

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

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**FORM 10-Q**

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(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 001-36310

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**CONCERT PHARMACEUTICALS, INC.**

(Exact name of registrant as specified in its charter)

**Delaware**

(State or other jurisdiction of  
incorporation or organization)

20-4839882

(I.R.S. Employer  
Identification No.)

65 Hayden Avenue, Suite 3000N 02421  
Lexington, Massachusetts (Zip Code)  
(Address of principal executive offices)

(781) 860-0045

(Registrant's telephone number, including area code)

(Former name, former address and former fiscal year, if changed since last report)

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

<u>Title of each class</u>	<u>Trading Symbol(s)</u>	<u>Name of each exchange on which registered</u>
Common Stock, par value \$0.001 per share	CNCE	Nasdaq Global 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.

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Large accelerated filer

Non-accelerated filer

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

The number of shares outstanding of the registrant's common stock as of July 31, 2020: 29,678,072

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## REFERENCES TO CONCERT

Throughout this Quarterly Report on Form 10-Q, “Concert,” “the Company,” “we,” “us” and “our,” except where the context requires otherwise, refer to Concert Pharmaceuticals, Inc. and its consolidated subsidiary, and “our board of directors” refers to the board of directors of Concert Pharmaceuticals, 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, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “will,” “would” 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:

- ongoing and planned clinical trials for our product candidates, whether conducted by us or by our collaborators, including the timing of initiation, enrollment and completion of these trials and of the anticipated results;
- our plans to identify, develop and commercialize novel small molecule drugs based on our knowledge of deuterium chemistry;
- our plans to enter into collaborations for the development and commercialization of product candidates;
- our expected benefits from our current and any future collaboration, development or license arrangements;
- our ability to receive research and development funding and achieve anticipated milestones under our collaborations;
- our expectations regarding any future milestone payments we may receive as part of our asset purchase agreement with Vertex Pharmaceuticals, Inc. with respect to VX-561 and payments from our other collaboration and license arrangements;
- the timing of and our ability to obtain and maintain marketing approvals for our product candidates;
- the rate and degree of market acceptance and clinical utilization of our products;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our intellectual property position and strategy;
- the outcome of our *inter partes* review proceeding regarding U.S. Patent No. 9,249,149 covering CTP-543;
- our freedom to operate with respect to third-party patents;
- our expectations regarding our DCE Platform<sup>®</sup> and the potential advantages of our product candidates;
- our estimates regarding expenses, future revenue, capital requirements and needs for additional financing;
- risks associated with the COVID-19 pandemic, which may adversely impact our business, clinical trials and supply chain;
- developments relating to our competitors and our industry; and
- the impact of government laws and regulations.

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, that 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, collaborations or investments that 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 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 law.

**PART I. FINANCIAL INFORMATION**

**Item 1. Financial Statements.**

**CONCERT PHARMACEUTICALS, INC.**  
**CONDENSED CONSOLIDATED BALANCE SHEETS (UNAUDITED)**  
(Amounts in thousands, except share and per share data)

	June 30, 2020	December 31, 2019
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 27,094	\$ 53,043
Investments, available for sale	117,623	53,395
Marketable equity securities	2,688	5,375
Interest receivable	244	260
Deferred offering costs	43	143
Accounts receivable	50	72
Income taxes receivable, current	2,446	—
Prepaid expenses and other current assets	4,397	4,567
<b>Total current assets</b>	<b>154,585</b>	<b>116,855</b>
Property and equipment, net	7,070	7,753
Restricted cash	1,157	1,157
Other assets	69	96
Income taxes receivable	—	2,358
Operating lease right-of-use assets, long-term	9,118	9,252
<b>Total assets</b>	<b>\$ 171,999</b>	<b>\$ 137,471</b>
<b>Liabilities and stockholders' equity</b>		
Current liabilities:		
Accounts payable	\$ 352	\$ 881
Accrued expenses and other liabilities	7,022	8,336
Deferred revenue, current portion	1,413	7,783
Lease liability, current portion	831	268
<b>Total current liabilities</b>	<b>9,618</b>	<b>17,268</b>
Deferred revenue, net of current portion	2,750	2,750
Lease liability, net of current portion	15,546	15,996
<b>Total liabilities</b>	<b>27,914</b>	<b>36,014</b>
Commitments (Note 11)		
Stockholders' equity:		
Preferred stock, \$0.001 par value per share; 5,000,000 shares authorized; no shares issued and outstanding as of June 30, 2020 and December 31, 2019	—	—
Common stock, \$0.001 par value per share; 100,000,000 shares authorized; 29,878,673 and 24,065,676 shares issued and 29,678,072 and 23,865,075 shares outstanding as of June 30, 2020 and December 31, 2019, respectively	29	24
Additional paid-in capital	372,029	296,145
Accumulated other comprehensive gain (loss)	176	(31)
Accumulated deficit	(228,149)	(194,681)
<b>Total stockholders' equity</b>	<b>144,085</b>	<b>101,457</b>
<b>Total liabilities and stockholders' equity</b>	<b>\$ 171,999</b>	<b>\$ 137,471</b>

See accompanying notes.

**CONCERT PHARMACEUTICALS, INC.**  
**CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS**  
**(UNAUDITED)**  
(Amounts in thousands, except per share data)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Revenue:				
License and research and development revenue	6,387	49	6,394	1,054
Operating expenses:				
Research and development	14,788	14,496	28,774	30,286
General and administrative	4,731	4,978	9,403	10,587
Total operating expenses	19,519	19,474	38,177	40,873
Loss from operations	(13,132)	(19,425)	(31,783)	(39,819)
Investment income	355	883	918	1,750
Unrealized loss on marketable equity securities	(299)	(126)	(2,688)	(2,425)
Loss before income taxes	(13,076)	(18,668)	(33,553)	(40,494)
Income tax benefit	(85)	—	(85)	—
Net loss	\$ (12,991)	\$ (18,668)	\$ (33,468)	\$ (40,494)
Other comprehensive income:				
Unrealized (loss) gain on investments, available for sale	(316)	44	207	141
Comprehensive loss	\$ (13,307)	\$ (18,624)	\$ (33,261)	\$ (40,353)
Net loss per share applicable to common stockholders - basic and diluted	\$ (0.41)	\$ (0.78)	\$ (1.11)	\$ (1.71)
Weighted-average number of common shares used in net loss per share applicable to common stockholders - basic and diluted	31,455	23,790	30,283	23,650

See accompanying notes.

**CONCERT PHARMACEUTICALS, INC.**  
**CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY (UNAUDITED)**

**Six Months Ended June 30, 2020**

	Common Stock			Additional paid-in capital	Accumulated other comprehensive income	Accumulated deficit	Total stockholders' equity
	Issued	In Treasury	Amount				
	(in thousands)						
Balance at December 31, 2019	24,066	200	\$ 24	\$ 296,145	\$ (31)	\$ (194,681)	\$ 101,457
Exercise of stock options	51	—	—	435	—	—	435
Unrealized gain on short-term investments	—	—	—	—	523	—	523
Stock-based compensation expense	—	—	—	2,477	—	—	2,477
Sale of common stock and pre-funded warrants, net of underwriters' discount and costs	5,735	—	5	70,059	—	—	70,064
Net loss	—	—	—	—	—	(20,477)	(20,477)
Balance at March 31, 2020	29,852	200	\$ 29	\$ 369,116	\$ 492	\$ (215,158)	\$ 154,479
Exercise of stock options	27	—	—	109	—	—	109
Unrealized loss on short-term investments	—	—	—	—	(316)	—	(316)
Stock-based compensation expense	—	—	—	2,804	—	—	2,804
Net loss	—	—	—	—	—	(12,991)	(12,991)
Balance at June 30, 2020	29,879	200	\$ 29	\$ 372,029	\$ 176	\$ (228,149)	\$ 144,085

**CONCERT PHARMACEUTICALS, INC.**  
**CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY (UNAUDITED)**

**Six Months Ended June 30, 2019**

	Common Stock			Additional paid-in capital	Accumulated other comprehensive income	Accumulated deficit	Total stockholders' equity
	Issued	In Treasury	Amount				
	(in thousands)						
Balance at December 31, 2018	23,519	81	\$ 23	\$ 284,369	\$ (137)	\$ (116,515)	\$ 167,740
Exercise of stock options	154	47	—	805	—	—	805
Release of restricted stock units	202	61	—	(741)	—	—	(741)
Unrealized gain on short-term investments	—	—	—	—	97	—	97
Stock-based compensation expense	—	—	—	2,929	—	—	2,929
Exercise of stock warrants	71	—	—	1,000	—	—	1,000
Offering expenses incurred	—	—	—	(206)	—	—	(206)
Net loss	—	—	—	—	—	(21,826)	(21,826)
Balance at March 31, 2019	23,946	189	\$ 23	\$ 288,156	\$ (40)	\$ (138,341)	\$ 149,798
Exercise of stock options	40	—	—	165	—	—	165
Unrealized gain on short-term investments	—	—	—	—	44	—	44
Stock-based compensation expense	—	—	—	2,362	—	—	2,362
Net loss	—	—	—	—	—	(18,668)	(18,668)
Balance at June 30, 2019	23,986	189	\$ 23	\$ 290,683	\$ 4	\$ (157,009)	\$ 133,701

See accompanying notes.

**CONCERT PHARMACEUTICALS, INC.**  
**CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS (UNAUDITED)**  
(Amounts in thousands)

	Six Months Ended June 30,	
	2020	2019
<b>Operating activities</b>		
Net loss	\$ (33,468)	(40,494)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	805	849
Stock-based compensation expense	5,281	5,291
Accretion of premiums and discounts on investments	(114)	(606)
Unrealized loss on marketable equity securities	2,688	2,425
Loss on disposal of asset	4	4
Non-cash lease expense	133	102
Changes in operating assets and liabilities:		
Accounts receivable	22	(23)
Deferred offering costs	(30)	—
Interest receivable	16	120
Prepaid expenses and other current assets	170	(365)
Contract asset	—	16,000
Other assets	27	(20)
Accounts payable	(529)	(508)
Accrued expenses and other liabilities	(1,184)	(83)
Income taxes receivable	(88)	(36)
Income taxes payable	—	(390)
Deferred revenue	(6,370)	—
Operating lease liability	113	(291)
Net cash used in operating activities	(32,524)	(18,025)
<b>Investing activities</b>		
Purchases of property and equipment	(126)	(472)
Purchases of investments	(145,152)	(49,208)
Maturities of investments	81,245	92,576
Net cash (used in) provided by investing activities	(64,033)	42,896
<b>Financing activities</b>		
Proceeds from exercises of stock options	544	970
Proceeds from exercise of warrants	—	1,000
Repurchase of common stock pursuant to share surrender	—	(741)
Proceeds from common stock and pre-funded warrants sold, net of underwriters' discount and costs	70,064	(156)
Net cash provided by financing activities	70,608	1,073
Net (decrease) increase in cash and cash equivalents and restricted cash	(25,949)	25,944
Cash, cash equivalents and restricted cash at beginning of period	54,200	18,927
Cash, cash equivalents and restricted cash at end of period	\$ 28,251	\$ 44,871
Supplemental cash flow information:		
Cash paid for income taxes	\$ —	\$ 444
Purchases of property and equipment unpaid at period end	\$ —	\$ 24
Public offering costs unpaid at period end	\$ 13	\$ 50
Cash paid included in measurement of lease liabilities	\$ 965	\$ 1,388
Pre-funded stock warrants issued	\$ 16,736	\$ —

See accompanying notes.

## **1. Nature of Business**

Concert Pharmaceuticals, Inc., or the Company, was incorporated on April 12, 2006 as a Delaware corporation and has its operations based in Lexington, Massachusetts. The Company is a clinical stage biopharmaceutical company that is developing small molecule drugs that it discovered through the application of its deuterated chemical entity platform, or DCE Platform<sup>®</sup>. Selective incorporation of deuterium into known molecules has the potential, on a case-by-case basis, to provide better pharmacokinetic or metabolic properties, thereby enhancing their clinical safety, tolerability or efficacy. The Company's pipeline consists of clinical stage candidates targeting autoimmune and central nervous system, or CNS, disorders, and a number of preclinical compounds that it is currently assessing.

The Company had cash and cash equivalents and investments of \$144.7 million as of June 30, 2020. The Company believes that its cash and cash equivalents and investments as of June 30, 2020 will be sufficient to allow the Company to fund its current operating plan for at least the next twelve months from the date of issuance of the financial statements. The Company may pursue additional cash resources through public or private financings, by establishing collaborations with or licensing its technology to other companies and through other arrangements.

Since its inception, the Company has generated an accumulated deficit of \$228.1 million through June 30, 2020. The Company's operating results may fluctuate significantly from year to year, depending on the timing and magnitude of clinical trial and other development activities under its current development programs. Substantially all of the Company's net losses have resulted from costs incurred in connection with its research and development programs and from general and administrative costs associated with its operations. The Company expects to continue to incur significant expenses and increasing operating losses for at least the next several years.

The Company is subject to risks common to companies in the biotechnology industry, including, but not limited to, risks of failure or unsatisfactory results of nonclinical studies and clinical trials, the need to obtain additional financing to fund the future development of its pipeline, the need to obtain marketing approval for its product candidates, the need to successfully commercialize and gain market acceptance of its product candidates, dependence on key personnel, protection of proprietary technology, compliance with government regulations, development by competitors of technological innovations and ability to transition from pilot-scale manufacturing to large-scale production of products.

Unless otherwise indicated, all amounts in the following tables are in thousands except share and per share amounts.

## **2. Basis of Presentation and Significant Accounting Policies**

### *Basis of Presentation*

The condensed consolidated financial statements have been prepared in accordance with generally accepted accounting principles for interim financial information and with the instructions to Form 10-Q and Article 10 of Regulation S-X. Accordingly, they do not include all of the information and footnotes required by generally accepted accounting principles for complete financial statements. In the opinion of management, all adjustments, consisting of normal recurring accruals and revisions of estimates, considered necessary for a fair presentation of the condensed consolidated financial statements have been included. Interim results for the six months ended June 30, 2020 are not necessarily indicative of the results that may be expected for the fiscal year ending December 31, 2020 or any other future period.

The accompanying condensed consolidated financial statements reflect the accounts of the Company and its subsidiaries. All intercompany transactions between the Company and its subsidiaries have been eliminated. Management has determined that the Company operates in one segment: the development of pharmaceutical products on its own behalf or in collaboration with others. The information included in this Quarterly Report on Form 10-Q should be read in conjunction with the Company's consolidated financial statements and the accompanying notes included in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2019 filed with the Securities and Exchange Commission, or SEC, on February 27, 2020.

### *Use of Estimates and Summary of Significant Accounting Policies*

The preparation of the consolidated financial statements in conformity with U.S. generally accepted accounting principles, or GAAP, requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, equity, revenue, expenses and the disclosure of contingent assets and liabilities and the Company's ability to continue as a going concern. In preparing the consolidated financial statements, management used estimates in the following areas, among others: revenue recognition; prepaid and accrued research and development expenses; stock-based compensation expense; and the

**CONCERT PHARMACEUTICALS, INC.**  
**NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (UNAUDITED)**

evaluation of the existence of conditions and events that raise substantial doubt regarding the Company's ability to continue as a going concern. Actual results could differ from those estimates.

During the six months ended June 30, 2020, there have been no material changes to the significant accounting policies previously disclosed in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2019.

*Recently Adopted Accounting Pronouncements*

In August 2018, the Financial Accounting Standards Board, or FASB, issued Accounting Standards Update, or ASU, 2018-15, *Intangible-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*. This standard aligns the requirements for capitalizing implementation costs in a cloud computing arrangement service contract with the requirements for capitalizing implementation costs incurred for internal-use software. The new guidance also prescribes the balance sheet, income statement and cash flow classification of the capitalized implementation costs and related amortization expense, and requires additional quantitative and qualitative disclosures. ASU 2018-15 is effective for fiscal years beginning after December 15, 2019, and early adoption is permitted. The Company adopted this new standard effective January 1, 2020, on a prospective basis, and it did not have a material effect on the consolidated financial statements and related disclosures.

In December 2019, the FASB issued ASU 2019-12, *Income Taxes (Topic 740): Simplifying the Accounting for Income Taxes*. This standard includes removal of certain exceptions to the general principles of Accounting Standards Codification 740, *Income Taxes*, and simplification in several other areas. ASU 2019-12 is effective for public business entities for annual reporting periods beginning after December 15, 2020, and interim periods within those reporting periods, and early adoption is permitted. The Company adopted this new standard effective January 1, 2020, and it did not have a material effect on the consolidated financial statements and related disclosures. For a detailed discussion of the adoption of ASU 2019-12, refer to Note 7.

*Pending Accounting Pronouncements*

In June 2016, the FASB issued ASU 2016-13, *Financial Instruments-Credit Losses*. This standard requires entities to measure all expected credit losses for financial assets held at the reporting date based on historical experience, current conditions and reasonable and supportable forecasts. As a smaller reporting company, ASU 2016-13 will become effective for the Company for fiscal years beginning after December 15, 2022, and early adoption is permitted. The Company is currently evaluating the impact that ASU 2016-13 will have on its financial statements and related disclosures.

### **3. Fair Value Measurements**

The Company has certain financial assets and liabilities that are recorded at fair value which have been classified as Level 1, 2 or 3 within the fair value hierarchy as described in the accounting standards for fair value measurements:

- Level 1—quoted prices for identical instruments in active markets;
- Level 2—quoted prices for similar instruments in active markets, quoted prices for identical or similar instruments in markets that are not active and model-derived valuations in which all significant inputs and significant value drivers are observable in active markets; and
- Level 3—valuations derived from valuation techniques in which one or more significant value drivers are unobservable.

The tables below present information about the Company's financial assets and liabilities that are measured and carried at fair value as of June 30, 2020 and December 31, 2019 and indicate the level within the fair value hierarchy where each measurement is classified. The carrying amounts reflected in the condensed consolidated balance sheets for cash, prepaid expenses and other current assets, restricted cash, accounts payable and accrued expenses approximate their fair value due to their short-term nature.

**CONCERT PHARMACEUTICALS, INC.**  
**NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (UNAUDITED)**

	Level 1	Level 2	Level 3	Total
<b>June 30, 2020</b>				
Cash equivalents:				
Money market funds	\$ 16,926	\$ —	\$ —	\$ 16,926
Investments, available for sale:				
U.S. Treasury obligations	48,724	—	—	48,724
Government agency securities	35,913	32,987	—	68,900
Marketable equity securities:				
Corporate equity securities (Note 8)	2,688	—	—	2,688
<b>Total</b>	<b>\$ 104,251</b>	<b>\$ 32,987</b>	<b>\$ —</b>	<b>\$ 137,238</b>

	Level 1	Level 2	Level 3	Total
<b>December 31, 2019</b>				
Cash equivalents:				
Money market funds	\$ 40,782	\$ —	\$ —	\$ 40,782
Government agency securities	—	2,000	—	2,000
Investments, available for sale:				
U.S. Treasury obligations	34,499	—	—	34,499
Government agency securities	10,997	7,899	—	18,896
Marketable equity securities:				
Corporate equity securities (Note 8)	5,375	—	—	5,375
<b>Total</b>	<b>\$ 91,653</b>	<b>\$ 9,899</b>	<b>\$ —</b>	<b>\$ 101,552</b>

**4. Cash, Cash Equivalents, Investments and Marketable Equity Securities**

Cash equivalents include all highly liquid investments maturing within 90 days from the date of purchase. Investments consist of securities with original maturities greater than 90 days when purchased. The Company classifies these investments as available-for-sale and records them at fair value in the accompanying consolidated balance sheets. Unrealized gains or losses from equity securities are included in net income. Unrealized gains or losses from other investments, including debt securities, are included in accumulated other comprehensive income (loss). Premiums or discounts from par value are amortized to investment income over the life of the underlying investment.

Cash, cash equivalents, available for sale investments and marketable equity securities included the following as of June 30, 2020 and December 31, 2019:

	Average Maturity	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
<b>June 30, 2020</b>					
Cash		\$ 10,168	\$ —	\$ —	\$ 10,168
Money market funds		16,926	—	—	16,926
Cash and cash equivalents		<u>\$ 27,094</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 27,094</u>
U.S. Treasury obligations	169 days	\$ 48,583	\$ 142	\$ (1)	\$ 48,724
Government agency securities	156 days	68,788	112	(1)	68,899
Investments, available for sale		<u>\$ 117,371</u>	<u>\$ 254</u>	<u>\$ (2)</u>	<u>\$ 117,623</u>
<b>June 30, 2020</b>					
Marketable equity securities (Note 8)		<u>\$ 10,451</u>	<u>\$ —</u>	<u>\$ (7,763)</u>	<u>\$ 2,688</u>

**CONCERT PHARMACEUTICALS, INC.**  
**NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (UNAUDITED)**

	Average Maturity	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
<b>December 31, 2019</b>					
Cash		\$ 10,261	\$ —	\$ —	\$ 10,261
Money market funds		40,782	—	—	40,782
Government agency securities	8 days	2,000	—	—	2,000
Cash and cash equivalents		<u>\$ 53,043</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 53,043</u>
U.S. Treasury obligations	108 days	\$ 34,475	\$ 24	\$ —	\$ 34,499
Government agency securities	74 days	18,874	22	—	18,896
Investments, available for sale		<u>\$ 53,349</u>	<u>\$ 46</u>	<u>\$ —</u>	<u>\$ 53,395</u>

	Acquisition Value	Unrealized Gains	Unrealized Losses	Fair Value
<b>December 31, 2019</b>				
Marketable equity securities (Note 8)	\$ 10,451	\$ —	\$ (5,076)	\$ 5,375

Although available to be sold to meet operating needs or otherwise, securities are generally held through maturity. The Company classifies all investments as current assets, as these assets are readily available for use in current operations. The cost of securities sold is determined based on the specific identification method for purposes of recording realized gains and losses. During 2020 and 2019, there were no realized gains or losses on sales of investments, and no investments were adjusted other than for temporary declines in fair value.

#### 5. Restricted Cash

Restricted cash as of June 30, 2020 and 2019 was held as collateral for stand-by letters of credit issued by the Company to its landlord in connection with the current lease for its principal facilities located at 65 Hayden Avenue, Lexington, Massachusetts. For additional information regarding the Company's lease, refer to Note 11. Cash, cash equivalents and restricted cash consisted of the following as of June 30, 2020 and 2019:

	June 30, 2020	June 30, 2019
Cash and cash equivalents	\$ 27,094	\$ 43,714
Restricted cash	1,157	1,157
Total cash, cash equivalents and restricted cash shown in the statements of cash flows	<u>\$ 28,251</u>	<u>\$ 44,871</u>

#### 6. Accrued Expenses and Other Liabilities

Accrued expenses and other liabilities consisted of the following as of June 30, 2020 and December 31, 2019:

	June 30, 2020	December 31, 2019
Accrued professional fees and other	\$ 514	\$ 862
Employee compensation and benefits	1,741	3,222
Research and development expenses	4,767	4,252
Total	<u>\$ 7,022</u>	<u>\$ 8,336</u>

#### 7. Income Taxes

Deferred tax assets and deferred tax liabilities are recognized based on temporary differences between the financial reporting and tax basis of assets and liabilities using statutory rates. A valuation allowance is recorded against deferred tax assets if it is more likely than not that some or all of the deferred tax assets will not be realized.

The Company's ability to use its operating loss carryforwards and tax credits to offset future taxable income is subject to restrictions under Sections 382 and 383 of the U.S. Internal Revenue Code. Net operating loss and tax credit carryforwards may

become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant stockholders over a three-year period in excess of 50%, as defined under Sections 382 and 383 of the Internal Revenue Code. Such changes would limit the Company's use of its operating loss carryforwards and tax credits. In such a situation, the Company may be required to pay income taxes, even though significant operating loss carryforwards and tax credits exist.

The Company records a provision or benefit for income taxes on ordinary pre-tax income or loss based on its estimated effective tax rate for the year. As of June 30, 2020, the Company forecasts an ordinary pre-tax loss for the year ended December 31, 2020 and, since it maintains a full valuation allowance on its deferred tax assets, the Company did not record an income tax benefit relating to this period. During the six months ended June 30, 2020, the Company recorded a benefit for income taxes of \$85 thousand for interest accrued in a prior period under the installment sales method for the sale of CTP-656 to Vertex Pharmaceuticals, Inc., or Vertex.

The Company adopted ASU 2019-12, *Income Taxes (Topic 740): Simplifying the Accounting for Income Taxes*, effective January 1, 2020. Under ASU 2019-12, the Company, having a full valuation and a loss in continuing operations, will no longer include the impacts of items in other comprehensive income in determining intra-period allocation of tax expense for continuing operations. Under ASU 2019-12, the Company can apply this change to intra-period tax allocation on a prospective basis. For the six months ended June 30, 2020, the Company applied the tax allocation rules of ASU 2019-12 to the \$207 thousand of unrealized gains on available for sale investments recognized in other comprehensive income, which did not have a material impact on the consolidated financial statements or related disclosures.

## **8. Revenue**

The Company's revenue is generated through collaborative licensing agreements, patent assignments and sales of intellectual property. The Company generates its revenue through one segment. The revenue recognized under each of the Company's arrangements during the current and prior periods is described below.

### ***Contract Assets***

In February 2019, the Company received \$16.0 million that had previously been held in escrow for indemnification purposes related to the asset purchase by Vertex. The Company did not have a contract asset as of June 30, 2020 or December 31, 2019.

### ***Contract Liabilities***

As of June 30, 2020 and December 31, 2019, the Company had \$4.2 million and \$10.6 million, respectively, in contract liabilities related to unsatisfied performance obligations as well as variable consideration paid in advance but currently constrained from recognition.

The contract liabilities as of June 30, 2020 consisted of the following deferred revenue:

- \$1.4 million related to the Company's collaboration with Celgene Corporation, or Celgene, which is attributable to research and development activities associated with the CTP-730 program; and
- \$2.8 million related to a payment received from GlaxoSmithKline that the Company will not recognize as revenue until all repayment obligations lapse.

During the three and six months ended June 30, 2020, the Company recognized \$6.4 million in deferred revenue upon the expiration of two licensing options under the Company's collaboration agreement with Celgene.

### ***Revenue Arrangements***

#### *Vertex*

On March 3, 2017, the Company and Vertex entered into an Asset Purchase Agreement, or the Vertex Agreement, pursuant to which, subject to the satisfaction or waiver of the conditions therein, the Company sold and assigned to Vertex CTP-656, a deuterated analog of ivacaftor, now known as VX-561, and other cystic fibrosis assets of the Company. On July 25, 2017, or the Vertex Closing Date, the transaction contemplated by the Vertex Agreement closed and Vertex paid the Company \$160 million in cash consideration. In addition, Vertex has agreed to pay the Company an aggregate of up to \$90 million upon the achievement of certain milestone events.

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As of December 31, 2018, the Vertex indemnification variable consideration represented a contract asset to be released from escrow 18 months following the Vertex Closing Date and was classified as a current asset in the accompanying consolidated balance sheet. In February 2019, the \$16.0 million that had previously been held in escrow was released to the Company. Additionally, the variable consideration related to the regulatory milestone payments are fully constrained due to the uncertainty associated with the achievement of the respective milestones. Accordingly, no contract asset was recorded as of June 30, 2020 or December 31, 2019.

*Processa*

On October 4, 2017, the Company entered into an Option and License Agreement, or the Option, with Promet Therapeutics, LLC, or Promet, pursuant to which the Company granted Promet an option to obtain an exclusive license to CTP-499, a deuterated analog of 1-(S)-5-hydroxyhexyl-3,7-dimethylxanthine, or HDX, an active metabolite of pentoxifylline, provided certain conditions were met. On October 5, 2017, Promet closed an asset purchase agreement with Heatwux, Inc., a public company, creating Processa Pharmaceuticals, Inc., or Processa.

On March 21, 2018, the Company entered into an Amendment to the Option, or the Amendment, and a Securities Purchase Agreement, with both Promet and Processa. Pursuant to the Amendment, the Company granted Promet, who then assigned to Processa, an exclusive, worldwide, royalty-bearing license to develop, manufacture and commercialize CTP-499, now known as PCS-499. Upon transfer of the license and as consideration for the license, the Company received 2,090,301 shares of common stock of Processa. In December 2019, Processa implemented a reverse stock split, and the Company now owns 298,615 shares of common stock of Processa.

The Company is also eligible to receive royalties on worldwide net sales.

The Amendment contained one performance obligation: an exclusive, worldwide, royalty-bearing license to develop, manufacture and commercialize CTP-499. The Company determined that the transaction price was \$10.5 million, which was based on the fair value of the non-cash consideration received on March 19, 2018, which consisted of 2,090,301 shares of publicly traded common stock of Processa. The transaction price of \$10.5 million was allocated to the single performance obligation. The performance obligation was considered satisfied at contract inception, as the exclusive license transferred control to the customer at this point in time. Accordingly, revenue of \$10.5 million was recognized during the first quarter of 2018.

Subsequent changes to the fair value of the underlying securities are recognized as unrealized gains or losses on marketable equity securities within the condensed consolidated statements of operations and comprehensive loss.

The Amendment contains consideration that is variable based on royalties upon the customer's commercial success with the licensed product. The consideration related to royalty payments is considered variable consideration that is fully constrained in accordance with the royalty recognition constraint. The variable consideration related to royalties will be recognized in the period the products are sold by Processa and the Company has a present right to payment.

For the three and six months ended June 30, 2020, the Company recognized \$9 thousand and \$10 thousand in revenue, respectively, related to intellectual property cost reimbursements. For the three and six months ended June 30, 2019, the Company recognized \$20 thousand and \$22 thousand in revenue, respectively, related to intellectual property cost reimbursements.

*Cipla*

The Company entered into a License Agreement, or the Cipla Agreement, with Cipla Technologies LLC, or Cipla, on January 16, 2019, or the Cipla Closing Date, pursuant to which the Company granted Cipla an exclusive, worldwide, royalty-bearing license to develop, manufacture and commercialize CTP-354, a novel GABA<sub>A</sub> receptor subtype-selective modulator. As consideration for the license, the Company received an upfront payment of \$1.0 million.

The Cipla Agreement also provides Cipla the option to purchase the Company's existing inventory of CTP-354 held as of the Cipla Closing Date, valued in the aggregate at \$0.3 million. Additionally, upon the achievement of certain milestone events, Cipla has agreed to pay the Company an aggregate of up to \$57.0 million. The first milestone payment the Company may be entitled to receive is \$3.0 million when the first investigational new drug application, or IND, for the first CTP-354 product goes into effect.

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Furthermore, the Company is eligible to receive royalties on worldwide net sales of future product sales at defined percentages ranging from the mid-single to high-single digits.

The Cipla Agreement contained one performance obligation: an exclusive, worldwide, royalty-bearing license to develop, manufacture and commercialize CTP-354, referred to as the Transfer of License Performance Obligation. The Company concluded that the option to purchase existing inventory did not provide Cipla a material right, and as such, was treated as a separate contract. The transaction price was determined to be \$1.0 million based on the upfront consideration received as of the Cipla Closing Date.

As of the Cipla Closing Date, the Transfer of License Performance Obligation was satisfied, as the control of CTP-354 transferred to Cipla, the customer. As a result, the full transaction price was recognized as revenue on the Cipla Closing Date. The sale of existing inventory is recognized as goods are transferred to the customer.

The arrangement with Cipla contains consideration that is variable based on the customer's achievement of certain development and regulatory milestones in addition to royalties upon the customer's commercial success with the licensed product. The next milestone payment the Company may be entitled to receive of \$3.0 million related to the first IND for the first CTP-354 product going into effect is considered variable consideration that is fully constrained due to the uncertainty associated with the achievement of the development milestone. The consideration related to royalties is also variable consideration that is fully constrained in accordance with the royalty recognition constraint. The variable consideration related to royalties will be recognized in the period the products are sold by Cipla and the Company has a present right to payment.

The Company did not recognize revenue related to the Cipla Agreement for the three and six months ended June 30, 2020. For the three and six months ended June 30, 2019, the Company recognized \$22 thousand and \$1.0 million, respectively, in revenue associated with the sale of existing inventory and the Transfer of License Performance Obligation.

### **9. Stock-Based Compensation**

The Company's equity incentive plans provide for the issuance of a variety of stock-based awards, including incentive stock options, nonstatutory stock options and awards of stock, to directors, officers and employees of the Company, as well as consultants and advisors to the Company. As of June 30, 2020, the Company has granted awards in the form of stock options and restricted stock units, or RSUs. The stock options generally have been granted with an exercise price equal to the closing market price of the Company's common stock on the date of grant, a vesting period of one, three or four years, and an expiration date no later than ten years from the date of grant.

Effective January 1, 2020, an additional 954,603 shares were added to the Company's 2014 Stock Incentive Plan, or the 2014 Plan, for future issuance pursuant to the terms of the 2014 Plan. As of June 30, 2020, there were 1,159,446 shares of common stock available for future awards under the 2014 Plan.

Total stock-based compensation expense related to all stock-based options and awards recognized in the condensed consolidated statements of operations and comprehensive loss consisted of:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Research and development	\$ 1,416	\$ 1,063	\$ 2,671	\$ 2,538
General and administrative	1,388	1,299	2,610	2,753
Total stock-based compensation expense	\$ 2,804	\$ 2,362	\$ 5,281	\$ 5,291

### **Stock Options**

Stock options are valued using the Black-Scholes-Merton option valuation model, and compensation cost is recognized based on such fair value over the period of vesting. The weighted-average fair value of options granted in the three and six months ended June 30, 2020 and 2019 reflect the following weighted-average assumptions:

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	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Expected volatility	71.00 %	76.89 %	68.58 %	77.13 %
Expected term	6.0 years	6.0 years	6.0 years	6.0 years
Risk-free interest rate	0.42 %	1.82 %	1.33 %	2.22 %
Expected dividend yield	— %	— %	— %	— %

For the three and six months ended June 30, 2019, expected volatility was estimated using a weighted average of the Company's historical volatility of its common stock and the historical volatility of the common stock of a group of similar companies that were publicly traded. For the three and six months ended June 30, 2020, expected volatility was estimated using solely the historical volatility of the Company's common stock because the Company had accumulated sufficient historical pricing data.

The following table provides certain information related to the Company's outstanding stock options:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
	<b>(in thousands, except per share data)</b>			
Weighted-average fair value of options granted, per option	\$ 5.68	\$ 7.41	\$ 6.54	\$ 9.23
Aggregate grant date fair value of options vested during the period	\$ 2,051	\$ 2,394	\$ 4,236	\$ 4,836
Total cash received from exercises of stock options	\$ 109	\$ 165	\$ 544	\$ 970
Total intrinsic value of stock options exercised	\$ 190	\$ 228	\$ 283	\$ 1,108

The following is a summary of stock option activity for the six months ended June 30, 2020:

	Number of Option Shares	Weighted Average Exercise Price per Share	Weighted Average Remaining Contractual Term  (in years)	Aggregate Intrinsic Value  (in thousands)
Outstanding at December 31, 2019	4,112,609	\$ 15.01		
Granted	713,754	\$ 10.63		
Exercised	(77,714)	\$ 7.01		
Forfeited or expired	(57,950)	\$ 19.26		
Outstanding at June 30, 2020	<u>4,690,699</u>	\$ 14.43	7.02	\$ 1,477
Exercisable at June 30, 2020	<u>2,878,717</u>	\$ 14.40	5.98	\$ 1,383
Vested and expected to vest at June 30, 2020 <sup>(1)</sup>	<u>4,555,272</u>	\$ 14.46	6.96	\$ 1,472

<sup>(1)</sup> Represents the number of vested stock option shares as of June 30, 2020, plus the number of unvested stock option shares that the Company estimated as of June 30, 2020 would vest, based on the unvested stock option shares as of June 30, 2020 and an estimated forfeiture rate of 6%.

As of June 30, 2020, there was \$15.5 million of unrecognized compensation cost related to stock options that are expected to vest. The stock option costs are expected to be recognized over a weighted-average remaining vesting period of 2.5 years.

**Restricted Stock Units**

On August 15, 2019, or the 2019 RSU grant date, the Company granted 0.4 million RSUs, or the 2019 RSUs, to certain executives and employees. All of the 2019 RSUs are service-based and vest ratably over two years. On the first anniversary of the 2019 RSU grant date, 35% of the 2019 RSUs will vest. The remainder of the 2019 RSUs will vest on the second anniversary of the 2019 RSU grant date.

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On February 14, 2020, or the 2020 RSU grant date, the Company granted 0.4 million RSUs, or the 2020 RSUs, to certain executives and employees. All of the 2020 RSUs are service-based and vest ratably over three years, with one third of the 2020 RSUs vesting on each anniversary of the 2020 RSU grant date through February 14, 2023.

RSUs are not included in issued and outstanding common stock until the shares have vested and settled. As of June 30, 2020, none of the 2019 RSUs or 2020 RSUs had vested. The fair value of an RSU is measured based on the market price of the underlying common stock as of the date of grant.

The following is a summary of RSU activity for the six months ended June 30, 2020:

	<b>Number of RSUs</b>	<b>Weighted- Average Grant Date Fair Value</b>
Outstanding at December 31, 2019	393,629	\$ 10.27
Granted	409,295	\$ 10.87
Released	—	\$ —
Forfeited	(4,168)	\$ 10.27
Outstanding at June 30, 2020	<u>798,756</u>	<u>\$ 10.58</u>

As of June 30, 2020, there was \$6.7 million of unrecognized compensation cost related to RSUs that are expected to vest. The RSU costs are expected to be recognized over a weighted-average remaining vesting period of 1.9 years.

#### 10. Loss Per Share

Basic net loss per common share is calculated by dividing net loss allocable to common stockholders by the weighted-average common shares outstanding during the period, without consideration of stock options and RSUs as common stock equivalents. The weighted-average common shares outstanding as of June 30, 2020 includes pre-funded warrants to purchase up to an aggregate of 1.8 million shares of common stock that were issued in connection with the January 2020 public offering, as discussed in Note 13. For purposes of the diluted net loss per share calculation, common stock equivalents are excluded from the calculation if their effect would be anti-dilutive. As such, basic and diluted net loss per share applicable to common stockholders are the same for periods with a net loss.

The following table illustrates the determination of loss per share for each period presented.

	<b>Three Months Ended June 30,</b>		<b>Six Months Ended June 30,</b>	
	<b>2020</b>	<b>2019</b>	<b>2020</b>	<b>2019</b>
	(in thousands, except per share amounts)			
Numerator:				
Net loss applicable to common stockholders - basic and diluted \$	(12,991)	\$ (18,668)	\$ (33,468)	\$ (40,494)
Denominator:				
Weighted-average shares outstanding - basic and diluted	31,455	23,790	30,283	23,650
Net loss per share applicable to common stockholders - basic and diluted	<u>\$ (0.41)</u>	<u>\$ (0.78)</u>	<u>\$ (1.11)</u>	<u>\$ (1.71)</u>
Anti-dilutive potential common stock equivalents excluded from the calculation of net loss per share:				
Stock options	135	227	145	326
Restricted stock units	82	—	68	91
Warrants	61	61	61	78

## 11. Lease

The Company currently has a lease, or the Lease, for approximately 56,000 square feet of office and laboratory space located at 65 Hayden Avenue, Lexington, Massachusetts, or the Premises. The Lease is classified as an operating lease. The lease term extends ten years following January 1, 2019. The Company is entitled to two five-year options to extend the Lease. The Lease provides for annual base rent of approximately \$2.8 million in the first year following January 1, 2019, which increases on a yearly basis by 3.0% (subject to an abatement of base rent of approximately \$0.5 million at the beginning of the second year of the lease term). There are no variable payments, exercise purchase options, penalties, fees or residual value guarantees under the Lease. The Company is also obligated to pay the landlord for certain costs, taxes and operating expenses related to the Premises, subject to certain exclusions.

The Company recorded a liability for the Lease of \$16.9 million on January 1, 2019. This lease liability is amortized over the remaining lease term in an amount equal to the difference between the cash rent paid and the monthly interest calculated on the remaining lease liability. As of June 30, 2020, the Company had a current lease liability of \$0.8 million and a non-current lease liability of \$15.5 million recorded in its condensed consolidated balance sheets.

On January 1, 2019, the Company recorded a right-of-use asset in the amount of \$9.5 million, which represents the lease liability of \$16.9 million, adjusted for previously accrued rent of \$2.9 million and previously recorded unamortized lease incentives in the amount of \$4.5 million. The right-of-use asset is amortized over the remaining lease term in an amount equal to the difference between the calculated straight-line expense of the total lease payments less the monthly interest calculated on the remaining lease liability. As of June 30, 2020, the Company had a long-term lease asset of \$9.1 million recorded in its condensed consolidated balance sheets.

The Company recognizes lease expense, calculated as the remaining cost of the Lease allocated over the remaining lease term, on a straight-line basis. Lease expense is presented as part of continuing operations in the condensed consolidated statements of operations and comprehensive loss. For the six months ended June 30, 2020, the Company recognized \$1.2 million in lease expense.

The six months ended June 30, 2020 included two months of rent abatement according to the terms of the Lease, and the Company paid \$1.0 million in rent. As a payment arising from an operating lease, the \$1.0 million is classified within operating activities in the condensed consolidated statements of cash flows.

For the six months ended June 30, 2020 and 2019, the weighted-average remaining lease term was 8.5 years and 9.5 years, respectively, and the weighted-average discount rate was 13.08%.

## 12. Open Market Sale Agreement

On March 1, 2019, the Company entered into an Open Market Sale Agreement, or the ATM Agreement, with Jefferies LLC, or Jefferies, with respect to an at-the-market offering program under which the Company may offer and sell, from time to time at its sole discretion, shares of its common stock, par value \$0.001 per share, having an aggregate offering price of up to \$50.0 million, referred to as Placement Shares, through Jefferies as its sales agent. The Company will pay Jefferies a commission equal to 3.0% of the gross sales proceeds of any Placement Shares sold through Jefferies under the ATM Agreement, and also has provided Jefferies with customary indemnification and contribution rights. In addition, the Company has agreed to reimburse certain legal expenses and fees incurred by Jefferies in connection with the offering up to a maximum of \$50 thousand, in addition to certain ongoing disbursements of Jefferies' counsel. In 2019, the Company sold 36,167 shares of its common stock in accordance with the ATM Agreement for net proceeds of \$0.4 million, after payment of cash commissions of 3.0% of the gross proceeds to Jefferies. Additionally, in 2019, the Company incurred approximately \$0.3 million related to legal, accounting and other fees in connection with the ATM Agreement. The Company has not sold any shares of common stock under the ATM Agreement in 2020.

## 13. Sale of Common Stock and Pre-Funded Warrants

In January 2020, the Company closed an underwritten public offering of 5,735,283 shares of its common stock to the public at \$9.92 per share, which included the full exercise of the underwriters' option to purchase 982,863 additional shares of common stock. In addition, and in lieu of common stock, the Company sold to a certain existing investor pre-funded warrants to purchase up to an aggregate of 1,800,000 shares of common stock at a purchase price of \$9.919 per pre-funded warrant, which represents the per share public offering price for the common stock less the \$0.001 per share exercise price for each pre-funded

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warrant. The aggregate net proceeds to the Company from this offering were approximately \$70.1 million, after deducting underwriting discounts and commissions and other offering expenses payable by the Company.

The pre-funded warrants are exercisable at any time by either (i) payment in full in immediately available funds for the number of shares of common stock purchased upon such exercise or (ii) a cashless exercise, in which case the holder would receive upon such exercise the net number of shares of common stock determined according to the formula set forth in the pre-funded warrant. A holder will not be entitled to exercise any portion of any pre-funded warrant if the holder's ownership of the Company's common stock would exceed 19.99% following such exercise.

In the event of certain fundamental transactions, the holders of the pre-funded warrants will be entitled to receive upon exercise of the pre-funded warrants the kind and amount of securities, cash or other property that the holders would have received had they exercised the pre-funded warrants immediately prior to such fundamental transaction without regard to any limitations on exercise contained in the pre-funded warrants.

The pre-funded warrants were classified as a component of permanent stockholders' equity within additional paid-in capital and were recorded at the issuance date using a relative fair value allocation method. The pre-funded warrants are equity classified because they are freestanding financial instruments that are legally detachable and separately exercisable from the equity instruments, are immediately exercisable, do not embody an obligation for the Company to repurchase its shares, permit the holders to receive a fixed number of shares of common stock upon exercise, are indexed to the Company's common stock and meet the equity classification criteria. In addition, such pre-funded warrants do not provide any guarantee of value or return. The Company valued the pre-funded warrants at issuance, concluding that their sales price approximated their fair value, and allocated net proceeds from the sale proportionately to the common stock and pre-funded warrants, of which \$16.7 million was allocated to the pre-funded warrants and recorded as a component of additional paid-in capital.

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

You should read the following discussion and analysis of our financial condition and results of operations together with our condensed consolidated financial statements and the related notes appearing elsewhere in this Quarterly Report on Form 10-Q. This discussion and analysis contains forward-looking statements that involve significant risks and uncertainties. You should read the "Risk Factors" section in Part II, Item 1A. of this Quarterly Report on Form 10-Q for a discussion of important factors that could cause our actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

**OVERVIEW**

We are a clinical stage biopharmaceutical company that is developing small molecule drugs that we discovered through the application of our DCE Platform. Selective incorporation of deuterium into known molecules has the potential, on a case-by-case basis, to provide better pharmacokinetic or metabolic properties, thereby enhancing their clinical safety, tolerability or efficacy. Our pipeline consists of clinical stage candidates targeting autoimmune and CNS disorders, and a number of preclinical compounds that we are currently assessing.

Product Candidate	Lead Indications	Phase 1	Phase 2	Phase 3	Market	Worldwide Rights
<b>CTP-543</b> Deuterated ruxolitinib	Alopecia Areata: Phase 3 Expected to Begin Q4 2020					
	Alopecia Areata: Dose Ranging Completed		●			
	Alopecia Areata: Dose Regimen Completed (8 BID vs 16 QD)		●			
	Alopecia Areata: Dose Regimen Completed (12 BID vs 24 QD)		●			
	Alopecia Areata: Long Term Extension			→		
<b>CTP-692</b> Deuterated D-serine	Schizophrenia: Dose Ranging Ongoing		●			
	Phase 1 Crossover Completed		●			
	Phase 1 SAD/MAD Completed		●			
	Additional CNS Indications	●				

**CTP-543**

**CTP-543 Opportunity**

CTP-543 is an oral JAK1 and JAK2 inhibitor that we are developing for the treatment of moderate-to-severe alopecia areata, a disease that currently has no approved treatments. Alopecia areata is a serious, chronic autoimmune disease affecting approximately 700,000 Americans at any given time that results in partial or complete loss of hair on the scalp and/or body. CTP-543 was discovered by applying our deuterium chemistry technology to modify ruxolitinib, a JAK inhibitor, which is commercially available under the name Jakafi® in the United States for the treatment of certain blood disorders and for graft versus host disease. The FDA has granted CTP-543 Breakthrough Therapy designation for the treatment of adult patients with moderate-to-severe alopecia areata and Fast Track designation for the treatment of alopecia areata.

**Clinical Development of CTP-543**

We have completed three Phase 2 trials of CTP-543 for the treatment of moderate-to-severe alopecia areata to support the advancement of the program into Phase 3 development. In September 2019, we announced topline results from a Phase 2 double-blind, randomized, dose-ranging trial to evaluate three sequential doses of CTP-543 (4, 8 and 12 mg twice-daily) and a placebo control in 149 patients with moderate-to-severe alopecia areata. Patients treated with either 8 mg twice-daily or 12 mg twice-daily of CTP-543 met the primary efficacy endpoint with statistically significant differences (p <0.001) relative to

placebo in the percentage of patients achieving a  $\geq 50\%$  relative change from baseline at 24 weeks. The 8 mg twice-daily and 12 mg twice-daily groups were also significantly different from placebo in the number of patients achieving  $\geq 75\%$  and  $\geq 90\%$  relative change in Severity of Alopecia Tool, or SALT, score between baseline at 24 weeks. A numerically but not statistically greater percentage of patients treated with the 4 mg twice-daily dose of CTP-543 met the primary efficacy endpoint. At 24 weeks, patients treated with 8 mg twice-daily and 12 mg twice-daily compared to placebo also rated significantly greater improvement in their alopecia areata on the Patient Global Impression of Improvement Scale. Treatment with CTP-543 was generally well tolerated. The most common side effects in the 8 mg or 12 mg twice-daily groups were headache, nasopharyngitis, upper respiratory tract infection, acne, nausea and low-density lipoprotein increase. One serious adverse event of facial cellulitis was reported in the 12 mg twice-daily group as possibly related to treatment; however, after a brief interruption, treatment continued and this patient completed the trial. No thromboembolic events were reported during the trial.

In June 2020, we released new data analyses from our Phase 2 dose-ranging trial of CTP-543. The new data analyses revealed that statistically significant results were reported for the 8 mg twice-daily and 12 mg twice-daily doses of CTP-543 at more stringent response thresholds, which may be more clinically meaningful to patients, and positive findings were reported for clinician and patient reported outcome measures of scalp hair loss. At 24 weeks, 26% and 42% of patients who received CTP-543 in the 8 mg twice-daily and 12 mg twice-daily cohorts, respectively, achieved an absolute SALT score  $\leq 20$  ( $p < 0.05$  vs. placebo), indicating a clinically-meaningful 80 percent or greater scalp hair present. Data from the Clinician Global Impression of Improvement scale showed 75% of clinicians rated the response in the 12 mg twice-daily cohort and 61% of clinicians rated the response in the 8 mg twice-daily cohort as "much improved" or "very much improved" at 24 weeks. For both doses, there was a statistically significant difference from placebo ( $p < 0.001$ ).

In December 2019, we announced that we completed an open label Phase 2 trial evaluating 8 mg twice-daily compared to 16 mg once-daily dosing of CTP-543 in 57 patients with moderate-to-severe alopecia areata. Results in the 8 mg twice-daily arm were consistent with the previously-reported 8 mg twice-daily results from our Phase 2 dose-ranging trial of CTP-543. The trial measured the relative change in SALT score between baseline and 24 weeks. Treatment was generally well tolerated in both arms of the study. All but one of the patients who completed this trial elected to continue in an ongoing open label long-term extension study. A second open label Phase 2 trial evaluating 12 mg twice-daily compared to 24 mg once-daily dosing of CTP-543 in patients with moderate-to-severe alopecia areata was completed in 2020. We intend to utilize the 8 mg twice-daily and 12 mg twice-daily doses in our clinical development program for CTP-543 going forward.

As of April 2019, all patients who complete efficacy and safety studies with CTP-543 are eligible to enroll into an open label long-term extension study.

We conducted an end of Phase 2 meeting with the FDA in March 2020 and announced our plans to advance CTP-543 into Phase 3 evaluation, which we expect to initiate in the fourth quarter of 2020. The planned Phase 3 program for CTP-543 will include two randomized, double-blind, placebo-controlled clinical trials in adults at sites in the United States, Canada and Europe. The CTP-543 Phase 3 program will evaluate the percentage of patients receiving 8 mg twice-daily, 12 mg twice-daily or placebo twice-daily that achieve a SALT score of 20 or less after 24 weeks of dosing in patients with moderate-to-severe alopecia areata.

## **CTP-692**

### **CTP-692 Opportunity**

CTP-692 is a selective deuterated analog of the endogenous amino acid D-serine, a key molecule that activates NMDA receptors in areas of the brain that are widely believed to play key roles in schizophrenia. Based on published nonclinical and clinical effects of D-serine, we believe that CTP-692 has the potential to help restore NMDA receptor activity in key areas of the brain to improve clinical outcomes in patients with schizophrenia. Population studies have shown that levels of D-serine measured in the plasma and cerebrospinal fluid of patients with schizophrenia are, on average, significantly lower than healthy controls. Genome-wide association studies demonstrate that genetic changes that alter glutamatergic transmission, including by reducing NMDA neurotransmission and by reducing D-serine exposure, substantially increase the likelihood of becoming schizophrenic. Academic studies have demonstrated that oral dosing of D-serine can result in dose-dependent improvement in positive, negative and cognitive symptoms in patients with schizophrenia when added to D2-modulating antipsychotics, with the exception of clozapine. However, nonclinical studies have demonstrated that D-serine can cause nephrotoxicity in rats. In addition, in some patients who received high doses of D-serine, clinical findings suggesting renal impairment were observed. As a result, the clinical development of D-serine has historically been limited.

In nonclinical studies in rats, when administered at doses where D-serine resulted in causing significantly increased blood levels of serum creatinine and blood urea nitrogen, indicating renal toxicity, CTP-692 did not cause changes in blood levels of those

markers, suggesting that CTP-692 could have reduced toxicity and a larger therapeutic window. Administration of CTP-692 in rats also resulted in greater plasma exposure and higher brain to plasma ratios than did similar doses of D-serine. It therefore may be better-suited for development as a human therapeutic agent. CTP-692 will initially be developed as an adjunctive therapy along with standard antipsychotic medicines in patients with schizophrenia.

### **Clinical Development of CTP-692**

The Phase 1 program was designed to assess the safety, tolerability and pharmacokinetics of CTP-692 in healthy volunteers. The Phase 1 program included three studies: a crossover comparison of CTP-692 versus D-serine, a single-ascending dose study that also assessed the effect of food on the pharmacokinetics of CTP-692, and a multiple-ascending dose trial assessing CTP-692 dosed orally over seven days.

In the crossover study, a single dose of CTP-692 was found to result in greater plasma exposure than did the same amount of D-serine. In the single- and multiple-ascending dose trials, CTP-692 was evaluated across doses ranging from 0.5 to 4 grams compared to placebo in a total of 72 volunteers. CTP-692 demonstrated a favorable safety, tolerability and pharmacokinetic profile with no serious adverse events reported. Importantly, key blood and urine markers of kidney function did not indicate any signs of renal impairment.

In December 2019, we initiated a double-blind, randomized, placebo-controlled Phase 2 trial to evaluate the safety and efficacy of CTP-692 as an adjunctive treatment for schizophrenia. Approximately 300 adult patients who are stable on an antipsychotic medication will be randomized to receive 1, 2 or 4 grams of CTP-692 or placebo once-daily. The primary outcome measure is the change in the Positive and Negative Syndrome Scale (PANSS) total score at 12 weeks compared to baseline. We currently expect to complete enrollment of this clinical trial by year-end 2020.

### **Preclinical Pipeline**

We are currently assessing a number of preclinical assets as potential development candidates.

### **COLLABORATION PRODUCT CANDIDATES**

In addition to our wholly owned development programs, we have entered into collaborative arrangements with companies to develop deuterium-modified versions of their marketed products. Our partners are currently responsible for all development and future commercialization activities under these arrangements. In each of these collaborations, the deuterium-modified compound was independently discovered by us. For example, on February 24, 2012, we entered into a development and license agreement with Avanir Pharmaceuticals, Inc., or Avanir, a subsidiary of Otsuka Pharmaceuticals Co., Ltd. for the worldwide rights to develop, manufacture and commercialize AVP-786. AVP-786 is a combination of deudextromethorphan hydrobromide (d6-DM) and quinidine sulfate (Q), a CYP2D6 inhibitor, being investigated for the treatment of neurologic and psychiatric disorders. In 2019, Avanir completed two Phase 3 trials evaluating AVP-786 for the treatment of agitation associated with dementia of the Alzheimer's type. The second of the Phase 3 trials did not meet its primary or key secondary endpoints; however, following additional data analysis, Avanir decided to continue developing AVP-786 for the treatment of agitation associated with dementia of the Alzheimer's type. Two additional Phase 3 trials and an open label long-term extension study for Alzheimer's agitation are ongoing, and an additional Phase 3 trial is planned. Additionally, Avanir is conducting a Phase 2/3 trial evaluating AVP-786 for the treatment of negative symptoms of schizophrenia.

### **ASSET PURCHASE AGREEMENT WITH VERTEX PHARMACEUTICALS FOR CTP-656**

In July 2017, we completed the sale of worldwide development and commercialization rights to CTP-656 and other assets related to the treatment of cystic fibrosis to Vertex. CTP-656, now known as VX-561, is an investigational cystic fibrosis transmembrane conductance regulator, or CFTR, potentiator that has the potential to be used as part of future once-daily combination regimens of CFTR modulators that treat the underlying cause of cystic fibrosis. We received \$160 million in cash upon closing, and if VX-561 is approved as part of a combination regimen to treat cystic fibrosis, we are eligible to receive up to \$90 million in the form of two additional milestones based on marketing approval in the United States and agreement for reimbursement in the first of the United Kingdom, Germany or France.

### **COVID-19 PANDEMIC**

In March 2020, the World Health Organization declared the outbreak of a novel strain of virus named SARS-CoV-2 (severe acute respiratory syndrome coronavirus 2), which causes coronavirus disease 2019, or COVID-19, as a pandemic, which continues to spread throughout the United States and worldwide. We could be materially and adversely affected by the risks, or the public perception of the risks, related to an epidemic, pandemic or other public health crisis, such as the COVID-19 pandemic, including but not limited to potential delays in our clinical trials. The ultimate extent of the impact of any epidemic,

pandemic or other public health crisis on our business, financial condition and results of operations will depend on future developments, which are highly uncertain and cannot be predicted, including new information that may emerge concerning the severity of such epidemic, pandemic or other public health crisis and actions taken to contain or prevent the further spread, among others. Accordingly, we cannot predict the extent to which our business, financial condition and results of operations may be affected by the COVID-19 pandemic, but we are monitoring the situation closely.

## FINANCIAL OPERATIONS OVERVIEW

Since our inception in 2006, we have devoted substantially all of our resources to our research and development efforts, including activities to develop our DCE Platform and our core capabilities in deuterium chemistry, identify potential product candidates, undertake nonclinical studies and clinical trials, manufacture clinical trial material in compliance with current good manufacturing practices, or cGMPs, provide general and administrative support for these operations and establish our intellectual property. We have generated an accumulated deficit of \$228.1 million since inception through June 30, 2020 and will require substantial additional capital to fund our research and development. We do not have any products approved for sale and have not generated any revenue from product sales. We have funded our operations primarily through the public offering and private placement of our equity, debt financing, funding from collaborations and patent assignments, an asset sale and other arrangements.

Our operating results may fluctuate significantly from year to year, depending on the timing and magnitude of cash payments received pursuant to collaboration and licensing arrangements and other agreements and the timing and magnitude of clinical trial and other development activities under our current development programs. Substantially all of our net losses have resulted from costs incurred in connection with our research and development programs and from general and administrative costs associated with our operations.

We expect to continue to incur significant expenses and increasing operating losses for at least the next several years. We expect our expenses will increase substantially in connection with our ongoing activities as we continue research and development efforts and develop and conduct additional nonclinical studies and clinical trials with respect to our product candidates.

We do not expect to generate revenue from product sales unless and until we, or our collaborators, obtain marketing approval for one or more of our product candidates, which we expect will take a number of years and is subject to significant uncertainty. If we obtain, or believe that we are likely to obtain, marketing approval for any product candidates for which we retain commercialization rights, and intend to commercialize a product, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution. We expect to seek to fund our operations through a combination of equity offerings, debt financings, collaboration and licensing arrangements and other sources for at least the next several years. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. Our failure to raise capital or enter into such other arrangements as and when needed would force us to delay, limit, reduce or terminate our research and development programs and could have a material adverse effect on our financial condition and our ability to develop our products. We will need to generate significant revenues to achieve sustained profitability, and we may never do so.

### Revenue

We have not generated any revenue from the sales of products. All of our revenue to date has been generated through collaboration, license and research arrangements with collaborators and nonprofit organizations for the development and commercialization of product candidates, a patent assignment agreement and an asset sale.

The terms of these agreements may include one or more of the following types of payments: non-refundable license fees, payments for research and development activities, payments based upon the achievement of specified milestones, payment of license exercise or option fees relating to product candidates and royalties on any net product sales. To date, we have received non-refundable upfront payments, several milestone payments, payments for research and development services provided to our collaborators, a change in control payment pursuant to a patent assignment agreement and a payment for the sale of an asset. However, we have not yet earned any license exercise or option fees, sales-based milestone payments or royalty revenue as a result of product sales.

In the future, we will seek to generate revenue from a combination of product sales and milestone payments and royalties on product sales in connection with our current collaborations, our asset sale with Vertex or other collaborations we may enter into.

## Research and development expenses

Research and development expenses consist primarily of costs incurred for the development of our product candidates, which include:

- employee-related expenses, including salary, benefits, travel and stock-based compensation expense;
- expenses incurred under agreements with contract research organizations and investigative sites that conduct our clinical trials;
- the cost of acquiring, developing and manufacturing clinical trial materials;
- facilities, depreciation and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance and other supplies;
- platform-related lab expenses, which includes costs related to synthesis, analysis and *in vitro* and *in vivo* characterization of deuterated compounds to support the selection and progression of potential product candidates;
- expenses related to consultants and advisors; and
- costs associated with nonclinical activities and regulatory operations.

Research and development costs are expensed as incurred. Costs for certain development activities are recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and our clinical sites.

A significant portion of our research and development costs have been external costs, which we track on a program-by-program basis. These external costs include fees paid to investigators, consultants, central laboratories and contract research organizations in connection with our clinical trials, and costs related to acquiring and manufacturing clinical trial materials. Our internal research and development costs are primarily personnel-related costs, depreciation and other indirect costs. We do not track our internal research and development expenses on a program-by-program basis, as they are deployed across multiple projects under development.

The successful development of any of our product candidates is highly uncertain. As such, at this time, we cannot reasonably predict with certainty the duration and completion costs of the current or future clinical trials of any of our product candidates or if, when or to what extent we will generate revenues from the commercialization and sale of any of our product candidates that obtain marketing approval. We may never succeed in achieving marketing approval for any of our product candidates. The duration, costs and timing of clinical trials and development of our product candidates will depend on a variety of factors, including:

- the scope and rate of progress of our ongoing as well as any additional clinical trials and other research and development activities;
- successful enrollment in and completion of clinical trials, including on account of the COVID-19 pandemic and its impact on clinical trial sites;
- conduct of and results from ongoing as well as any additional clinical trials and research and development activities;
- significant and changing government regulation;
- the terms and timing and receipt of any marketing approvals;
- the performance of our collaborators;
- our ability to manufacture any of our product candidates that we are developing or may develop in the future; and
- the expense and success of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights, including potential claims that we infringe other parties' intellectual property.

A change in the outcome of any of these variables with respect to the development of a product candidate could mean a significant change in the cost and timing associated with the development of that product candidate. For example, if the FDA or another regulatory authority were to require us to conduct clinical trials or other research and development activities beyond those that we currently anticipate will be required for the completion of clinical development of a product candidate, 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.

Research and development activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, due to the increased size and duration of later-stage clinical trials and the manufacturing that is typically required for those later-stage clinical trials. We expect research and development costs to increase significantly for the foreseeable future as our product candidate development programs progress, but we do not believe that it is possible at this time to accurately project total program-specific expenses through commercialization. There are numerous factors associated with the successful commercialization of any of our product candidates, including future trial design and various regulatory requirements, many of which cannot be determined

with accuracy at this time based on our stage of development. Additionally, future commercial and regulatory factors beyond our control will impact our clinical development programs and plans.

### **General and administrative expenses**

General and administrative expenses consist primarily of salaries and related costs for personnel, including stock-based compensation and travel expenses for our employees in executive, operational, finance, legal, business development and human resource functions. Other general and administrative expenses include facility-related costs, depreciation and other expenses not allocated to research and development expense and professional fees for directors, accounting and legal services and expenses associated with obtaining and maintaining patents. In both 2020 and 2019, we incurred expenses for intellectual property matters related to CTP-543.

We anticipate that our general and administrative expenses will increase in the future as our pipeline grows and matures. Additionally, if and when we believe that a marketing approval of the first product candidate that we intend to commercialize on our own appears likely, we anticipate an increase in payroll and related expenses as a result of our preparation for commercial operations, especially as it relates to the sales, marketing and distribution of our product candidates.

### **Investment income**

Investment income consists of interest income earned on cash equivalents and investments. The amount of investment income earned in any particular period may vary primarily as a result of the amount of cash equivalents and investments held during the period and the types of securities included in our portfolio during the period. Our current investment policy is to maintain a diversified investment portfolio of U.S. government-backed securities and money market mutual funds consisting of U.S. government-backed securities.

### **Unrealized loss on marketable equity securities**

Unrealized loss on marketable equity securities consists of changes in the fair value of shares of common stock of Processa held by us, as discussed further in Note 8 in the condensed consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q.

### **Income taxes**

We record a provision or benefit for income taxes on pre-tax income or loss based on our estimated effective tax rate for the year. As of June 30, 2020, we forecast an ordinary pre-tax loss for the year ended December 31, 2020 and maintain a full valuation allowance on our deferred tax assets. During the three and six months ended June 30, 2020, we recorded a benefit for income taxes of \$85 thousand for interest accrued under the installment sales method for the sale of CTP-656 to Vertex.

## **CRITICAL ACCOUNTING POLICIES AND SIGNIFICANT JUDGMENTS AND ESTIMATES**

Our critical accounting policies are those policies which require the most significant judgments and estimates in the preparation of our condensed consolidated financial statements.

There have been no material changes to our critical accounting policies as detailed in our Annual Report on Form 10-K for the fiscal year ended December 31, 2019 filed with the SEC on February 27, 2020.

## **PENDING AND RECENTLY ADOPTED ACCOUNTING PRONOUNCEMENTS**

For detailed information regarding recently issued accounting pronouncements and the actual and expected impact on our condensed consolidated financial statements, see Note 2 in the condensed consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q.

## **RESULTS OF OPERATIONS**

### **Discussion of the three and six months ended June 30, 2020**

The following table summarizes our results of operations for the three and six months ended June 30, 2020.

(in thousands)	Three Months Ended June 30,		Six Months Ended June 30,	
	2020		2020	
<b>Revenue:</b>				
License and research and development revenue	\$	6,387	\$	6,394
<b>Operating expenses:</b>				
Research and development		14,788		28,774
General and administrative		4,731		9,403
Total operating expenses		19,519		38,177
Loss from operations		(13,132)		(31,783)
Investment income		355		918
Unrealized loss on marketable equity securities		(299)		(2,688)
Loss before income taxes		(13,076)		(33,553)
Income tax benefit		(85)		(85)
Net loss	\$	(12,991)	\$	(33,468)

#### *License and research and development revenue*

Total revenue was \$6.4 million for both the three and six months ended June 30, 2020. The revenue recognized in the 2020 periods was primarily attributable to the expiration of two licensing options under our collaboration agreement with Celgene.

#### *Research and development expenses*

The following table summarizes our research and development expenses for the three and six months ended June 30, 2020, with our external research expenses separately classified by program and our internal research expenses separately classified by category.

(in thousands)	Three Months Ended June 30,		Six Months Ended June 30,	
	2020		2020	
CTP-543 external costs	\$	3,626	\$	6,556
CTP-692 external costs		4,951		9,259
External expenses for other programs		151		495
Employee and contractor-related expenses		4,728		9,784
Facility and other expenses		1,332		2,680
Total research and development expenses	\$	14,788	\$	28,774

Research and development expenses were \$14.8 million and \$28.8 million for the three and six months ended June 30, 2020, respectively. CTP-543 expenses primarily related to clinical development, including preparations for the Phase 3 clinical trial that is expected to begin in the fourth quarter of 2020. CTP-543 expenses decreased by \$1.4 million and \$2.4 million in the three and six months ended June 30, 2020 compared to the three and six months ended June 30, 2019, respectively, primarily due to the completion of the Phase 2 dose-ranging clinical trial in the third quarter of 2019. CTP-692 expenses increased by \$2.2 million and \$2.5 million in the three and six months ended June 30, 2020 compared to the three and six months ended June 30, 2019, respectively. These CTP-692 expenses, and their increase from the prior period, were primarily attributable to the ongoing Phase 2 dose-ranging clinical trial. External expenses for other programs consisted of costs incurred to develop our research pipeline. External expenses for other programs decreased by \$0.5 million and \$1.5 million in the three and six months ended June 30, 2020 compared to the three and six months ended June 30, 2019, respectively, primarily due to a decrease in lab activity due to the COVID-19 pandemic, and due to the payment made during the first quarter of 2019 to the non-profit organization Fast Forward related to the Cipla Agreement, as discussed further in Note 8 in the condensed consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q. Employee-related expenses consisted primarily of cash compensation and non-cash stock-based compensation expenses. Facility-related expenses consisted primarily of rent and maintenance of our premises.

#### *General and administrative expenses*

The following table summarizes our general and administrative expenses for the three and six months ended June 30, 2020.

(in thousands)	Three Months Ended June 30,	Six Months Ended June 30,
	2020	2020
Employee salaries and benefits	\$ 2,688	\$ 5,397
External professional service and legal expenses	998	1,886
Facility, technology and other expenses	970	1,970
Depreciation and amortization	75	150
Total general and administrative expenses	<u>\$ 4,731</u>	<u>\$ 9,403</u>

General and administrative expenses decreased by \$0.2 million and \$1.2 million in the 2020 periods compared to the three and six months ended June 30, 2019, respectively, primarily due to a decrease in legal expenses.

#### *Investment income*

Investment income was \$0.4 million and \$0.9 million for the three and six months ended June 30, 2020, respectively, and consisted of interest income earned on cash equivalents and investments.

#### *Unrealized loss on marketable equity securities*

Unrealized loss on marketable equity securities was \$0.3 million and \$2.7 million for the three and six months ended June 30, 2020, respectively, and consisted of changes in the fair value of shares of common stock of Processa held by us, as discussed further in Note 8 in the condensed consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q.

#### *Income Tax Benefit*

During the three and six months ended June 30, 2020, we recorded a benefit for income taxes of \$85 thousand for interest accrued under the installment sales method for the sale of CTP-656 to Vertex.

### **Discussion of the three and six months ended June 30, 2019**

The following table summarizes our results of operations for the three and six months ended June 30, 2019.

(in thousands)	Three Months Ended June 30,	Six Months Ended June 30,
	2019	2019
<b>Revenue:</b>		
License and research and development revenue	\$ 49	\$ 1,054
<b>Operating expenses:</b>		
Research and development	14,496	30,286
General and administrative	4,978	10,587
Total operating expenses	19,474	40,873
Loss from operations	(19,425)	(39,819)
Investment income	883	1,750
Unrealized loss on marketable equity securities	(126)	(2,425)
Net loss	<u>\$ (18,668)</u>	<u>\$ (40,494)</u>

#### *License and research and development revenue*

Total revenue was \$49 thousand and \$1.1 million for the three and six months ended June 30, 2019, respectively. The revenue recognized in the 2019 periods was primarily related to the Cipla Agreement, as discussed further in Note 8 in the condensed consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q.

#### Research and development expenses

The following table summarizes our research and development expenses for the three and six months ended June 30, 2019, with our external research expenses separately classified by program and our internal research expenses separately classified by category.

(in thousands)	Three Months Ended June 30,		Six Months Ended June 30,	
	2019		2019	
CTP-543 external costs	\$	5,018	\$	8,951
CTP-692 external costs		2,758		6,789
External expenses for other programs		629		1,988
Employee and contractor-related expenses		4,692		9,761
Facility and other expenses		1,399		2,797
Total research and development expenses	\$	14,496	\$	30,286

Research and development expenses were \$14.5 million and \$30.3 million for the three and six months ended June 30, 2019, respectively. CTP-543 expenses primarily related to clinical development, including multiple Phase 2 clinical trials. CTP-692 expenses were primarily attributable to the Phase 1 clinical trials and manufacturing costs to support the continued advancement of the program. External expenses for other programs consisted of costs incurred to develop our research pipeline and a \$0.5 million payment to the non-profit organization Fast Forward in the first quarter of 2019 under a pre-existing CTP-354 agreement, which was triggered by the upfront payment that Cipla paid to us under the Cipla Agreement. Employee-related expenses consisted primarily of cash compensation and non-cash stock-based compensation expenses. Facility-related expenses consisted primarily of rent and maintenance of our premises.

#### General and administrative expenses

The following table summarizes our general and administrative expenses for the three and six months ended June 30, 2019.

(in thousands)	Three Months Ended June 30,		Six Months Ended June 30,	
	2019		2019	
Employee salaries and benefits	\$	2,747	\$	5,747
External professional service and legal expenses		1,197		2,808
Facility, technology and other expenses		959		1,876
Depreciation and amortization		75		156
Total general and administrative expenses	\$	4,978	\$	10,587

#### Investment income

Investment income was \$0.9 million and \$1.8 million for the three and six months ended June 30, 2019, respectively, and consisted of interest income earned on cash equivalents and investments.

#### Unrealized loss on marketable equity securities

Unrealized loss on marketable equity securities was \$0.1 million and \$2.4 million for the three and six months ended June 30, 2019, respectively, and consisted of changes in the fair value of shares of common stock of Processa held by us, as discussed further in Note 8 in the condensed consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q.

#### OFF-BALANCE SHEET ARRANGEMENTS

**CONCERT PHARMACEUTICALS, INC.**  
**NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (UNAUDITED)**

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

## LIQUIDITY AND CAPITAL RESOURCES

We have incurred cumulative losses and negative cash flows from operations since our inception in April 2006, and as of June 30, 2020, we had an accumulated deficit of \$228.1 million. We generated net income for the fiscal year ended December 31, 2015 due to a one-time payment from Auspex Pharmaceuticals, Inc., or Auspex, under a change in control provision described in our patent assignment agreement and again for the fiscal year ended December 31, 2017 from the closing of our sale of CTP-656 to Vertex, but we anticipate that we will continue to incur losses for at least the next several years. We expect that our research and development and general and administrative expenses will continue to increase and, as a result, we will need additional capital to fund our operations, which we may raise through a combination of equity offerings, debt financings, additional collaborations and licensing arrangements, and other sources.

We have financed our operations to date primarily through the public offering and private placement of our equity, debt financing and funding from collaborations, patent assignments and an asset sale. In February 2014, we completed our initial public offering whereby we sold 6,649,690 shares of common stock at a price to the public of \$14.00 per share, raising aggregate net proceeds of \$83.1 million. In March 2015, we sold 3,300,000 shares of common stock through an underwritten public offering at a price to the public of \$15.15 per share, raising aggregate net proceeds of \$46.7 million. In January 2020, we sold 5,735,283 shares of common stock through an underwritten public offering at a price to the public of \$9.92 per share. At the same time, we sold to a certain existing investor pre-funded warrants to purchase up to an aggregate of 1,800,000 shares of common stock at a purchase price of \$9.919 per pre-funded warrant, which represents the per share public offering price for the common stock less the \$0.001 per share exercise price for each pre-funded warrant. The aggregate net proceeds from the January 2020 offering was \$70.1 million.

In June 2015, we received proceeds of \$50.2 million in connection with the change in control payment from Auspex, relating to Teva Pharmaceutical Industries Ltd.'s acquisition of Auspex.

In July 2017, the asset purchase contemplated by the Vertex Agreement was completed, and Vertex paid us \$160 million in cash consideration.

As a result of the COVID-19 pandemic, enrollment of new patients in our clinical trials has slowed and the timelines to complete our clinical trials may be delayed. Our assessment of our liquidity and capital resources includes an estimate of the financial impacts of these changes.

As of June 30, 2020, we had cash and cash equivalents and investments of \$144.7 million. Cash in excess of immediate requirements is invested in accordance with our investment policy, primarily with a view to liquidity and capital preservation. Currently, our funds are held in U.S. government-backed securities and money market mutual funds consisting of U.S. government-backed securities.

### Cash flows

The following table sets forth the primary sources and uses of cash for each of the periods set forth below:

(in thousands)	Six Months Ended June 30,	
	2020	2019
Net cash (used in) provided by:		
Operating activities	\$ (32,524)	\$ (18,025)
Investing activities	(64,033)	42,896
Financing activities	70,608	1,073
Net (decrease) increase in cash and cash equivalents and restricted cash	\$ (25,949)	\$ 25,944

*Operating activities.* The cash used for operating activities generally approximates our net loss adjusted for non-cash items and changes in operating assets and liabilities. During the six months ended June 30, 2020, our operating activities used cash of \$32.5 million as compared to cash used by operating activities of \$18.0 million during the six months ended June 30, 2019. Cash used in operating activities during both the six months ended June 30, 2020 and 2019 was primarily driven by our development activities associated with CTP-543 and CTP-692, our wholly owned development programs.

*Investing activities.* Net cash provided by investing activities consisted of proceeds from the maturity of investments, purchases of investments and purchases of fixed assets. Net cash provided by maturities of investments for the six months ended June 30, 2020 and 2019 was \$81.2 million and \$92.6 million, respectively. Net cash used in purchases of investments for the six months ended June 30, 2020 and 2019 was \$145.2 million and \$49.2 million, respectively. Purchases of fixed assets for the six months ended June 30, 2020 and 2019 were \$0.1 million and \$0.5 million, respectively. The increase in the purchases of investments during the six months ended June 30, 2020 is primarily due to the management of funds received from the January 2020 public offering of common stock and pre-funded warrants, as discussed further in Note 13 in the condensed consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q.

*Financing activities.* During the six months ended June 30, 2020 and 2019, our financing activities provided cash of \$70.6 million and \$1.1 million, respectively. The cash provided by financing activities during the six months ended June 30, 2020 was primarily attributable to the proceeds from the January 2020 public offering of common stock and pre-funded warrants. Additionally, the Company received proceeds of \$0.5 million and \$1.0 million from the exercise of stock options during the six months ended June 30, 2020 and 2019, respectively.

### **Operating capital requirements**

We do not anticipate commercializing any of our product candidates for several years. Although we generated net income for the fiscal years ended December 31, 2017 and 2015 due to one-time payments from Vertex and Auspex, respectively, we anticipate that we will continue to generate losses for the foreseeable future, and we expect the losses to increase as we continue the development of, and seek marketing approvals for, our product candidates, and begin to commercialize any approved products for which we retain commercialization rights. We are subject to all of the risks incident in the development of new drug products, and we may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business, as well as additional risks stemming from the unproven nature of deuterated drugs.

Based on our current expectations, including with respect to our development plans, we believe that our existing cash and cash equivalents and investments as of June 30, 2020 will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2021. However, we will require additional capital for the further development of our existing product candidates and may also need to raise additional funds sooner to pursue other development activities related to additional product candidates.

To date, we have not generated any revenue from product sales. We do not expect to generate significant revenue from product sales unless and until we, or our collaborators, obtain marketing approval of and commercialize one of our current or future product candidates. Because our product candidates are in various stages of development and the outcome of these efforts is uncertain, we cannot estimate the actual amounts necessary to successfully complete development and commercialization of our product candidates or whether or when we will achieve profitability.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings and additional collaborations, strategic alliances and licensing arrangements, and other arrangements. Except for any obligations of our collaborators to reimburse us for research and development expenses or to make milestone payments under our agreements with them, we do not have any additional committed external sources of funds. Additional capital may not be available on reasonable terms, if at all. If we are unable to raise additional funds when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves. If we raise additional funds through the issuance of additional equity or debt securities, it could result in dilution to our existing stockholders, increased fixed payment obligations and the issuance of securities with rights senior to those of our common stock. We may become subject to covenants under any future indebtedness that could limit our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, which could adversely impact our ability to conduct our business.

Our expectation with respect to the period of time through which our financial resources will be adequate to support our operations is a forward-looking statement and involves risks and uncertainties, and actual results could vary as a result of a number of factors, including those discussed in the "Risk Factors" section of this Quarterly Report on Form 10-Q. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we currently expect. If we cannot expand our operations or otherwise capitalize on our business opportunities because we lack sufficient capital, our business, financial condition and results of operations could be materially adversely affected.

**Item 3. Quantitative and Qualitative Disclosures About Market Risk.**

We are exposed to market risk related to changes in interest rates. Our current investment policy is to maintain a diversified investment portfolio in U.S. government-backed securities and money market mutual funds consisting of U.S. government-backed securities. Our cash is deposited in and invested through highly rated financial institutions in North America. As of June 30, 2020 and December 31, 2019, we had \$144.7 million and \$106.4 million of cash, cash equivalents and investments, respectively. The fair value of cash equivalents and short-term investments is subject to change as a result of potential changes in market interest rates. Due to the short-term maturities of our cash equivalents and the low risk profile of these investments, an immediate 100 basis point change in interest rates at levels as of June 30, 2020 would not have a material effect on the fair market value of our cash equivalents and short-term investments.

We contract with suppliers of raw materials, contract manufacturers and contract research organizations internationally. Transactions with these providers are predominantly settled in U.S. dollars and, therefore, we believe that we have only minimal exposure to foreign currency exchange risks. We do not hedge against foreign currency risks.

Inflation generally affects us by increasing our cost of labor and clinical trial costs. We do not believe that inflation had a material effect on our business, financial condition or results of operations during the six months ended June 30, 2020.

**Item 4. Controls and Procedures.****Evaluation of Disclosure Controls and Procedures**

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, refers to controls and procedures 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 SEC'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, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure. Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and our management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our disclosure controls and procedures are designed to provide reasonable assurance of achieving their control objectives.

Our management, with the participation of our Principal Executive Officer and Principal Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of June 30, 2020, the end of the period covered by this Quarterly Report on Form 10-Q. Based upon such evaluation, our Principal Executive Officer and Principal Financial Officer concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of such date.

**Changes in Internal Control over Financial Reporting**

There were no changes in our internal control over financial reporting that occurred during the six months 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 1A. Risk Factors.

Our business is subject to numerous risks. The following important factors, among others, could cause our actual results to differ materially from those expressed in forward-looking statements made by us or on our behalf in this Quarterly Report on Form 10-Q and other filings with the SEC, press releases, communications with investors and oral statements. Actual future results may differ materially from those anticipated in our forward-looking statements. We undertake no obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise.

#### RISKS RELATED TO THE COVID-19 PANDEMIC

##### *Our business may be adversely affected by the ongoing COVID-19 pandemic.*

Our business could be adversely affected by health epidemics in regions where we have concentrations of clinical trial sites or other business activities and could cause significant disruption in the operations of clinical research organizations and contract manufacturers upon whom we rely. For example, the COVID-19 pandemic has grown to affect most regions of the world.

As a result of the COVID-19 pandemic, we may experience disruptions that could severely impact our business, clinical trials and supply chain, including:

- We believe that the COVID-19 pandemic has had, and will continue to have, an impact on our clinical trials, including our Phase 2 clinical trial of CTP-692 for schizophrenia and our open label long-term extension study of CTP-543 for alopecia areata. Due to changes to study site operations and local travel restrictions, in some cases, these impacts include the potential need for remote assessments and delivery of study medication directly to patients. In addition, enrollment of new patients in our clinical trials has slowed as a result of state and local restrictions. Some patients may choose to withdraw from our studies or we may choose to, or be required to, pause enrollment or patient dosing in order to preserve health resources and protect trial participants. As a result, the timelines to complete our clinical trials may be delayed. For example, due to delays in enrollment in our Phase 2 clinical trial of CTP-692 for schizophrenia as a result of the COVID-19 pandemic, in late April 2020, we updated our expectation regarding the completion of enrollment of this clinical trial to expecting enrollment to be complete by year-end 2020.
- We believe that the COVID-19 pandemic may also have an impact on the clinical trials of our collaborators. For instance, AVP-786 is being developed under a collaboration with Avanir. Screening and enrollment in ongoing AVP-786 clinical trials were temporarily paused due to restrictions associated with the COVID-19 pandemic, but have since resumed for many of the clinical trials. As a result, our collaborators' timelines to complete clinical trials may be delayed.
- We currently rely on third parties to, among other things, manufacture raw materials, manufacture our product candidates for our clinical trials, ship our product candidates to study sites, perform quality testing and supply other goods and services to run our business. If any such third party in our supply chain is adversely impacted by restrictions resulting from the COVID-19 pandemic, including staffing shortages, production slowdowns or disruptions in delivery systems, our supply chain may be disrupted, limiting our ability to manufacture our product candidates for our clinical trials and conduct our research and development operations.
- We have limited our on-site staff to personnel that work in our laboratories or perform other essential activities on-site, and have requested that all other personnel continue to work remotely. Our increased reliance on personnel working from home may negatively impact productivity or disrupt, delay or otherwise adversely impact our business. In addition, this could increase our cyber security risk, create data accessibility concerns and make us more susceptible to communication disruptions, any of which could adversely impact our business operations or delay necessary interactions with local and federal regulators, ethics committees, manufacturing sites, clinical trial sites and other important agencies and contractors. General protective measures put into place at various governmental levels, including quarantines, travel restrictions and business shutdowns, may also negatively affect our operations.
- Health regulatory agencies globally may experience disruptions in their operations as a result of the COVID-19 pandemic. The FDA and comparable foreign regulatory agencies may have slower response times or be under-resourced. It is unknown how long these disruptions could continue, were they to occur. Any elongation or de-prioritization of our clinical trials or delay in regulatory review resulting from such disruptions could materially affect the development and study of our product candidates.

- The trading prices for our common stock and the stock of other biopharmaceutical companies have been highly volatile as a result of the COVID-19 pandemic. As a result, we may face difficulties raising capital through sales of our common stock or such sales may be on unfavorable terms. In addition, a recession, depression or other sustained adverse market event resulting from the COVID-19 pandemic could materially and adversely affect our business and the value of our common stock.

The COVID-19 pandemic continues to rapidly evolve. The ultimate impact of the COVID-19 pandemic on our business operations is highly uncertain and subject to change and will depend on future developments, which cannot be accurately predicted, including the duration of the pandemic, additional or modified government actions, new information that will emerge concerning the severity and impact of COVID-19 and the actions taken to contain or address its impact in the short and long term, among others. We do not yet know the full extent of potential delays or impacts on our business, our clinical trials, healthcare systems or the global economy. We will continue to monitor the situation closely.

#### **RISKS RELATED TO OUR FINANCIAL POSITION AND NEED FOR ADDITIONAL CAPITAL**

***We have incurred significant losses since inception, expect to incur losses for at least the next several years and may never sustain profitability.***

As of June 30, 2020, we had an accumulated deficit of \$228.1 million. We have not generated any revenues from product sales and have financed our operations to date primarily through the public offering of our common stock, private placements of our preferred stock, debt financings and funding from collaborations or other agreements. We have not completed development of any product candidate and have devoted substantially all of our financial resources and efforts to research and development, including nonclinical studies and our clinical development programs. We expect to continue to incur significant expenses and increasing operating losses for at least the next several years. Our net losses may fluctuate significantly from quarter to quarter and year to year. Net losses and negative cash flows have had, and will continue to have, an adverse effect on our stockholders' equity and working capital.

We anticipate that our expenses will increase substantially if and as we:

- continue to develop and conduct nonclinical studies and clinical trials with respect to our product candidates;
- seek to identify additional product candidates;
- in-license or acquire additional product candidates;
- seek marketing approvals for our product candidates that successfully complete clinical trials;
- establish sales, marketing, distribution and other commercial infrastructure in the future to commercialize various products for which we may obtain marketing approval;
- require the manufacture of larger quantities of product candidates for clinical development and potentially commercialization;
- maintain, expand and protect our intellectual property portfolio;
- incur delays to the initiation or completion of our clinical trials due to the COVID-19 pandemic;
- incur any disruptions or delays to the supply of our product candidates due to the COVID-19 pandemic;
- hire additional personnel;
- add equipment and physical infrastructure to support our research and development; and
- continue to implement the infrastructure necessary to support our product development and help us comply with our obligations as a public company.

Our ability to become and remain profitable depends on our ability to generate revenue. We do not expect to generate significant revenue unless and until we are, or one of our collaborators is, able to successfully commercialize one or more of our product candidates. Doing so will require success in a range of challenging activities, including completing clinical trials of our product candidates, obtaining marketing approval for these product candidates, manufacturing, marketing and selling those products for which we, or our collaborators, may obtain marketing approval, satisfying any post-marketing requirements and obtaining reimbursement for our products from private insurance or government payors. We, and our collaborators, may never succeed in these activities and, even if we do, or one of our collaborators does, we may never generate revenues that are large enough for us to achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our Company and could impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our pipeline of product candidates or continue our operations. A decline in the value of our Company could cause our stockholders to lose all or part of their investments in us.

***We have a limited operating history and no history of commercializing pharmaceutical products, which may make it difficult to evaluate the prospects for our future viability.***

We began operations in April 2006. Our operations to date have been limited to financing and staffing our Company, developing our technology and product candidates and establishing collaborations. We have not yet demonstrated an ability to successfully conduct an international multi-center clinical trial, conduct a large-scale pivotal clinical trial, obtain marketing approvals, manufacture product on a commercial scale or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. Consequently, predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing pharmaceutical products.

***We will need substantial additional funding. If we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product development programs or commercialization efforts.***

Developing pharmaceutical products, including conducting nonclinical studies and clinical trials, is a very time-consuming, expensive and uncertain process that takes years to complete. We expect our expenses to increase in connection with our ongoing activities, particularly as we initiate new clinical trials of, initiate new research and nonclinical development efforts for and seek marketing approval for our product candidates, or if we in-license or acquire product candidates. In addition, if we obtain marketing approval for any of our product candidates, we may incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution to the extent that such sales, marketing and distribution are not the responsibility of one of our collaborators. In particular, the costs that we may be required to incur for the manufacture of any product candidate that receives marketing approval may be substantial. Manufacturing a drug at commercial scale may require specialized facilities, processes and materials. Furthermore, we will continue to incur costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we may be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts.

In any event, our existing cash and cash equivalents and investments will not be sufficient to fund all of the efforts that we plan to undertake or to fund the completion of development of any of our product candidates. Accordingly, we will be required to obtain further funding through a combination of equity offerings, debt financings, collaboration and licensing arrangements and other sources. Adequate additional financing may not be available to us on acceptable terms, or at all. Market volatility resulting from the COVID-19 pandemic or other factors could also adversely impact our ability to access capital as and when needed. Our failure to raise capital when needed would have a negative impact on our financial condition and our ability to pursue our business strategy.

We believe that our cash and cash equivalents and investments as of June 30, 2020 will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2021. Our estimate as to how long we expect our cash and cash equivalents and investments to be able to continue to fund our operations is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Changing circumstances could cause us to consume capital significantly faster than we currently anticipate, and we may need to spend more money than currently expected because of circumstances beyond our control. Our future funding requirements, both short-term and long-term, will depend on many factors, including:

- the progress, timing, costs and results of clinical trials of, and research and nonclinical development efforts for, our product candidates and potential product candidates, including current and future clinical trials, including on account of the COVID-19 pandemic and its impact on our clinical trial sites;
- our current collaboration agreements and achievement of milestones under these agreements;
- our ability to enter into and the terms and timing of any additional collaborations, licensing, product acquisition or other arrangements that we may establish;
- the number of product candidates that we pursue and their development requirements;
- the outcome, timing and costs of seeking marketing approvals;
- our headcount growth and associated costs as we expand our research and development and establish a commercial infrastructure;
- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against intellectual property related claims;
- potential litigation costs; and
- the costs of operating as a public company.

***Raising additional capital may cause dilution to our stockholders or require us to relinquish rights to our technologies or product candidates.***

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaboration and licensing arrangements and other sources. We do not have any committed external source of funds, other than potential milestone payments under the Vertex Agreement and potential milestone payments and royalties under our existing license agreements, each of which is subject to the achievement of development, regulatory and/or sales-based milestones with respect to our product candidates. To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity securities, the ownership interests of our stockholders may be materially diluted, and the terms of these securities could include liquidation or other preferences and anti-dilution protections that could adversely affect the rights of our stockholders. In addition, debt financing, if available, would result in increased fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, that could adversely impact our ability to conduct our business.

If we raise additional funds through collaborations or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates, or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

***Any future indebtedness could adversely affect our ability to operate our business.***

We could in the future incur indebtedness containing financial obligations and restrictive covenants, which could have significant adverse consequences, including:

- requiring us to dedicate a portion of our cash resources to the payment of interest and principal, reducing money available to fund working capital, capital expenditures, product development and other general corporate purposes;
- increasing our vulnerability to adverse changes in general economic, industry and market conditions;
- subjecting us to restrictive covenants that may reduce our ability to take certain corporate actions or obtain further debt or equity financing;
- limiting our flexibility in planning for, or reacting to, changes in our business and the industry in which we compete; and
- placing us at a competitive disadvantage compared to our competitors that have less debt or better debt servicing options.

Any financial obligations or restrictive covenants could negatively impact our ability to conduct our business.

**RISKS RELATED TO THE DISCOVERY, DEVELOPMENT AND COMMERCIALIZATION OF OUR PRODUCT CANDIDATES**

***Clinical drug development involves a lengthy and expensive process with an uncertain outcome.***

Clinical testing is expensive, time-consuming and uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. The clinical development of our product candidates is susceptible to the risk of failure inherent at any stage of drug development, including failure to demonstrate efficacy in a clinical trial or across a broad or definable population of patients, the occurrence of severe or medically or commercially unacceptable adverse events, fraudulent conduct by clinical investigators, failure to comply with protocols, applicable regulatory requirements or other determinations made by the FDA, or any comparable foreign regulatory authority that a drug product is not approvable. It is possible that even if one or more of our product candidates has a beneficial effect, that effect will not be detected during clinical evaluation as a result of one or more of a variety of factors, including the size, duration, design, measurements, conduct or analysis of our clinical trials. Conversely, as a result of the same factors, our clinical trials may indicate an apparent positive effect of a product candidate that is greater than the actual positive effect, if any. Similarly, in our clinical trials, we may fail to detect toxicity of or intolerance caused by our product candidates, or mistakenly believe that our product candidates are toxic or not well tolerated when that is not in fact the case.

In addition to the risk of failure inherent in drug development, certain of the deuterated compounds that we, and our collaborators, are developing and may develop in the future may be particularly susceptible to failure to the extent they are

based on compounds that others have previously studied or tested, but did not progress in development due to safety, tolerability or efficacy concerns or otherwise. Deuteration of these compounds may not be sufficient to overcome the problems experienced with the corresponding non-deuterated compound.

***We may not be able to continue further clinical development of our wholly owned development programs, including CTP-543 and CTP-692. If we are unable to develop, obtain marketing approval for or commercialize our wholly owned development programs, ourselves or through a collaboration, or experience significant delays in doing so, our business could be materially harmed.***

We currently have no products approved for sale. The success of our wholly owned development programs will depend on several factors, including:

- in the case of CTP-543, our ability to treat moderate-to-severe alopecia areata with acceptable safety and efficacy;
- in the case of CTP-692, our ability to treat schizophrenia, in patients concurrently receiving antipsychotic medication, with acceptable safety and efficacy;
- successful and timely completion of clinical trials, including the impact of the COVID-19 pandemic on the initiation or completion of our clinical trials and the supply of our product candidates;
- receipt of marketing approvals from applicable regulatory authorities;
- the performance of our future collaborators, if any, for our programs;
- the extent of any required post-marketing approval commitments to applicable regulatory authorities;
- establishment of supply arrangements with third-party raw materials suppliers and manufacturers;
- our ability to manufacture or arrange for the manufacture of our active pharmaceutical ingredients and drug products with sufficient quality, quantity and reproducibility to support clinical trials and potential future commercialization;
- establishment of arrangements with third-party manufacturers to obtain finished drug products that are appropriately packaged for sale;
- obtaining and maintaining patent, trade secret protection, regulatory exclusivity and freedom to operate, both in the United States and internationally;
- amount of commercial sales, if and when approved;
- a continued acceptable safety profile of our programs following any marketing approval; and
- agreement by third-party payors to reimburse patients for the costs of treatment with our products, and the terms of such reimbursement.

If we are unable to successfully develop, receive marketing approval for and commercialize our wholly owned development programs, or experience delays as a result of any of these factors or otherwise, our business could be materially harmed.

***If clinical trials of our product candidates fail to satisfactorily demonstrate safety and efficacy to the FDA and other regulators, we, or our collaborators, may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of these product candidates.***

We, or our collaborators, must complete nonclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of our product candidates in humans in order to obtain marketing approval from regulatory authorities for the sale of our product candidates. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is inherently uncertain as to outcome. Further, the outcome of nonclinical studies and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. Moreover, nonclinical and clinical data are often susceptible to varying interpretations and analyses. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials after achieving positive results in earlier development, and we cannot be certain that we will not face similar setbacks.

Any inability to successfully complete nonclinical and clinical development could result in additional costs to us, or our collaborators, and impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. In addition, if (1) we, or our collaborators, are required to conduct additional or larger clinical trials or other testing of our product candidates beyond the trials and testing that we, or they, contemplate, (2) we, or our collaborators, are unable to successfully complete clinical trials of our product candidates or other testing, (3) the results of these trials or tests are unfavorable, uncertain or are only modestly favorable, or (4) there are unacceptable safety concerns associated with our product candidates, we, or our collaborators, in addition to incurring additional costs, may:

- be delayed in obtaining marketing approval for our product candidates;
- not obtain marketing approval at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;

- obtain approval with labeling that includes significant use or distribution restrictions or significant safety warnings, including boxed warnings;
- be subject to additional post-marketing testing or other requirements; or
- be required to remove the product from the market after obtaining marketing approval.

For instance, AVP-786 is being developed under a collaboration with Avanir. In 2019, Avanir completed two Phase 3 trials evaluating AVP-786 for the treatment of agitation associated with dementia of the Alzheimer's type. The second of the Phase 3 trials did not meet its primary or key secondary endpoints; however, following additional data analysis, Avanir decided to continue developing AVP-786 for the treatment of agitation associated with dementia of the Alzheimer's type in a number of ongoing and planned Phase 3 trials. Additionally, Avanir is conducting a Phase 2/3 trial evaluating AVP-786 for the treatment of negative symptoms of schizophrenia. However, given the results of Avanir's second Phase 3 trial of AVP-786 for the treatment of agitation associated with dementia of the Alzheimer's type, there is no guarantee that any future trials of AVP-786 will meet their primary or key secondary endpoints.

Even if we, or our collaborators, believe that the results of clinical trials for our product candidates warrant marketing approval, the FDA or comparable foreign regulatory authorities may disagree and may not grant marketing approval of our product candidates.

***If we, or our collaborators, experience any of a number of possible unforeseen events in connection with clinical trials of our product candidates, potential marketing approval or commercialization of our product candidates could be delayed or prevented.***

We, or our collaborators, may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent marketing approval of our product candidates, including:

- toxicity or serious adverse effects may be observed in our nonclinical studies causing us to delay or abandon clinical trials;
- clinical trials of our product candidates may produce unfavorable or inconclusive results;
- unexpectedly high placebo response rates;
- rater variability in the assessment of clinical endpoints;
- we, or our collaborators, may decide, or regulators may require us or them, to conduct additional clinical trials and or develop and or validate new clinical endpoints for our clinical trials, or abandon product development programs;
- the number of patients required for clinical trials of our product candidates may be larger than we, or our collaborators, anticipate, patient enrollment in these clinical trials may be slower than we, or our collaborators, anticipate or participants may drop out of these clinical trials at a higher rate than we, or our collaborators, anticipate;
- our third-party contractors, or those of our collaborators, including those manufacturing our product candidates or components or ingredients thereof or conducting clinical trials on our behalf or on behalf of our collaborators, may fail to comply with regulatory requirements or meet their contractual obligations to us or our collaborators in a timely manner or at all;
- criminal or unauthorized misuse of computer systems may result in disruption to our, or our partners' or vendors', clinical trials, nonclinical activities or manufacturing, or may compromise data from our, or our partners' or vendors', clinical or nonclinical studies;
- regulators or institutional review boards may not authorize us, our collaborators or our or their investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- we, or our collaborators, may have delays in reaching or fail to reach agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites;
- patients that enroll in a clinical trial may misrepresent their eligibility to do so or may otherwise not comply with the clinical trial protocol, resulting in the need to drop the patients or the sites from the clinical trial, increase the needed enrollment size for the clinical trial, extend the clinical trial's duration or cause spurious results;
- investigators may provide inaccurate or false data, resulting in spurious clinical results, an inadequate data set or regulators' unwillingness to approve a product;
- regulators, institutional review boards or data monitoring committees may require that we, or our collaborators, or our or their investigators suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or their standards of conduct, a finding that the participants are being exposed to unacceptable health risks, undesirable side effects or other unexpected characteristics of the product candidate or findings of undesirable effects caused by a chemically or mechanistically similar drug or drug candidate;
- the FDA or comparable foreign regulatory authorities may disagree with our, or our collaborators', clinical trial design or our or their interpretation of data from nonclinical studies and clinical trials;

- the FDA or comparable foreign regulatory authorities may change their requirements for approvability for a given product or for an indication after we have initiated work based on their previous guidance;
- the COVID-19 pandemic may impact the FDA's or comparable foreign regulatory authorities' ability to continue its normal operations;
- the supply or quality of raw materials or manufactured product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient, inadequate or not available at an acceptable cost, or we may experience interruptions in supply, including as a result of shipping delays or vendor personnel shortages due to the COVID-19 pandemic;
- we, or our manufacturing vendors, may not produce, or may not consistently produce material, that meets necessary specifications for commercialization;
- the FDA or comparable foreign regulatory authorities may determine that our, or our manufacturing vendors, manufacturing or quality control processes fail to meet their specifications or guidelines; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient to obtain marketing approval.

Product development costs for us, or our collaborators, will increase if we, or they, experience delays in testing or pursuing marketing approvals and we, or they, may be required to obtain additional funds to complete clinical trials and prepare for possible commercialization of our product candidates. We, and our collaborators, do not know whether any nonclinical tests or clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant nonclinical or clinical trial delays also could shorten any periods during which we, or our collaborators, may have the exclusive right to commercialize our product candidates or allow our competitors, or the competitors of our collaborators, to bring products to market before we, or our collaborators, do and impair our ability, or the ability of our collaborators, to successfully commercialize our product candidates and may harm our business and results of operations. In addition, many of the factors that cause, or lead to, clinical trial delays may ultimately lead to the denial of marketing approval of any of our product candidates.

Additionally, timely enrollment, conduct, progress and completion of clinical trials are reliant on clinical trial sites, which may be adversely affected by global health matters, including, among other things pandemics. For example, some of our clinical trial sites have been impacted by the COVID-19 pandemic. As the COVID-19 pandemic continues to evolve, the conduct of our clinical trials may be adversely affected, despite efforts to mitigate this impact.

***If we, or our collaborators, experience delays or difficulties in the enrollment of patients in clinical trials, our, or their, receipt of necessary marketing approvals could be delayed or prevented.***

We, or our collaborators, may not be able to initiate or continue clinical trials for any of our product candidates if we, or they, are unable to locate and enroll a sufficient number of eligible patients to participate in clinical trials as required by the FDA or comparable foreign regulatory authorities, such as the European Medicines Agency. Patient enrollment is a significant factor in the timing of clinical trials and is affected by many factors, including:

- the size and nature of the patient population;
- the severity of the disease under investigation;
- the availability or interest of clinical sites to enroll patients into our trials;
- the willingness or availability of patients to participate in our clinical trials, including due to the COVID-19 pandemic;
- the proximity of patients to clinical sites;
- the eligibility criteria for the trial;
- the design of the clinical trial, including any requirement to halt current therapy in connection with the trial;
- the potential need to discontinue investigational treatment at the completion of the study;
- the availability of other effective treatments for the indication we are assessing;
- access to relevant clinical trial sites;
- efforts to facilitate timely enrollment;
- competing clinical trials;
- support by relevant industry or patient organizations with influence over clinical trial sites; and
- clinicians' and patients' perceptions as to the potential advantages and risks of the drug being studied in relation to other available therapies, including any new drugs that may be approved or used for the indications we are investigating.

Our inability, or the inability of our collaborators, to enroll a sufficient number of patients for our, or their, clinical trials could result in significant delays or may require us or them to abandon one or more clinical trials altogether. Enrollment delays in our, or their, clinical trials may result in increased development costs for our product candidates, delay or halt the development of and approval processes for our product candidates and jeopardize our, or our collaborators', ability to commence sales of and

generate revenues from our product candidates, which could cause the value of our Company to decline and limit our ability to obtain additional financing, if needed.

***Breakthrough Therapy and Fast Track designations by the FDA may not lead to a faster development, regulatory review or approval.***

Although the FDA has granted CTP-543 Breakthrough Therapy designation for the treatment of adult patients with moderate-to-severe alopecia areata and Fast Track designation for the treatment of alopecia areata, Breakthrough Therapy and Fast Track designations do not necessarily lead to a faster development pathway or regulatory review process and do not increase the likelihood of marketing approval. The FDA may later withdraw the designations if it believes that CTP-543 no longer meets the necessary conditions.

***Serious adverse events, undesirable side effects or other unexpected properties of our product candidates, including those that we have licensed to collaborators, may be identified during development that could delay or prevent the product candidate's marketing approval.***

All of our product candidates are in nonclinical and clinical development stages and their risk of failure is high. Serious adverse events or undesirable side effects caused by our product candidates, or competitor products with similar mechanisms of action, could cause us, one of our collaborators, an institutional review board, data monitoring committee or regulatory authorities to interrupt, amend, delay or halt clinical trials of one or more of our product candidates and could result in a more restrictive label or the delay or denial of marketing approval by the FDA or comparable foreign regulatory authorities. A dose of a deuterated compound could, in comparison to an equal dose of the corresponding non-deuterated compound, result in altered exposure levels, distribution and half-life in the body and alter the levels of particular metabolites that are present in the body. These changes may cause serious adverse events or undesirable side effects that we, or our collaborators, did not anticipate, whether based on the characteristics of the corresponding non-deuterated compound or otherwise. If any of our product candidates is associated with serious adverse events or undesirable side effects or have properties that are unexpected, we, or our collaborators, may need to abandon development or limit development of that product candidate to certain uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. Many compounds that initially showed promise in clinical or earlier stage testing have later been found to cause undesirable or unexpected side effects that prevented further development of the compound. In addition, unexpected adverse clinical effects of a deuterated product candidate, including either those identified by us or deuterated analogs of approved drugs being developed by any third parties, may create general concerns regarding deuterium technology that could delay the development of our product candidates.

***High placebo rates are common in neuropsychiatric clinical trials, which may result in failed clinical trials.***

Placebo responses are common in clinical trials of many neuropsychiatric agents, and the rate of such responses has generally been increasing for decades in the United States and Europe. High placebo response rates can cause even effective agents to fail to achieve statistical significance on key endpoints, resulting in failed clinical trials. Even if our compounds demonstrate efficacy in Phase 2 studies, higher than expected placebo response rates may result in their failure in pivotal studies and our inability to obtain marketing approval. The COVID-19 pandemic may also have unforeseen effects on placebo responses in our clinical trials, particularly our Phase 2 clinical trial of CTP-692 for schizophrenia.

***Even if one of our product candidates receives marketing approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success and the market opportunity for the product candidate may be smaller than we estimate.***

Even if one of our product candidates, including those licensed to our collaborators, is approved by the appropriate regulatory authorities for marketing and sale, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors, formulary decision-makers and others in the medical or patient communities. For example, physicians are often reluctant to switch their patients from existing therapies even when new and potentially more effective or convenient treatments enter the market. Further, patients often acclimate to the therapy that they are currently taking and do not want to switch unless their physicians recommend switching products or they are required to switch therapies due to lack of reimbursement for existing therapies. If any of our product candidates receive negative publicity, patients may choose not to request them even if approved, or may not comply with taking them as prescribed.

Efforts to educate the medical community, patients, formulary decision-makers and third-party payors on the benefits of our product candidates may require significant resources and may not be successful. If any of our product candidates is approved but does not achieve an adequate level of market acceptance, we may not generate significant revenues and we may not become

profitable. The degree of market acceptance of our product candidates, including those licensed to our collaborators, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of the product;
- the potential advantages of the product compared to alternative treatments;
- the prevalence and severity of any side effects;
- the clinical indications for which the product is approved;
- whether the product is designated under physician treatment guidelines as a first-line therapy or as a second- or third-line therapy;
- limitations or warnings, including distribution or use restrictions or burdensome prescription requirements contained in the product's approved labeling;
- our ability, or the ability of our collaborators, to offer the product for sale at commercially acceptable prices;
- the product's convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try, and of physicians to prescribe, the product;
- the strength of sales, marketing and distribution support;
- the approval of other new products for the same indications;
- the extent and success of counter-detailing efforts by our competitors;
- the pricing, extent of discounts or bundled products offered by our competitors;
- the organization stability of our collaborators, if any;
- changes in the standard of care for the targeted indications for the product;
- the timing of market introduction of our approved products as well as competitive products; and
- the availability and amount of reimbursement from government payors, managed care plans and other third-party payors.

The potential market opportunities for our product candidates are difficult to precisely estimate. Our estimates of the potential market opportunities are predicated on many assumptions, including industry knowledge and publications, third-party research reports and other surveys. While we believe that our internal assumptions are reasonable, these assumptions involve the exercise of significant judgment on the part of our management, are inherently uncertain and the reasonableness of these assumptions has not been assessed by an independent source. If any of the assumptions proves to be inaccurate, the actual markets for our product candidates could be smaller than our estimates of the potential market opportunities.

***If any of our product candidates receives marketing approval and we, or others, later discover that the drug is less pure, homogeneous or stable than believed, less effective than previously believed, or causes undesirable side effects that were not previously identified or at a higher rate than was projected during clinical development, our ability to market the drug, or that of our collaborators, could be compromised.***

Clinical trials of our product candidates are conducted in carefully defined subsets of patients who have agreed to enter into clinical trials. Consequently, it is possible that these individuals are not representative of the actual patient population or that our clinical trials may indicate an apparent positive effect of a product candidate that is greater than the actual positive effect, if any, or alternatively fail to identify undesirable side effects. If, following approval of a product candidate, we, or others, discover that the drug is less effective than previously believed or causes undesirable side effects that were not previously identified, any of the following adverse events could occur:

- regulatory authorities may withdraw their approval of the drug and/or seize the drug;
- we, or our collaborators, may need to recall the drug or change the way the drug is administered;
- additional restrictions may be imposed on the marketing of, or the manufacturing processes for, the particular drug, including the addition of labeling statements, such as a "black box" warning or a contraindication;
- we may be subject to fines, injunctions or the imposition of civil or criminal penalties;
- we, or our collaborators, may be required to operate under a Risk Evaluation and Mitigation Strategy, or REMS;
- we, or our collaborators, could be sued and held liable for harm caused to patients; and
- the drug may become less competitive.

Any of these events could have a material and adverse effect on our operations and business and could adversely impact our stock price.

***If we are unable to establish sales, marketing and distribution capabilities or enter into sales, marketing and distribution arrangements with third parties, we may not be successful in commercializing any product candidates that we develop if and when those product candidates are approved.***

We do not have a sales, marketing or distribution infrastructure and as a company have no experience in the sale, marketing or distribution of pharmaceutical products. To achieve commercial success for any approved product, we must either develop a sales and marketing organization or outsource these functions to third parties. We expect to use a combination of third-party collaboration, licensing and distribution arrangements and a focused in-house commercialization capability to sell any products that receive marketing approval.

We generally plan to seek to retain full commercialization rights for the United States for products that we can commercialize with a specialized sales force and to retain co-promotion or similar rights for the United States when feasible in indications requiring a larger commercial infrastructure. The development of sales, marketing and distribution capabilities will require substantial resources, will be time-consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing and distribution capabilities is delayed or does not occur for any reason, we could have prematurely or unnecessarily incurred these commercialization costs. This may be costly, and our investment could be lost if we cannot retain or reposition our sales and marketing personnel. In addition, we may not be able to hire or retain a sales force in the United States that is sufficient in size or has adequate expertise in the medical markets that we plan to target. If we are unable to establish or retain a sales force and marketing and distribution capabilities, our operating results may be adversely affected. If a potential partner has development or commercialization expertise that we believe is particularly relevant to one of our products, then we may seek to collaborate with that potential partner even if we believe we could otherwise develop and commercialize the product independently.

We currently expect to collaborate with third parties for commercialization in the United States of any products that require a large sales, marketing and product distribution infrastructure. We also expect to commercialize our product candidates outside the United States through collaboration, licensing and distribution arrangements with third parties, if at all. As a result of entering into arrangements with third parties to perform sales, marketing and distribution services, our product revenues or the profitability of these product revenues may be lower, perhaps substantially lower, than if we were to directly market and sell products in those markets. Furthermore, we may be unsuccessful in entering into the necessary arrangements with third parties or may be unable to do so on terms that are favorable to us. In addition, we may have little or no control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively, or may actively sell a competing product at the expense of selling ours.

If we do not establish sales and marketing capabilities, either on our own or in collaboration with third parties, we will not be successful in commercializing any of our product candidates that receive marketing approval.

***We face substantial competition from other pharmaceutical and biotechnology companies and our operating results may suffer if we fail to compete effectively.***

The development and commercialization of new drug products is highly competitive. We expect that we, and our collaborators, will face significant competition from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide with respect to our product candidates that we, or they, may seek to develop or commercialize in the future. Specifically, there are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of product candidates for the treatment of autoimmune and CNS disorders, which are key indications for our development programs. Our competitors may succeed in developing, acquiring or licensing technologies and drug products that attain preferred reimbursement by payors or are more effective, simpler to use, have fewer or more tolerable side effects or are less costly than any product candidates that we are currently developing or that we may develop or acquire, or which are marketed more effectively, which could render our product candidates obsolete and noncompetitive.

We are developing CTP-543 as an oral agent for the treatment of moderate-to-severe alopecia areata. If CTP-543 receives marketing approval for this indication, it may face competition from a number of other product candidates that are being studied for alopecia areata. A number of companies are pursuing development of oral JAK inhibitors with a range of subtype selectivities for the treatment of alopecia areata, including Eli Lilly and Company and Pfizer Inc.

We are developing CTP-692 as an oral adjunctive treatment of schizophrenia. A number of companies are pursuing development for adjunctive treatment of schizophrenia, exploring cognitive or negative symptoms of the disease, including SyneuRx International [Taiwan] Corp.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we, or our collaborators, may develop. Our competitors also may obtain FDA or other marketing approval for their products before we, or our collaborators, are able to obtain approval for ours, which could reduce our ability to utilize expedited

regulatory pathways and could result in our competitors establishing a strong market position before we, or our collaborators, are able to enter the market.

Many of our existing and potential future competitors have significantly greater financial resources and expertise in research and development, manufacturing, nonclinical testing, conducting clinical trials, obtaining marketing approvals and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

***If the FDA or comparable foreign regulatory authorities approve generic versions of any of our products that receive marketing approval, or such authorities do not grant our products appropriate periods of data exclusivity before approving generic versions of our products, the sales of our products could be adversely affected.***

Once a new drug application, or NDA, is approved, the product covered thereby becomes a "reference listed drug" in the FDA's publication, "Approved Drug Products with Therapeutic Equivalence Evaluations." Manufacturers may seek approval of generic versions of reference listed drugs through submission of abbreviated new drug applications, or ANDAs, in the United States. In support of an ANDA, a generic manufacturer need not conduct clinical studies. Rather, the applicant generally must show that its product has the same active ingredient(s), dosage form, strength, route of administration and conditions of use or labeling as the reference listed drug and that the generic version is bioequivalent to the reference listed drug, meaning it is absorbed in the body at the same rate and to the same extent. Generic products may be significantly less costly to bring to market than the reference listed drug and companies that produce generic products are generally able to offer them at lower prices. Thus, following the introduction of a generic drug, a significant percentage of the sales of any branded product or reference listed drug is typically lost to the generic product.

The FDA may not approve an ANDA for a generic product until any applicable period of non-patent exclusivity for the reference listed drug has expired. The Federal Food, Drug, and Cosmetic Act, or FDCA, provides a period of five years of non-patent exclusivity for a new drug containing a new chemical entity. Specifically, in cases where such exclusivity has been granted, an ANDA may not be filed with the FDA until the expiration of five years unless the submission is accompanied by a Paragraph IV certification that a patent covering the reference listed drug is either invalid or will not be infringed by the generic product, in which case the applicant may submit its application four years following approval of the reference listed drug. While we believe that our product candidates contain active ingredients that would be treated as new chemical entities by the FDA and, therefore, if approved, should be afforded at least five years of data exclusivity, the FDA may disagree with that conclusion and may approve generic products after a period that is less than five years. Manufacturers may seek to launch these generic products following the expiration of the applicable marketing exclusivity period, even if we still have patent protection for our product.

Competition that our products may face from generic versions of our products could materially and adversely impact our future revenue, profitability and cash flows and substantially limit our ability to obtain a return on the investments we have made in those product candidates.

***To the extent we, or our collaborators, market products that are deuterated analogs of generic drugs that are approved or will be approved while we market our products in territories in which the generic drug is available, our products may compete against these generic products and the sales of our products could be adversely affected.***

We anticipate that some of the products that we, or our collaborators, may develop will be deuterated analogs of approved drugs that are or will then be available on a generic basis. In addition, if we develop a product that is a deuterated analog of a non-generic approved drug, the FDA or comparable foreign regulatory authorities may also approve generic versions of the corresponding non-deuterated drug. If approved, we expect that our deuterated products will compete against these generic non-deuterated compounds if they are used in the same indications. Even if the approved indications are different for the deuterated and non-deuterated drugs, the generic non-deuterated drug may be used off-label, negatively affecting sales of our product. Efforts to educate the medical community and third-party payors on the benefits of any product that we develop as compared to the corresponding non-deuterated compound, or generic versions of it, may require significant resources and may not be successful. If physicians, rightly or wrongly, do not believe that a product that we, or our collaborators, develop offers substantial advantages over the corresponding non-deuterated compound, or generic versions of the corresponding non-deuterated compound, or that the advantages offered by our product as compared to the corresponding non-deuterated compound, or its generic versions, are not sufficient to merit the increased price over the corresponding non-deuterated

compound, or its generic versions, that we, or our collaborators, would seek, physicians might not prescribe that product. In addition, third-party payors may refuse to provide reimbursement for a product that we, or our collaborators, develop when the corresponding non-deuterated compound, or generic versions of the corresponding non-deuterated compound, offer a cheaper alternative therapy in the same indication, or may otherwise encourage use of the corresponding non-deuterated compound, or generic versions of the corresponding non-deuterated compound, over our product, even if our product possesses favorable pharmaceutical properties or is labeled for a different indication.

Competition that our product candidates may face from any generic non-deuterated product on which our product candidate is based or a later-approved generic version of a branded non-deuterated product on which our product is based, could materially and adversely impact our future revenue, profitability and cash flows and substantially limit our ability to obtain a return on the investments we have made in those product candidates.

***Even if we, or our collaborators, are able to commercialize any product candidate that we, or they, develop, the product may become subject to unfavorable pricing regulations, third-party payor reimbursement practices or healthcare reform initiatives that could harm our business.***

The commercial success of our product candidates will depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations or reimbursed by government health administration authorities, private health coverage insurers and other third-party payors. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will cover and establish reimbursement levels. The healthcare industry is acutely focused on cost containment, both in the United States and elsewhere. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications, which could affect our ability or that of our collaborators to sell our product candidates profitably. These payors may not view our products, if any, as cost-effective, and coverage and reimbursement may not be available to our customers, or those of our collaborators, or may not be sufficient to allow our products, if any, to be marketed on a competitive basis. Cost-control initiatives could cause us, or our collaborators, to decrease the price we, or they, might establish for products, which could result in lower than anticipated product revenues. If reimbursement is not available, or is available only to limited levels, we, or our collaborators, may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us, or our collaborators, to establish or maintain pricing sufficient to realize a sufficient return on our, or their, investments.

There is significant uncertainty related to third-party payor coverage and reimbursement of newly approved drugs. Marketing approvals, pricing and reimbursement for new drug products vary widely from country to country. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we, or our collaborators, might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay commercial launch of the product, possibly for lengthy time periods, which may negatively impact the revenues we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability, or the ability of our collaborators, to recoup our, or their, investment in one or more product candidates, even if our product candidates obtain marketing approval.

Third-party payor coverage of newly approved drugs may be more limited than the indications for which the drugs are approved by the FDA or comparable foreign regulatory authorities. Moreover, eligibility for reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Reimbursement rates may vary, by way of example, according to the use of the drug and the clinical setting in which it is used. Reimbursement rates may also be based on reimbursement levels already set for lower cost drugs or may be incorporated into existing payments for other services.

In addition, increasingly, third-party payors are requiring higher levels of evidence of the benefits and clinical outcomes of new technologies, requiring burdensome comparison studies with currently approved drugs and challenging the prices charged. We, and our collaborators, cannot be sure that coverage will be available for any product candidate that we, or they, commercialize and, if available, that the reimbursement rates will be adequate. Further, the net reimbursement for drug products may be subject to additional reductions if there are changes to laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. An inability to promptly obtain coverage and adequate payment rates from both government-funded and private payors for any product candidates for which we, or our collaborators, obtain marketing approval could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

***We may not be successful in our efforts to identify or discover additional potential product candidates.***

If we are unable to identify suitable additional compounds for nonclinical and clinical development, our ability to develop product candidates and obtain product revenues in future periods could be compromised, which could result in significant harm to our financial position and adversely impact our stock price.

***Product liability lawsuits against us could divert our resources, cause us to incur substantial liabilities and limit commercialization of any products that we may develop.***

We face an inherent risk of product liability claims as a result of the clinical testing of our product candidates despite obtaining appropriate informed consents from our clinical trial participants. We will face an even greater risk if we, or our collaborators, commercially sell any product that we may, or they may, develop. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our product candidates. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased demand for our product candidates or products that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend litigation;
- distraction to our management diverting focus from business operations and strategy;
- initiation of investigations by regulators;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- substantial monetary awards to trial participants or patients;
- loss of revenue; and
- the inability to commercialize any products that we may develop.

Although we maintain product liability insurance coverage, it may not fully cover potential liabilities that we may incur. The cost of any product liability litigation or other proceeding, even if resolved in our favor, could be substantial. We will need to increase our insurance coverage if and when we begin selling any product candidate that receives marketing approval. In addition, insurance coverage is becoming increasingly expensive. If we are unable to obtain or maintain sufficient insurance coverage at an acceptable cost or to otherwise protect against potential product liability claims, it could prevent or inhibit the development and commercial production and sale of our product candidates, which could adversely affect our business, financial condition, results of operations and prospects.

***The increasing use of social media platforms presents risks and challenges.***

The increasing use of social media platforms presents risks and challenges. Social media increasingly is being used by third parties to communicate about our product candidates and the diseases they are designed to treat. We believe that members of the alopecia areata community may be more active on social media as compared to other patient populations due to the demographics of this patient population. Social media practices in the pharmaceutical and biotechnology industries are evolving, which creates uncertainty and risk of noncompliance with regulations applicable to our business. For example, patients in clinical trials may use social media platforms to comment on the effectiveness of, or adverse experiences with, a product candidate, which could result in reporting obligations. In addition, there is a risk of inappropriate disclosure of sensitive information or negative or inaccurate posts or comments about us on any social networking website. If any of these events were to occur or we otherwise fail to comply with applicable regulations, we could incur liability, face restrictive regulatory actions or incur other harm to our business.

## **RISKS RELATED TO OUR DEPENDENCE ON THIRD PARTIES**

***We depend on collaborations with third parties for the development and commercialization of some of our product candidates and expect to continue to do so in the future. Our prospects with respect to those product candidates will depend in significant part on the success of those collaborations.***

We have entered into collaborations for the development and commercialization of certain of our product candidates and expect to enter into additional collaborations in the future. We have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our product candidates, and our ability to generate revenues from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements. In addition, our collaborators have the right to abandon research or development projects and terminate applicable agreements, including funding obligations, prior to or upon the expiration of the agreed upon terms.

Collaborations involving our product candidates pose a number of risks, including:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;
- collaborators may not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs, based on clinical trial results, changes in the collaborators' strategic focus or available funding or external factors, such as an acquisition, that divert resources or create competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators may conduct their clinical trials poorly or inadequately, harming our products, including our products' development in other territories;
- product candidates developed in collaboration with us, including in particular product candidates based on deuteration of a collaborator's marketed drugs or advanced clinical candidates, may be viewed by our collaborators as competitive with their own product candidates or products, which may cause collaborators to cease to devote resources to the commercialization of our product candidates;
- a collaborator with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such product or products;
- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or termination of the research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- collaborators may steal our trade secrets or may hire valuable employees from us;
- collaborators may fail to protect our trade secrets;
- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.

Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If a collaborator of ours is involved in a business combination, it could decide to delay, diminish or terminate the development or commercialization of any product candidate licensed to it by us.

***We expect to seek to establish additional collaborations, and if we are not able to establish them on commercially reasonable terms, we may have to alter our development and commercialization plans.***

Our drug development programs and the potential commercialization of our product candidates will require substantial additional cash to fund expenses. We may seek one or more collaborators for the development and commercialization of one or more of our product candidates.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the potential differentiation of our product candidate from its corresponding non-deuterated analog, design or results of clinical trials, the likelihood of approval by the FDA or comparable foreign regulatory authorities and the regulatory pathway for any such approval, the potential market for the product candidate, the proposed collaborator's perception of our freedom to operate in a particular market or markets without challenge, the costs and complexities of manufacturing and delivering the product to patients and the potential of competing products. The collaborator may also consider alternative product candidates or technologies that may be available for collaboration and whether such collaboration could be more attractive than the one with us for our product candidate.

Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators. We are also restricted under the terms of certain of our existing collaboration agreements from entering

into collaborations regarding or otherwise developing specified compounds that are similar to the compounds that are subject to those agreements and collaboration agreements that we enter into in the future may contain further restrictions on our ability to enter into potential collaborations or to otherwise develop specified compounds.

We may not be able to negotiate collaborations for our product candidates on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to limit the development of the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate product revenue. In cases where we seek a collaborator for a product compound that is a deuterated analog of a compound that has been previously developed, failure to enter into a collaboration with the developer of the corresponding non-deuterated compound may result in a loss of the potential to obtain clearance from the FDA to follow expedited development programs that reference and rely on findings previously obtained from the developer's prior nonclinical or clinical studies of the corresponding non-deuterated compound.

***We rely on third parties to conduct our clinical trials and some aspects of our research and nonclinical testing. If they terminate their relationships with us or do not perform satisfactorily, our business may be materially harmed.***

We do not independently conduct clinical trials of any of our product candidates. We rely on third parties, such as contract research organizations, clinical data management organizations, medical institutions and clinical investigators, to conduct these clinical trials and expect to rely on these third parties to conduct clinical trials of any other product candidate that we develop. We also rely on third parties to conduct some aspects of our research and nonclinical testing and expect to rely on these third parties in the future. Any of these third parties may terminate their engagements with us under certain circumstances. If any of our relationships with these third parties terminate, we may not be able to enter into arrangements with alternative third parties on commercially reasonable terms, or at all. Switching to or adding additional third parties would involve additional cost and require management time and focus. In addition, there is a natural transition period when a new third party commences work, which could result in delays in our product development activities. Although we seek to carefully manage our relationships with our contract research organizations, any such challenges or delays could have a material adverse impact on our business, financial condition and prospects.

Our reliance on these third parties for clinical development activities limits our control over these activities but we remain responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol, legal and regulatory requirements and scientific standards. For example, notwithstanding the obligations of a contract research organization for a trial of one of our product candidates, we remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with standards commonly referred to as current Good Clinical Practices, or GCPs, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. The FDA enforces these GCPs through periodic inspections of trial sponsors, principal investigators, clinical trial sites and institutional review boards. If we or our third-party contractors fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA may require us to perform additional clinical trials before approving our product candidates, which would delay the marketing approval process. We cannot be certain that, upon inspection, the FDA will determine that any of our clinical trials comply with GCPs.

Furthermore, these third parties are not our employees, and except for remedies available to us under our agreements with such contractors, we cannot control whether or not they devote sufficient time, skill and resources to our ongoing development programs. These contractors may also have relationships with other commercial entities, including our competitors, which could impede their ability to devote appropriate time to our clinical programs. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct their services in accordance with our contracts, regulatory requirements or our stated protocols, we may not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates. If that occurs, we will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates. In such an event, our financial results and the commercial prospects for any product candidates that we seek to develop could be harmed, our costs could increase and our ability to generate revenues could be delayed, impaired or foreclosed.

We also rely on other third parties to store, label and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of our product candidates or commercialization of any resulting products, producing additional losses and depriving us of potential product revenue.

We are also required to register clinical trials and post the results of completed clinical trials on a government-sponsored database, such as ClinicalTrials.gov, within certain timeframes. Failure to do so can result in the inability to report our clinical results in certain publications, fines, adverse publicity and civil and criminal sanctions.

***Because there are limited sources of deuterium, we, and our collaborators, are exposed to a number of risks and uncertainties associated with our deuterium supply.***

We believe that all of the deuterium that we use in manufacturing our product candidates is currently derived, directly or indirectly, from deuterium oxide. For most of our deuterium supply, we rely on bulk supplies of deuterium oxide which we currently source from multiple suppliers, including two located in North America, one of which is in the United States.

In order to internationally transport any deuterium oxide that we purchase from our current or potential future foreign suppliers, we, or our suppliers, may be required to obtain an export license from the country of origin and we may be required to obtain an International Import Certificate or other governmental approvals or assurances from the country of destination. We are also required to obtain an export license from the Nuclear Regulatory Commission before shipping deuterium oxide from the United States to any contract manufacturer in another country. Export licenses and certain other required documents may specify the maximum amount of deuterium oxide that we, or our suppliers, are permitted to either import or export. In order for us to obtain supplies of deuterium oxide from foreign suppliers, they may be required to obtain an export license from the country of origin and we may be required to obtain domestic governmental approvals or assurances. In addition, our current U.S. export licenses may be insufficient to meet our future requirements. We, or our suppliers, may not be able to obtain such licenses, approvals or assurances in a timely manner or at all.

Certain of our manufacturing processes for our product candidates incorporate deuterium by using deuterated chemical intermediates or reagents that are derived from deuterium oxide. For the deuterated chemical intermediates and reagents, we are not subject to the license requirements applicable to deuterium oxide; however, the manufacturer of the deuterated chemical intermediate or reagent may themselves be required to obtain deuterium oxide under applicable licensing requirements. Many of the manufacturers of these deuterated chemical intermediates and reagents are not located in countries that produce bulk quantities of deuterium oxide. Therefore, our ability to source these deuterated chemical intermediates may depend on the ability of these manufacturers to obtain deuterium oxide from other countries. Certain countries may also limit or prohibit the export of deuterium-containing products or intermediates. In the future we may arrange for supplies of deuterated chemical intermediates or reagents from manufacturers located in countries from which they can source deuterium oxide in bulk. However, contract manufacturers in these countries may not represent a viable alternative to our current suppliers. We do not have long-term agreements with our suppliers of deuterated chemical intermediates or reagents and we obtain some of these deuterated chemical intermediates or reagents from single sources, putting us at risk of uncontrolled cost increases or supply interruptions if we cannot establish alternative sourcing arrangements. Deuterated chemical intermediates may be expensive or difficult to obtain or may be produced by specialized techniques that are not widely practiced, and we may not be able to enter into arrangements for larger scale supply of deuterated chemical intermediates on acceptable terms, or at all.

We estimate that our current sources of deuterium oxide will be sufficient to meet our anticipated requirements; however, we do not have long-term agreements with our current suppliers. If we are not able to establish or maintain supply arrangements, or any relevant foreign governments decide to withhold authorizations for the export of deuterium oxide that we seek, we may be unable to secure alternative sources. If we are unable to obtain sufficient supplies of deuterium oxide from our current suppliers or our potential future foreign supplier, we would be forced to either seek alternative suppliers of deuterium oxide, likely in other countries, or alternative sources of deuterium. Such alternative supplies may not be available to us on acceptable terms, or at all.

If we are unable to obtain sufficient supplies of deuterium, our ability to produce our product candidates would be impeded and our business, financial condition and prospects could be harmed. In particular, certain of our manufacturing processes are projected to require particularly large quantities of deuterium for late-stage clinical trials and for commercialization. Consequently, any adverse impact on our ability to obtain deuterium oxide from our current suppliers, import deuterium oxide into the United States or export deuterium oxide to our contract manufacturers could have a particularly severe impact on our ability to develop or commercialize those product candidates.

Similarly, to develop and commercialize any of our licensed product candidates, our collaborators will need to obtain supplies of deuterium and will be subject to risks and requirements in connection with sourcing deuterium that are similar to the ones that we face. In addition, if any of our product candidates is approved by the FDA, then the FDA will also have regulatory jurisdiction over the manufacture and use of deuterium oxide and deuterated chemical intermediates or reagents in such products. Any adverse impact on our, or our collaborators', ability to obtain deuterium could delay or prevent the development or commercialization of our product candidates, which could have a material adverse effect on our business.

***We contract with third parties for the manufacture and distribution of our product candidates for nonclinical and clinical testing and expect to continue to do so in connection with our future development and commercialization efforts. This reliance on third parties increases the risk that we will not have sufficient quantities of our product candidates or such quantities at an acceptable cost, or that the product candidates will not be of sufficient quality or reproducibility or produced on our desired schedule, which could delay, prevent or impair our development or commercialization efforts.***

We currently rely, and expect to continue to rely, on third-party contractors to manufacture nonclinical and clinical supplies of our product candidates and to package, label and ship these supplies. We expect to rely on third-party contractors to manufacture, formulate, package, label and distribute commercial quantities of any product candidate that we commercialize following approval for marketing by applicable regulatory authorities. Reliance on such third-party contractors entails risks, including:

- manufacturing delays, including if our third-party contractors give greater priority to the supply of other products over our product candidates or if they otherwise do not satisfactorily perform according to the terms of the agreements between us and them;
- potentially incorrect data analysis, resulting in falsely-positive, falsely-negative or misleading or uninterpretable results;
- potential industrial accidents such as fires or explosions that compromise our product candidates or the ability of the contractors to timely deliver them;
- natural disasters, public health crises, pandemics and epidemics, including the COVID-19 pandemic;
- the possible termination or non-renewal of agreements by our third-party contractors at a time that is costly or inconvenient for us;
- potentially limited numbers of available contractors due to the need for uncommon equipment or expertise, or pre-existing conflicts of interest;
- the possible breach by the third-party contractors of our agreements with them;
- possible theft of intellectual property or trade secrets;
- possible theft of our materials, including starting materials, intermediates, active pharmaceutical ingredients or drug products;
- the failure of third-party contractors to comply with applicable regulatory requirements;
- the possible mislabeling of clinical supplies, potentially resulting in the wrong dose amounts being supplied or active drug or placebo not being properly identified;
- possible contamination, or non-conformance with product or packaging specifications, of our product during or after its manufacture;
- possible interruptions in our contractors' operations, including departure of key personnel, disruption due to merger and acquisitions activities or supply chain disruptions;
- the possibility of clinical supplies not being delivered to clinical sites on time, leading to clinical trial interruptions, or of drug supplies not being distributed to commercial vendors in a timely manner, resulting in lost sales; and
- the possible misappropriation of our proprietary information, including our trade secrets and know-how.

If any of our product candidates are approved by any regulatory agency, we plan to enter into agreements with third-party contract manufacturers for the commercial production and distribution of those products. It may be difficult for us to reach agreement with a contract manufacturer on satisfactory terms or in a timely manner, especially if the manufacturer believes it is uniquely suited to use our deuterium chemistry manufacturing processes or otherwise has unusual market power, or that our deuterium chemistry manufacturing processes bear greater production risks than manufacture of non-deuterated compounds. In addition, we may face competition for access to manufacturing facilities as there are a limited number of contract manufacturers operating under cGMPs that are capable of manufacturing our product candidates. Consequently, we may not be able to reach agreement with third-party manufacturers on satisfactory terms, which could delay our commercialization efforts.

Third-party manufacturers are required to comply with cGMPs and similar regulatory requirements outside the United States. Facilities used by our third-party manufacturers must be inspected by the FDA after we submit an NDA and before potential approval of the product candidate. Similar regulations apply to manufacturers of our product candidates for use or sale in foreign countries. We do not directly control the manufacturing process and are completely dependent on our third-party manufacturers for compliance with the applicable regulatory requirements for the manufacture of our product candidates. If our manufacturers fail to consistently manufacture material that conforms to the strict regulatory requirements of the FDA and any applicable foreign regulatory authority, or if they unacceptably deviate from standard operating procedures in the production of our product candidates, they will not be able to secure the applicable approval for or a regulatory authority may find deficiencies with their manufacturing facilities. If deficiencies are found at these facilities or if these facilities are not approved for commercial manufacture, we may need to find alternative manufacturing facilities, which could result in delays in obtaining approval for the applicable product candidate.

In addition, our manufacturers are subject to ongoing periodic inspections by the FDA and corresponding state and foreign agencies for compliance with cGMPs and similar regulatory requirements both prior to and following the receipt of marketing approval for any of our product candidates. Some of these inspections may be unannounced. Failure by any of our manufacturers to comply with applicable cGMPs or other regulatory requirements could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspensions or withdrawals of approvals, operating restrictions, interruptions in supply and criminal prosecutions, any of which could significantly and adversely affect supplies of our product candidates and have a material adverse impact on our business, financial condition and results of operations.

Our current and anticipated future dependence upon others for the manufacture of our product candidates may adversely affect our future profit margins and our ability to commercialize any products that receive marketing approval on a timely and competitive basis.

#### **RISKS RELATED TO OUR INTELLECTUAL PROPERTY**

***If we are unable to obtain and maintain sufficient patent protection for our product candidates, or if the scope of the patent protection is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours, and our ability to successfully commercialize our product candidates may be adversely affected.***

Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary product candidates. If we do not adequately protect our intellectual property, competitors may be able to erode or negate any competitive advantage we may have, which could harm our business and ability to achieve profitability. To protect our proprietary position, we file patent applications in the United States and abroad related to our novel product candidates that are important to our business. The patent application and approval process is expensive, uncertain and time-consuming. We may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. Neither deuterium itself, nor the general concept of selective substitution of deuterium for hydrogen in existing pharmaceutical compounds, is patentable; therefore, we usually seek patents on a compound-by-compound basis or on a relatively narrow genus of compounds. We are not guaranteed that patents will issue protecting any particular deuterated compound for which we seek patent protection. We also cannot guarantee that another company will not be able to find a different pattern of deuterium substitution that is equally or more effective in improving the characteristics of a non-deuterated compound, then patenting that deuterated compound and competing with us.

Our ability to obtain and maintain patent protection for our product candidates may be limited if disclosures of non-deuterated compounds are held to anticipate or make obvious claims of deuterated analogs of the same or similar compounds in any given territory. In addition, several large pharmaceutical and biotechnology companies have begun to pursue patent protection for deuterated analogs of their products and product candidates, and may in the future obtain patent protection that covers deuterated analogs of those product candidates. If patents directed primarily to non-deuterated compounds are deemed to protect deuterated analogs of those compounds or patent claims on deuterated analogs of compounds become common in the biotechnology and pharmaceutical industries, these factors may substantially limit our ability to seek and obtain patent protection for new product candidates based on deuterium modification of compounds. It may also limit our ability to develop new product candidates based on deuterium modification of such compounds without obtaining a license from those patent holders. In certain cases, a company that owns the patent on a non-deuterated compound may be able to file a continuation or divisional patent on deuterated analogs of their compounds that successfully claims priority to the original filing date of the non-deuterated composition, causing their patent to have priority over ours, even if filed later than ours was.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain. No consistent policy regarding the breadth of claims allowed in biotechnology and pharmaceutical patents has emerged to date in the United States or in many foreign jurisdictions. In addition, the determination of patent rights with respect to pharmaceutical compounds commonly involves complex legal and factual questions, which has in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain.

Assuming the other requirements for patentability are met, currently, the first to file a patent application is generally entitled to the patent. However, prior to March 16, 2013, in the United States, the first to invent was entitled to the patent. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions.

We may also become involved in opposition, derivation, reexamination, post grant review, or PGR, inter partes review, or IPR, or interference proceedings in the United States or elsewhere, challenging our patent rights or the patent rights of others. For

example, in April 2018, the Patent Trial and Appeal Board, or PTAB, instituted an IPR brought against our U.S. Patent No. 9,249,149, or the '149 patent, by Incyte Corporation, or Incyte. The '149 patent covers the composition of matter of deuterated analogs of ruxolitinib, including CTP-543. In April 2019, the PTAB issued a final written decision in connection with the IPR that held that the claims of the '149 patent were unpatentable as obvious. In January 2020, the U.S. Court of Appeals for the Federal Circuit, or Federal Circuit, granted our motion to vacate and remand the PTAB final written decision in light of the recent Federal Circuit ruling on the Constitution's Appointments Clause in *Arthrex, Inc. v. Smith & Nephew, Inc.*, 941 F.3d 1320 (Fed. Cir. 2019). As a result, the IPR was remanded for reconsideration by a new panel of PTAB judges. However, in May 2020, the PTAB issued an Order putting on hold the reconsideration of any IPR proceedings, including our IPR, that had been remanded by the Federal Circuit under *Arthrex* until the Supreme Court acts on the petitions for certiorari that have been filed related to *Arthrex*. The '149 patent remains valid and enforceable while the IPR is being reconsidered by the PTAB and until any future appeals by us have been exhausted in the event that the PTAB reaches a similar decision to invalidate the '149 patent. Adverse determinations in any such submission, proceeding or litigation such as this could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or product candidates and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights.

Our pending and future patent applications may not result in patents being issued that protect our product candidates, in whole or in part, or which effectively prevent others from commercializing competitive products. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection. In addition, the laws of foreign countries may not protect our rights to the same extent or in the same manner as the laws of the United States. For example, European patent law restricts the patentability of methods of treatment of the human body more than U.S. law does.

Even if our patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner. Our competitors may also seek approval to market their own products similar to or otherwise competitive with our products. Alternatively, our competitors may seek to market generic versions of any approved products by submitting ANDAs to the FDA in which they claim that patents owned or licensed by us are invalid, unenforceable or not infringed. In these circumstances, we may need to defend or assert our patents, or both, including by filing lawsuits alleging patent infringement. In any of these types of proceedings, a court or other agency with jurisdiction may find our patents invalid or unenforceable, or that our competitors are competing in a non-infringing manner. In certain territories, losses to an infringing product may not be sufficiently great to justify the costs of challenging the infringer and asserting our rights. In some situations, governments have allowed or enabled the sale of competing products that infringe a company's intellectual property. Thus, even if we have valid and nominally enforceable patents, these patents still may not provide protection against competing products or processes sufficient to achieve our business objectives.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad, including challenges through the PTO's PGR proceedings. Such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. In addition, given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized.

***If we are unable to protect the confidentiality of our trade secrets, the value of our technology could be materially adversely affected and our business would be harmed.***

While we have obtained composition of matter patents with respect to our most advanced product candidates, our DCE Platform is not patented. In seeking to develop and maintain a competitive position through our DCE Platform and as to other aspects of our business, we rely on trade secrets, including unpatented know-how, technology and other proprietary information. We seek to protect these trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our consultants, independent contractors, advisors, corporate collaborators, outside scientific collaborators, contract manufacturers, suppliers and other third parties. We also enter into confidentiality and invention or patent assignment agreements with employees and certain consultants. Any party with whom we have executed such an agreement may breach that agreement and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, if any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such third

party, or those to whom they communicate such technology or information, from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our business and competitive position could be harmed.

***Third parties may sue us alleging that we are infringing their intellectual property rights, and such litigation could be costly and time consuming and could prevent or delay us from developing or commercializing our product candidates.***

Our commercial success depends, in part, on our ability to develop, manufacture, market and sell our product candidates without infringing the intellectual property and other proprietary rights of third parties. Our CTP-543 compound is based, and potential future product candidates may be based, on products that are covered by issued patents or patent applications, the holders of which may attempt to assert claims against us. To date, we are not aware of any judicial decision holding that a patent that covers a non-deuterated compound should be construed to also cover deuterated analogs thereof, absent specific claims with respect to the deuterated analogs. However, any such judicial decision, or legal proceedings asserting such claims, could increase the likelihood of potential infringement claims being asserted against us. If any third-party patents or patent applications are found to cover our product candidates or their methods of use, we may not be free to manufacture or market our product candidates as planned without obtaining a license, which may not be available on commercially reasonable terms, or at all.

For example, CTP-543 is a deuterated analog of ruxolitinib. Incyte owns patents covering ruxolitinib that may be unexpired if and when we seek marketing approval for CTP-543. Incyte also owns a U.S. patent that broadly claims deuterated analogs of ruxolitinib. On June 27, 2017, we filed a PGR with the PTAB seeking to invalidate all claims of Incyte's U.S. patent that covers deuterated analogs of ruxolitinib. In January 2018, the PTAB did not grant our petition to challenge the validity of Incyte's patent. In May 2018, our request for reconsideration was denied.

In addition, Columbia University is the assignee of patents licensed to Aclaris Therapeutics, Inc. claiming the use of ruxolitinib, isotopic forms of ruxolitinib and other named JAK inhibitors for the treatment of hair loss disorders, including alopecia areata, which may be unexpired if and when we seek marketing approval for CTP-543.

There is a substantial amount of intellectual property litigation in the biotechnology and pharmaceutical industries, and we may become party to, or threatened with, litigation or other adversarial proceedings with respect to our product candidates. Third parties may assert infringement claims against us based on existing or future intellectual property rights. The outcome of intellectual property litigation is subject to uncertainties that cannot be adequately quantified in advance. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform. If we are sued for patent infringement, we would need to demonstrate that our product candidates, products or methods either do not infringe the relevant patent claims or that these patent claims are invalid or unenforceable, and we may not be able to do this. Proving invalidity is difficult. For example, in the United States, proving invalidity under most circumstances requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. We may also assert that a patent claim for a corresponding non-deuterated compound does not cover our product. Even if we are successful in these proceedings, we may incur substantial costs and the time and attention of our management and scientific personnel could be diverted in pursuing these proceedings, which could have a material adverse effect on us. In addition, we may not have sufficient resources to bring these actions to a successful conclusion.

If we are found to infringe a third party's intellectual property rights, we could be forced, including by court order, to cease developing, manufacturing or commercializing the infringing product candidate or product and could be required to pay potentially significant damages. Alternatively, we may be required to obtain a license from such third party in order to use the infringing technology and continue developing, manufacturing or marketing the infringing product candidate. However, we may not be able to obtain any required license on commercially reasonable terms, or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

***We may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time consuming and unsuccessful.***

Competitors may infringe our patents, trademarks, copyrights or other intellectual property. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time consuming and divert the time and attention of our management and scientific personnel. In any patent infringement proceeding, there is a risk that a

court will decide that a patent of ours is invalid or unenforceable, in whole or in part, and that we do not have the right to stop the other party from using the invention at issue. There is also a risk that, even if the validity and enforceability of such patents is upheld, the court will construe the patent's claims narrowly or decide that we do not have the right to stop the other party from using the invention at issue on the grounds that our patent claims do not cover the invention. An adverse outcome in a litigation or proceeding involving our patents could limit our ability to assert our patents against those parties or other competitors, and may curtail or preclude our ability to exclude third parties from making and selling similar or competitive products. Any of these occurrences could adversely affect our competitive business position, business prospects and financial condition.

Even if we establish infringement, the court may decide not to grant an injunction against further infringing activity and instead award only monetary damages, which may not be an adequate remedy. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during litigation. Moreover, there can be no assurance that we will have sufficient financial or other resources to file and pursue such infringement claims, which typically last for years before they are concluded. Even if we ultimately prevail in such claims, the monetary cost of such litigation and the diversion of the attention of our management and scientific personnel could outweigh any benefit we receive as a result of the proceedings.

## **RISKS RELATED TO MARKETING APPROVAL AND OTHER LEGAL COMPLIANCE MATTERS**

***Even if we complete the necessary nonclinical studies and clinical trials, the marketing approval process is expensive, time consuming and uncertain and we may not obtain approvals for the commercialization of some or all of our product candidates. As a result, we cannot predict when or if, and in which territories, we, or our collaborators, will obtain marketing approval to commercialize a product candidate.***

The research, testing, manufacturing, labeling, approval, selling, marketing, promotion and distribution of drug products are subject to extensive regulation by the FDA and comparable foreign regulatory authorities, which regulations differ from country to country. Failure to obtain marketing approval for a product candidate in a given territory will prevent us, and our collaborators, from commercializing the product candidate in that territory. Our product candidates are in various stages of development and are subject to the risks of failure inherent in drug development. We, and our collaborators, have not submitted an application for or received marketing approval for any of our product candidates in the United States or in any other jurisdiction. We have limited experience in filing and supporting the applications necessary to gain marketing approvals.

The process of obtaining marketing approvals, both in the United States and abroad, is lengthy, expensive and uncertain. It may take many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. This is the case even though the deuterated compounds that we produce and seek to develop can have similar pharmacological properties as their corresponding non-deuterated compounds. Even if, as a result of any such similarities, we, or our collaborators, obtain clearance from the FDA and other regulatory authorities to follow expedited development programs for some deuterated compounds that reference and rely on previous findings for non-deuterated compounds, the review and approval of our product candidates may still take a substantial period of time. Conversely, in certain countries regulators may consider our deuterated compounds to be equivalent to non-deuterated compounds that possess regulatory exclusivity and therefore refuse to approve our compounds until the expiration of that exclusivity.

In addition, changes in marketing approval policies during the development period, changes in or the enactment or promulgation of additional statutes, regulations or guidance or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional nonclinical, clinical or other studies. In addition, varying interpretations of the data obtained from nonclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate. Any marketing approval we, or our collaborators, ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable.

Any delay in obtaining or failure to obtain required approvals could materially adversely affect our ability, or that of our collaborators, to generate revenue from the particular product candidate, which likely would result in significant harm to our financial position and adversely impact our stock price.

***Failure to obtain marketing approval in international jurisdictions would prevent our product candidates from being marketed abroad.***

In order to market and sell our products in the European Union and many other jurisdictions, we, or our collaborators, must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The marketing approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many territories outside the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that territory. Our products may not receive commercially feasible prices in any given territory, or the price offered for our products in a territory may have an adverse effect on their prices in other territories if we were to accept. We, and our collaborators, may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA.

***Even if we, or our collaborators, obtain marketing approvals for our product candidates, the terms of approvals and ongoing regulation of our products may limit how we, or they, manufacture and market our products, which could materially impair our ability to generate revenue.***

Once marketing approval has been granted, an approved product and its manufacturer and marketer are subject to ongoing review and extensive regulation. We, and our collaborators, must therefore comply with requirements concerning advertising and promotion for any of our product candidates for which we or they obtain marketing approval. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved labeling. Thus, we, and our collaborators, will not be able to promote any products we develop for indications or uses for which they are not approved.

In addition, manufacturers of approved products and those manufacturers' facilities are required to comply with extensive FDA requirements, including ensuring that quality control and manufacturing procedures conform to cGMPs, which include requirements relating to quality control and quality assurance as well as the corresponding maintenance of records and documentation and reporting requirements. We, our contract manufacturers, our collaborators and their contract manufacturers could be subject to periodic unannounced inspections by the FDA to monitor and ensure compliance with cGMPs.

Accordingly, assuming we, or our collaborators, receive marketing approval for one or more of our product candidates, we, and our collaborators, and our and their contract manufacturers will continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance and quality control.

If we, and our collaborators, are not able to comply with post-approval regulatory requirements, we, and our collaborators, could have the marketing approvals for our products withdrawn by regulatory authorities and our, or our collaborators', ability to market any future products could be limited, which could adversely affect our ability to achieve or sustain profitability. Further, the cost of compliance with post-approval regulations may have a negative effect on our operating results and financial condition.

***Any of our product candidates for which we, or our collaborators, obtain marketing approval in the future could be subject to post-marketing restrictions or withdrawal from the market and we, or our collaborators, may be subject to substantial penalties if we, or they, fail to comply with regulatory requirements or if we, or they, experience unanticipated problems with our products following approval.***

Any of our product candidates for which we, or our collaborators, obtain marketing approval in the future, as well as the manufacturing processes, post-approval studies and measures, labeling, advertising and promotional activities for such product, among other things, will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, including the requirement to implement a REMS.

The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of a product. The FDA and other agencies, including the Department of Justice, closely regulate and monitor the post-approval marketing and promotion of products to ensure that they are manufactured, marketed and distributed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we, or our collaborators, do not market any of our product candidates for which we, or they, receive marketing approval for only their approved indications, we, or they, may be

subject to warnings or enforcement action for off-label marketing. Violation of the FDCA and other statutes, including the False Claims Act, relating to the promotion and advertising of prescription drugs may lead to investigations or allegations of violations of federal and state health care fraud and abuse laws and state consumer protection laws.

In addition, later discovery of previously unknown adverse events or other problems with our products or their manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on such products, manufacturers or manufacturing processes;
- restrictions on the indication, patient population or other parameters for which the drug is approved;
- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenues;
- reputational damage;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of products;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

***Recently enacted and future legislation may increase the difficulty and cost for us and our collaborators to obtain marketing approval of and commercialize our product candidates and affect the prices we, or they, may obtain.***

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability, or the ability of our collaborators, to profitably sell any products for which we, or they, obtain marketing approval.

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or the MMA, changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for drug purchases by the elderly and introduced a new reimbursement methodology based on average sales prices for physician administered drugs. In addition, this legislation provided authority for limiting the number of drugs that will be covered in any therapeutic class. Cost reduction initiatives and other provisions of this legislation could decrease the coverage and price that we receive for any approved products. While the MMA only addresses drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from the MMA may result in a similar reduction in payments from private payors.

In March 2010, President Obama signed into law the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act, or collectively the PPACA.

Among the provisions of the PPACA of potential importance to our product candidates are the following:

- an annual, non-deductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program;
- expansion of healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, new government investigative powers and enhanced penalties for noncompliance;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices;
- extension of manufacturers' Medicaid rebate liability;
- expansion of eligibility criteria for Medicaid programs;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program new requirements to report financial arrangements with physicians and teaching hospitals;
- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in and conduct comparative clinical effectiveness research, along with funding for such research.

In addition, other legislative changes have been proposed and adopted since the PPACA was enacted. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, starting in 2013. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us and our collaborators to more stringent product labeling and post-marketing testing and other requirements.

***Our future relationships with customers and third-party payors will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.***

Healthcare providers, physicians and third-party payors will play a primary role in the recommendation and prescription of any products for which we obtain marketing approval. Our future arrangements with third-party payors and customers, if any, will subject us to broadly applicable fraud and abuse and other healthcare laws and regulations. The laws and regulations may constrain the business or financial arrangements and relationships through which we market, sell and distribute any products for

which we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations in the United States include the following:

- *Anti-Kickback Statute.* The federal Healthcare Anti-Kickback statute prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation or arranging of, any good or service, for which payment may be made, in whole or in part, under a federal healthcare program such as Medicare and Medicaid;
- *False Claims Act.* The federal False Claims Act imposes criminal and civil penalties, including through civil whistleblower or *qui tam* actions, against individuals or entities for, among other things, knowingly presenting, or causing to be presented false or fraudulent claims for payment by a federal healthcare program or making a false statement or record material to payment of a false claim or avoiding, decreasing or concealing an obligation to pay money to the federal government, with potential liability including mandatory treble damages and significant per-claim penalties;
- *HIPAA.* The federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services and, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, also imposes obligations, including mandatory contractual terms and technical safeguards, with respect to maintaining the privacy, security and transmission of individually identifiable health information;
- *Transparency Requirements.* Federal transparency laws require certain manufacturers of covered drugs to report payments and other transfers of value to physicians, other healthcare providers and teaching hospitals, as well as ownership and investment interests held by physicians and other healthcare providers and their immediate family members; and
- *Analogous State and Foreign Laws.* Analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, can apply to sales or marketing arrangements and claims involving healthcare items or services. In addition, some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other health care providers or marketing expenditures and govern the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures. State and foreign laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not pre-empted by HIPAA, thus complicating compliance efforts.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion of products from government funded healthcare programs, such as Medicare and Medicaid and the curtailment or restructuring of our operations. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

***If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on our business.***

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. From time to time, our operations may involve the use of hazardous materials, including chemicals and biological materials, and may also produce hazardous waste products. Even if we contract with third parties for the disposal of these materials and waste products, we cannot completely eliminate the risk of contamination or injury resulting from these materials. In the event of contamination or injury resulting from the use or disposal of our hazardous materials, we could be held liable for any resulting damages, and any

liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

We maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, but this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations, including any laws and regulations that may be imposed as a result of the COVID-19 pandemic. Current or future environmental laws and regulations may impair our research, development or production efforts, which could adversely affect our business, financial condition, results of operations or prospects. In addition, failure to comply with these laws and regulations may result in substantial fines, penalties or other sanctions.

***Governments outside the United States tend to impose strict price controls, which may adversely affect our revenues, if any.***

In some countries, such as the countries of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we, or our collaborators, may be required to conduct a clinical trial that compares the cost-effectiveness of our product to other available therapies. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be materially harmed.

## **RISKS RELATED TO DATA PROTECTION AND CYBERSECURITY**

***Our failure to comply with data protection laws and regulations could lead to government enforcement actions, private litigation and/or adverse publicity and could negatively affect our operating results and business.***

We are subject to data protection laws and regulations that address privacy and data security. The legislative and regulatory landscape for data protection continues to evolve, and in recent years there has been an increasing focus on privacy and data security issues. In the United States, numerous federal and state laws and regulations, including state data breach notification laws, state health information privacy laws and federal and state consumer protection laws govern the collection, use, disclosure and protection of health-related and other personal information. Failure to comply with data protection laws and regulations could result in government enforcement actions, which could include civil or criminal penalties, private litigation and/or adverse publicity and could negatively affect our operating results and business. In addition, we may obtain health information from third parties that are subject to privacy and security requirements under HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act. We could be subject to criminal penalties if we knowingly obtain or disclose individually identifiable health information in a manner that is not authorized or permitted.

The collection and use of personal health data in the European Union is governed by the provisions of the General Data Protection Regulation, or GDPR, which came into effect in May 2018. This regulation imposes several requirements relating to the consent of the individuals to whom the personal data relates, the information provided to the individuals, notification of data processing obligations to the competent national data protection authorities and the security and confidentiality of the personal data. The GDPR also imposes strict rules on the transfer of personal data out of the European Union to the United States. Failure to comply with the requirements of the GDPR and the related national data protection laws of the European Union Member States may result in significant fines and other administrative penalties.

***Significant disruptions of information technology systems or security breaches could adversely affect our business.***

We are increasingly dependent upon information technology systems, infrastructure and data to operate our business. In the ordinary course of business, we collect, store and transmit large amounts of confidential information (including, among other things, trade secrets or other intellectual property, proprietary business information and personal information). It is critical that we do so in a secure manner to maintain the confidentiality and integrity of such confidential information. We also have outsourced elements of our operations to third parties, and as a result we manage a number of third-party vendors who may or could have access to our confidential information. The size and complexity of our information technology systems, and those of third-party vendors with whom we contract, and the large amounts of confidential information stored on those systems, make such systems vulnerable to service interruptions or to security breaches from inadvertent or intentional actions by our employees, consultants, third-party vendors, and/or business partners, or from cyber-attacks by malicious third parties. Cyber-

attacks are increasing in their frequency, sophistication and intensity, and have become increasingly difficult to detect. Cyber-attacks could include the deployment of harmful malware, ransomware, denial-of-service attacks, social engineering and other means to affect service reliability and threaten the confidentiality, integrity and availability of information. Cyber-attacks could also include phishing attempts or e-mail fraud to cause payments or information to be transmitted to an unintended recipient.

Significant disruptions of our information technology systems, or those of our third-party vendors, or security breaches could adversely affect our business operations and/or result in the loss, misappropriation and/or unauthorized access, use or disclosure of, or the prevention of access to, confidential information, including, among other things, trade secrets or other intellectual property, proprietary business information and personal information, and could result in financial, legal, business and reputational harm to us. For example, any such event that leads to unauthorized access, use or disclosure of personal information, including personal information regarding our patients or employees, could harm our reputation, require us to comply with federal and/or state breach notification laws and foreign law equivalents, and otherwise subject us to liability under laws and regulations that protect the privacy and security of personal information. Security breaches and other inappropriate access can be difficult to detect, and any delay in identifying them may lead to increased harm of the type described above. While we have implemented security measures to protect our information technology systems and infrastructure, there can be no assurance that such measures will prevent service interruptions or security breaches that could adversely affect our business.

## **RISKS RELATED TO EMPLOYEE MATTERS AND MANAGING GROWTH**

***Our future success depends on our ability to retain our Chief Executive Officer and other key executives and to attract, retain and motivate qualified personnel.***

Our industry has experienced a high rate of turnover of management personnel in recent years. Our ability to compete in the highly competitive biotechnology and pharmaceuticals industries depends upon our ability to attract and retain highly qualified managerial, scientific and clinical personnel. We are highly dependent on the pharmaceutical research and development and business development expertise of Roger D. Tung, our President and Chief Executive Officer, as well as the other principal members of our management, scientific and development team. Although we have formal employment agreements with our executive officers, these agreements do not prevent them from terminating their employment with us at any time. In addition, although we maintain a key-man insurance policy with respect to Dr. Tung, we do not carry key-man insurance on any of our other executive officers or employees and may not carry any key-man insurance in the future.

If we lose one or more of our executive officers, our ability to implement our business strategy successfully could be seriously harmed. Furthermore, replacing executive officers may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to develop, gain marketing approval of and commercialize products successfully. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these additional key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to develop and commercialize product candidates will be limited.

***We expect to grow our organization and, as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.***

As our pipeline grows and matures, we expect to experience significant growth in the number of our employees and the scope of our operations, including in the areas of drug manufacturing, regulatory affairs and sales, clinical development, marketing and distribution. Our management may need to divert a disproportionate amount of its attention away from our day-to-day activities to devote time to managing these growth activities. To manage these growth activities, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. Moreover, the expected expansion of our operations may lead to significant costs and may divert our business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

## RISKS RELATED TO OUR COMMON STOCK

***The price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for purchasers of our common stock.***

The trading price of our common stock has been, and may continue to be, volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control. The stock market in general and the market for smaller pharmaceutical and biotechnology companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. The market price for our common stock may be influenced by many factors, including:

- the success or failure of existing or new competitive products or technologies;
- the timing, advancement of and results of nonclinical studies and clinical trials of any of our product candidates;
- commencement or termination of collaborations for our development programs;
- failure, delays, changes to or discontinuation of any of our development programs;
- regulatory or legal developments in the United States and other countries;
- regulatory actions relating to our product candidates;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- disclosures by our collaborators relating to our product candidates or competitive programs;
- merger or acquisition activity of our collaborators;
- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to develop additional product candidates or products;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- announcement or expectation of additional financing efforts;
- receipt or expectation of receipt of revenues such as milestones, royalties, grants and license fees;
- sales of our common stock by us, our insiders or other stockholders;
- programmed trading based on technical stock chart or other inputs;
- portfolio restructuring by large stockholders or decisions by stockholders to rapidly acquire or sell our shares;
- addition or removal of our stock from stock indices;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in estimates or recommendations by securities analysts that cover our stock;
- actions by short-sellers or supporters of our stock, including social media postings or reports;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- legalization or the anticipation of possible legalization of drug reimportation from other countries;
- actual or anticipated changes in FDA practices;
- general economic, industry and market conditions; and
- the other factors described in this “Risk Factors” section.

***An active trading market for our common stock may not be sustained.***

Although we have listed our common stock on the Nasdaq Global Market, an active trading market for our common stock may not be sustained. In the absence of an active trading market for our common stock, investors may not be able to sell their common stock at or above the price at which they acquired their shares or at the times that they would like to sell. An inactive trading market may also impair our ability to raise capital to continue to fund operations by selling shares and may impair our ability to acquire other companies or technologies by using our shares as consideration.

***We have broad discretion in the use of our cash reserves and may not use them effectively.***

Our management has broad discretion to use our cash reserves and could use our cash reserves in ways that do not improve our results of operations or enhance the value of our common stock. The failure by our management to apply these funds effectively could result in financial losses that could have a material adverse effect on our business, cause the price of our common stock to decline and delay the development of our product candidates. Pending their use, we may invest our cash reserves in a manner that does not produce income or that loses value.

***We are a “smaller reporting company,” and the reduced disclosure requirements applicable to smaller reporting companies may make our common stock less attractive to investors.***

We are a smaller reporting company under applicable SEC regulations. For so long as we remain a smaller reporting company, we are permitted and plan to rely on exemptions from certain disclosure requirements applicable to other public companies, including reduced disclosure obligations regarding executive compensation. We cannot predict whether investors will find our common stock less attractive if we rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

***We will continue to incur increased costs as a result of operating as a public company.***

As a public company, we are incurring and expect to continue to incur significant legal, accounting and other expenses. We expect that these expenses will further increase now that we are no longer an “emerging growth company,” as defined in the Jumpstart Our Business Startups Act, as of January 1, 2020.

Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or SOX, we are required to evaluate the effectiveness of our internal control over financial reporting as of the end of each fiscal year and to report on this evaluation in our Annual Report on Form 10-K for the year. We will need to continue to dedicate internal resources, engage outside consultants and maintain a detailed work plan to assess and document the adequacy of our internal control over financial reporting, continue to improve control processes as appropriate, validate through testing that controls are functioning as documented and maintain a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that in the future we will not be able to conclude that our internal control over financial reporting is effective as required by SOX Section 404. If we identify one or more material weaknesses, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

***A significant portion of our total outstanding shares may be sold into the market in the near future, which could cause the market price of our common stock to decline significantly, even if our business is doing well.***

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares of common stock intend to sell shares, could reduce the market price of our common stock.

In addition, as of July 31, 2020, there were 29,678,072 shares subject to outstanding options and restricted stock units under our equity compensation plans, all of which shares are registered under the Securities Act of 1933, as amended, or the Securities Act. These shares will be able to be freely sold in the public market upon exercise, as permitted by any applicable vesting requirements, except to the extent they are held by our affiliates, in which case such shares will become eligible for sale in the public market as permitted by Rule 144 under the Securities Act. Furthermore, as of July 31, 2020, there were 1,861,273 shares subject to outstanding warrants to purchase common stock, 1,800,000 shares of which are registered under the Securities Act. The remaining 61,273 shares will become eligible for sale in the public market, to the extent such warrant is exercised, as permitted by Rule 144 under the Securities Act.

***We do not anticipate paying any cash dividends on our capital stock in the foreseeable future, accordingly, stockholders must rely on capital appreciation, if any, for any return on their investment.***

We have never declared or paid cash dividends on our capital stock. We currently plan to retain all of our future earnings, if any, to finance the operation, development and growth of our business. Furthermore, any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be the sole source of gain for our stockholders for the foreseeable future.

***Our executive officers, directors and principal stockholders, if they choose to act together, have the ability to substantially influence all matters submitted to stockholders for approval.***

Our executive officers and directors, combined with our stockholders who own more than 5% of our outstanding common stock, and all affiliates, in the aggregate, beneficially own a substantial percentage of our capital stock. As a result, if these stockholders were to choose to act together, they would be able to substantially influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would substantially influence the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of ownership control may:

- delay, defer or prevent a change in control;
- entrench our management or the board of directors; or
- impede a merger, consolidation, takeover or other business combination involving us that other stockholders may desire.

***Future sales of a substantial number of our common shares by our principal stockholders could depress the trading price of our common stock.***

If our principal stockholders sell substantial amounts of shares of our common stock in the public market or if the market anticipates that these sales could occur, the market price of shares of our common stock could decline. These sales may make it more difficult for us to sell equity or equity-related securities in the future at a time and price that we deem appropriate, or to use equity as consideration for future acquisitions.

***Provisions in our corporate charter documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.***

Provisions in our corporate charter and our by-laws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which our stockholders might otherwise receive a premium for their shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that all members of the board of directors are not elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for nominations for election to the board of directors or for proposing matters that can be acted on at stockholder meetings;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call a special meeting of stockholders;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a “poison pill” that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or by-laws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner. This could discourage, delay or prevent someone from acquiring us or merging with us, whether or not it is desired by, or beneficial to, our stockholders.

***If securities or industry analysts do not publish research or publish inaccurate or unfavorable research about our business, our share price and trading volume could decline.***

The trading market for our common stock depends on the research and reports that securities or industry analysts publish about us or our business. We do not have any control over these analysts. There can be no assurance that analysts will cover us, or provide favorable coverage. If one or more analysts downgrade our stock or change their opinion of our stock, our share price may decline. In addition, if one or more analysts cease coverage of our Company or fail to regularly publish reports on us, we could lose visibility in the financial markets, which could cause our share price or trading volume to decline.

***Changes in tax law could adversely affect our business and financial condition or holders of our common stock.***

The rules dealing with U.S. federal, state and local income taxation are constantly under review by persons involved in the legislative process and by the Internal Revenue Service and Treasury Department. Changes to tax laws (which changes may have retroactive application) could adversely affect us or holders of our common stock. In recent years, many such changes have been made and changes are likely to continue to occur in the future. Future changes in tax laws could have a material adverse effect on our business, cash flow, financial condition or results of operations. We urge stockholders to consult with their legal and tax advisers regarding the implications of potential changes in tax laws on an investment in our common stock.

**Item 6. Exhibits.**

<u>Exhibit number</u>	<u>Description</u>
<a href="#"><u>31.1*</u></a>	<a href="#"><u>Principal Executive Officer — Certification pursuant to Rule 13a-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002</u></a>
<a href="#"><u>31.2*</u></a>	<a href="#"><u>Principal Financial Officer — Certification pursuant to Rule 13a-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002</u></a>
<a href="#"><u>32.1**</u></a>	<a href="#"><u>Principal Executive Officer — Certification pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002</u></a>
<a href="#"><u>32.2**</u></a>	<a href="#"><u>Principal Financial Officer — Certification pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002</u></a>
101.INS*	XBRL Instance Document
101.SCH*	XBRL Taxonomy Extension Schema Document
101.CAL*	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF*	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB*	XBRL Taxonomy Extension Label Linkbase Document
101.PRE*	XBRL Taxonomy Extension Presentation Linkbase Document

\* Filed herewith.

\*\* Furnished herewith.

**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.

CONCERT PHARMACEUTICALS, INC.

Date: August 6, 2020

By: /s/ Marc A. Becker

Marc A. Becker  
Chief Financial Officer  
(Principal Financial Officer)

**CERTIFICATION PURSUANT TO RULE 13a-14(a)  
OF THE SECURITIES EXCHANGE ACT OF 1934, AS ADOPTED PURSUANT TO  
SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002**

I, Roger D. Tung, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Concert Pharmaceuticals, 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(s) 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(s) 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 6, 2020

/s/ Roger D. Tung

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Roger D. Tung

President and Chief Executive Officer

(Principal Executive Officer)

**CERTIFICATION PURSUANT TO RULE 13a-14(a)  
OF THE SECURITIES EXCHANGE ACT OF 1934, AS ADOPTED PURSUANT TO  
SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002**

I, Marc A. Becker, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Concert Pharmaceuticals, 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(s) 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(s) 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 6, 2020

/s/ Marc A. Becker

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Marc A. Becker

Chief Financial Officer

(Principal Financial Officer)

**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 Concert Pharmaceuticals, Inc. (the "Company") for the period ended June 30, 2020, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Roger D. Tung, President and Chief Executive Officer of the Company, hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to my 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.

Dated: August 6, 2020

/s/ Roger D. Tung

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Roger D. Tung

President and Chief Executive Officer

(Principal Executive Officer)

*A signed original of this written statement required by Section 906 has been provided to Concert Pharmaceuticals, Inc. and will be retained by Concert Pharmaceuticals, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.*

**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 Concert Pharmaceuticals, Inc. (the "Company") for the period ended June 30, 2020, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Marc A. Becker, Chief Financial Officer of the Company, hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to my 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.

Dated: August 6, 2020

/s/ Marc A. Becker

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Marc A. Becker

Chief Financial Officer

(Principal Financial Officer)

*A signed original of this written statement required by Section 906 has been provided to Concert Pharmaceuticals, Inc. and will be retained by Concert Pharmaceuticals, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.*