored Research Agreement by the Seller under the License Agreement or the Sponsored Research Agreement, the Seller shall give written notice thereof to the Buyer, including delivering the Buyer a copy of any such written notice. The Seller shall use its reasonable efforts to cure any breach or default by the Seller under the License Agreement or the Sponsored Research Agreement, as applicable, as reasonably instructed by the Buyer, and shall give written notice to the Buyer upon curing any such breach or default. The Seller shall pay all of its costs and expenses (including of counsel) in connection with any such breach or default.

(ii) In connection with any dispute regarding any such alleged breach or default by Licensee that is related to the Royalty or primarily related to any Product or that would reasonably be expected (with or without the giving of notice or passage of time, or both) to result in a Product MAE, the Seller shall employ such counsel reasonably acceptable to the Seller as the Buyer shall select for such purpose, provided that the Buyer shall pay its Pro Rata Portion of the reasonable and documented costs and expenses incurred by the Seller in connection with any such dispute (net of any amounts recovered by the Seller).

(iii) The Seller shall consult with the Buyer prior to appointing to or withdrawing members of the JSC and any JOC and prior to appointing or removing an Alliance Director, or withdrawing from participation in the JSC and any JOT or other committee under the License Agreement.

(iv) The Seller shall not, except as mutually agreed by the parties, (a) forgive, release, settle or compromise any amount owed to or becoming owed to the Seller under the License Agreement in respect of the Royalty or (b) waive any obligation of, or grant any consent to, the Licensee under, in respect of or

related to the Royalty; provided, that neither the occurrence of a Credit Event nor any automatic effect of a Credit Event under the License Agreement without an affirmative action of the Seller shall itself be deemed any forgiving, release, settlement, compromise, waiver or consent by the Seller.

(v) If the Seller is provided with an opportunity to assist with the establishment of any budgets in connection with New Product development as provided in Section 5.2 of the License Agreement, or to agree to matters related to a diagnostic Product as provided in Section 11.7 of the License Agreement, the Seller shall undertake such activities in consultation with the Buyer. The Seller further agrees to consult with and give due consideration to the Buyer's input in connection with the matters provided in Sections 7.8 and 10 of the License Agreement.

(vi) The Seller shall not exercise its rights under the License Agreement or the Sponsored Research Agreement in any manner that would result in a breach of this Agreement or would reasonably be expected (with or without the giving of notice or the passage of time, or both) to result in a Product MAE.

(d) Enforcement of License Agreement.

(i) Notice of Breaches by Licensee. Promptly (and in any event within [**]) after the Seller becomes aware of, or comes to believe in good faith that there has been, a breach of the License Agreement or the Sponsored Research Agreement by Licensee or the Foundation, the Seller shall provide notice of such breach to the Buyer. In addition, the Seller shall provide to the Buyer a copy of any written notice of such breach or alleged breach of the License Agreement or the Sponsored Research Agreement delivered by the Seller to Licensee as soon as practicable and in any event not less than [**] following such delivery.

(ii) Enforcement of License Agreement. In the case of any breach by Licensee or the Foundation referred to in Section 5.6(d)(i), the Seller shall consult with the Buyer regarding the timing, manner and conduct of any enforcement of Licensee's obligations under the License Agreement or the Foundation's obligations under the Sponsored Research Agreement. Following such consultation, the Seller shall, (i) as reasonably instructed by the Buyer, exercise such rights and remedies relating to any such breach as shall be available to the Seller, whether under the License Agreement, the Sponsored Research Agreement or by operation of law and, (ii) if such breach is related to the Royalty or would reasonably be expected (with or without the giving of notice or passage of time, or both) to result in a Product MAE, employ such counsel reasonably acceptable to the Seller as the Buyer shall select for such purpose.

(iii) Allocation of Proceeds and Costs of Enforcement. Each of the Buyer and the Seller shall bear its own fees and expenses incurred in enforcing Licensee's obligations under the License Agreement pursuant to this Section

5.6(d), provided that the Buyer shall pay its Pro Rata Portion of the reasonable and documented costs and expenses incurred by the Seller in connection with the Seller's enforcement of the License Agreement in accordance with Section 5.6(d)(ii) (net of any amounts reimbursable by Licensee). The Proceeds resulting from any enforcement of Licensee's obligations under the License Agreement shall be applied first to reimburse the Seller and the Buyer for any expenses incurred by them in connection with such enforcement, and the remainder of the Proceeds shall be allocated between the Seller and the Buyer as though such Proceeds constituted Royalty payments. The Seller hereby assigns and, if not presently assignable, agrees to assign to the Buyer the amount of Proceeds due to the Buyer in accordance with this Section 5.6(d).

Section 5.7 Inspections and Audits of Licensee. If either party desires to cause an inspection as provided under Section 14 of the License Agreement, then the Seller and the Buyer agree to consult in good faith with each other in connection therewith. Following such consultation, the Seller may, or if requested by the Buyer, shall, promptly provide written notice to Licensee to cause such an inspection. The Seller shall, for purposes of Section 14 of the License Agreement, select such independent certified public accounting firm as is reasonably acceptable to both the Seller and the Buyer (as long as such independent certified public accountant is reasonably acceptable to Licensee as required by Section 14 of the License Agreement). Each of the Buyer and the Seller shall be responsible for its Pro Rata Portion of the expense of any inspection or audit undertaken by either the Buyer or the Seller (including the fees and expenses of such independent certified public accounting firm designated for such purpose) that would otherwise be borne by the Seller pursuant to the License Agreement (if and as such expenses are actually incurred by the Seller). The Seller shall deliver to the Buyer a copy of the results of any audit conducted pursuant to Section 14 of the License Agreement within [**] following the Seller's receipt thereof (and the results of any such audit shall be considered Product Information). If an audit reveals an underpayment by the Licensee, then the Royalty shall include the amount paid by the Licensee to the Seller to compensate for such underpayment in accordance with Section 14.2 of the License Agreement, with interest thereon as set forth in Section 12.2 of the License Agreement. If an audit reveals an overpayment by the Licensee, then the Seller shall comply with its obligations set forth in Section 14.2 of the License Agreement and, in furtherance thereof, may, in its sole discretion, require that the Buyer pay the Seller an amount equal to the Buyer's Pro Rata Portion of such overpayment or credit an amount equal to the Buyer's Pro Rata Portion of such overpayment against future Assigned Royalty Payments.

Section 5.8 Termination of License Agreement.

(a) The Seller shall not, without the prior written consent of the Buyer, (i) exercise any right to terminate the License Agreement, in whole or in part, (ii) agree with Licensee to terminate the License Agreement, in whole or in part, or (iii) take, or permit any Affiliate or sublicensee to take, any action that would reasonably be expected to give Licensee the right to terminate the License Agreement, in whole or in part. The Seller shall not take any action, fail to take an action or permit an action to be taken, that would give Licensee the right to terminate the License Agreement under Section 20.3.1 thereof, provided that in no event shall the

Seller be obligated to prevent any termination of the License Agreement as a result of a Credit Event so long as such termination is not also a result of any affirmative action of the Seller.

(b) Effect of Termination.

(i) In the event that the License Agreement is terminated while the Seller is the Control Party and prior to a Change of Control of the Seller, and the Seller has rights under Section 20.4 of the License Agreement, the Seller shall have the right in its sole discretion to determine whether and how to continue the development and commercialization of Compounds and Products as provided in Section 20.4 of the License Agreement (including via direct sales by the Seller or its Affiliates and licenses to one or more distributors and other Third Parties on a regional basis); provided, that, the Seller shall consult with the Buyer as to its plans (a "Seller Direct Undertaking"). If the Seller, in its sole discretion, determines to completely out-license to one or more Third Parties (each, a "Seller New Licensee") the global development and commercialization of Compounds and Products (a "Seller New Arrangement"), the Seller shall notify the Buyer in writing and shall promptly provide copies of any license agreement with a Seller New Licensee for the continued commercialization of the Compounds and Products, and commercialization plans therefor.

(ii) If the License Agreement is terminated in whole or in part by Licensee pursuant to Section 20.3.3 (Termination Without a Cause) or by the Seller pursuant to Section 20.3.1 (Termination for Breach), Section 20.3.4 (Termination for Patent Challenge) or Section 20.3.5 (Termination for Post-Change of Control Material Change) in each case of the License Agreement while the Buyer is the Control Party or following a Change of Control of the Seller (any such termination while the Buyer is the Control Party or following a Change of Control of the Seller, a "Triggering Termination"), then the Seller shall have the first right to continue development and commercialization of Compounds and Products as provided in Section 20.4.1 of the License Agreement and pursue its rights under Sections 20.4.1.1, 20.4.1.2, 20.4.1.3, 20.4.1.4(i), 20.4.1.4(ii), 20.4.1.4(iii), 20.4.1.4(iv), 20.4.1.4(v), 20.4.1.5, 20.4.1.6, 20.4.1.7, 20.4.1.8 and 20.4.3 of the License Agreement (collectively, the "Reversionary Rights"). The Seller shall inform the Buyer in writing within [**] of a Triggering Termination whether or not it has elected to pursue the Reversionary Rights and shall thereafter keep Buyer reasonably informed as to the status of, and shall use commercially reasonable efforts in connection with, the pursuit of such development and commercialization under the Reversionary Rights, including delivering to the Buyer concurrently with deliver of the same to Licensee, notice pursuant to Section 20.4.1.9 of the License Agreement. In the event that the Seller does not notify the Buyer in writing that the Seller has elected to pursue the Reversionary Rights within [**] of a Triggering Termination, the Buyer shall have the right to cause the Seller to pursue the Reversionary Rights by delivering written notice to the Seller and the Seller shall act as reasonably instructed by the

Buyer to pursue the Reversionary Rights. The Buyer shall indemnify, defend and hold harmless the Seller and its Affiliates against and in respect of all Losses suffered or incurred by the Seller or its Affiliates to the extent arising out of or resulting from the Seller's or its Affiliates' exercise of the Reversionary Rights solely at the Buyer's direction following a Triggering Termination, other than (A) the Seller's Pro Rata Portion of any out of pocket fees, including attorneys', consultants' or other professional advisor costs and expenses incurred in connection with exercising its Reversionary Rights pursuant to this Section 5.8(b)(ii) or as contemplated by Section 5.8(b)(iii) and (B) costs, expenses or other amounts attributable to the development, manufacture and commercialization of Compounds and Products under the License Agreement or the Sponsored Research Agreement that would otherwise have been borne by the Seller and its Affiliates. The Seller shall promptly, and in any event within [**], provide the Buyer with access to all materials, reports, information and property delivered, provided or otherwise made available to the Seller in connection with such Triggering Termination, the Licensee's ongoing obligations and the Reversionary Rights.

(iii) If there is a Triggering Termination and the Buyer is pursuing the Reversionary Rights pursuant to Section 5.8(b)(ii), the Buyer shall have the exclusive right to negotiate one or more licenses or sublicenses to continue to commercialize any Product that has obtained Regulatory Approval (each, a "Marketed Product") with one or more Third Parties under the Reversionary Rights (each such license or sublicense, a "Buyer Negotiated Arrangement"). The Seller shall reasonably cooperate with the Buyer, at the Buyer's direction, in connection with the negotiation, execution and delivery of any Buyer Negotiated Arrangement. Except with respect to the license or sublicense of any intellectual property rights included in the Reversionary Rights (which licenses or sublicenses shall be limited to the commercialization of the Marketed Product and any related development activities specifically with respect to such Marketed Product), no Buyer Negotiated Arrangement shall include terms, conditions and limitations that are, in the aggregate, more burdensome to the Seller than the licensing or sublicensing terms contained in the License Agreement. The Buyer shall reimburse the Seller for the Buyer's Pro Rata Portion of any out of pocket fees, including attorneys', consultants' or other profession advisor costs and expenses incurred in connection with the pursuit, negotiation or execution of any Buyer Negotiated Arrangement.

(iv) In connection with any Seller Direct Undertaking, Seller New Arrangement or Buyer Negotiated Arrangement, (a) the Buyer shall be provided with economic rights equivalent to those remaining under this Agreement and with substantially the same other rights as provided in this Agreement, and (b) the Seller's rights and obligations under this Agreement in respect of the License Agreement shall otherwise apply to any Seller Direct Undertaking, any Seller New Arrangement or any Buyer Negotiated Arrangement, in each case *mutatis*

mutandis. In connection with any Buyer Negotiated Arrangement, the Seller shall be provided with economic rights equivalent to those remaining under this Agreement and with substantially the same other rights as provided in this Agreement. As soon as practicable following the Seller delivering written notice to the Buyer of any Seller Direct Undertaking or the execution of any Seller New Arrangement or Buyer Negotiated Arrangement by each party thereto, the Buyer and the Seller shall cooperate with one another to enter into new agreements (or to make amendments or modifications to this Agreement, the Bill of Sale and such other documents and the Buyer may reasonably request) to effect this clause (iv).

Section 5.9 No Liens. The Seller shall not hereafter mortgage, pledge, hypothecate or grant a security interest or other Lien of any kind in (i) the Assigned Royalty Payments (other than a Permitted Lien) or (ii) any of its interest in any portion of the PTC Patent Rights, Joint Patent Rights, any Product or the License Agreement (other than a Permitted Lien).

Section 5.10 Enforcement; Defense; Prosecution and Maintenance.

(a) The Seller shall promptly inform the Buyer of any suspected infringement by a Third Party of any of the Licensed IP or if any Third Party alleges that manufacture, use, sale, offer for sale or import of a Product infringes the intellectual property rights of a Third Party. The Seller shall (i) provide to the Buyer a copy of any written notice of any such suspected infringement and all pleadings filed in such action and (ii) notify the Buyer of any material developments in any claim, suit or proceeding resulting from such infringement that are delivered by or to Licensee or the Seller under Sections 15.8 and 15.9 of the License Agreement or otherwise as soon as practicable and in any event not less than [**] following such delivery.

(b) In the event the Buyer is the Control Party, and if the Seller has the right to join or assume the defense or pursuit of an enforcement action pursuant to Section 15.8 or 15.9 of the License Agreement, the Seller shall, if requested in writing by the Buyer, promptly, and in any event within [**] after receipt of such request, exercise such right as reasonably instructed by the Buyer and, if requested by the Buyer, the Seller shall employ such counsel reasonably acceptable to the Seller as the Buyer shall select for such purpose, provided that the Buyer shall pay its Pro Rata Portion of the costs and expenses of any such counsel. In the event the Buyer is the Control Party, the Seller shall not, except as reasonably instructed by the Buyer, exercise any consent or consultation rights under Section 15.8 or 15.9 of the License Agreement. In the event the Buyer is the Control Party, the Seller shall not, except as reasonably instructed by the Buyer, join any infringement action under Section 15.8 or 15.9 of the License Agreement. To the extent the Seller exercises its right to join or assume the defense or pursue an action while the Buyer is the Control Party pursuant to this Section 5.10(b), the Seller shall act as reasonably instructed by the Buyer satisfy its obligations under Section 15.8 or 15.9 of the License Agreement, including to (1) diligently defend or enforce any Licensed Patents (including by bringing and defending any counterclaim of invalidity or unenforceability, defending against any action of a Third Party for declaratory judgment of non-infringement or non-interference or bringing any legal action for infringement) and (2) not disclaim or abandon, or fail to take any

action necessary or desirable to prevent the disclaimer, abandonment or dismissal (including through lack of enforcement against Third Party infringers) of any defense of invalidity or unenforceability or any legal action for infringement of any Licensed Patents in the Territory for which it has elected to join or assume the defense or pursue an enforcement action pursuant to this Section 5.10(b).

(c) Promptly (and in any event within [**]) following the Seller's receipt of written notice from the Licensee pursuant to Section 15.4 or 15.5 of the License Agreement of the Licensee's intention to allow any of the Roche Patent Rights or the Joint Patent Rights in the Territory to lapse or become abandoned or to not file patent applications for any of the Roche Patent Rights or Joint Patent Rights in the Territory (such Patent Rights, the "Applicable Listed Patents"), the Seller shall inform the Buyer of such notice and, if the Buyer is the Control Party, the Seller shall, as mutually agreed with the Buyer, exercise its rights under Section 15.4 or 15.5 of the License Agreement to assume the prosecution and maintenance of any such Applicable Listed Patents and each of the Buyer and the Seller shall pay its Pro Rata Portion of the expenses in connection therewith. If the Seller, in its reasonable discretion and after consultation with the Buyer, determines to abandon or not file any patent applications, the Buyer shall have the right, at its sole expense, to direct the Seller to assume the prosecution and maintenance of any such Applicable Listed Patents and the Buyer shall be solely responsible for the expenses in connection therewith.

(d) The Seller shall satisfy, at its own expense, its prosecution obligations under Sections 15.3, 15.4 and 15.5 of the License Agreement, which actions while the Buyer is the Control Party shall be reasonably determined by the Buyer after consultation with the Seller, including to (i) take any and all actions, and prepare, execute, deliver and file any and all agreements, documents and instruments, that are reasonably necessary or desirable to diligently prosecute, preserve and maintain any Licensed Patents in the Territory for which it controls the prosecution and maintenance, including payment of maintenance fees or annuities on any such Licensed Patents, (ii) prosecute any corrections, substitutions, reissues, reviews, interferences and reexaminations of any Licensed Patents in the Territory, for which it controls the prosecution and maintenance, and file any other forms of patent term restoration in any applicable jurisdiction in the Territory, (iii) diligently defend any Licensed Patents for which it controls the prosecution and maintenance against any opposition, *inter partes* review, post-grant review or other proceeding before any Patent Office, and (iv) not disclaim or abandon, or fail to take any action necessary or desirable to prevent the disclaimer or abandonment, of any Licensed Patents in the Territory for which it controls the prosecution and maintenance. To the extent the Seller does not control the prosecution and maintenance obligations under Sections 15.3, 15.4 and 15.5 of the License Agreement, the Seller shall use commercially reasonable efforts to cause Licensee (or the Foundation, as applicable) to satisfy its prosecution and maintenance obligations thereunder and the Seller shall at its own expense exercise its consulting, request, consent, approval or similar rights, with such exercise to be reasonably determined by the Buyer after consultation with the Seller in the event the Buyer is the Control Party.

(e) Nothing in this Section 5.10 shall be construed to limit the Seller's obligations pursuant to Section 5.5(a) while the Seller is the Control Party.

(f) The Proceeds resulting from any enforcement actions under Section 15.8 of the License Agreement shall be applied first to reimburse for any expenses incurred by the Seller and the Buyer in connection with such enforcement, and the remainder of the Proceeds shall be allocated between the Seller and the Buyer as though such Proceeds constituted Royalty payments; provided, however, that in the event that the Seller initiates an action pursuant to Section 15.8 of the License Agreement, the Seller shall promptly notify the Buyer of such action and the Proceeds of any such action shall be allocated between the Seller and the Buyer as though such Proceeds constituted Royalty payments only if the Buyer funds its Pro Rata Portion of the costs of such action.

Section 5.11 Additional Monetizations. The Seller agrees to notify the Buyer in writing at least [**] prior to entering into a definitive agreement with a Third Party to assign, convey, monetize, impose a Lien upon or otherwise transfer or encumber any or all of its retained interest in the Royalty (a "Definitive Monetization Agreement"). For the avoidance of doubt, nothing herein shall prevent or limit the ability of the Seller to enter into or consummate any such Definitive Monetization Agreement; provided, that, if the Seller is the Control Party, the Seller agrees that if any Definitive Monetization Agreement provides a Third Party with information rights that are more favorable than the information rights provided to the Buyer in this Agreement, the Seller shall provide the Buyer with information rights that are as favorable to the Buyer as those provided to such Third Party, and the Seller shall effect such amendments or modifications to this Agreement or execute such other documents as the Buyer may reasonable request to effect the foregoing and provided further, that, for the avoidance of doubt, nothing in this Section 5.11 shall in any way limit the Buyer's rights pursuant to Section 5.5(a).

Section 5.12 Efforts to Consummate Transactions. Subject to the terms and conditions of this Agreement, each of the Seller and the Buyer shall use its commercially reasonable efforts to take, or cause to be taken, all actions and to do, or cause to be done, all things reasonably necessary under applicable law to consummate the transactions contemplated by this Agreement. Each of the Buyer and the Seller agrees to execute and deliver such other documents, certificates, agreements and other writings and to take such other actions as may be reasonably necessary in order to consummate or implement expeditiously the transactions contemplated by this Agreement.

Section 5.13 Further Assurances. After the Closing, the Seller and the Buyer agree to execute and deliver such other documents, certificates, agreements and other writings and to take such other actions as may be reasonably necessary in order to give effect to the transactions contemplated by this Agreement.

Section 5.14 Tax Matters.

(a) The Seller and Buyer shall treat the transactions contemplated by the Transaction Documents as a sale of the Assigned Royalty Payments for all United States federal state, local and non-U.S. Tax purposes. Accordingly, any and all Assigned Royalty Payments made after the Closing Date shall be treated as made to the Buyer for United States federal, state, local and non-U.S. Tax purposes.

(b) If there is a Permitted Withholding Tax Royalty Reduction, the Seller shall reasonably cooperate with, and assist, Buyer in delivering the prescribed forms necessary to reduce the applicable rate of, or to eliminate, withholding to the extent Buyer is entitled under any applicable tax treaty, in accordance with Section 13 of the License Agreement.

Section 5.15 Standstill. The Buyer agrees that during the term of this Agreement, or upon an earlier Standstill Termination, without the consent of the Seller, the Buyer shall not, and shall cause its Affiliates not to, (a) propose any merger, consolidation, business combination, tender or exchange offer or other business combination or change of control transaction involving all or substantially all of the Seller's equity, businesses or assets, on a consolidated basis (a "Seller Business Combination"); (b) provide financing to any Third Party in connection with a Seller Business Combination; (c) propose or seek, whether alone or in concert with others, any "solicitation" (as such term is used in the rules of the Securities and Exchange Commission) of proxies or consents to vote any securities of the Seller; (d) nominate any person as a director of the Seller; (e) propose any matter to be voted upon by the stockholders of the Seller; (f) directly or indirectly, form, join or in any way participate in a Third Party "group" (as such term is used in the rules of the Securities and Exchange Commission) (or discuss with any Third Party the potential formation of a group) with respect to any securities of the Seller or a Seller Business Combination; provided that, for the avoidance of doubt, nothing in this Section 5.15 shall prohibit the Buyer from agreeing to purchase any pharmaceutical royalties from the Seller or any Third Party. The term "Standstill Termination" shall mean the earliest of (1) the public announcement that the Seller has entered into a definitive agreement with a Third Party for a Change of Control or a transaction involving a Seller Business Combination; (2) if any person(s) or "group" publicly announces or commences a tender or exchange offer to acquire voting securities of the Seller, that, if successful, would result in such person or group beneficially owning more than 50% of the then outstanding voting securities of the Seller; (3) if any person(s) or "group" commences a proxy contest with respect to the Seller and (4) if any person(s) or "group" files a Schedule 13D with respect to the beneficial ownership of at least [**]% of the then outstanding voting securities of the Seller by such person(s) or "group".

ARTICLE 6.

CONFIDENTIALITY

Section 6.1 Confidentiality. Except as provided in this Article 6 or otherwise agreed in writing by the parties, the parties hereto agree that each party (the "Receiving Party") shall keep confidential and shall not publish or otherwise disclose and shall not use for any purpose other than as provided for in this Agreement (which includes the exercise of any rights or the performance of any obligations hereunder) any information furnished to it by or on behalf of the other party (the "Disclosing Party") pursuant to this Agreement (such information, "Confidential Information" of the Disclosing Party), except for that portion of such information that:

(a) was already known to the Receiving Party, other than under an obligation of confidentiality, at the time of disclosure by the Disclosing Party;

(b) was generally available to the public or otherwise part of the public domain at the time of its disclosure to the Receiving Party;

(c) became generally available to the public or otherwise part of the public domain after its disclosure and other than through any act or omission of the Receiving Party in breach of this Agreement;

(d) is independently developed by the Receiving Party or any of its Affiliates, as evidenced by written records, without the use of or reference of the Confidential Information; or

(e) is subsequently disclosed to the Receiving Party on a non-confidential basis by a Third Party without obligations of confidentiality with respect thereto.

Section 6.2 Authorized Disclosure.

(a) Either party may disclose Confidential Information with the prior written consent of the Disclosing Party or to the extent such disclosure is reasonably necessary in the following situations:

(i) prosecuting or defending litigation;

(ii) complying with applicable laws and regulations, including regulations promulgated by securities exchanges;

(iii) complying with a valid order of a court of competent jurisdiction or other Governmental Entity;

(iv) for regulatory, tax or customs purposes;

(v) for audit purposes, provided that each recipient of Confidential Information must be bound by customary obligations of confidentiality and non-use prior to any such disclosure (which obligations must be consistent with the obligations of confidentiality set forth in the License Agreement and no less protective than those in this Agreement);

(vi) disclosure to its Affiliates and Representatives on a need-to-know basis, provided that each recipient of Confidential Information must be bound by customary obligations of confidentiality and non-use prior to any such disclosure (which obligations must be consistent with the obligations of confidentiality set forth in the License Agreement and no less protective than those in this Agreement); or

(vii) disclosure to its actual or potential investors and co-investors, and other sources of funding, including debt financing, or potential partners, collaborators or acquirers, and their respective accountants, financial advisors and other professional representatives, provided, that such disclosure shall be made

only to the extent customarily required to consummate such investment, financing transaction partnership, collaboration or acquisition and that each recipient of Confidential Information must be bound by customary obligations of confidentiality and non-use prior to any such disclosure (which obligations must be consistent with the obligations of confidentiality set forth in the License Agreement and no less protective than those in this Agreement).

(b) Notwithstanding the foregoing, in the event the Receiving Party is required to make a disclosure of the Disclosing Party's Confidential Information pursuant to Sections 6.2(a)(i), (ii), (iii) or (iv), it will, except where impracticable, give reasonable advance notice to the Disclosing Party of such disclosure and use reasonable efforts to secure confidential treatment of such information. In any event, the Buyer shall not file any patent application based upon or using the Confidential Information of the Seller provided hereunder.

ARTICLE 7.

TERMINATION

Section 7.1 Automatic Termination. This Agreement shall continue in full force and effect until the earlier of sixty (60) days after such time as (A) the Buyer has received payments hereunder in an amount equal to the Assigned Royalty Cap and (B) Licensee (or any other applicable Third Party, including a New Licensee or a Buyer Selected Licensee) is no longer obligated to make any payments of the Royalty, at which point this Agreement, the Escrow Agreement and the Licensee Instruction Letter shall automatically terminate, except with respect to any rights that shall have accrued prior to such termination.

Section 7.2 Survival. Notwithstanding anything to the contrary in this Article 7, the following provisions shall survive termination of this Agreement: Section 5.1 (Disclosures), Section 5.2(a) (Payments; Adjustments for Pre-Closing Royalty Reductions), Article 6 (Confidentiality), Section 7.2 (Survival) and Article 8 (Miscellaneous). Termination of the Agreement shall not relieve any party of liability in respect of breaches under this Agreement by any party on or prior to termination.

ARTICLE 8.

MISCELLANEOUS

Section 8.1 Notices. All notices and other communications under this Agreement shall be in writing and shall be by email with PDF attachment, courier service or personal delivery to the following addresses, or to such other addresses as shall be designated from time to time by a party hereto in accordance with this Section 8.1:

If to the Seller, to it at:

PTC Therapeutics, Inc.

100 Corporate Court
South Plainfield, New Jersey 07080
Attention: Legal Department
Email: [**]

With a copy to:

Wilmer Cutler Pickering Hale and Dorr LLP

60 State Street
Boston, Massachusetts 02109
Attention: Steven D. Barrett and George W. Shuster, Jr.
Email: steven.barrett@wilmerhale.com; george.shuster@wilmerhale.com

If to the Buyer, to it at:

RP Management, LLC

110 E. 59th Street, Suite 3300
New York, New York 0022
Attention: George Lloyd
Email: [**]

With a copy to:

Goodwin Procter LLP

100 Northern Avenue
Boston, Massachusetts 02210
Attention: Arthur R. McGivern & Robert M. Crawford
Email: amcgivern@goodwinlaw.com; rcrawford@goodwinlaw.com

All notices and communications under this Agreement shall be deemed to have been duly given (i) when delivered by hand, if personally delivered, (ii) when received by a recipient, if sent by e mail with delivery confirmation thereof, (iii) when sent, if sent by facsimile, with an acknowledgement of sending being produced by the sending facsimile machine or (iv) one (1) Business Day following sending within the United States by overnight delivery via commercial one- (1-)day overnight courier service.

Section 8.2 Expenses. Except as otherwise provided herein, all fees, costs and expenses (including any legal, accounting and banking fees) incurred in connection with the preparation, negotiation, execution and delivery of this Agreement and to consummate the transactions contemplated hereby shall be paid by the party hereto incurring such fees, costs and expenses.

Section 8.3 Assignment.

(a) The Seller shall not sell, assign or otherwise transfer all or any portion of its interest in the Licensed PTC IP, any Product, the License Agreement, the Sponsored

Research Agreement or this Agreement to any Third Party, the Licensee or the Foundation by operation of law, merger, change of control, or otherwise, unless in connection therewith (a) such Person acquires all of the Seller's interest in all of the Licensed PTC IP, all Products, the License Agreement, the Sponsored Research Agreement and this Agreement and (b) prior to closing any such transaction, the Seller causes such Person to deliver to the Buyer a writing in which (i) if such Person is not the Licensee, such Person assumes all of the obligations of the Seller to the Buyer under this Agreement, or (ii) if such Person is the Licensee, the Licensee assumes all of the obligations of the Seller to the Buyer hereunder and agrees to pay the Assigned Royalty Payments directly to the Buyer notwithstanding any subsequent termination of the License Agreement by the Licensee.

(b) The Buyer may assign this Agreement in whole or in part; provided that in connection with any such assignment of this Agreement in whole or substantially in whole to a Third Party, or if a Third Party acquires all of the assets or securities of Royalty Pharma plc, on a consolidated basis, in a merger, tender offer, asset acquisition or similar transaction, the Buyer shall destroy or return to the Seller the Data Room and shall not transfer any rights to the Data Room in connection with such assignment or transaction.

(c) This Agreement shall be binding upon, inure to the benefit of and be enforceable by, the parties hereto and their respective permitted successors and assigns. Any purported assignment in violation of this Section 8.3 shall be null and void.

Section 8.4 Amendment and Waiver.

(a) This Agreement may be amended, modified or supplemented only in a writing signed by each of the parties hereto. Any provision of this Agreement may be waived only in a writing signed by the parties hereto granting such waiver.

(b) No failure or delay on the part of any party hereto in exercising any right, power or remedy hereunder shall operate as a waiver thereof, nor shall any single or partial exercise of any such right, power or remedy preclude any other or further exercise thereof or the exercise of any other right, power or remedy. No course of dealing between the parties hereto shall be effective to amend, modify, supplement or waive any provision of this Agreement.

Section 8.5 Independent Nature of Relationship. The relationship between the Seller and the Buyer is solely that of seller and buyer, and neither the Seller nor the Buyer has any fiduciary or other special relationship with the other party hereto or any of its Affiliates. Nothing in this Agreement shall be deemed in any way or for any purpose as constituting or creating any partnership or joint venture between the Seller and the Buyer or any other party for U.S. federal income tax or any other purpose. Each of the Seller and the Buyer agree that they shall not take any inconsistent position with respect to such treatment in any filing with any Governmental Entity.

Section 8.6 Entire Agreement. This Agreement, the Exhibits annexed hereto and the Disclosure Schedule constitute the entire understanding between the parties hereto with

respect to the subject matter hereof and supersede all other understandings and negotiations with respect thereto.

Section 8.7 No Third Party Beneficiaries. This Agreement is for the sole benefit of the Seller and the Buyer and their permitted successors and assigns and nothing herein expressed or implied shall give or be construed to give to any Person, other than the parties hereto and such successors and assigns, any legal or equitable rights hereunder.

Section 8.8 Governing Law. This Agreement shall be governed by, and construed in accordance with, the laws of the State of New York without giving effect to any choice or conflict of law provision or rule that would cause the application of the laws of any other jurisdiction.

Section 8.9 JURISDICTION; VENUE.

(a) EACH OF THE PARTIES HERETO HEREBY IRREVOCABLY AND UNCONDITIONALLY SUBMITS, FOR ITSELF AND ITS RESPECTIVE PROPERTY AND ASSETS, TO THE EXCLUSIVE JURISDICTION OF ANY NEW YORK STATE COURT OR FEDERAL COURT OF THE UNITED STATES OF AMERICA SITTING IN NEW YORK COUNTY, NEW YORK, AND ANY APPELLATE COURT THEREOF, IN ANY ACTION OR PROCEEDING ARISING OUT OF OR RELATING TO THIS AGREEMENT, OR FOR RECOGNITION OR ENFORCEMENT OF ANY JUDGMENT IN RESPECT THEREOF, AND THE BUYER AND THE SELLER HEREBY IRREVOCABLY AND UNCONDITIONALLY AGREE THAT ALL CLAIMS IN RESPECT OF ANY SUCH ACTION OR PROCEEDING MAY BE HEARD AND DETERMINED IN ANY SUCH NEW YORK STATE COURT OR, TO THE FULLEST EXTENT PERMITTED BY APPLICABLE LAW, IN SUCH FEDERAL COURT. THE BUYER AND THE SELLER HEREBY AGREE THAT A FINAL JUDGMENT IN ANY SUCH ACTION OR PROCEEDING SHALL BE CONCLUSIVE AND MAY BE ENFORCED IN OTHER JURISDICTIONS BY SUIT ON THE JUDGMENT OR IN ANY OTHER MANNER PROVIDED BY APPLICABLE LAW. EACH OF THE BUYER AND THE SELLER HEREBY SUBMITS TO THE EXCLUSIVE PERSONAL JURISDICTION AND VENUE OF SUCH NEW YORK STATE AND FEDERAL COURTS. THE BUYER AND THE SELLER AGREE, TO THE FULLEST EXTENT PERMITTED BY APPLICABLE LAW, THAT PROCESS MAY BE SERVED ON THE BUYER OR THE SELLER IN THE SAME MANNER THAT NOTICES MAY BE GIVEN PURSUANT TO SECTION 8.1 HEREOF.

(b) EACH OF THE PARTIES HERETO HEREBY IRREVOCABLY AND UNCONDITIONALLY WAIVES, TO THE FULLEST EXTENT IT MAY LEGALLY AND EFFECTIVELY DO SO, ANY OBJECTION THAT IT MAY NOW OR HEREAFTER HAVE TO THE LAYING OF VENUE OF ANY ACTION OR PROCEEDING ARISING OUT OF OR RELATING TO THIS AGREEMENT IN ANY NEW YORK STATE OR FEDERAL COURT. EACH OF THE BUYER AND THE SELLER HEREBY IRREVOCABLY WAIVES, TO THE FULLEST EXTENT PERMITTED BY APPLICABLE LAW, THE DEFENSE OF AN INCONVENIENT FORUM TO THE MAINTENANCE OF SUCH ACTION OR PROCEEDING IN ANY SUCH COURT.

Section 8.10 Severability. If any term or provision of this Agreement shall for any reason be held to be invalid, illegal or unenforceable in any situation in any jurisdiction, then, to the extent that the economic and legal substance of the transactions contemplated hereby is not affected in a manner that is materially adverse to either party hereto, all other terms and provisions of this Agreement shall nevertheless remain in full force and effect and the enforceability and validity of the offending term or provision shall not be affected in any other situation or jurisdiction.

Section 8.11 Specific Performance. Each of the parties acknowledges and agrees that the other parties would be damaged irreparably in the event any of the provisions of this Agreement are not performed in accordance with their specific terms or otherwise are breached or violated. Accordingly, each of the parties agrees that, without posting bond or other undertaking, the other parties shall be entitled to an injunction or injunctions to prevent breaches or violations of the provisions of this Agreement and to enforce specifically this Agreement and the terms and provisions hereof in any action, suit or other proceeding instituted in any court of the United States or any state thereof having jurisdiction over the parties and the matter in addition to any other remedy to which it may be entitled, at law or in equity.

Section 8.12 Limitations on Liability; Prevailing Party.

(a) Each party shall be liable to the other for damages arising out of or relating to such party's performance or failure to perform under this Agreement; provided, however, that the liability of a party to the other party, whether based on an action or claim in contract, equity, negligence, tort or otherwise, for all events, acts or omissions shall not exceed in the aggregate an amount equal to the Assigned Royalty Cap less the amount of Assigned Royalty Payments received by the Buyer. No party hereto shall be liable for any consequential, punitive, special, indirect or incidental damages as a result of any breach or violation of any covenant or agreement of such party in or pursuant to this Agreement. For the avoidance of doubt, and notwithstanding anything to the contrary in this Agreement, the Buyer shall have no recourse against the Company as a result of any Credit Event, any Permitted Royalty Reduction or any breach of the License Agreement by Licensee.

(b) In connection with the foregoing, the parties hereto acknowledge and agree that (i) the Buyer's damages, if any, for any such action or claim will typically include Losses for Assigned Royalty Payments that the Buyer was entitled to receive in respect of its ownership of the Assigned Royalty Payments but did not receive timely or at all due to the Seller's breach, default or failure to perform under this Agreement and (ii) the Buyer shall be entitled to make claims for all such missing or delayed Assigned Royalty Payments as damages hereunder, and such missing or delayed Assigned Royalty Payments shall not be deemed consequential, punitive, special, indirect or incidental damages.

(c) If any dispute, litigation or other court action, arbitration or similar adjudicatory proceeding is commenced by any party to enforce its rights or to seek damages under this Agreement against the other party, all fees, costs and expenses, including, reasonable attorneys' fees and court and collection costs, incurred by the prevailing party in such dispute, litigation, action, arbitration or proceeding shall be reimbursed by the non-prevailing party;

provided, that if a party to such dispute litigation, action, arbitration or proceeding prevails in part, and loses in part, the court, arbitrator or other adjudicator presiding over such litigation, action, arbitration or proceeding shall award a reimbursement of the fees, costs and expenses incurred by such Party on an equitable basis.

Section 8.13 Trustee Capacity of Wilmington Trust, National Association. Notwithstanding anything contained herein to the contrary, it is expressly understood and agreed by the parties hereto that (i) this Agreement is executed and delivered by Wilmington Trust, National Association, not individually or personally but solely in its trustee capacity, in the exercise of the powers and authority conferred and vested in it under the trust deed of the Buyer, (ii) each of the representations, undertakings and agreements herein made on the part of the Buyer is made and intended not as a personal representation, undertaking and agreement by Wilmington Trust, National Association but is made and intended for the purpose of binding only the Buyer and (iii) under no circumstances shall Wilmington Trust, National Association be personally liable for the payment of any indebtedness or expenses of the Buyer or be liable for the breach or failure of any obligation, representation, warranty or covenant made or undertaken by the Buyer under this Agreement or any related documents.

Section 8.14 Counterparts. This Agreement may be executed in any number of counterparts and by the parties hereto in separate counterparts, each of which when so executed shall be deemed to be an original and all of which taken together shall constitute one and the same agreement. Copies of executed counterparts transmitted by telecopy, facsimile or other similar means of electronic transmission, including "PDF," shall be considered original executed counterparts, provided receipt of such counterparts is confirmed.

[*Signature Page Follows*]

IN WITNESS WHEREOF, the parties hereto have caused this Royalty Purchase Agreement to be executed and delivered by their respective representatives thereunto duly authorized as of the date first above written.

PTC THERAPEUTICS, INC.

By: /s/ Stuart Peltz

Name: Stuart Peltz

Title: CEO

RPI 2019 INTERMEDIATE FINANCE TRUST

By: Wilmington Trust, National Association, not in its individual capacity but solely in its capacity as owner trustee

By: /s/ Cynthia L. Major

Name: Cynthia L. Major

Title: Banking Officer

ROYALTY PHARMA PLC

(solely for the purposes of Section 5.15 hereof)

By: /s/ George W. Lloyd

Name: George W. Lloyd

Title: EVP, Investments & General Counsel

Exhibit E

License Agreement

Incorporated by reference to Exhibit 10.14 of the Company's Form S-1 Registration Statement filed with the Securities and Exchange Commission on May 16, 2013

Exhibit F

Sponsored Research Agreement

Incorporated by reference to Exhibit 10.15 of the Company's Form S-1 Registration Statement filed with the Securities and Exchange Commission on May 16, 2013

Exhibit F

EMPLOYMENT AGREEMENT

This EMPLOYMENT AGREEMENT (the "Agreement") is made as of October 28, 2019 (the "Effective Date"), by and between PTC Therapeutics, Inc., a Delaware corporation (the "Company") and Matthew Klein ("Executive"). In consideration of the mutual covenants contained in this Agreement, the Company and Executive agree as follows:

1. Employment. The Company agrees to employ Executive and Executive agrees to be employed by the Company on the terms and conditions set forth in this Agreement.

(a) Capacity. Executive shall serve the Company as Global Head of Gene and Mitochondrial Therapies, reporting to Marcio Souza, Chief Operating Officer, or such senior executive as the Company shall specify. Executive shall have the responsibilities, duties and authority commensurate with the position of Global Head of Gene and Mitochondrial Therapies. In addition to Executive's primary duties, Executive shall perform such other services for the Company that are consistent with his/her position as Global Head of Gene and Mitochondrial Therapies as may be reasonably assigned to Executive from time to time by the individual to whom s/he reports or the Board of Directors of the Company (the "Board") or their respective designees. The principal location at which Executive shall perform such services shall be the Company's corporate headquarters currently located at 100 Corporate Court, Middlesex Business Center, South Plainfield, New Jersey , subject to Section 2(c)(i) of this Agreement.

(b) Devotion of Duties; Representations. During the Term (as defined below) of Executive's employment with the Company, Executive shall devote his/her best efforts and full business time and energies to the business and affairs of the Company, and shall endeavor to perform the duties and services contemplated hereunder to the reasonable satisfaction of the individual to whom s/he reports and the Board. During the Term, Executive shall adhere to, and perform all of Executive's duties in accordance with, all applicable laws, rules and regulations and all policies and procedures of the Company, as may be in effect from time to time. During the Term of Executive's employment with the Company, Executive shall not, without the prior written approval of the Company (by action of the Board), undertake any other employment from any person or entity or serve as a director of any other company; provided, however, that (i) the Company will entertain requests as to such other employment or directorships in good faith and (ii) Executive will be eligible to participate in any policy relating to outside activities that is applicable to the senior executives of the Company and approved by the Board after the date hereof.

2. Term of Employment.

(a) Executive's employment hereunder shall commence on the Effective Date. Executive's employment hereunder shall be terminated upon the first to occur of the following:

a. Immediately upon Executive's death;

(ii) By the Company:

(A) By written notice to Executive effective the date of such notice, following the Disability of Executive. “Disability” means that Executive (i) is unable to engage in any substantial gainful activity by reason of any medically determinable physical or mental impairment which can be expected to result in death or can be expected to last for a continuous period of not less than 12 months, or (ii) is, by reason of any medically determinable physical or mental impairment which can be expected to last for a continuous period of not less than 12 months, receiving income replacement benefits for a period of not less than three months under an accident and health plan covering employees of the Company. Such incapacity shall be determined by a physician chosen by the Company and reasonably satisfactory to Executive (or Executive’s legal representative) upon examination requested by the Company (to which Executive hereby agrees to submit). Notwithstanding the foregoing, such Disability must result in Executive becoming “Disabled” within the meaning of Section 409A(a)(2)(C) of the Internal Revenue Code of 1986, as amended (the “Code”) and the guidance issued thereunder. (In this Agreement we refer to Section 409A of the Code and any guidance issued thereunder as “Section 409A”).

(B) By written notice to Executive, effective the date of such notice, for Cause (as defined below); or

(C) By written notice to Executive, effective ninety (90) days after the date of such notice and subject to Section 4 hereof, without Cause; or

(iii) By Executive:

(A) At any time by written notice to the Company, effective forty-five (45) days after the date of such notice**;** or

(B) By written notice to the Company for Good Reason (as defined below), effective on the date specified in such notice.

The term of Executive’s employment by the Company under this Agreement is referred to herein as the “Term.”

(b) Definition of “Cause”. For purposes of this Agreement, “Cause” shall, pursuant to the reasonable good faith determination by a majority of the Board (excluding Executive) as documented in writing, include: (i) the willful and continued failure by Executive to substantially perform Executive’s material duties or responsibilities under this Agreement (other than such a failure as a result of Disability); (ii) any action or omission by Executive involving willful misconduct or gross negligence with regard to the Company, which has a detrimental effect on the Company; (iii) Executive’s conviction of a felony, either in connection with the performance of Executive’s obligations to the Company or which otherwise shall adversely affect Executive’s ability to perform such obligations or shall materially adversely affect the business activities, reputation, goodwill or image of the Company; (iv) the material breach of a fiduciary duty to the Company; or (v) the material breach by Executive of any of the provisions of this Agreement,

provided that any breach of Executive's obligations with respect to Sections 5 or 6 of this Agreement, subject to the cure provision in the next sentence, shall be deemed "material." In respect of the events described in clauses (i) and (v) above, the Company shall give Executive notice of the failure of performance or breach, reasonable as to time, place and manner in the circumstances, and a 30-day opportunity to cure, provided that such failure of performance or breach is reasonably amenable to cure as determined by the Board in its sole discretion.

(c) Definition of "Good Reason". For purposes of this Agreement, a "Good Reason" shall mean any of the following, unless (i) the basis for such Good Reason is cured within a reasonable period of time (determined in the light of the cure appropriate to the basis of such Good Reason, but in no event less than thirty (30) nor more than ninety (90) days) after the Company receives written notice (which must be received from Executive within ninety (90) days of the initial existence of the condition giving rise to such Good Reason) specifying the basis for such Good Reason or (ii) Executive has consented to the condition that would otherwise be a basis for Good Reason:

(i) A change in the principal location at which Executive provides services to the Company to a location more than fifty (50) miles from such principal location and/or to a location in New York City (either of which change, the Company has reasonably determined as of the date hereof, would constitute a material change in the geographic location at which Executive provides services to the Company), provided that such a relocation shall not be deemed to occur under circumstances where Executive's responsibilities require him/her to work at a location other than the corporate headquarters for a reasonable period of time;

(ii) A material adverse change by the Company in Executive's duties, authority or responsibilities as Global Head of Gene and Mitochondrial Therapies which causes Executive's position with the Company to become of materially less responsibility or authority than Executive's position immediately following the Effective Date. For purposes of this definition of "Good Reason," a "material adverse change" following a Corporate Change shall not include any diminution in authority, duties or responsibilities that is solely attributable to the change in the Company's ownership structure but does not otherwise change Executive's authority, duties or responsibilities (except in a positive manner) otherwise with respect to the Company's business.

(iii) A material reduction in Executive's base compensation (including Base Salary) except if the reduction is in connection with a general reduction of not more than 20% in compensation of senior executives of the Company generally that occurs prior to the effective date of any Corporate Change;

(iv) A material breach of this Agreement by the Company which has not been cured within thirty (30) days after written notice thereof by Executive; or

(v) Failure to obtain the assumption (assignment) of this Agreement by any successor to the Company.

(d) Definition of "Corporate Change". For purposes of this Agreement, "Corporate Change" shall mean any circumstance in which (i) the Company is not the surviving entity in any merger, consolidation or other reorganization (or survives only as a subsidiary or affiliate of an entity other than a previously wholly-owned subsidiary of the Company); (ii) the Company sells, leases or exchanges or agrees to sell, lease or exchange all or substantially all of its assets to any other person or entity (other than a wholly-owned subsidiary of the Company); (iii) any person or entity, including a "group" as contemplated by Section 13(d)(3) of the Securities Exchange Act of 1934 (excluding, for this purpose, the Company or any Subsidiary, or any employee benefit plan of the Company or any Subsidiary, or any "group" in which all or substantially all of its members or its members' affiliates are individuals or entities who are or were beneficial owners of the Company's outstanding shares prior to the initial public offering, if any, of the Company's stock), acquires or gains ownership or control (including, without limitations, powers to vote) of more than 50% of the outstanding shares of the Company's voting stock (based upon voting power); or (iv) as a result of or in connection with a contested election of directors, the persons who were directors of the Company before such election shall cease to constitute a majority of the Board of Directors of the Company. Notwithstanding the foregoing, a "Corporate Change" shall not occur as a result of an initial public offering of the Company's common stock, or as a result of a merger, consolidation, reorganization or restructuring after which either (1) a majority of the Board of Directors of the controlling entity consists of persons who were directors of the Company prior to the merger, consolidation, reorganization or restructuring or (2) Executive forms part of an executive management team that consists of substantially the same group of individuals and Executive is performing in a similar role, with similar authority and responsibility (other than changes solely attributable to the change in ownership structure), to that which existed prior to the reorganization or restructuring. Notwithstanding the foregoing, for any payments or benefits hereunder that are subject to Section 409A, the Corporate Change must constitute a "change in control event" within the meaning of Treasury Regulation Section 1.409A-3(i)(5)(i).

3. Compensation.

(a) Base Salary. Executive's minimum base salary during the Term shall be at the rate of $400,000 per year (the "Base Salary"). Base Salary shall be payable in substantially equal installments in accordance with the Company's payroll practices as in effect from time to time, less any amounts required to be withheld under applicable law. The Base Salary will be subject to adjustment from time to time in the sole discretion of the Board; provided that, the Company covenants that it shall not reduce the Base Salary below $400,000 or the Base Salary then in effect immediately prior to the reduction unless (i) Executive consents to such reduction, or (ii) the reduction is in connection with a general reduction of not more than 20% in compensation of senior executives of the Company generally that occurs prior to the effective date of any Corporate Change.

(b) Bonus. In addition to the Base Salary, the Company may pay Executive an annual bonus (the "Bonus") as determined by the Board, solely in its discretion (it being understood that Executive's target annual bonus shall be at 40% of the Base Salary, but may be higher or lower in any year in the Board's discretion). The Board's decision to issue a Bonus to Executive in any

particular year shall have no effect on the absolute discretion of the Board to grant or not to grant a Bonus in subsequent years. Any Bonus for a particular year shall be paid or provided to Executive in a lump sum no later than March 15th of the calendar year following the calendar year in which the Bonus was earned. Executive will not be eligible for a 2019 Bonus.

(c) Equity Compensation. Executive will receive an inducement grant of 100,000 options to purchase shares of common stock of PTC, subject to formal approval by the Compensation Committee of the Board (or a majority of the Company's independent directors) (the "Inducement Grant"). Such award is granted pursuant to the inducement grant exception under NASDAQ rules and is intended to serve as a material inducement to Executive entering into employment with PTC. Executive shall be eligible to participate in PTC's annual equity and long term incentive plan(s) and may be eligible to receive discretionary awards under such plan(s), subject to the terms and conditions of such plan(s). Executive shall not be eligible to participate in PTC's annual equity program for the current calendar year. For the avoidance of doubt, Executive shall not be eligible to receive a discretionary award in 2020. Except as explicitly set forth below, Executive's rights with respect to equity (including stock options) shall be covered in PTC's equity and long term incentive plan(s) and separate stock option certificates or agreements for each grant.

(i) Accelerated Vesting.

(A) For the avoidance of doubt, in the event that Executive's employment hereunder is terminated by the Company without Cause or by Executive for Good Reason, neither the unvested portion of the Inducement Grant nor any unvested equity awards granted under the Company's equity and long- term incentive plan(s) shall be subject to any accelerated vesting except as otherwise provided for in the applicable award agreement or in Section 3(c)(i)(B) below.

(B) Except as otherwise provided in the applicable award, in the event that Executive's employment hereunder is terminated by the Company without Cause or by Executive for Good Reason within the period of three (3) months prior to (but only if negotiations relating to the particular Corporate Change that occurs are ongoing at the date of the notice of termination) or twelve (12) months after a Corporate Change that occurs during the Term (such fifteen-month period, the "Protected Period"), one hundred percent (100%) of the unvested portion of the Inducement Grant and all of Executive's outstanding unvested equity awards granted under the Company's equity and long-term incentive plan(s) shall vest immediately.

(d) Vacation. Executive is eligible for time off programs outlined in the Company's Time Off Policies. Executive shall accrue over the calendar year 160 hours of paid vacation. Executive may accrue up to 200 hours of vacation. Once Executive has reached the maximum accrual, no further vacation time will be accrued unless and until the Executive uses vacation time. Upon termination of employment, the value of Executive's current balance of accrued but unused vacation shall be paid out based on his/her Base Salary that was in effect immediately prior to his/her termination of employment.

(e) Fringe Benefits. Executive shall be entitled to participate in any employee benefit plans that the Company makes available to its senior executives (including, without limitation, group life, disability, medical, dental and other insurance, retirement, pension, profit-sharing and similar plans) (collectively, the "Fringe Benefits"), provided that the Fringe Benefits shall not include any stock option or similar plans relating to the grant of equity securities of the Company. These benefits may be modified or changed from time to time at the sole discretion of the Company. Where a particular benefit is subject to a formal plan (for example, medical or life insurance), eligibility to participate in and receive any particular benefit is governed solely by the applicable plan document, and eligibility to participate in such plan(s) may be dependent upon, among other things, a physical examination.

(f) Reimbursement of Expenses. Executive shall be entitled to reimbursement for all ordinary and reasonable out-of-pocket business expenses that are reasonably incurred by him/her in furtherance of the Company's business in accordance with reasonable policies adopted from time to time by the Company for senior executives. The Company agrees to reimburse Executive for reasonable out-of-pocket fees related to maintaining Executive's medical license.

(g) Relocation. Executive is eligible to be reimbursed for moving-related expenses incurred by Executive in relocating his residence from Menlo Park, California to such domicile that is within a reasonable commuting distance of PTC's offices in South Plainfield, New Jersey in an amount not to exceed $250,000, subject to PTC's applicable policies and procedures with respect to relocation and reimbursements; *provided*, however, that, Executive will be required to refund 100% such relocation expenses and reimbursements to the Company in the event that, prior to the first anniversary of Effective Date, Executive resigns his employment for any reason or PTC terminates Executive's employment for Cause. Executive will be required to refund 50% of such relocation expenses and reimbursements to the Company in the event that, after the first anniversary of the Start Date but prior to the second anniversary of the Start Date Executive resigns his employment for any reason or PTC terminates Executive's employment for Cause. Unless otherwise extended in writing by the Company, Executive's relocation must be completed by June 30, 2020 and Executive will not be reimbursed for any relocation expenses that are incurred after such date. Relocation-related expenses may be considered taxable income as specified by IRS tax regulations. Executive agrees relocation to New Jersey is a condition of employment and that a failure to complete his relocation to New Jersey by June 30, 2020 (unless otherwise extended by the Company in

writing) will constitute a breach of this Agreement and could cause the Executive's employment to be terminated for Cause as defined in section 2(b).

(h) Cost of Living Adjustment. Executive will be eligible to receive a cost of living adjustment of $8,200, per month, commencing on the Effective Date and ending on the earlier of the completion of Executive's relocation to New Jersey or March 31, 2020. The cost of living allowance will be paid in accordance with the company's regular payroll practices.

(i) Sign-On Bonus. Executive will be eligible to receive a one-time, lump sum bonus of $61,823, payable on March 15, 2020; *provided*, however, that Executive must be employed by PTC and must not be in breach of this Agreement (as determined by the Board, in its discretion) at the time such bonus is to be paid in order to receive such bonus.

(j) Taxes and Withholdings. The Company shall deduct and withhold from all compensation and benefits under this Agreement all social security and other federal, state and local taxes and charges which currently are or which hereafter may be required by law to be so deducted and withheld.

4. Severance Compensation.

(a) In the event of any termination of Executive's employment for any reason the Company shall pay Executive (or Executive's estate) such portions of Executive's Base Salary as have accrued prior to such termination and have not yet been paid, together with (i) amounts for accrued unused vacation days (as provided above), (ii) any amounts for expense reimbursement which have been properly incurred or the Company has become obligated to pay prior to termination and have not been paid as of the date of such termination and (iii) the amount of any Bonus previously granted to Executive by the Board but not yet paid, which amount shall not include any pro rata portion of any Bonus which would have been earned if such termination had not occurred (the "Accrued Obligations").

(b) In the event that Executive's employment hereunder is terminated (i) by Executive for a Good Reason or (ii) by the Company without Cause, the Company shall pay to Executive the Accrued Obligations. In addition, the Company shall pay to Executive the severance benefits set forth in Section (b)(i) below for twelve (12) months following Executive's termination of employment (the "Severance Period"), and pay to Executive the severance benefits set forth in Section (b)(ii). The receipt of any severance benefits provided in this Section shall be dependent upon Executive's execution (and, as applicable, nonrevocation) of a standard separation agreement and general release of claims, substantially in the form attached hereto as Exhibit A (the "Release"). The Company will also consider in good faith (but without any

binding commitment) requests from Executive that the Company include in the Release a release of Executive by the Company from matters specifically disclosed to the Company by Executive in writing in advance of execution of the Release and not involving any illegality, fraud, concealment, criminal acts or acts outside the scope of Executive's employment. The distribution of severance benefits in this Section 4 is subject to section (iii) of this Section 4(b).

(i) If Executive's employment is terminated (A) by Executive for a Good Reason or (B) by the Company without Cause, in either case before or after the Protected Period, the Company shall pay Executive his/her Base Salary, less any amounts required to be withheld under applicable law, for the Severance Period in substantially equal installments in accordance with the Company's payroll practices as in effect from time to time, commencing no later than sixty (60) days following the effective date of such termination. If Executive's employment is terminated (A) by Executive for a Good Reason or (B) by the Company without Cause, in either case during the Protected Period, the Company shall pay Executive his/her Base Salary for the Severance Period, which total amount shall be payable in a lump sum no later than sixty (60) days following Executive's termination of employment. In each case, payments shall commence or be paid provided that the Release has been executed and any applicable revocation period has expired as of the 60th day following Executive's termination.

(ii) The Company shall provide Executive with a lump sum payment representing the net value of the contributions to Executive's current group health premiums that PTC would have paid on Executive's behalf (had Executive continued to be an employee of PTC) for the Severance Period, less any amounts required to be withheld under applicable law. Such payment shall be made no later than sixty (60) days following the effective date of Executive's termination; provided that the Release has been executed and any applicable revocation period has expired as of the 60th day following Executive's termination. The foregoing shall not be construed to extend any period of continuation coverage (e.g., COBRA) required by Federal law.

(iii) Compliance with Section 409A. Subject to the provisions in this Section 4(b)(iii), any severance payments or benefits under this Agreement shall begin only upon the date of Executive's "separation from service" (determined as set forth below) which occurs on or after the date of termination of Executive's employment. The following rules shall apply with respect to the distribution of the severance payments and benefits, if any, to be provided to Executive under this Agreement:

(1) It is intended that each installment of the severance payments and benefits provided under this Agreement shall be treated as a separate "payment" for purposes of Section 409A. Neither the Company nor Executive

shall have the right to accelerate or defer the delivery of any such payments or benefits except to the extent specifically permitted or required by Section 409A.

(2) If, as of the date of Executive's "separation from service" from the Company, Executive is not a "specified employee" (within the meaning of Section 409A), then each installment of the severance payments and benefits shall be made on the dates and terms set forth in this Agreement.

(3) If, as of the date of Executive's "separation from service" from the Company, Executive is a "specified employee" (within the meaning of Section 409A), then:

(A) Each installment of the severance payments and benefits due under this Agreement that, in accordance with the dates and terms set forth herein, will in all circumstances, regardless of when the separation from service occurs, be paid within the short-term deferral period (as defined under Section 409A) shall be treated as a short-term deferral within the meaning of Treasury Regulation Section 1.409A-1(b)(4) to the maximum extent permissible under Section 409A and such payments and benefits shall be paid or provided on the dates and terms set forth in this Agreement; and

(B) Each installment of the severance payments and benefits due this Agreement that is not described in Section 4(b)(iii)(3)(A) above and that would, absent this subsection, be paid within the six-month period following Executive's "separation from service" from the Company shall not be paid until the date that is six months and one day after such separation from service (or, if earlier, Executive's death), with any such installments that are required to be delayed being accumulated during the six-month period and paid in a lump sum on the date that is six months and one day following Executive's separation from service and any subsequent installments, if any, being paid in accordance with the dates and terms set forth herein; provided, however, that the preceding provisions of this sentence shall not apply to any installment of severance payments and benefits if and to the maximum extent that such installment is deemed to be paid under a separation pay plan that does not provide for a deferral of compensation by reason of the application of Treasury Regulation 1.409A-1(b)(9)(iii) (relating to separation pay upon an involuntary separation from service). Any installments that qualify for the exception under Treasury Regulation Section 1.409A-1(b)(9)(iii) must be paid no later than the last day of Executive's second taxable year following the taxable year in which the separation from service occurs.

(4) The determination of whether and when Executive's separation from service from the Company has occurred shall be made in a manner consistent with, and based on the presumptions set forth in, Treasury Regulation Section 1.409A-1(h). Solely for purposes of this Section 4(b)(iii), "Company" shall include all persons with whom the Company would be considered a single employer under Section 414(b) and 414(c) of the Code.

(5) All reimbursements and in-kind benefits provided under this Agreement shall be made or provided in accordance with the requirements of Sections 409A to the extent that such reimbursements or in-kind benefits are subject to Section 409A, including, where applicable, the requirements that (i) any reimbursement is for expenses incurred during Executive's lifetime (or during a shorter period of time specified in this Agreement), (ii) the amount of expenses eligible for reimbursement during a calendar year may not affect the expenses eligible for reimbursement in any other calendar year, (iii) the reimbursement of an eligible expense will be made on or before the last day of the calendar year following the year in which the expense is incurred and (iv) the right to reimbursement is not subject to set off or liquidation or exchange for any other benefit.

(6) Notwithstanding anything herein to the contrary, the Company shall have no liability to Executive or to any other person if the payments and benefits provided hereunder that are intended to be exempt from or compliant with Section 409A are not so exempt or compliant.

(c) In the event that Executive's employment hereunder is terminated (i) by Executive for other than a Good Reason, or (ii) by the Company for Cause, or (iii) as a result of Executive's death or Disability, then the Company will pay to Executive the Accrued Obligations. The Company shall have no obligation to pay Executive (or Executive's estate) any other compensation following such termination except as provided in Section 4(a).

(d) Modified Section 280G Cutback.

(i) Notwithstanding any other provision of this Agreement, except as set forth in Section 4(d)(ii), in the event that the Company undergoes a "Change in Ownership or Control" (as defined below), the Company shall not be obligated to provide to Executive a portion of any "Contingent Compensation Payments" (as defined below) that Executive would otherwise be entitled to receive to the extent necessary to eliminate any "excess parachute payments" (as defined in Section 280G(b)(1) of the Code) for Executive. For purposes of this Section 4(d), the Contingent Compensation Payments so eliminated shall be referred to as the "Eliminated Payments" and the aggregate amount (determined in accordance with Treasury Regulation Section 1.280G-1, Q/A-30 or any

successor provision) of the Contingent Compensation Payments so eliminated shall be referred to as the "Eliminated Amount."

(ii) Notwithstanding the provisions of Section 4(d)(i), no such reduction in Contingent Compensation Payments shall be made if (1) the Eliminated Amount (computed without regard to this sentence) exceeds (2) 100% of the aggregate present value (determined in accordance with Treasury Regulation Section 1.280G-1, Q/A-31 and Q/A-32 or any successor provisions) of the amount of any additional taxes that would be incurred by Executive if the Eliminated Payments (determined without regard to this sentence) were paid to him/her (including, state and federal income taxes on the Eliminated Payments, the excise tax imposed by Section 4999 of the Code payable with respect to all of the Contingent Compensation Payments in excess of Executive's "base amount" (as defined in Section 280G(b)(3) of the Code), and any withholding taxes). The override of such reduction in Contingent Compensation Payments pursuant to this Section 4(d)(ii) shall be referred to as a "Section 4(d)(ii) Override." For purpose of this paragraph, if any federal or state income taxes would be attributable to the receipt of any Eliminated Payment, the amount of such taxes shall be computed by multiplying the amount of the Eliminated Payment by the maximum combined federal and state income tax rate provided by law.

(iii) For purposes of this Section 4(d) the following terms shall have the following respective meanings:

(1) "Change in Ownership or Control" shall mean a change in the ownership or effective control of the Company or in the ownership of a substantial portion of the assets of the Company determined in accordance with Section 280G(b)(2) of the Code.

(2) "Contingent Compensation Payment" shall mean any payment (or benefit) in the nature of compensation that is made or made available (under this Agreement or otherwise) to a "disqualified individual" (as defined in Section 280G(c) of the Code) and that is contingent (within the meaning of Section 280G(b)(2)(A)(i) of the Code) on a Change in Ownership or Control of the Company.

(iv) Any payments or other benefits otherwise due to Executive following a Change in Ownership or Control that could reasonably be characterized (as determined by the Company) as Contingent Compensation Payments (the "Potential Payments") shall not be made until the dates provided for in this Section 4(d)(iv). Within 30 days after each date on which Executive first becomes entitled to receive (whether or not then due) a Contingent Compensation Payment relating to such Change in Ownership or Control, the Company shall determine and notify Executive (with reasonable detail

regarding the basis for its determinations) (1) which Potential Payments constitute Contingent Compensation Payments, (2) the Eliminated Amount and (3) whether the Section 4(d)(ii) Override is applicable. Within 30 days after delivery of such notice to Executive, Executive shall deliver a response to the Company (the "Executive Response") stating either (A) that s/he agrees with the Company's determination pursuant to the preceding sentence or (B) that s/he disagrees with such determination, in which case s/he shall set forth (x) which Potential Payments should be characterized as Contingent Compensation Payments, (y) the Eliminated Amount, and (z) whether the Section 4(d)(ii) Override is applicable. In the event that Executive fails to deliver an Executive Response on or before the required date, the Company's initial determination shall be final. If Executive states in the Executive Response that s/he agrees with the Company's determination, the Company shall make the Potential Payments to Executive within three business days following delivery to the Company of the Executive Response (except for any Potential Payments which are not due to be made until after such date, which Potential Payments shall be made on the date on which they are due). If Executive states in the Executive Response that s/he disagrees with the Company's determination, then, for a period of 60 days following delivery of the Executive Response, Executive and the Company shall use good faith efforts to resolve such dispute. If such dispute is not resolved within such 60-day period, such dispute shall be settled exclusively by arbitration in South Plainfield, New Jersey, in accordance with the rules of the American Arbitration Association then in effect. Judgment may be entered on the arbitrator's award in any court having jurisdiction. The Company shall, within three business days following delivery to the Company of the Executive Response, make to Executive those Potential Payments as to which there is no dispute between the Company and Executive regarding whether they should be made (except for any such Potential Payments which are not due to be made until after such date, which Potential Payments shall be made on the date on which they are due). The balance of the Potential Payments shall be made within three business days following the resolution of such dispute.

(v) The Contingent Compensation Payments to be treated as Eliminated Payments shall be determined by the Company by determining the "Contingent Compensation Payment Ratio" (as defined below) for each Contingent Compensation Payment and then reducing the Contingent Compensation Payments in order beginning with the Contingent Compensation Payment with the highest Contingent Compensation Payment Ratio. For Contingent Compensation Payments with the same Contingent Compensation Payment Ratio, such Contingent Compensation Payment shall be reduced based on the time of payment of such Contingent Compensation Payments with amounts having later payment dates being reduced first. For Contingent Compensation Payments with the same Contingent Compensation Payment Ratio and the same time of payment, such Contingent Compensation Payments shall be reduced on a

pro rata basis (but not below zero) prior to reducing Contingent Compensation Payment with a lower Contingent Compensation Payment Ratio. The term "Contingent Compensation Payment Ratio" shall mean a fraction the numerator of which is the value of the applicable Contingent Compensation Payment that must be taken into account by Executive for purposes of Section 4999(a) of the Code, and the denominator of which is the actual amount to be received by Executive in respect of the applicable Contingent Compensation Payment. For example, in the case of an equity grant that is treated as contingent on the Change in Ownership or Control because the time at which the payment is made or the payment vests is accelerated, the denominator shall be determined by reference to the fair market value of the equity at the acceleration date, and not in accordance with the methodology for determining the value of accelerated payments set forth in Treasury Regulation Section 1.280G-1Q/A-24(b) or (c)).

(vi) The provisions of this Section 4(d) are intended to apply to any and all payments or benefits available to Executive under this Agreement or any other agreement or plan of the Company under which Executive receives Contingent Compensation Payments.

(vii) Notwithstanding Sections 4(d)(i)-(vi) hereof, until the closing of the first underwritten public offering of common stock of the Company, in the event that it shall be determined that any payment or benefit (including any accelerated vesting of options or other equity awards) made or provided, or to be made or provided, by the Company (or any successor thereto or affiliate thereof) to or for the benefit of Executive, whether pursuant to the terms of this Agreement, any other agreement, plan, program or arrangement of or with the Company (or any successor thereto or affiliate thereof) or otherwise, may be subject to the excise tax imposed by Section 4999 of the Code or any comparable tax imposed by any replacement or successor provision of United States tax law, then upon the request of Executive, the Company shall use reasonable efforts to procure a shareholder vote in satisfaction of the shareholder approval requirements described in Treas. Reg. Section 1.280G-1, Q&A-7.

5. Executive Covenants.

(a) Confidential Information. Executive recognizes and acknowledges the competitive and proprietary aspects of the business of the Company, and that as a result of Executive's employment, Executive recognizes and acknowledges that s/he will have access to, and will be involved in the development of, Confidential Information (as defined below) of the Company. As used herein, "Confidential Information" shall mean and include trade secrets, knowledge and other confidential information of the Company, which Executive has acquired, no matter from whom or on what matter such knowledge or information may have been acquired, heretofore or hereafter, concerning the content and details of the business of the

Company, and which is not known to the general public, including but not limited to: (a) new products, product betterments and other inventions, formulas, processes, methods, materials, material combinations, manner of preparations, technical production procedures and information, alarm and security codes and procedures, sources of technology, and sources of supply of raw and finished materials and other products; (b) financial and accounting records; (c) the identity of employees, consultants, independent contractors, customers, business development partners, licensees, suppliers, creditors or other parties with which the Company has business dealings, the nature of the relationship with such persons, or any other information relating to such persons or the Company's dealings with such persons; and (d) computer software used by the Company or provided to the customers of the Company unless publicly available.

(i) For as long as Executive is employed and at all times thereafter, Executive shall not, directly or indirectly, communicate, disclose or divulge to any person or entity, or use for Executive's own benefit or the benefit of any person (other than the Company), any Confidential Information, except as permitted in subparagraph (iii) below. Upon termination of Executive's employment, or at any other time at the request of the Company, Executive agrees to deliver promptly to the Company all Confidential Information, including, but not limited to, customer and supplier lists, files and records, in Executive's possession or under Executive's control. Executive further agrees that s/he will not make or retain any copies of any of the foregoing and will so represent to the Company upon termination of Executive's employment.

(ii) Executive shall disclose immediately to the Company any trade secrets or other Confidential Information conceived or developed by Executive at any time during Executive's employment. Executive hereby assigns and agrees to assign to the Company Executive's entire right, title and interest in and to all Confidential Information. Such assignment shall include, without limitation, the rights to obtain patent or copyright protection, thereon in the United States and foreign countries. Executive agrees to provide all reasonable assistance to enable the Company to prepare and prosecute any application before any governmental agency for patent or copyright protection or any similar application with respect to any Confidential Information. Executive further agrees to execute all documents and assignments and to make all oaths necessary to vest ownership of such intellectual property rights in the Company, as the Company may request. These obligations shall apply whether or not the subject thereof was conceived or developed at the suggestion of the Company, and whether or not developed during regular hours of work or while on the premises of the Company. Executive shall at all times, both during and after termination of this Agreement by either Executive or the Company, maintain in confidence and shall not, without prior written consent of the Company, use, except in the course of performance of Executive's duties for the Company or as required by legal process (provided that Executive will promptly notify the Company of such legal process except with respect to any confidential government

investigation), disclose or give to others any Confidential Information. In the event Executive is questioned by anyone not employed by the Company or by an employee of or a consultant to the Company not authorized to receive such information, in regard to any such information or any other secret or confidential work of the Company, or concerning any fact or circumstance relating thereto, Executive will promptly notify the Company.

(iii) Nothing in this Agreement, including but not limited to Section 5 (Executive Covenants), including sub-sections 5(a) (Confidential Information) and 5(b) (Non-Competition and Non-Solicitation) and Section 6 (Ownership of Ideas, Copyrights and Patents (Inventions), shall prohibit or restrict Executive, or be construed to prohibit or restrict Executive, from filing a charge or complaint with, reporting possible violations of any law or regulation, making disclosures to (including providing documents or other information), and/or participating in any investigation or proceeding conducted by any self-regulatory organization or governmental agency, authority or legislative body, including, but not limited to, the Securities and Exchange Commission and/or Equal Employment Opportunity Commission or as otherwise required by law.

(iv) Executive is hereby notified that under the Defend Trade Secrets Act: (a) no individual will be held criminally or civilly liable under Federal or State trade secret law for disclosure of a trade secret (as defined in the Economic Espionage Act) that is: (1) made in confidence to a Federal, State, or local government official, either directly or indirectly, or to an attorney, and made solely for the purpose of reporting or investigating a suspected violation of law; or, (2) made in a complaint or other document filed in a lawsuit or other proceeding, if such filing is made under seal so that it is not made public; and (b) an individual who pursues a lawsuit for retaliation by an employer for reporting a suspected violation of the law may disclose the trade secret to the attorney of the individual and use the trade secret information in the court proceeding, if the individual files any document containing the trade secret under seal, and does not disclose the trade secret, except as permitted by court order.

(b) Non-Competition and Non-Solicitation. Executive recognizes that the Company is engaged in a competitive business and that the Company has a legitimate interest in protecting its trade secrets, confidential business information, and customer, business development partner, licensee, supplier, and credit and/or financial relationships. Accordingly, in exchange for valuable consideration, including without limitation Executive’s access to confidential business information and continued at-will employment, Executive agrees that, during the Term hereof and for a period of eighteen (18) months thereafter, Executive shall not:

(i) directly or indirectly, whether for himself or for any other person or entity, and whether as a proprietor, principal, shareholder, partner, agent, employee, consultant, independent contractor, or in any other capacity whatsoever, undertake or have any interest in (other than the passive ownership of publicly registered securities

representing an ownership interest of less than 1%), engage in or assume any role directly competitive with the Company's Field of Interest (or any portion thereof) or any other business in which the Company is engaged and for which the employee has rendered services while employed by the Company, or enter into any agreement to do any of the foregoing; or

(ii) initiate contact with (including without limitation phone calls, press releases and the sending or delivering of announcements), or in any manner solicit, directly or indirectly, any customers, business development partners, licensors, licensees, or creditors (including institutional lenders, bonding companies and trade creditors) of the Company in an attempt to induce or motivate them either to discontinue or modify their then prevailing or future relationship with the Company or to transfer any of their business with the Company to any person or entity other than the Company; or

(iii) initiate contact with, or in any manner solicit, directly or indirectly, any supplier of goods, services or materials to the Company in an attempt to induce or motivate them either to discontinue or modify their then prevailing or future relationship with the Company or to supply the same or similar inventory, goods, services or materials (except generally available inventory, goods, services or materials) to any person or entity other than the Company; or

(iv) directly or indirectly recruit, solicit or otherwise induce or influence any employee or independent contractor of the Company to discontinue or modify his or her employment or engagement with the Company, or employ or contract with any such employee or contractor for the provision of services.

(c) Definition of "Field of Interest". The term Company's "Field of Interest" shall mean the research, development and commercialization of products and strategies relating to: (i) therapies for genetic disorders or specific diseases within each of AADC deficiency, Friedreich Ataxia, Angelman, Mitochondrial diseases, Duchenne muscular dystrophy, and (ii) any other diseases or products, in each case, that Executive directly managed or supported during his/her employment with the Company.

(d) Definition of "Customer". The term "customer" or "customers" shall include any person or entity (a) that is a current customer of the Company, (b) that was a customer of the Company at any time during the preceding twenty-four (24) months or (c) to which the Company made a written presentation for the solicitation of business at any time during the preceding twenty-four (24) months.

(e) Reasonableness of Restrictions. Executive further recognizes and acknowledges that (i) the types of employment which are prohibited by this Section 5 are narrow and reasonable in relation to the skills which represent Executive's principal salable asset both to

the Company and to Executive's other prospective employers, and (ii) the broad geographical scope of the provisions of this Section 5 is reasonable, legitimate and fair to Executive in light of the global nature of the Company's business, particularly pharmaceutical research and development, and in light of the limited restrictions on the type of employment prohibited herein compared to the types of employment for which Executive is qualified to earn Executive's livelihood.

(f) Remedies. Executive acknowledges that a breach of this Section 5 will cause great and irreparable injury and damage, which cannot be reasonably or adequately compensated by money damages. Accordingly, Executive acknowledges that the remedies of injunction and specific performance shall be available in the event of such a breach, in addition to money damages, costs and attorneys' fees, and other legal or equitable remedies, and that the Company shall be entitled as a matter of course to an injunction pending trial, without the posting of bond or other security. Any period of restriction set forth in this Section 5 shall be extended for a period of time equal to the duration of any breach or violation hereof.

(g) Notification. Any person employing Executive or evidencing any intention to employ Executive may be notified as to the existence and provisions of this Agreement.

(h) Modification of Covenants; Enforceability. In the event that any provision of this Section 5 is held to be in any respect an unreasonable restriction, then the court so holding may modify the terms thereof, including the period of time during which it operates or the geographic area to which it applies, or effect any other change to the extent necessary to render this section enforceable, it being acknowledged by the parties that the representations and covenants set forth herein are of the essence of this Agreement.

(i) Subsidiaries. For purposes of Sections 5 and 6 of this Agreement, "Company" shall include all direct and indirect subsidiaries of the Company. An entity shall be deemed to be a subsidiary of the Company if the Company directly or indirectly owns or controls 50% or more of the equity interest in such entity.

6. Ownership of Ideas, Copyrights and Patents.

(a) Property of the Company. Executive agrees that all ideas, discoveries, creations, manuscripts and properties, innovations, improvements, know-how, inventions, designs, developments, apparatus, techniques, methods, biological processes, cell lines, laboratory notebooks and formulae, whether patentable, copyrightable or not, which Executive may conceive, reduce to practice or develop, alone or in conjunction with another, or others, whether during or out of regular business hours, and whether at the request or upon the suggestion of the Company, or otherwise, in the course of performing services for the Company in any capacity, whether heretofore or hereafter, (collectively, "the Inventions") are and shall be the sole and exclusive property of the Company, and that Executive shall not publish any of the

Inventions without the prior written consent of the Company. Executive hereby assigns to the Company all of Executive's right, title and interest in and to all of the foregoing. Executive further represents and agrees that to the best of Executive's knowledge and belief none of the Inventions will violate or infringe upon any right, patent, copyright, trademark or right of privacy, or constitute libel or slander against or violate any other rights of any person, firm or corporation and that Executive will use his/her best efforts to prevent any such violation.

(b) Cooperation. At any time during or after the Term, Executive agrees that s/he will fully cooperate with the Company, its attorneys and agents in the preparation and filing of all papers and other documents as may be required to perfect the Company's rights in and to any of such Inventions, including, but not limited to, executing any lawful document (including, but not limited to, applications, assignments, oaths, declarations and affidavits) and joining in any proceeding to obtain letters patent, copyrights, trademarks or other legal rights of the United States and of any and all other countries on such Inventions, provided that any patent or other legal right so issued to Executive, personally, shall be assigned by Executive to the Company without charge by Executive. Executive further designates the Company as his/her agent for, and grants to the Company a power of attorney with full power of substitution, which power of attorney shall be deemed coupled with an interest, for the purpose of effecting the foregoing assignments from Executive to the Company. Company will bear the reasonable expenses which it causes to be incurred in Executive's assisting and cooperating hereunder. Executive waives all claims to moral rights in any Inventions.

7. Disclosure to Future Employers. The Company may provide in its discretion, a copy of this Agreement (in whole or in part, including the covenants contained in Sections 5 and 6 of this Agreement) to: (a) any business or enterprise which Executive may directly, or indirectly, own, manage, operate, finance, join, control or in which Executive participates in the ownership, management, operation, financing, or control, or with which Executive may be connected as an officer, director, employee, partner, principal, agent, representative, consultant or otherwise, or (b) any third party who may be affected by the restrictive covenants in this Agreement.

8. Records. Upon termination of Executive's relationship with the Company, and at any time requested by the Company, Executive shall deliver to the Company any property of the Company which may be in Executive's possession including products, materials, memoranda, notes, records, reports, or other documents or photocopies of the same.

9. Insurance. The Company, in its sole discretion, may apply for and procure in its own name (whether or not for its own benefit) policies of insurance insuring Executive's life. Executive agrees to submit to reasonable medical or other examinations and to execute and deliver any applications or other instruments in writing that are reasonably necessary to effectuate such insurance. No adverse employment actions may be based upon the results of any such exam or the failure by the Company to obtain such insurance.

10. No Conflicting Agreements. Executive hereby represents and warrants that Executive has no commitments or obligations inconsistent with this Agreement.

11. "Market Stand-Off" Agreement. Executive agrees, if requested by the Company and an underwriter of common stock (or other securities) of the Company, not to sell or otherwise transfer or dispose of any common stock (or other securities) of the Company held by Executive during a period not to exceed one hundred and eighty (180) days following the effective date of the first underwritten public offering of common stock of the Company, offered on a firm commitment basis pursuant to a registration statement filed with the Securities and Exchange Commission (or any successor agency of the Federal government administrating the Securities Act of 1933, as amend, and the Securities Exchange Act of 1934, as amended) under the Securities Act of 1933, as amended, on Form S-1 or its then equivalent, and to enter into an agreement to such effect. The Company may impose stop-transfer instructions with respect to the shares (or securities) subject to the foregoing restriction until the end of said period.

12. General.

(a) Notices. All notices, requests, consents and other communications hereunder shall be in writing, shall be addressed to the receiving party's address as follows:

If to the Company: PTC Therapeutics Inc.
100 Corporate Court
South Plainfield, NJ 07080
USA
Attention: Legal Department
Telephone: (908) 222-7000

With an email copy to: legal@ptcbio.com

If to Executive: Matthew Klein
184 Sand Hill Circle
Menlo Park, CA 94025

or to such other address as a party may designate by notice hereunder, and shall be either (i) delivered by hand, (ii) sent by overnight courier, or (iii) sent by registered or certified mail, return receipt requested, postage prepaid. All notices, requests, consents and other communications hereunder shall be deemed to have been given either (i) if by hand, at the time of the delivery thereof to the receiving party at the address of such party set forth above, (ii) if sent by overnight courier, on the next business day following the day such notice is delivered to the courier service, or (iii) if sent by registered or certified mail, on the fifth (5th) business day following the day such mailing is made.

(b) Entire Agreement. This Agreement embodies the entire agreement and understanding between the parties hereto with respect to the subject matter hereof and supersedes all prior oral or written agreements and understandings relating to the subject matter hereof, except with respect to the equity and fringe benefit arrangements referred to in Subsections 3(c) and (e) above. No statement, representation, warranty, covenant or agreement of any kind not expressly set forth in this Agreement shall affect, or be used to interpret, change or restrict, the express terms and provisions of this Agreement.

(c) Modifications and Amendments. The terms and provisions of this Agreement may be modified or amended only by written agreement executed by the parties hereto.

(d) Waivers and Consents. The terms and provisions of this Agreement may be waived, or consent for the departure therefrom granted, only by written document executed by the party entitled to the benefits of such terms or provisions. No such waiver or consent shall be deemed to be or shall constitute a waiver or consent with respect to any other terms or provisions of this Agreement, whether or not similar. Each such waiver or consent shall be effective only in the specific instance and for the purpose for which it was given, and shall not constitute a continuing waiver or consent.

(e) Assignment. The Company shall assign its rights and obligations hereunder to any person or entity that succeeds to all or substantially all of the Company's business or that aspect of the Company's business in which Executive is principally involved. Executive may not assign Executive's rights and obligations under this Agreement without the prior written consent of the Company.

(f) Benefit. All statements, representations, warranties, covenants and agreements in this Agreement shall be binding on the parties hereto and shall inure to the benefit of the respective successors and permitted assigns of each party hereto. Nothing in this Agreement shall be construed to create any rights or obligations except among the parties hereto, and no person or entity shall be regarded as a third□party beneficiary of this Agreement.

(g) Governing Law. This Agreement and the rights and obligations of the parties hereunder shall be construed in accordance with and governed by the laws of The State of New Jersey, without giving effect to the conflict of law principles thereof.

(h) Arbitration. Executive and the Company hereby agree that the sole remedy for any and all disputes arising out of or based on this Agreement or Executive's employment with the Company ("Arbitrable Claims"), shall be binding arbitration, which shall be conducted in New Jersey, before a single arbitrator, in accordance with the then applicable rules of the Judicial Arbitration and Mediation Service ("JAMS") or by a non- JAMS process to which the parties may otherwise agree. By agreeing to arbitrate, the parties are waiving their respective rights to a jury trial with regard to any of the above referenced claims. Executive understands and agrees

that notwithstanding the foregoing, the Company may pursue legal or equitable relief against Executive in the event of a breach of a restrictive covenant as per Section 5(f) above.

(i) Severability. The parties intend this Agreement to be enforced as written. However, (i) if any portion or provision of this Agreement shall to any extent be declared illegal or unenforceable by a duly authorized court having jurisdiction, then the remainder of this Agreement, or the application of such portion or provision in circumstances other than those as to which it is so declared illegal or unenforceable, shall not be affected thereby, and each portion and provision of this Agreement shall be valid and enforceable to the fullest extent permitted by law; and (ii) if any provision, or part thereof, is held to be unenforceable because of the duration of such provision or the geographic area covered thereby or otherwise, the Company and Executive agrees that the court or arbitrator making such determination shall have the power to reduce the duration and/or geographic area of such provision, and/or to delete specific words and phrases ("blue-penciling"), and in its reduced or blue-penciled form such provision shall then be enforceable and shall be enforced.

(j) Headings and Captions; Interpretation. The headings and captions of the various subdivisions of this Agreement are for convenience of reference only and shall in no way modify, or affect the meaning or construction of any of the terms or provisions hereof. The provisions of the following Sections of this Agreement are in addition to, and do not limit, each other: Sections 6 and 5(a); Sections 7 and 5(g); Sections 12(k) and 12(f); and Sections 12(l) and 12(d).

(k) Injunctive Relief. Executive hereby expressly acknowledges that any breach or threatened breach of any of the terms and/or conditions set forth in Section 5 or 6 of this Agreement will result in substantial, continuing and irreparable injury to the Company. Therefore, Executive hereby agrees that, in addition to any other remedy that may be available to the Company, the Company shall be entitled to injunctive or other equitable relief by a court of appropriate jurisdiction.

(l) No Waiver of Rights, Powers and Remedies. No failure or delay by a party hereto in exercising any right, power or remedy under this Agreement, and no course of dealing between the parties hereto, shall operate as a waiver of any such right, power or remedy of the party. No single or partial exercise of any right, power or remedy under this Agreement by a party hereto, nor any abandonment or discontinuance of steps to enforce any such right, power or remedy, shall preclude such party from any other or further exercise thereof or the exercise of any other right, power or remedy hereunder. The election of any remedy by a party hereto shall not constitute a waiver of the right of such party to pursue other available remedies. No notice to or demand on a party not expressly required under this Agreement shall entitle the party receiving such notice or demand to any other or further notice or demand in similar or other

circumstances or constitute a waiver of the rights of the party giving such notice or demand to any other or further action in any circumstances without such notice or demand.

(m) Counterparts. This Agreement may be executed in one or more counterparts, and by different parties hereto on separate counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument.

(n) Survival. The provisions of Sections 4, 5, 6, 7, 8, 11 and 12 shall survive the termination of this Agreement and Executive's employment hereunder in accordance with their terms.

(o) Knowing and Voluntary Nature of Agreement. Executive acknowledges and agrees that Executive is executing this Agreement knowingly and voluntarily and without any duress or undue influence by PTC or anyone else. Executive further acknowledges and agrees that Executive has carefully read this Agreement and fully understands it, including that Executive is waiving the right to a jury trial. Executive further agrees that Executive has been provided an opportunity to seek the advice of an attorney of Executive's choice before signing this Agreement.

IN WITNESS THEREOF, the parties hereto have executed this Agreement as of the day and year first above written.

PTC Therapeutics, Inc.

/s/ Mark E. Boulding
Name: Mark E. Boulding
Title: EVP, Chief Legal Officer

Agreed and Accepted

/s/ Matthew Klein
Name: Matthew Klein

EXHIBIT A

Sample Separation and Release Agreement

[Insert Date]

[Insert Employee Name]
[Insert Employee Address]

Dear **[Insert Employee Name]**:

In connection with the termination of your employment with PTC Therapeutics, Inc. (the "Company") on **[Termination Date]**, you are eligible to receive the Severance Compensation as described in Section 4 of the Employment Agreement executed between you and the Company on [**Insert Date**] (the "Employment Agreement") if you sign and return this letter agreement to me by **[Return Date – e.g., 21 days from date of receipt of this letter agreement]** and it becomes binding between you and the Company. By signing and returning this letter agreement and not revoking your acceptance, you will be agreeing to the terms and conditions set forth in the numbered paragraphs below, including the release of claims set forth in paragraph 3. Therefore, you are advised to consult with an attorney before signing this letter agreement and you may take up to twenty-one (21) days to do so. If you sign this letter agreement, you may change your mind and revoke your agreement during the seven (7) day period after you have signed it by notifying me in writing. If you do not so revoke, this letter agreement will become a binding agreement between you and the Company upon the expiration of the seven (7) day period.

If you choose not to sign and return this letter agreement by **[Return Date-Same as Above]**, or if you timely revoke your acceptance in writing, you shall not receive any Severance Compensation from the Company. You will, however, receive payment for your final wages and any unused vacation time accrued through the Termination Date, as defined below. Also, regardless of signing this letter agreement, you may elect to continue receiving group medical insurance pursuant to the federal "COBRA" law, 29 U.S.C. § 1161 et seq. If you so elect, you shall pay all premium costs on a monthly basis for as long as, and to the extent that, you remain eligible for COBRA continuation. You should consult the COBRA materials to be provided by the Company for details regarding these benefits. All other benefits will cease upon your Termination Date in accordance with the plan documents.

The following numbered paragraphs set forth the terms and conditions that will apply if you timely sign and return this letter agreement and do not revoke it in writing within the seven (7) day period.

1. **Termination Date** – Your effective date of termination from the Company is **[Insert Date]** (the "Termination Date").

2. **Release** – In consideration of the payment of the Severance Compensation, which you acknowledge you would not otherwise be entitled to receive, you hereby fully, forever, irrevocably and unconditionally release, remise and discharge the Company, its affiliates, subsidiaries, parent companies, predecessors, and successors, and all of their respective past

and present officers, directors, stockholders, partners, members, employees, agents, representatives, plan administrators, attorneys, insurers and fiduciaries (each in their individual and corporate capacities) (collectively, the "Released Parties") from any and all claims, charges, complaints, demands, actions, causes of action, suits, rights, debts, sums of money, costs, accounts, reckonings, covenants, contracts, agreements, promises, doings, omissions, damages, executions, obligations, liabilities, and expenses (including attorneys' fees and costs), of every kind and nature that you ever had or now have against any or all of the Released Parties, including, but not limited to, any and all claims arising out of or relating to your employment with and/or separation from the Company, including, but not limited to, all claims under Title VII of the Civil Rights Act of 1964, 42 U.S.C. § 2000e et seq., the Americans With Disabilities Act of 1990, 42 U.S.C. § 12101 et seq., the Age Discrimination in Employment Act, 29 U.S.C. § 621 et seq., the Genetic Information Nondiscrimination Act of 2008, 42 U.S.C. § 2000ff et seq., the Family and Medical Leave Act, 29 U.S.C. § 2601 et seq., the Worker Adjustment and Retraining Notification Act ("WARN"), 29 U.S.C. § 2101 et seq., the Rehabilitation Act of 1973, 29 U.S.C. § 701 et seq., Executive Order 11246, Executive Order 11141, the Fair Credit Reporting Act, 15 U.S.C. § 1681 et seq., and the Employee Retirement Income Security Act of 1974 ("ERISA"), 29 U.S.C. § 1001 et seq., all as amended; all claims arising out of the New Jersey Law Against Discrimination, N.J. Stat. Ann. § 10:5-1 et seq., the New Jersey Family Leave Act, N.J. Stat. Ann. § 34:11B-1 et seq., the New Jersey Conscientious Employee Protection Act, N.J. Stat. Ann. § 34:19-1 et seq., and the N.J. Stat. Ann. § 34:11-56.1 et seq. (New Jersey equal pay law), all as amended; all common law claims including, but not limited to, actions in defamation, intentional infliction of emotional distress, misrepresentation, fraud, wrongful discharge, and breach of contract, including without limitation, all claims arising from the Employment Agreement; all state and federal whistleblower claims to the maximum extent permitted by law; all claims to any non-vested ownership interest in the Company, contractual or otherwise; and any claim or damage arising out of your employment with and/or separation from the Company (including a claim for retaliation) under any common law theory or any federal, state or local statute or ordinance not expressly referenced above; provided, however, that nothing in this letter agreement shall (i) prevent you from filing a charge with, cooperating with, or participating in any proceeding before the Equal Employment Opportunity Commission or a state fair employment practices agency (except that you acknowledge that you may not recover any monetary benefits in connection with any such claim, charge or proceeding) or (ii) deprive you of any rights you may have to be indemnified by the Company as provided in any agreement between the Company and you or pursuant to the Company's Certificate of Incorporation or by-laws.

3. **Non-Disclosure, Non-Competition, Confidential Information and Non-Solicitation and Inventions** – You acknowledge and reaffirm your obligations to keep confidential and not disclose all non-public information concerning the Company with respect to Confidential Information, non-solicitation, and Inventions and its clients that you acquired during the course of your employment with the Company, as stated more fully in Sections 5 and 6 of the Employment Agreement, which remains in full force and effect.

4. **Return of Company Property** – You acknowledge and reaffirm your obligations to the Company with respect to Company property, as stated more fully in Section 6 and 8 of the Employment Agreement. You confirm that you have returned to the Company all keys, files, records (and copies thereof), equipment (including, but not limited to, computer hardware, software and printers, wireless handheld devices, cellular phones, smartphones, tablets, etc.), Company identification, and any other Company-owned property in your possession or control and have left intact all electronic Company documents, including but not limited to those which you developed or helped to develop during your employment. You further confirm that you have cancelled all accounts for your benefit, if any, in the Company's name, including but not limited to, credit cards, telephone charge cards, cellular phone and/or wireless data accounts and computer accounts.

5. **Business Expenses and Final Compensation** – You acknowledge that you have been reimbursed by the Company for all business expenses incurred in conjunction with the performance of your employment and that no other reimbursements are owed to you. You further acknowledge that you have received payment in full for all services rendered in conjunction with your employment by the Company, including payment for all wages, bonuses and accrued, unused vacation time, and that no other compensation is owed to you except as provided herein.

6. **Non-Disparagement** – To the extent permitted by law, you understand and agree that as a condition for payment to you of the Severance Compensation herein described, you shall not make any false, disparaging or derogatory statements to any person or entity, including any media outlet, regarding the Company or any of its directors, officers, employees, agents or representatives or about the Company’s business affairs and financial condition.

7. **Continued Assistance** – You acknowledge and reaffirm your obligations to the Company with respect to cooperation, as stated more fully in Section 6 of the Employment Agreement. You agree that after the Termination Date you will provide all reasonable cooperation to the Company, including but not limited to, assisting the Company transition your job duties, assisting the Company in defending against and/or prosecuting any litigation or threatened litigation, and performing any other tasks as reasonably requested by the Company.

8. **Cooperation** – To the extent permitted by law, you agree to cooperate fully with the Company in the defense or prosecution of any claims or actions which already have been brought, are currently pending, or which may be brought in the future against or on behalf of the Company**,** whether before a state or federal court, any state or federal government agency, or a mediator or arbitrator. Your full cooperation in connection with such claims or actions shall include, but not be limited to, being available to meet with counsel to prepare its claims or defenses, to prepare for trial or discovery or an administrative hearing or a mediation or arbitration and to act as a witness when requested by the Company at reasonable times designated by the Company. You agree that you will notify the Company promptly in the event that you are served with a subpoena or in the event that you are asked to provide a third party with information concerning any actual or potential complaint or claim against the Company.

9. **Amendment and Waiver** – This letter agreement shall be binding upon the parties and may not be modified in any manner, except by an instrument in writing of concurrent or subsequent date signed by duly authorized representatives of the parties hereto. This letter agreement is binding upon and shall inure to the benefit of the parties and their respective agents, assigns, heirs, executors, successors and administrators. No delay or omission by the Company in exercising any right under this letter agreement shall operate as a waiver of that or any other right. A waiver or consent given by the Company on any one occasion shall be effective only in that instance and shall not be construed as a bar to or waiver of any right on any other occasion.

10. **Validity** – Should any provision of this letter agreement be declared or be determined by any court of competent jurisdiction to be illegal or invalid, the validity of the remaining parts, terms or provisions shall not be affected thereby and said illegal or invalid part, term or provision shall be deemed not to be a part of this letter agreement.

11. **Confidentiality** – To the extent permitted by law, you understand and agree that as a condition for payment to you of the Severance Compensation herein described, the terms and contents of this letter agreement, and the contents of the negotiations and discussions resulting in this letter agreement, shall be maintained as confidential by you and your agents and representatives and shall not be disclosed except to the extent required by federal or state law or as otherwise agreed to in writing by the Company.

12. **Nature of Agreement** – You understand and agree that this letter agreement is a severance agreement and does not constitute an admission of liability or wrongdoing on the part of the Company.

13. **Acknowledgments –** You acknowledge that you have been given at least [twenty-one (21) days] to consider this letter agreement, and that the Company advised you to consult with an attorney of your own choosing prior to signing this letter agreement. [You understand that you may revoke this letter agreement for a period of seven (7) days after you sign this letter agreement by notifying me in writing, and the letter agreement shall not be effective or enforceable until the expiration of this seven (7) day revocation period.] You understand and agree that by entering into this agreement, you are waiving any and all rights or claims you might have under the Age Discrimination in Employment Act, as amended by the Older Workers Benefits Protection Act, and that you have received consideration beyond that to which you were previously entitled.

14. **Voluntary Assent** – You affirm that no other promises or agreements of any kind have been made to or with you by any person or entity whatsoever to cause you to sign this letter agreement, and that you fully understand the meaning and intent of this letter agreement. You state and represent that you have had an opportunity to fully discuss and review the terms of this letter agreement with an attorney. You further state and represent that you have carefully read this letter agreement, understand the contents herein, freely and voluntarily assent to all of the terms and conditions hereof and sign your name of your own free act.

15. **Protected Conduct** – Nothing in this Agreement shall prohibit or restrict you, or be cnstrued to prohibit or restrict you, from filing a charge or complaint with, reporting possible violations of any law or regulation, making disclosures to (including providing documents or other information), and/or participating in any investigation or proceeding conducted by any self-regulatory organization or governmental agency, authority or legislative body, including, but not limited to, the Securities and Exchange Commission and/or Equal Employment Opportunity Commission or as otherwise required by law.

16. **Applicable Law** – This letter agreement shall be interpreted and construed by the laws of the State of New Jersey, without regard to conflict of laws provisions. You hereby irrevocably submit to and acknowledge and recognize the jurisdiction of the courts of the State of New Jersey, or if appropriate, a federal court located in the State of New Jersey (which courts, for purposes of this letter agreement, are the only courts of competent jurisdiction), over any suit, action or other proceeding arising out of, under or in connection with this letter agreement or the subject matter hereof.

17. **Entire Agreement** – This letter agreement contains and constitutes the entire understanding and agreement between the parties hereto with respect to your Severance Compensation and the settlement of claims against the Company and cancels all previous oral and written negotiations, agreements and commitments in connection therewith, except as otherwise set forth herein. For example, nothing in this paragraph shall modify, cancel or supersede your obligations set forth in paragraph 3 herein.

18. **Tax Acknowledgement** – In connection with the payments and consideration provided to you pursuant to this letter agreement, the Company shall withhold and remit to the tax authorities the amounts required under applicable law, and you shall be responsible for all applicable taxes with respect to such payments and consideration under applicable law. You acknowledge that you are not relying upon the advice or representation of the Company with respect to the tax treatment of any of the Severance Compensation set forth in Section 4 of the Employment Agreement.

If you have any questions about the matters covered in this letter agreement, please call me at **[Insert Phone Number]**.

Very truly yours,

By: _____

[Name]

[Title]

I hereby agree to the terms and conditions set forth above. I have been given at least [twenty-one (21) days] to consider this letter agreement and I have chosen to execute this on the date below.

I intend that this letter agreement will become a binding agreement between me and the Company [if I do not revoke my acceptance in seven (7) days].

_____ _____

[Insert Employee Name] Date

To be returned to me by **[Return Date – e.g., 21 days from date of receipt of this letter]**.

April 10, 2020

Matthew Klein
304 Franklin Avenue
Princeton, NJ 08540

Dear Matthew,

Congratulations on your proposed appointment to Chief Development Officer of PTC Therapeutics, Inc. Your success with PTC has been impressive and we look forward to your continued contributions to PTC's business and mission. The effective date of your appointment is the date that your appointment is approved by the Board of Directors of PTC following receipt of a signed copy of this letter from you indicating your acceptance of your appointment. In this new role you will report to our CEO and Founder, Stu Peltz.

Outlined below are details of your appointment:

- Your annual base salary will be increased to $450,000 annually, subject to deductions for taxes and other withholdings as required by law. Please allow 1 to 2 pay periods for your new base salary to be reflected in your paycheck.

- Your bonus target will increase to 45.00% of your annual salaried earnings paid in accordance with the terms of conditions of PTC's annual incentive compensation plan.

- You will receive a one-time grant of 50,000 stock options to purchase shares of common stock of PTC, and 10,000 restricted stock units**,** subject to formal approval by the Compensation Committee of the Board of Directors (or a majority of the PTC's independent directors) and to the terms of the applicable grant agreements. The options will vest over four years, with 25% vesting on the one-year anniversary of your grant date and 6.25% vesting every three-month period thereafter over the following three years. The restricted stock units will vest over four years, with 25% vesting annually on the anniversary of the grant date.

- This letter supersedes any other recent discussions or communications from PTC with respect to changes in your pay, title, role in the organization, or equity grants. All other terms of your employment with PTC will remain consistent with existing signed employment and equity agreements and applicable policies.

On behalf of PTC, let me again congratulate you on your proposed appointment. Please return a signed copy of this letter to me by close of business on Monday, April 13th, 2020. Feel free to contact me if you have any questions concerning this letter.

Sincerely,
/s/ Martin Rexroad

Martin Rexroad
SVP, Human Resources

Cc: Stuart Peltz

Accepted by:
/s/ Matthew Klein

Matthew Klein
April 11, 2020
Date

EXHIBIT 10.4

EMPLOYMENT AGREEMENT

This EMPLOYMENT AGREEMENT (the "Agreement") is made as of March 9, 2015 (the "Effective Date"), by and between PTC Therapeutics, Inc., a Delaware corporation (the "Company") and Eric Pauwels ("Executive"). In consideration of the mutual covenants contained in this Agreement, the Company and Executive agree as follows:

1. Employment. The Company agrees to continue to employ Executive and Executive agrees to continue to be employed by the Company on the terms and conditions set forth in this Agreement.

(a) Capacity. Executive shall serve the Company as Senior Vice President & General Manager - Americas reporting to Mark Rothera, Chief Commercial Officer, or such senior executive as the Company shall specify. Executive shall have the responsibilities, duties and authority commensurate with the position of Senior Vice President & General Manager - Americas. In addition to Executive's primary duties, Executive shall perform such other services for the Company that are consistent with his/her position as Senior Vice President & General Manager - Americas as may be reasonably assigned to Executive from time to time by the individual to whom s/he reports or the Board of Directors of the Company (the "Board") or their respective designees. The principal location at which Executive shall perform such services shall be the Company's corporate headquarters currently located at 100 Corporate Court, Middlesex Business Center, South Plainfield, NJ 07080, subject to relocation and Section 2(c)(i) of this Agreement.

(b) Devotion of Duties; Representations. During the Term (as defined below) of Executive's employment with the Company, Executive shall devote his/her best efforts and full business time and energies to the business and affairs of the Company, and shall endeavor to perform the duties and services contemplated hereunder to the reasonable satisfaction of the individual to whom s/he reports and the Board. During the Term of Executive's employment with the Company, Executive shall not, without the prior written approval of the Company (by action of the Board), undertake any other employment from any person or entity or serve as a director of any other company; provided, however, that (i) the Company will entertain requests as to such other employment or directorships in good faith and (ii) Executive will be eligible to participate in any policy relating to outside activities that is applicable to the senior executives of the Company and approved by the Board after the date hereof.

2. Term of Employment.

(a) Executive's employment hereunder shall continue on the Effective Date. Executive's employment hereunder shall be terminated upon the first to occur of the following:

a. Immediately upon Executive's death;

(ii) By the Company:

(A) By written notice to Executive effective the date of such notice, following the Disability of Executive. “Disability” means that Executive (i) is unable to engage in any substantial gainful activity by reason of any medically determinable physical or mental impairment which can be expected to result in death or can be expected to last for a continuous period of not less than 12 months, or (ii) is, by reason of any medically determinable physical or mental impairment which can be expected to last for a continuous period of not less than 12 months, receiving income replacement benefits for a period of not less than three months under an accident and health plan covering employees of the Company. Such incapacity shall be determined by a physician chosen by the Company and reasonably satisfactory to Executive (or Executive’s legal representative) upon examination requested by the Company (to which Executive hereby agrees to submit). Notwithstanding the foregoing, such Disability must result in Executive becoming “Disabled” within the meaning of Section 409A(a)(2)(C) of the Internal Revenue Code of 1986, as amended (the “Code”) and the guidance issued thereunder. (In this Agreement we refer to Section 409A of the Code and any guidance issued thereunder as “Section 409A”).

(B) By written notice to Executive, effective the date of such notice, for Cause (as defined below); or

(C) By written notice to Executive, effective ninety (90) days after the date of such notice and subject to Section 4 hereof, without Cause; or

(iii) By Executive:

(A) At any time by written notice to the Company, effective forty-five (45) days after the date of such notice**;** or

(B) By written notice to the Company for Good Reason (as defined below), effective on the date specified in such notice.

The term of Executive’s employment by the Company under this Agreement is referred to herein as the “Term.”

(b) Definition of “Cause”. For purposes of this Agreement, “Cause” shall, pursuant to the reasonable good faith determination by a majority of the Board (excluding Executive) as documented in writing, include: (i) the willful and continued failure by Executive to substantially perform Executive’s material duties or responsibilities under this Agreement (other than such a failure as a result of Disability); (ii) any action or omission by Executive

involving willful misconduct or gross negligence with regard to the Company, which has a detrimental effect on the Company; (iii) Executive's conviction of a felony, either in connection with the performance of Executive's obligations to the Company or which otherwise shall adversely affect Executive's ability to perform such obligations or shall materially adversely affect the business activities, reputation, goodwill or image of the Company; (iv) the material breach of a fiduciary duty to the Company; or (v) the material breach by Executive of any of the provisions of this Agreement, provided that any breach of Executive's obligations with respect to Sections 5 or 6 of this Agreement, subject to the cure provision in the next sentence, shall be deemed "material." In respect of the events described in clauses (i) and (v) above, the Company shall give Executive notice of the failure of performance or breach, reasonable as to time, place and manner in the circumstances, and a 30-day opportunity to cure, provided that such failure of performance or breach is reasonably amenable to cure as determined by the Board in its sole discretion.

(c) Definition of "Good Reason". For purposes of this Agreement, a "Good Reason" shall mean any of the following, unless (i) the basis for such Good Reason is cured within a reasonable period of time (determined in the light of the cure appropriate to the basis of such Good Reason, but in no event less than thirty (30) nor more than ninety (90) days) after the Company receives written notice (which must be received from Executive within ninety (90) days of the initial existence of the condition giving rise to such Good Reason) specifying the basis for such Good Reason or (ii) Executive has consented to the condition that would otherwise be a basis for Good Reason:

(i) A change in the principal location at which Executive provides services to the Company to a location more than fifty (50) miles from such principal location and/or to a location in New York City (either of which change, the Company has reasonably determined as of the date hereof, would constitute a material change in the geographic location at which Executive provides services to the Company), provided that such a relocation shall not be deemed to occur under circumstances where Executive's responsibilities require him/her to work at a location other than the corporate headquarters for a reasonable period of time;

(ii) A material adverse change by the Company in Executive's duties, authority or responsibilities as Senior Vice President & General Manager - Americas of the Company which causes Executive's position with the Company to become of materially less responsibility or authority than Executive's position immediately following the Effective Date. For purposes of this definition of "Good Reason," a "material adverse change" following a Corporate Change shall not include any diminution in authority, duties or responsibilities that is solely attributable to the change in the Company's ownership structure but does not otherwise change Executive's

authority, duties or responsibilities (except in a positive manner) otherwise with respect to the Company's business.

(iii) A material reduction in Executive's base compensation (including Base Salary) except if the reduction is in connection with a general reduction of not more than 20% in compensation of senior executives of the Company generally that occurs prior to the effective date of any Corporate Change;

(iv) A material breach of this Agreement by the Company which has not been cured within thirty (30) days after written notice thereof by Executive; or

(v) Failure to obtain the assumption (assignment) of this Agreement by any successor to the Company.

(d) Definition of "Corporate Change". For purposes of this Agreement, "Corporate Change" shall mean any circumstance in which (i) the Company is not the surviving entity in any merger, consolidation or other reorganization (or survives only as a subsidiary or affiliate of an entity other than a previously wholly-owned subsidiary of the Company); (ii) the Company sells, leases or exchanges or agrees to sell, lease or exchange all or substantially all of its assets to any other person or entity (other than a wholly-owned subsidiary of the Company); (iii) any person or entity, including a "group" as contemplated by Section 13(d)(3) of the Securities Exchange Act of 1934 (excluding, for this purpose, the Company or any Subsidiary, or any employee benefit plan of the Company or any Subsidiary, or any "group" in which all or substantially all of its members or its members' affiliates are individuals or entities who are or were beneficial owners of the Company's outstanding shares prior to the initial public offering, if any, of the Company's stock), acquires or gains ownership or control (including, without limitations, powers to vote) of more than 50% of the outstanding shares of the Company's voting stock (based upon voting power); or (iv) as a result of or in connection with a contested election of directors, the persons who were directors of the Company before such election shall cease to constitute a majority of the Board of Directors of the Company. Notwithstanding the foregoing, a "Corporate Change" shall not occur as a result of an initial public offering of the Company's common stock, or as a result of a merger, consolidation, reorganization or restructuring after which either (1) a majority of the Board of Directors of the controlling entity consists of persons who were directors of the Company prior to the merger, consolidation, reorganization or restructuring or (2) Executive forms part of an executive management team that consists of substantially the same group of individuals and Executive is performing in a similar role, with similar authority and responsibility (other than changes solely attributable to the change in ownership structure), to that which existed prior to the reorganization or restructuring. Notwithstanding the foregoing, for any payments or benefits hereunder that are subject to Section 409A, the Corporate Change must constitute a "change in control event" within the meaning of Treasury Regulation Section 1.409A-3(i)(5)(i).

3. Compensation.

(a) Base Salary. Executive's minimum base salary during the Term shall be at the rate of $335,000 per year (the "Base Salary"). Base Salary shall be payable in substantially equal installments in accordance with the Company's payroll practices as in effect from time to time, less any amounts required to be withheld under applicable law. The Base Salary will be subject to adjustment from time to time in the sole discretion of the Board; provided that, the Company covenants that it shall not reduce the Base Salary below $335,000 or the Base Salary then in effect immediately prior to the reduction unless (i) Executive consents to such reduction, or (ii) the reduction is in connection with a general reduction of not more than 20% in compensation of senior executives of the Company generally that occurs prior to the effective date of any Corporate Change.

(b) Bonus. In addition to the Base Salary, the Company may pay Executive an annual bonus (the "Bonus") as determined by the Board, solely in its discretion (it being understood that Executive's target annual bonus shall be at 40% of Base Salary, but may be higher or lower in any year in the Board's discretion). The Board's decision to issue a Bonus to Executive in any particular year shall have no effect on the absolute discretion of the Board to grant or not to grant a Bonus in subsequent years. Any Bonus for a particular year shall be paid or provided to Executive in a lump sum no later than March 15th of the calendar year following the calendar year in which the Bonus was earned.

(c) Equity Compensation. Executive will receive an inducement grant of 70,000 options to purchase shares of common stock of PTC, subject to formal approval by the Compensation Committee of the Board (or a majority of the Company's independent directors) (the "Inducement Grant"). Such award is granted pursuant to the inducement grant exception under NASDAQ rules and is intended to serve as a material inducement to Executive entering into employment with PTC. Executive shall be eligible to participate in PTC's annual equity and long term incentive plan(s) and may be eligible to receive discretionary awards under such plan(s), subject to the terms and conditions of such plan(s). Except as explicitly set forth below, Executive's rights with respect to equity (including stock options) shall be covered in PTC's equity and long term incentive plan(s) and separate stock option certificates or agreements for each grant.

(i) Accelerated Vesting.

(A) For the avoidance of doubt, in the event that Executive's employment hereunder is terminated by the Company without Cause or by Executive for Good Reason, neither the unvested portion of the Inducement Grant nor any unvested equity awards granted under the Company's equity and long-term incentive plan(s) following the date hereof shall be subject to any accelerated

vesting except as otherwise provided for in the applicable award agreement or in Section 3(c)(i)(B) below.

(B) Except as otherwise provided in the applicable award, in the event that Executive's employment hereunder is terminated by the Company without Cause or by Executive for Good Reason within the period of three (3) months prior to (but only if negotiations relating to the particular Corporate Change that occurs are ongoing at the date of the notice of termination) or twelve (12) months after a Corporate Change that occurs during the Term (such fifteen-month period, the "Protected Period"), one hundred percent (100%) of all of Executive's outstanding unvested equity awards granted under the Company's equity and long-term incentive plan(s) following the date hereof shall vest immediately.

(d) Vacation. Executive is eligible for time off programs outlined in the Company's Time Off Policy. Executive shall accrue over the calendar year 160 hours of paid vacation. Executive may accrue up to 200 hours of vacation. Once Executive has reached the maximum accrual, no further vacation time will be accrued unless and until the Executive uses vacation time. Upon termination of employment, the value of Executive's current balance of accrued but unused vacation shall be paid out in cash based on his/her Base Salary that was in effect immediately prior to his/her termination of employment.

(e) Fringe Benefits. Executive shall be entitled to participate in any employee benefit plans that the Company makes available to its senior executives (including, without limitation, group life, disability, medical, dental and other insurance, retirement, pension, profit-sharing and similar plans) (collectively, the "Fringe Benefits"), provided that the Fringe Benefits shall not include any stock option or similar plans relating to the grant of equity securities of the Company. These benefits may be modified or changed from time to time at the sole discretion of the Company. Where a particular benefit is subject to a formal plan (for example, medical or life insurance), eligibility to participate in and receive any particular benefit is governed solely by the applicable plan document, and eligibility to participate in such plan(s) may be dependent upon, among other things, a physical examination.

(f) Reimbursement of Expenses. Executive shall be entitled to reimbursement for all ordinary and reasonable out-of-pocket business expenses that are reasonably incurred by him/her in furtherance of the Company's business in accordance with reasonable policies adopted from time to time by the Company for senior executives.

(g) Taxes and Withholdings. The Company shall deduct and withhold from all compensation and benefits under this Agreement all social security and other federal, state and local taxes and charges which currently are or which hereafter may be required by law to be so deducted and withheld.

4. Severance Compensation.

(a) In the event of any termination of Executive's employment for any reason the Company shall pay Executive (or Executive's estate) such portions of Executive's Base Salary as have accrued prior to such termination and have not yet been paid, together with (i) amounts for accrued unused vacation days (as provided above), (ii) any amounts for expense reimbursement which have been properly incurred or the Company has become obligated to pay prior to termination and have not been paid as of the date of such termination and (iii) the amount of any Bonus previously granted to Executive by the Board but not yet paid, which amount shall not include any pro rata portion of any Bonus which would have been earned if such termination had not occurred (the "Accrued Obligations"). Such amounts shall be paid as soon as possible after termination.

(b) In the event that Executive's employment hereunder is terminated (i) by Executive for a Good Reason or (ii) by the Company without Cause, the Company shall pay to Executive the Accrued Obligations. In addition, the Company shall pay to Executive the severance benefits set forth below for twelve (12) months following Executive's termination of employment (the "Severance Period"). The receipt of any severance benefits provided in this Section shall be dependent upon Executive's execution (and, as applicable, non-revocation) of a standard separation agreement and general release of claims, substantially in the form attached hereto as Exhibit A (the "Release"). The Company will also consider in good faith (but without any binding commitment) requests from Executive that the Company include in the Release a release of Executive by the Company from matters specifically disclosed to the Company by Executive in writing in advance of execution of the Release and not involving any illegality, fraud, concealment, criminal acts or acts outside the scope of Executive's employment. The distribution of severance benefits in this Section 4 is subject to section (iii) of this Section 4(b).

(i) If Executive's employment is terminated (A) by Executive for a Good Reason or (B) by the Company without Cause, in either case before or after the Protected Period, the Company shall pay Executive his/her Base Salary, less any amounts required to be withheld under applicable law, for the Severance Period in substantially equal installments in accordance with the Company's payroll practices as in effect from time to time, commencing no later than sixty (60) days following the effective date of such termination. If Executive's employment is terminated (A) by Executive for a Good Reason or (B) by the Company without Cause, in either case during the Protected Period, the Company shall pay Executive his/her Base Salary for the Severance Period, which total amount shall be payable in a lump sum no later than sixty (60) days following Executive's termination of employment. In each case, payments shall commence or be paid provided that the Release has been executed and any applicable revocation period has expired as of the 60th day following Executive's termination.

(ii) Only if Executive's employment is terminated (A) by Executive for a Good Reason or (B) by the Company without Cause, in either case during the Protected Period, the Company shall pay Executive his target annual bonus, described in section 3(b) hereof, for the year in which the termination of employment occurs, which total amount shall be payable in a lump sum 30 days following Executive's termination of employment, provided that the Release has been executed and any applicable revocation period has expired as of such date.

(iii) The Company shall continue to provide Executive and his/her then-enrolled eligible dependents with group health insurance and shall continue to pay the amount of the premium as in effect on the date of such termination for the Severance Period commencing on the effective date of such termination, subject to applicable law and the terms of the respective policies; provided that the Company's obligation to provide the benefits contemplated herein shall terminate upon Executive's becoming eligible for coverage under the medical benefits program of a subsequent employer. The foregoing shall not be construed to extend any period of continuation coverage (e.g., COBRA) required by Federal law.

(iv) Compliance with Section 409A. Subject to the provisions in this Section 4(b)(iii), any severance payments or benefits under this Agreement shall begin only upon the date of Executive's "separation from service" (determined as set forth below) which occurs on or after the date of termination of Executive's employment. The following rules shall apply with respect to the distribution of the severance payments and benefits, if any, to be provided to Executive under this Agreement:

(1) It is intended that each installment of the severance payments and benefits provided under this Agreement shall be treated as a separate "payment" for purposes of Section 409A. Neither the Company nor Executive shall have the right to accelerate or defer the delivery of any such payments or benefits except to the extent specifically permitted or required by Section 409A.

(2) If, as of the date of Executive's "separation from service" from the Company, Executive is not a "specified employee" (within the meaning of Section 409A), then each installment of the severance payments and benefits shall be made on the dates and terms set forth in this Agreement.

(3) If, as of the date of Executive's "separation from service" from the Company, Executive is a "specified employee" (within the meaning of Section 409A), then:

(A) Each installment of the severance payments and benefits due under this Agreement that, in accordance with the dates and terms set

forth herein, will in all circumstances, regardless of when the separation from service occurs, be paid within the short-term deferral period (as defined under Section 409A) shall be treated as a short-term deferral within the meaning of Treasury Regulation Section 1.409A-1(b)(4) to the maximum extent permissible under Section 409A and such payments and benefits shall be paid or provided on the dates and terms set forth in this Agreement; and

(B) Each installment of the severance payments and benefits due this Agreement that is not described in Section 4(b)(iii)(3)(A) above and that would, absent this subsection, be paid within the six-month period following Executive's "separation from service" from the Company shall not be paid until the date that is six months and one day after such separation from service (or, if earlier, Executive's death), with any such installments that are required to be delayed being accumulated during the six-month period and paid in a lump sum on the date that is six months and one day following Executive's separation from service and any subsequent installments, if any, being paid in accordance with the dates and terms set forth herein; provided, however, that the preceding provisions of this sentence shall not apply to any installment of severance payments and benefits if and to the maximum extent that such installment is deemed to be paid under a separation pay plan that does not provide for a deferral of compensation by reason of the application of Treasury Regulation 1.409A-1(b)(9)(iii) (relating to separation pay upon an involuntary separation from service). Any installments that qualify for the exception under Treasury Regulation Section 1.409A-1(b)(9)(iii) must be paid no later than the last day of Executive's second taxable year following the taxable year in which the separation from service occurs.

(4) The determination of whether and when Executive's separation from service from the Company has occurred shall be made in a manner consistent with, and based on the presumptions set forth in, Treasury Regulation Section 1.409A-1(h). Solely for purposes of this Section 4(b)(iii), "Company" shall include all persons with whom the Company would be considered a single employer under Section 414(b) and 414(c) of the Code.

(5) All reimbursements and in-kind benefits provided under this Agreement shall be made or provided in accordance with the requirements of Sections 409A to the extent that such reimbursements or in-kind benefits are subject to Section 409A, including, where applicable, the requirements that (i) any reimbursement is for expenses incurred during Executive's lifetime (or during a shorter period of time specified in this Agreement), (ii) the amount of expenses eligible for reimbursement during a calendar year may not affect the expenses

eligible for reimbursement in any other calendar year, (iii) the reimbursement of an eligible expense will be made on or before the last day of the calendar year following the year in which the expense is incurred and (iv) the right to reimbursement is not subject to set off or liquidation or exchange for any other benefit.

(6) Notwithstanding anything herein to the contrary, the Company shall have no liability to Executive or to any other person if the payments and benefits provided hereunder that are intended to be exempt from or compliant with Section 409A are not so exempt or compliant.

(c) In the event that Executive's employment hereunder is terminated (i) by Executive for other than a Good Reason, or (ii) by the Company for Cause, or (iii) as a result of Executive's death or Disability, then the Company will pay to Executive the Accrued Obligations. The Company shall have no obligation to pay Executive (or Executive's estate) any other compensation following such termination except as provided in Section 4(a).

(d) Modified Section 280G Cutback.

(i) Notwithstanding any other provision of this Agreement, except as set forth in Section 4(d)(ii), in the event that the Company undergoes a "Change in Ownership or Control" (as defined below), the Company shall not be obligated to provide to Executive a portion of any "Contingent Compensation Payments" (as defined below) that Executive would otherwise be entitled to receive to the extent necessary to eliminate any "excess parachute payments" (as defined in Section 280G(b)(1) of the Code) for Executive. For purposes of this Section 4(d), the Contingent Compensation Payments so eliminated shall be referred to as the "Eliminated Payments" and the aggregate amount (determined in accordance with Treasury Regulation Section 1.280G-1, Q/A-30 or any successor provision) of the Contingent Compensation Payments so eliminated shall be referred to as the "Eliminated Amount."

(ii) Notwithstanding the provisions of Section 4(d)(i), no such reduction in Contingent Compensation Payments shall be made if (1) the Eliminated Amount (computed without regard to this sentence) exceeds (2) 100% of the aggregate present value (determined in accordance with Treasury Regulation Section 1.280G-1, Q/A-31 and Q/A-32 or any successor provisions) of the amount of any additional taxes that would be incurred by Executive if the Eliminated Payments (determined without regard to this sentence) were paid to him/her (including, state and federal income taxes on the Eliminated Payments, the excise tax imposed by Section 4999 of the Code payable with respect to all of the Contingent Compensation Payments in excess of Executive's "base amount" (as defined in Section 280G(b)(3) of the Code), and any withholding taxes). The override of such reduction in Contingent Compensation Payments pursuant to this

Section 4(d)(ii) shall be referred to as a “Section 4(d)(ii) Override.” For purpose of this paragraph, if any federal or state income taxes would be attributable to the receipt of any Eliminated Payment, the amount of such taxes shall be computed by multiplying the amount of the Eliminated Payment by the maximum combined federal and state income tax rate provided by law.

(iii) For purposes of this Section 4(d) the following terms shall have the following respective meanings:

(1) “Change in Ownership or Control” shall mean a change in the ownership or effective control of the Company or in the ownership of a substantial portion of the assets of the Company determined in accordance with Section 280G(b)(2) of the Code.

(2) “Contingent Compensation Payment” shall mean any payment (or benefit) in the nature of compensation that is made or made available (under this Agreement or otherwise) to a “disqualified individual” (as defined in Section 280G(c) of the Code) and that is contingent (within the meaning of Section 280G(b)(2)(A)(i) of the Code) on a Change in Ownership or Control of the Company.

(iv) Any payments or other benefits otherwise due to Executive following a Change in Ownership or Control that could reasonably be characterized (as determined by the Company) as Contingent Compensation Payments (the “Potential Payments”) shall not be made until the dates provided for in this Section 4(d)(iv). Within 30 days after each date on which Executive first becomes entitled to receive (whether or not then due) a Contingent Compensation Payment relating to such Change in Ownership or Control, the Company shall determine and notify Executive (with reasonable detail regarding the basis for its determinations) (1) which Potential Payments constitute Contingent Compensation Payments, (2) the Eliminated Amount and (3) whether the Section 4(d)(ii) Override is applicable. Within 30 days after delivery of such notice to Executive, Executive shall deliver a response to the Company (the “Executive Response”) stating either (A) that s/he agrees with the Company’s determination pursuant to the preceding sentence or (B) that s/he disagrees with such determination, in which case s/he shall set forth (x) which Potential Payments should be characterized as Contingent Compensation Payments, (y) the Eliminated Amount, and (z) whether the Section 4(d)(ii) Override is applicable. In the event that Executive fails to deliver an Executive Response on or before the required date, the Company’s initial determination shall be final. If Executive states in the Executive Response that s/he agrees with the Company’s determination, the Company shall make the Potential Payments to Executive within three business days following delivery to the Company of the Executive Response

(except for any Potential Payments which are not due to be made until after such date, which Potential Payments shall be made on the date on which they are due). If Executive states in the Executive Response that s/he disagrees with the Company's determination, then, for a period of 60 days following delivery of the Executive Response, Executive and the Company shall use good faith efforts to resolve such dispute. If such dispute is not resolved within such 60-day period, such dispute shall be settled exclusively by arbitration in South Plainfield, New Jersey, in accordance with the rules of the American Arbitration Association then in effect. Judgment may be entered on the arbitrator's award in any court having jurisdiction. The Company shall, within three business days following delivery to the Company of the Executive Response, make to Executive those Potential Payments as to which there is no dispute between the Company and Executive regarding whether they should be made (except for any such Potential Payments which are not due to be made until after such date, which Potential Payments shall be made on the date on which they are due). The balance of the Potential Payments shall be made within three business days following the resolution of such dispute.

(v) The Contingent Compensation Payments to be treated as Eliminated Payments shall be determined by the Company by determining the "Contingent Compensation Payment Ratio" (as defined below) for each Contingent Compensation Payment and then reducing the Contingent Compensation Payments in order beginning with the Contingent Compensation Payment with the highest Contingent Compensation Payment Ratio. For Contingent Compensation Payments with the same Contingent Compensation Payment Ratio, such Contingent Compensation Payment shall be reduced based on the time of payment of such Contingent Compensation Payments with amounts having later payment dates being reduced first. For Contingent Compensation Payments with the same Contingent Compensation Payment Ratio and the same time of payment, such Contingent Compensation Payments shall be reduced on a pro rata basis (but not below zero) prior to reducing Contingent Compensation Payment with a lower Contingent Compensation Payment Ratio. The term "Contingent Compensation Payment Ratio" shall mean a fraction the numerator of which is the value of the applicable Contingent Compensation Payment that must be taken into account by Executive for purposes of Section 4999(a) of the Code, and the denominator of which is the actual amount to be received by Executive in respect of the applicable Contingent Compensation Payment. For example, in the case of an equity grant that is treated as contingent on the Change in Ownership or Control because the time at which the payment is made or the payment vests is accelerated, the denominator shall be determined by reference to the fair market value of the equity at the acceleration date, and not in accordance with the methodology for determining the value of accelerated payments set forth in Treasury Regulation Section 1.280G-1Q/A-24(b) or (c)).

(vi) The provisions of this Section 4(d) are intended to apply to any and all payments or benefits available to Executive under this Agreement or any other agreement or plan of the Company under which Executive receives Contingent Compensation Payments.

(vii) Notwithstanding Sections 4(d)(i)-(vi) hereof, until the closing of the first underwritten public offering of common stock of the Company, in the event that it shall be determined that any payment or benefit (including any accelerated vesting of options or other equity awards) made or provided, or to be made or provided, by the Company (or any successor thereto or affiliate thereof) to or for the benefit of Executive, whether pursuant to the terms of this Agreement, any other agreement, plan, program or arrangement of or with the Company (or any successor thereto or affiliate thereof) or otherwise, may be subject to the excise tax imposed by Section 4999 of the Code or any comparable tax imposed by any replacement or successor provision of United States tax law, then upon the request of Executive, the Company shall use reasonable efforts to procure a shareholder vote in satisfaction of the shareholder approval requirements described in Treas. Reg. Section 1.280G-1, Q&A-7.

5. Executive Covenants.

(a) Confidential Information. Executive recognizes and acknowledges the competitive and proprietary aspects of the business of the Company, and that as a result of Executive's employment, Executive recognizes and acknowledges that s/he has had and will continue to have access to, and has been and will continue to be involved in the development of, Confidential Information (as defined below) of the Company. As used herein, "Confidential Information" shall mean and include trade secrets, knowledge and other confidential information of the Company, which Executive has acquired, no matter from whom or on what matter such knowledge or information may have been acquired, heretofore or hereafter, concerning the content and details of the business of the Company, and which is not known to the general public, including but not limited to: (a) new products, product betterments and other inventions, formulas, processes, methods, materials, material combinations, manner of preparations, technical production procedures and information, alarm and security codes and procedures, sources of technology, and sources of supply of raw and finished materials and other products; (b) financial and accounting records; (c) the identity of employees, consultants, independent contractors, customers, business development partners, licensees, suppliers, creditors or other parties with which the Company has business dealings, the nature of the relationship with such persons, or any other information relating to such persons or the Company's dealings with such persons; and (d) computer software used by the Company or provided to the customers of the Company unless publicly available.

(i) For as long as Executive is employed and at all times thereafter, Executive shall not, directly or indirectly, communicate, disclose or divulge to any person or entity, or use for Executive's own benefit or the benefit of any person (other than the Company), any Confidential Information, except as permitted in subparagraph (iii) below. Upon termination of Executive's employment, or at any other time at the request of the Company, Executive agrees to deliver promptly to the Company all Confidential Information, including, but not limited to, customer and supplier lists, files and records, in Executive's possession or under Executive's control. Executive further agrees that s/he will not make or retain any copies of any of the foregoing and will so represent to the Company upon termination of Executive's employment.

(ii) Executive shall disclose immediately to the Company any trade secrets or other Confidential Information conceived or developed by Executive at any time during Executive's employment. Executive hereby assigns and agrees to assign to the Company Executive's entire right, title and interest in and to all Confidential Information. Such assignment shall include, without limitation, the rights to obtain patent or copyright protection, thereon in the United States and foreign countries. Executive agrees to provide all reasonable assistance to enable the Company to prepare and prosecute any application before any governmental agency for patent or copyright protection or any similar application with respect to any Confidential Information. Executive further agrees to execute all documents and assignments and to make all oaths necessary to vest ownership of such intellectual property rights in the Company, as the Company may request. These obligations shall apply whether or not the subject thereof was conceived or developed at the suggestion of the Company, and whether or not developed during regular hours of work or while on the premises of the Company.

(iii) Executive shall at all times, both during and after termination of this Agreement by either Executive or the Company, maintain in confidence and shall not, without prior written consent of the Company, use, except in the course of performance of Executive's duties for the Company or as required by legal process (provided that Executive will promptly notify the Company of such legal process except with respect to any confidential government investigation), disclose or give to others any Confidential Information. In the event Executive is questioned by anyone not employed by the Company or by an employee of or a consultant to the Company not authorized to receive such information, in regard to any such information or any other secret or confidential work of the Company, or concerning any fact or circumstance relating thereto, Executive will promptly notify the Company.

(b) Non-Competition and Non-Solicitation. Executive recognizes that the Company is engaged in a competitive business and that the Company has a legitimate interest in protecting its trade secrets, confidential business information, and customer, business

development partner, licensee, supplier, and credit and/or financial relationships. Accordingly, in exchange for valuable consideration, including without limitation Executive's access to confidential business information and continued at-will employment, Executive agrees that, during the Term hereof and for a period of eighteen (18) months thereafter, Executive shall not:

(i) directly or indirectly, whether for himself or for any other person or entity, and whether as a proprietor, principal, shareholder, partner, agent, employee, consultant, independent contractor, or in any other capacity whatsoever, undertake or have any interest in (other than the passive ownership of publicly registered securities representing an ownership interest of less than 1%), engage in or assume any role involving directly or indirectly the Company's Field of Interest (or any portion thereof) or any other business in which the Company is engaged and for which the employee has rendered services while employed by the Company, or enter into any agreement to do any of the foregoing; or

(ii) initiate contact with (including without limitation phone calls, press releases and the sending or delivering of announcements), or in any manner solicit, directly or indirectly, any customers, business development partners, licensors, licensees, or creditors (including institutional lenders, bonding companies and trade creditors) of the Company in an attempt to induce or motivate them either to discontinue or modify their then prevailing or future relationship with the Company or to transfer any of their business with the Company to any person or entity other than the Company; or

(iii) initiate contact with, or in any manner solicit, directly or indirectly, any supplier of goods, services or materials to the Company in an attempt to induce or motivate them either to discontinue or modify their then prevailing or future relationship with the Company or to supply the same or similar inventory, goods, services or materials (except generally available inventory, goods, services or materials) to any person or entity other than the Company; or

(iv) directly or indirectly recruit, solicit or otherwise induce or influence any employee or independent contractor of the Company to discontinue or modify his or her employment or engagement with the Company, or employ or contract with any such employee or contractor for the provision of services.

(c) Definition of "Field of Interest". The term Company's "Field of Interest" shall mean the research, development and commercialization of products and strategies relating to: (i) therapies for genetic disorders or diseases that include cystic fibrosis, Duchenne muscular dystrophy, other diseases caused in whole or part by nonsense (or stop) codons, and other genetic diseases as to which the Company engages in the research, development or commercialization of drugs; anti-angiogenic therapies that target VEGF protein production for cancer; and antiviral therapies for the Hepatitis C virus (HCV); and (ii) other therapeutic targets, mechanisms of

action and/or therapies in which the Company has a research, development or commercialization program.

(d) Definition of "Customer". The term "customer" or "customers" shall include any person or entity (a) that is a current customer of the Company, (b) that was a customer of the Company at any time during the preceding twenty-four (24) months or (c) to which the Company made a written presentation for the solicitation of business at any time during the preceding twenty-four (24) months.

(e) Reasonableness of Restrictions. Executive further recognizes and acknowledges that (i) the types of employment which are prohibited by this Section 5 are narrow and reasonable in relation to the skills which represent Executive's principal salable asset both to the Company and to Executive's other prospective employers, and (ii) the broad geographical scope of the provisions of this Section 5 is reasonable, legitimate and fair to Executive in light of the global nature of the Company's business, particularly pharmaceutical research and development, and in light of the limited restrictions on the type of employment prohibited herein compared to the types of employment for which Executive is qualified to earn Executive's livelihood.

(f) Remedies. Executive acknowledges that a breach of this Section 5 will cause great and irreparable injury and damage, which cannot be reasonably or adequately compensated by money damages. Accordingly, Executive acknowledges that the remedies of injunction and specific performance shall be available in the event of such a breach, in addition to money damages, costs and attorneys' fees, and other legal or equitable remedies, and that the Company shall be entitled as a matter of course to an injunction pending trial, without the posting of bond or other security. Any period of restriction set forth in this Section 5 shall be extended for a period of time equal to the duration of any breach or violation hereof.

(g) Notification. Any person employing Executive or evidencing any intention to employ Executive may be notified as to the existence and provisions of this Agreement.

(h) Modification of Covenants; Enforceability. In the event that any provision of this Section 5 is held to be in any respect an unreasonable restriction, then the court so holding may modify the terms thereof, including the period of time during which it operates or the geographic area to which it applies, or effect any other change to the extent necessary to render this section enforceable, it being acknowledged by the parties that the representations and covenants set forth herein are of the essence of this Agreement.

(i) Subsidiaries. For purposes of Sections 5 and 6 of this Agreement, "Company" shall include all direct and indirect subsidiaries of the Company. An entity shall be deemed to be a subsidiary of the Company if the Company directly or indirectly owns or controls 50% or more of the equity interest in such entity.

6. Ownership of Ideas, Copyrights and Patents.

(a) Property of the Company. Executive agrees that all ideas, discoveries, creations, manuscripts and properties, innovations, improvements, know‐how, inventions, designs, developments, apparatus, techniques, methods, biological processes, cell lines, laboratory notebooks and formulae, whether patentable, copyrightable or not, which Executive may conceive, reduce to practice or develop, alone or in conjunction with another, or others, whether during or out of regular business hours, and whether at the request or upon the suggestion of the Company, or otherwise, in the course of performing services for the Company in any capacity, whether heretofore or hereafter, (collectively, "the Inventions") are and shall be the sole and exclusive property of the Company, and that Executive shall not publish any of the Inventions without the prior written consent of the Company. Executive hereby assigns to the Company all of Executive's right, title and interest in and to all of the foregoing. Executive further represents and agrees that to the best of Executive's knowledge and belief none of the Inventions will violate or infringe upon any right, patent, copyright, trademark or right of privacy, or constitute libel or slander against or violate any other rights of any person, firm or corporation and that Executive will use his/her best efforts to prevent any such violation.

(b) Cooperation. At any time during or after the Term, Executive agrees that s/he will fully cooperate with the Company, its attorneys and agents in the preparation and filing of all papers and other documents as may be required to perfect the Company's rights in and to any of such Inventions, including, but not limited to, executing any lawful document (including, but not limited to, applications, assignments, oaths, declarations and affidavits) and joining in any proceeding to obtain letters patent, copyrights, trademarks or other legal rights of the United States and of any and all other countries on such Inventions, provided that any patent or other legal right so issued to Executive, personally, shall be assigned by Executive to the Company without charge by Executive. Executive further designates the Company as his/her agent for, and grants to the Company a power of attorney with full power of substitution, which power of attorney shall be deemed coupled with an interest, for the purpose of effecting the foregoing assignments from Executive to the Company. Company will bear the reasonable expenses which it causes to be incurred in Executive's assisting and cooperating hereunder. Executive waives all claims to moral rights in any Inventions.

7. Disclosure to Future Employers. The Company may provide in its discretion, a copy of the covenants contained in Sections 5 and 6 of this Agreement to any business or enterprise which Executive may directly, or indirectly, own, manage, operate, finance, join, control or in which Executive participates in the ownership, management, operation, financing, or control, or with which Executive may be connected as an officer, director, employee, partner, principal, agent, representative, consultant or otherwise.

8. Records. Upon termination of Executive's relationship with the Company, Executive shall deliver to the Company any property of the Company which may be in Executive's possession including products, materials, memoranda, notes, records, reports, or other documents or photocopies of the same.

9. Insurance. The Company, in its sole discretion, may apply for and procure in its own name (whether or not for its own benefit) policies of insurance insuring Executive's life. Executive agrees to submit to reasonable medical or other examinations and to execute and deliver any applications or other instruments in writing that are reasonably necessary to effectuate such insurance. No adverse employment actions may be based upon the results of any such exam or the failure by the Company to obtain such insurance.

10. No Conflicting Agreements. Executive hereby represents and warrants that Executive has no commitments or obligations inconsistent with this Agreement.

11. "Market Stand-Off" Agreement. Executive agrees, if requested by the Company and an underwriter of common stock (or other securities) of the Company, not to sell or otherwise transfer or dispose of any common stock (or other securities) of the Company held by Executive during a period not to exceed one hundred and eighty (180) days following the effective date of the first underwritten public offering of common stock of the Company, offered on a firm commitment basis pursuant to a registration statement filed with the Securities and Exchange Commission (or any successor agency of the Federal government administrating the Securities Act of 1933, as amend, and the Securities Exchange Act of 1934, as amended) under the Securities Act of 1933, as amended, on Form S-1 or its then equivalent, and to enter into an agreement to such effect. The Company may impose stop-transfer instructions with respect to the shares (or securities) subject to the foregoing restriction until the end of said period.

12. General.

(a) Notices. All notices, requests, consents and other communications hereunder shall be in writing, shall be addressed to the receiving party's address as follows:

If to the Company: PTC Therapeutics Inc.
100 Corporate Court
South Plainfield, NJ 07080
USA
Attention: Legal Department
Telephone: (908) 222-7000

With an email copy to: legal@ptcbio.com

If to Executive: Eric Pauwels
623 Spring Valley Road

Morristown, NJ 07960

or to such other address as a party may designate by notice hereunder, and shall be either (i) delivered by hand, (ii) sent by overnight courier, or (iii) sent by registered or certified mail, return receipt requested, postage prepaid. All notices, requests, consents and other communications hereunder shall be deemed to have been given either (i) if by hand, at the time of the delivery thereof to the receiving party at the address of such party set forth above, (ii) if sent by overnight courier, on the next business day following the day such notice is delivered to the courier service, or (iii) if sent by registered or certified mail, on the fifth (5th) business day following the day such mailing is made.

(b) Entire Agreement. This Agreement embodies the entire agreement and understanding between the parties hereto with respect to the subject matter hereof and supersedes all prior oral or written agreements and understandings relating to the subject matter hereof, except with respect to the equity and fringe benefit arrangements referred to in Subsections 3(c) and (e) above. No statement, representation, warranty, covenant or agreement of any kind not expressly set forth in this Agreement shall affect, or be used to interpret, change or restrict, the express terms and provisions of this Agreement.

(c) Modifications and Amendments. The terms and provisions of this Agreement may be modified or amended only by written agreement executed by the parties hereto.

(d) Waivers and Consents. The terms and provisions of this Agreement may be waived, or consent for the departure therefrom granted, only by written document executed by the party entitled to the benefits of such terms or provisions. No such waiver or consent shall be deemed to be or shall constitute a waiver or consent with respect to any other terms or provisions of this Agreement, whether or not similar. Each such waiver or consent shall be effective only in the specific instance and for the purpose for which it was given, and shall not constitute a continuing waiver or consent.

(e) Assignment. The Company shall assign its rights and obligations hereunder to any person or entity that succeeds to all or substantially all of the Company's business or that aspect of the Company's business in which Executive is principally involved. Executive may not assign Executive's rights and obligations under this Agreement without the prior written consent of the Company.

(f) Benefit. All statements, representations, warranties, covenants and agreements in this Agreement shall be binding on the parties hereto and shall inure to the benefit of the respective successors and permitted assigns of each party hereto. Nothing in this Agreement shall be construed to create any rights or obligations except among the parties hereto, and no person or entity shall be regarded as a third□party beneficiary of this Agreement.

(g) Governing Law. This Agreement and the rights and obligations of the parties hereunder shall be construed in accordance with and governed by the law of The State of New Jersey, without giving effect to the conflict of law principles thereof.

(h) Jurisdiction and Service of Process. Any legal action or proceeding with respect to this Agreement shall be brought in the courts of The State of New Jersey or of the United States of America for the District of New Jersey. By execution and delivery of this Agreement, each of the parties hereto accepts for itself and in respect of its property, generally and unconditionally, the jurisdiction of the aforesaid courts. Each of the parties hereto irrevocably consents to the service of process of any of the aforementioned courts in any such action or proceeding by the mailing of copies thereof by certified mail, postage prepaid, to the party at its address set forth in Section 12(a) hereof.

(i) Severability. The parties intend this Agreement to be enforced as written. However, (i) if any portion or provision of this Agreement shall to any extent be declared illegal or unenforceable by a duly authorized court having jurisdiction, then the remainder of this Agreement, or the application of such portion or provision in circumstances other than those as to which it is so declared illegal or unenforceable, shall not be affected thereby, and each portion and provision of this Agreement shall be valid and enforceable to the fullest extent permitted by law; and (ii) if any provision, or part thereof, is held to be unenforceable because of the duration of such provision or the geographic area covered thereby or otherwise, the Company and Executive agrees that the court making such determination shall have the power to reduce the duration and/or geographic area of such provision, and/or to delete specific words and phrases (“blue-penciling”), and in its reduced or blue-penciled form such provision shall then be enforceable and shall be enforced.

(j) Headings and Captions; Interpretation. The headings and captions of the various subdivisions of this Agreement are for convenience of reference only and shall in no way modify, or affect the meaning or construction of any of the terms or provisions hereof. The provisions of the following Sections of this Agreement are in addition to, and do not limit, each other: Sections 6 and 5(a); Sections 7 and 5(g); Sections 12(k) and 12(f); and Sections 12(l) and 12(d).

(k) Injunctive Relief. Executive hereby expressly acknowledges that any breach or threatened breach of any of the terms and/or conditions set forth in Section 5 or 6 of this Agreement will result in substantial, continuing and irreparable injury to the Company. Therefore, Executive hereby agrees that, in addition to any other remedy that may be available to the Company, the Company shall be entitled to injunctive or other equitable relief by a court of appropriate jurisdiction.

(l) No Waiver of Rights, Powers and Remedies. No failure or delay by a party hereto in exercising any right, power or remedy under this Agreement, and no course of dealing

between the parties hereto, shall operate as a waiver of any such right, power or remedy of the party. No single or partial exercise of any right, power or remedy under this Agreement by a party hereto, nor any abandonment or discontinuance of steps to enforce any such right, power or remedy, shall preclude such party from any other or further exercise thereof or the exercise of any other right, power or remedy hereunder. The election of any remedy by a party hereto shall not constitute a waiver of the right of such party to pursue other available remedies. No notice to or demand on a party not expressly required under this Agreement shall entitle the party receiving such notice or demand to any other or further notice or demand in similar or other circumstances or constitute a waiver of the rights of the party giving such notice or demand to any other or further action in any circumstances without such notice or demand.

(m) Counterparts. This Agreement may be executed in one or more counterparts, and by different parties hereto on separate counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument.

(n) Survival. The provisions of Sections 4, 5, 6, 7, 8, 11 and 12 shall survive the termination of this Agreement and Executive's employment hereunder in accordance with their terms.

(o) WAIVER OF TRIAL BY JURY. THE PARTIES IRREVOCABLY WAIVE THEIR RESPECTIVE RIGHT TO A TRIAL BY JURY REGARDING ANY DISPUTE, CLAIM OR CAUSE OF ACTION ARISING OUT OF, CONCERNING, OR RELATED TO EXECUTIVE'S EMPLOYMENT WITH THE COMPANY OR THIS AGREEMENT.

(p) Knowing and Voluntary Nature of Agreement. Executive acknowledges and agrees that Executive is executing this Agreement knowingly and voluntarily and without any duress or undue influence by PTC or anyone else. Executive further acknowledges and agrees that Executive has carefully read this Agreement and fully understands it, including that Executive is waiving the right to a jury trial. Executive further agrees that Executive has been provided an opportunity to seek the advice of an attorney of Executive's choice before signing this Agreement.

IN WITNESS THEREOF, the parties hereto have executed this Agreement as of the day and year first above written.

PTC Therapeutics, Inc.

/s/ Mark E. Boulding
Name: Mark E. Boulding
Title: EVP, Chief Legal Officer

Agreed and Accepted

/s/ Eric Pauwels
Name: Eric Pauwels

EXHIBIT A

Sample Separation and Release Agreement

[Insert Date]

[Insert Employee Name]
[Insert Employee Address]

Dear **[Insert Employee Name]**:

In connection with the termination of your employment with PTC Therapeutics, Inc. (the "Company") on **[Termination Date]**, you are eligible to receive the Severance Compensation as described in Section 4 of the Employment Agreement executed between you and the Company on [**Insert Date**] (the "Employment Agreement") if you sign and return this letter agreement to me by **[Return Date – e.g., 21 days from date of receipt of this letter agreement]** and it becomes binding between you and the Company. By signing and returning this letter agreement and not revoking your acceptance, you will be agreeing to the terms and conditions set forth in the numbered paragraphs below, including the release of claims set forth in paragraph 3. Therefore, you are advised to consult with an attorney before signing this letter agreement and you may take up to twenty-one (21) days to do so. If you sign this letter agreement, you may change your mind and revoke your agreement during the seven (7) day period after you have signed it by notifying me in writing. If you do not so revoke, this letter agreement will become a binding agreement between you and the Company upon the expiration of the seven (7) day period.

If you choose not to sign and return this letter agreement by **[Return Date-Same as Above]**, or if you timely revoke your acceptance in writing, you shall not receive any Severance Compensation from the Company. You will, however, receive payment for your final wages and any unused vacation time accrued through the Termination Date, as defined below, on the Company's regular payroll date immediately following the Termination Date. Also, regardless of signing this letter agreement, you may elect to continue receiving group medical insurance pursuant to the federal "COBRA" law, 29 U.S.C. § 1161 et seq. If you so elect, you shall pay all premium costs on a monthly basis for as long as, and to the extent that, you remain eligible for COBRA continuation. You should consult the COBRA materials to be provided by the Company for details regarding these benefits. All other benefits will cease upon your Termination Date in accordance with the plan documents.

The following numbered paragraphs set forth the terms and conditions that will apply if you timely sign and return this letter agreement and do not revoke it in writing within the seven (7) day period.

1. **Termination Date** – Your effective date of termination from the Company is **[Insert Date]** (the "Termination Date").

2. **Release** – In consideration of the payment of the Severance Compensation, which you acknowledge you would not otherwise be entitled to receive, you hereby fully, forever, irrevocably and unconditionally release, remise and discharge the Company, its affiliates, subsidiaries, parent companies, predecessors, and successors, and all of their respective past and present officers, directors, stockholders, partners, members, employees, agents, representatives, plan administrators, attorneys, insurers and fiduciaries (each in their individual and corporate capacities) (collectively, the "Released Parties") from any and all claims, charges, complaints, demands, actions, causes of action, suits, rights, debts, sums of money, costs, accounts, reckonings, covenants, contracts, agreements, promises, doings, omissions, damages, executions, obligations, liabilities, and expenses (including attorneys' fees and costs), of every kind and nature that you ever had or now have against any or all of the Released Parties, including, but not limited to, any and all claims arising out of or relating to your employment with and/or separation from the Company, including, but not limited to, all claims under Title VII of the Civil Rights Act of 1964, 42 U.S.C. § 2000e et seq., the Americans With Disabilities Act of 1990, 42 U.S.C. § 12101 et seq., the Age Discrimination in Employment Act, 29 U.S.C. § 621 et seq., the Genetic Information Nondiscrimination Act of 2008, 42 U.S.C. § 2000ff et seq., the Family and Medical Leave Act, 29 U.S.C. § 2601 et seq., the Worker Adjustment and Retraining Notification Act ("WARN"), 29 U.S.C. § 2101 et seq., the Rehabilitation Act of 1973, 29 U.S.C. § 701 et seq., Executive Order 11246, Executive Order 11141, the Fair Credit Reporting Act, 15 U.S.C. § 1681 et seq., and the Employee Retirement Income Security Act of 1974 ("ERISA"), 29 U.S.C. § 1001 et seq., all as amended; all claims arising out of the New Jersey Law Against Discrimination, N.J. Stat. Ann. § 10:5-1 et seq., the New Jersey Family Leave Act, N.J. Stat. Ann. § 34:11B-1 et seq., the New Jersey Conscientious Employee Protection Act, N.J. Stat. Ann. § 34:19-1 et seq., and the N.J. Stat. Ann. § 34:11-56.1 et seq. (New Jersey equal pay law), all as amended; all common law claims including, but not limited to, actions in defamation, intentional infliction of emotional distress, misrepresentation, fraud, wrongful discharge, and breach of contract, including without limitation, all claims arising from the Employment Agreement; all state and federal whistleblower claims to the maximum extent permitted by law; all claims to any non-vested ownership interest in the Company, contractual or otherwise; and any claim or damage arising out of your employment with and/or separation from the Company (including a claim for retaliation) under any common law theory or any federal, state or local statute or ordinance not expressly referenced above; provided, however, that nothing in this letter agreement shall (i) prevent you from filing a charge with, cooperating with, or participating in any proceeding before the Equal Employment Opportunity Commission or a state fair

employment practices agency (except that you acknowledge that you may not recover any monetary benefits in connection with any such claim, charge or proceeding) or (ii) deprive you of any rights you may have to be indemnified by the Company as provided in any agreement between the Company and you or pursuant to the Company's Certificate of Incorporation or by-laws.

3. **Non-Disclosure, Non-Competition and Non-Solicitation** – You acknowledge and reaffirm your obligation to keep confidential and not disclose all non-public information concerning the Company and its clients that you acquired during the course of your employment with the Company, as stated more fully in Section 5 of the Employment Agreement, which remains in full force and effect.

4. **Return of Company Property** – You confirm that you have returned to the Company all keys, files, records (and copies thereof), equipment (including, but not limited to, computer hardware, software and printers, wireless handheld devices, cellular phones, smartphones, tablets, etc.), Company identification, and any other Company-owned property in your possession or control and have left intact all electronic Company documents, including but not limited to those which you developed or helped to develop during your employment. You further confirm that you have cancelled all accounts for your benefit, if any, in the Company's name, including but not limited to, credit cards, telephone charge cards, cellular phone and/or wireless data accounts and computer accounts.

5. **Business Expenses and Final Compensation** – You acknowledge that you have been reimbursed by the Company for all business expenses incurred in conjunction with the performance of your employment and that no other reimbursements are owed to you. You further acknowledge that you have received payment in full for all services rendered in conjunction with your employment by the Company, including payment for all wages, bonuses and accrued, unused vacation time, and that no other compensation is owed to you except as provided herein.

6. **Non-Disparagement** – To the extent permitted by law, you understand and agree that as a condition for payment to you of the Severance Compensation herein described, for a period of five years following the date hereof you shall not make any false, disparaging or derogatory statements to any person or entity, including any media outlet, regarding the Company or any of its directors, officers, employees, agents or representatives or about the Company's business affairs and financial condition. Further, for a period of five years following the date hereof, neither the Company, nor any of its executive officers or members of its Board will directly or indirectly make, or cause to be made, any false statement, observation or opinion, disparaging your reputation.

7. **Continued Assistance** - You agree that after the Termination Date you will provide all reasonable cooperation to the Company, including but not limited to, assisting the Company transition your job duties, assisting the Company in defending against and/or prosecuting any litigation or threatened litigation, and performing any other tasks as reasonably requested by the Company.

8. **Cooperation** – To the extent permitted by law, you agree to cooperate fully with the Company in the defense or prosecution of any claims or actions which already have been brought, are currently pending, or which may be brought in the future against or on behalf of the Company**,** whether before a state or federal court, any state or federal government agency, or a mediator or arbitrator. Your full cooperation in connection with such claims or actions shall include, but not be limited to, being available to meet with counsel to prepare its claims or defenses, to prepare for trial or discovery or an administrative hearing or a mediation or arbitration and to act as a witness when requested by the Company at reasonable times designated by the Company. You agree that you will notify the Company promptly in the event that you are served with a subpoena or in the event that you are asked to provide a third party with information concerning any actual or potential complaint or claim against the Company.

9. **Amendment and Waiver** – This letter agreement shall be binding upon the parties and may not be modified in any manner, except by an instrument in writing of concurrent or subsequent date signed by duly authorized representatives of the parties hereto. This letter agreement is binding upon and shall inure to the benefit of the parties and their respective agents, assigns, heirs, executors, successors and administrators. No delay or omission by the Company in exercising any right under this letter agreement shall operate as a waiver of that or any other right. A waiver or consent given by the Company on any one occasion shall be effective only in that instance and shall not be construed as a bar to or waiver of any right on any other occasion.

10. **Validity** – Should any provision of this letter agreement be declared or be determined by any court of competent jurisdiction to be illegal or invalid, the validity of the remaining parts, terms or provisions shall not be affected thereby and said illegal or invalid part, term or provision shall be deemed not to be a part of this letter agreement.

11. **Confidentiality** – To the extent permitted by law, you understand and agree that as a condition for payment to you of the Severance Compensation herein described, the terms and contents of this letter agreement, and the contents of the negotiations and discussions resulting in this letter agreement, shall be maintained as confidential by you and your agents and representatives and shall not be disclosed except to the extent required by federal or state law or as otherwise agreed to in writing by the Company.

12. **Nature of Agreement** – You understand and agree that this letter agreement is a severance agreement and does not constitute an admission of liability or wrongdoing on the part of the Company.

13. **Acknowledgments –** You acknowledge that you have been given at least twenty-one (21) days to consider this letter agreement, and that the Company advised you to consult with an attorney of your own choosing prior to signing this letter agreement. You understand that you may revoke this letter agreement for a period of seven (7) days after you sign this letter agreement by notifying me in writing, and the letter agreement shall not be effective or enforceable until the expiration of this seven (7) day revocation period. You understand and agree that by entering into this agreement, you are waiving any and all rights or claims you might have under the Age Discrimination in Employment Act, as amended by the Older Workers Benefits Protection Act, and that you have received consideration beyond that to which you were previously entitled.

14. **Voluntary Assent** – You affirm that no other promises or agreements of any kind have been made to or with you by any person or entity whatsoever to cause you to sign this letter agreement, and that you fully understand the meaning and intent of this letter agreement. You state and represent that you have had an opportunity to fully discuss and review the terms of this letter agreement with an attorney. You further state and represent that you have carefully read this letter agreement, understand the contents herein, freely and voluntarily assent to all of the terms and conditions hereof and sign your name of your own free act.

15. **Applicable Law** – This letter agreement shall be interpreted and construed by the laws of the State of New Jersey, without regard to conflict of laws provisions. You hereby irrevocably submit to and acknowledge and recognize the jurisdiction of the courts of the State of New Jersey, or if appropriate, a federal court located in the State of New Jersey (which courts, for purposes of this letter agreement, are the only courts of competent jurisdiction), over any suit, action or other proceeding arising out of, under or in connection with this letter agreement or the subject matter hereof.

16. **Entire Agreement** – This letter agreement contains and constitutes the entire understanding and agreement between the parties hereto with respect to your Severance Compensation and the settlement of claims against the Company and cancels all previous oral and written negotiations, agreements and commitments in connection therewith. Nothing in this paragraph, however, shall modify, cancel or supersede your obligations set forth in paragraph 3 herein.

17. **Tax Acknowledgement** – In connection with the payments and consideration provided to you pursuant to this letter agreement, the Company shall withhold and remit to the tax

authorities the amounts required under applicable law, and you shall be responsible for all applicable taxes with respect to such payments and consideration under applicable law. You acknowledge that you are not relying upon the advice or representation of the Company with respect to the tax treatment of any of the Severance Compensation set forth in Section 4 of the Employment Agreement.

If you have any questions about the matters covered in this letter agreement, please call me at **[Insert Phone Number]**.

Very truly yours,

By: _____
[Name]
[Title]

I hereby agree to the terms and conditions set forth above. I have been given at least twenty-one (21) days to consider this letter agreement and I have chosen to execute this on the date below. I intend that this letter agreement will become a binding agreement between me and the Company if I do not revoke my acceptance in seven (7) days.

_____ _____
[Insert Employee Name] Date

To be returned to me by **[Return Date – e.g., 21 days from date of receipt of this letter]**.

April 10, 2020

Eric Pauwels
76 Lemoyne Lane
Johns Island, SC 29455

Dear Eric,

Congratulations on your proposed appointment to Chief Business Officer of PTC Therapeutics, Inc. Your success with PTC has been impressive and we look forward to your continued contributions to PTC's business and mission. The effective date of your appointment is the date that your appointment is approved by the Board of Directors of PTC following receipt of a signed copy of this letter from you indicating your acceptance of your appointment. In this new role you will report to our CEO and Founder, Stuart Peltz.

Outlined below are details of your appointment:

- Your annual base salary will be increased to $490,000 annually, subject to deductions for taxes and other withholdings as required by law. Please allow 1 to 2 pay periods for your new base salary to be reflected in your paycheck.

- Your bonus target will increase to 45.00% of your annual salaried earnings paid in accordance with the terms of conditions of PTC's annual incentive compensation plan.

- You will receive a one-time grant of 50,000 stock options to purchase shares of common stock of PTC, and 10,000 restricted stock units**,** subject to formal approval by the Compensation Committee of the Board of Directors (or a majority of PTC's independent directors) and to the terms of the applicable grant agreements. The options will vest over four years, with 25% vesting on the one-year anniversary of your grant date and 6.25% vesting every three-month period thereafter over the following three years. The restricted stock units will vest over four years, with 25% vesting annually on the anniversary of the grant date.

- This letter supersedes any other recent discussions or communications from PTC with respect to changes in your pay, title, role in the organization, or equity grants. All other terms of your employment with PTC will remain consistent with existing signed employment and equity agreements and applicable policies.

On behalf of PTC, let me again congratulate you on your proposed appointment. Please return a signed copy of this letter to me by close of business on Monday, April 13th, 2020. Feel free to contact me if you have any questions concerning this letter.

Sincerely,
/s/ Martin Rexroad

Martin Rexroad
SVP, Human Resources

Cc: Stuart Peltz

Accepted by:
/s/ Eric Pauwels

Eric Pauwels
April 12, 2020
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: August 5, 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: August 5, 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 June 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: August 5, 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 June 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: August 5, 2020

By: /s/ EMILY HILL

Emily Hill
Chief Financial Officer
(Principal Financial Officer)