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**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 March 31, 2014

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)

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**Delaware**  
(State or other jurisdiction of  
incorporation or organization)

**99 Hayden Avenue, Suite 500**  
**Lexington, Massachusetts**  
(Address of principal executive offices)

**20-4839882**  
(I.R.S. Employer  
Identification No.)

**02421**  
(Zip Code)

**(781) 860-0045**  
(Registrant's telephone number, including area code)

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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 and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted 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 and post such files). Yes  No

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

Large accelerated filer  Accelerated filer   
Non-accelerated filer  (Do not check if a smaller reporting company) Smaller reporting company

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 May 13, 2014: 17,899,585

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## PART I. FINANCIAL INFORMATION

## Item 1. Financial Statements.

## CONCERT PHARMACEUTICALS, INC.

## CONDENSED CONSOLIDATED BALANCE SHEETS (UNAUDITED)

	<u>March 31,</u> 2014	<u>December 31,</u> 2013
	(In thousands, except share and per share data)	
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 39,651	\$ 9,638
Investments, available for sale	68,379	23,039
Interest receivable	317	92
Accounts receivable	68	170
Prepaid expenses and other current assets	2,223	1,106
Total current assets	110,638	34,045
Property and equipment, net	2,247	2,473
Restricted cash	706	706
Other assets	34	2,549
Total assets	<u>\$ 113,625</u>	<u>\$ 39,773</u>
<b>Liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)</b>		
Current liabilities:		
Accounts payable	\$ 1,292	\$ 971
Accrued expenses and other liabilities	2,528	2,475
Deferred revenue, current portion	4,741	4,321
Leasehold improvement loan, current portion	332	332
Loan payable, net of discount	7,987	7,818
Total current liabilities	16,880	15,917
Deferred revenue, net of current portion	13,950	15,310
Leasehold improvement loan, net of current portion	166	249
Deferred lease incentive, net of current portion	257	385
Deferred rent, net of current portion	138	208
Warrant to purchase redeemable securities	—	463
Loan payable, net of current portion and discount	5,037	7,101
Total liabilities	36,428	39,633
Commitments		
Redeemable convertible preferred stock, \$0.001 par value per share; 5,000,000 and 62,916,667 shares (Series A, B, C, D) authorized, respectively; 0 shares and 56,047,067 shares issued and outstanding, respectively; aggregate liquidation preference of \$0 and \$112,993, respectively	—	112,244
Stockholders' equity (deficit):		
Common stock, \$0.001 par value per share; 100,000,000 and 83,716,667 shares authorized, respectively; 17,899,585 and 1,298,300 shares issued and outstanding, respectively	18	1
Additional paid-in capital	197,770	1,528
Accumulated other comprehensive (loss) income	(4)	4
Accumulated deficit	(120,587)	(113,637)
Total stockholders' equity (deficit)	77,197	(112,104)
Total liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)	<u>\$ 113,625</u>	<u>\$ 39,773</u>

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 March 31,	
	2014	2013
Revenue:		
License and research and development revenue	\$ 1,613	\$ 3,873
Milestone revenue	—	2,000
Total revenue	<u>1,613</u>	<u>5,873</u>
Operating expenses:		
Research and development	5,594	5,039
General and administrative	2,538	1,964
Total operating expenses	<u>8,132</u>	<u>7,003</u>
Loss from operations	(6,519)	(1,130)
Investment income	4	11
Interest and other expense	(435)	(660)
Net loss	<u>\$(6,950)</u>	<u>\$(1,779)</u>
Other comprehensive loss:		
Unrealized loss on investments	(8)	(1)
Comprehensive loss	<u>\$(6,958)</u>	<u>\$(1,780)</u>
Reconciliation of net loss to net loss applicable to common stockholders:		
Net loss	\$(6,950)	\$(1,779)
Accretion on redeemable convertible preferred stock	(55)	(98)
Net loss applicable to common stockholders—basic and diluted	<u>\$(7,005)</u>	<u>\$(1,877)</u>
Net loss per share applicable to common stockholders—basic and diluted	<u>\$ (0.76)</u>	<u>\$ (1.46)</u>
Weighted-average number of common shares used in net loss per share applicable to common stockholders—basic and diluted	9,188	1,290

See accompanying notes.

## CONCERT PHARMACEUTICALS, INC.

## CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS (UNAUDITED)

(Amounts in thousands)

	Three Months Ended March 31,	
	2014	2013
<b>Operating activities</b>		
Net loss	\$ (6,950)	\$ (1,779)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	326	338
Stock-based compensation expense	210	226
Accretion of premiums and discounts on investments	84	67
Amortization of discount on loan payable	24	24
Amortization of deferred financing costs	9	9
Re-measurement of warrant to purchase redeemable securities	117	211
Amortization of deferred lease incentive	(128)	(128)
Changes in operating assets and liabilities:		
Accounts receivable	102	(2,000)
Interest receivable	(225)	7
Prepaid expenses and other current assets	(1,126)	(224)
Other assets	98	47
Accounts payable	321	441
Accrued expenses and other liabilities	42	438
Deferred rent	(59)	(41)
Deferred revenue	(940)	275
Net cash used in operating activities	(8,095)	(2,089)
<b>Investing activities</b>		
Purchases of property and equipment	(100)	(76)
Purchases of investments	(51,432)	(2,526)
Maturities of investments	6,000	10,750
Net cash (used in) provided by investing activities	(45,532)	8,148
<b>Financing activities</b>		
Principal payments on loan payable	(1,919)	—
Repayment of leasehold improvement loan	(83)	(83)
Proceeds from initial public offering, net of underwriting discounts and commissions	86,579	—
Proceeds from issuance of common stock	105	—
Payment of initial public offering costs	(1,042)	—
Net cash provided by (used in) financing activities	83,640	(83)
Net increase in cash and cash equivalents	30,013	5,976
Cash and cash equivalents at beginning of period	9,638	7,490
Cash and cash equivalents at end of period	<u>\$ 39,651</u>	<u>\$ 13,466</u>
Supplemental cash flow information:		
Cash paid for interest	\$ 308	\$ 425
Initial public offering costs incurred but unpaid at period end	\$ 394	\$ —

See accompanying notes.

CONCERT PHARMACEUTICALS, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS (UNAUDITED)

**1. Nature of business**

Concert Pharmaceuticals, Inc. (Concert or the Company) was incorporated on April 12, 2006 (inception) as a Delaware corporation, with operations based in Lexington, Massachusetts. The Company is a clinical stage biopharmaceutical company that applies its extensive knowledge of deuterium chemistry to discover and develop novel small molecule drugs. The Company's approach starts with approved drugs, advanced clinical candidates or previously studied compounds that the Company believes can be improved with deuterium substitution to provide better pharmacokinetic or metabolic properties and thereby enhance clinical safety, tolerability or efficacy. The Company believes this approach may enable drug discovery and clinical development that is more efficient and less expensive than conventional small molecule drug development. The Company's pipeline includes three clinical-stage candidates and a number of preclinical compounds that it is actively assessing.

The condensed consolidated financial statements of the Company included herein have been prepared, without audit, pursuant to the rules and regulations of the Securities and Exchange Commission (SEC). Certain information and footnote disclosures normally included in financial statements prepared in accordance with accounting principles generally accepted in the United States of America have been condensed or omitted from this report, as is permitted by such rules and regulations. Accordingly, these condensed consolidated financial statements should be read in conjunction with the financial statements and notes thereto included in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2013, filed with the SEC on March 31, 2014.

The unaudited condensed consolidated financial statements include the accounts of Concert and its subsidiary. All intercompany transactions and balances of subsidiaries have been eliminated in consolidation. In the opinion of management, the information furnished reflects all adjustments, all of which are of a normal and recurring nature, necessary for a fair presentation of the results for the reported interim periods. The Company considers events or transactions that occur after the balance sheet date but before the financial statements are issued to provide additional evidence relative to certain estimates or to identify matters that require additional disclosure. The three months ended March 31, 2014 and 2013 are referred to as the first quarter of 2014 and 2013, respectively. The results of operations for interim periods are not necessarily indicative of results to be expected for the full year or any other interim period.

In the first quarter of 2014, the Company closed its initial public offering (IPO), in which the Company sold 6,649,690 shares of common stock, including shares sold under the underwriters' over-allotment option, at a price to the public of \$14.00 per share. The Company's net proceeds from the IPO were \$83.1 million after deducting underwriting discounts and offering expenses. In preparation for the IPO, the Company's Board of Directors and stockholders approved a one-for-5.65 reverse stock split of the Company's common stock, which stock split was effected on January 29, 2014. All share and per share amounts in the condensed consolidated financial statements and notes thereto have been retroactively adjusted, where necessary, to give effect to this reverse stock split. In conjunction with the IPO, all outstanding shares of the Company's preferred stock automatically converted into 9,919,821 shares of common stock and the outstanding warrant to purchase 400,000 shares of Series C redeemable convertible preferred stock converted into a warrant to purchase 70,796 shares of common stock at an exercise price of \$14.13 per share. Following these transactions, the Company's total issued common stock as of March 31, 2014 was 17,899,585 shares. The significant increase in shares outstanding in the first quarter of 2014 is expected to impact the year-over-year comparability of the Company's net (loss) earnings per share calculations for the next twelve months.

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

**2. Summary of significant accounting policies**

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, 2013. The Company did not adopt any new accounting pronouncements during the three months ended March 31, 2014 that had a material effect on the Company's condensed consolidated financial statements.

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### 3. Fair Value Measurements

The Company measures certain financial assets and liabilities at fair value on a recurring basis (principally cash equivalents, investments and the preferred stock warrant liability) that have been classified as Level 1, 2 or 3 within the fair value hierarchy as described below. Fair values determined by Level 1 inputs utilize quoted prices (unadjusted) in active markets for identical assets or liabilities that the Company has the ability to access. Fair values determined by Level 2 inputs utilize data points that are observable, such as quoted prices, interest rates and yield curves. Fair values determined by Level 3 inputs utilize unobservable data points for the asset or liability. The Company's investments in money market funds, U.S. treasury obligations and government agency securities have been classified as Level 1 because their fair values are based on quoted market prices. The preferred stock warrant liability is classified as Level 3 because certain inputs to the valuation of the warrant are based on unobservable inputs.

As of March 31, 2014 and December 31, 2013, the Company's financial assets recognized at fair value consisted of the following:

	<u>Level 1</u>	<u>Level 2</u>	<u>Level 3</u>	<u>Total</u>
<b>March 31, 2014</b>				
Money market funds, included in cash equivalents	\$ 38,631	\$ —	\$ —	\$ 38,631
Investments, available for sale:				
Government agency securities	58,710	—	—	58,710
U.S. Treasury obligation	9,669	—	—	9,669
	<u>\$107,010</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$107,010</u>
	<u>Level 1</u>	<u>Level 2</u>	<u>Level 3</u>	<u>Total</u>
<b>December 31, 2013</b>				
Money market funds, included in cash equivalents	\$ 7,450	\$ —	\$ —	\$ 7,450
Investments, available for sale:				
Government agency securities	22,539	—	—	22,539
U.S. Treasury obligation	500	—	—	500
	<u>\$ 30,489</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 30,489</u>

The fair value of the preferred stock warrant liability was determined based on "Level 3" inputs utilizing the Black-Scholes option pricing model. On February 19, 2014, upon completion of the IPO, the Company's outstanding warrant to purchase preferred stock converted into a warrant to purchase common stock and the Company reclassified the fair value of the warrant as of February 19, 2014 to additional paid-in capital. The following table presents activity in the preferred stock warrant liability during the three months ended March 31, 2014.

	<u>Balance</u>
Fair value at December 31, 2013	\$ 463
Increase in fair value recognized in net loss	117
Reclassification to additional paid-in capital in connection with IPO	(580)
Fair value at March 31, 2014	<u>\$ —</u>

The carrying amount of financial instruments not carried at fair value, including the loan payable and leasehold improvement loan approximate fair value. The carrying value of the Company's loan payable and leasehold improvement loan approximated fair value because of the length of the remaining terms of the loans and interest rate yields for the loans that were near then-current market yields. The disclosed fair values of the Company's loan payable and leasehold improvement loan are Level 3 liabilities within the fair value hierarchy.

[Table of Contents](#)**4. Cash, cash equivalents and investments, available for sale**

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 are included in accumulated other comprehensive income. Premiums or discounts from par value are amortized to investment income over the life of the underlying investment.

Cash, cash equivalents and investments, available for sale included the following at March 31, 2014 and December 31, 2013 (in thousands):

	<u>Average maturity</u>	<u>Amortized cost</u>	<u>Unrealized gains</u>	<u>Unrealized losses</u>	<u>Fair value</u>
<b>March 31, 2014</b>					
Cash		\$ 1,020	\$ —	\$ —	\$ 1,020
Money market funds		38,631	—	—	38,631
Cash and cash equivalents		<u>\$ 39,651</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$39,651</u>
U.S. Treasury obligations	316 days	\$ 9,670	\$ —	\$ (1)	\$ 9,669
Government agency securities	335 days	58,713	8	(11)	58,710
Investments		<u>\$ 68,383</u>	<u>\$ 8</u>	<u>\$ (12)</u>	<u>\$68,379</u>
<b>December 31, 2013</b>					
Cash		\$ 2,188	\$ —	\$ —	\$ 2,188
Money market funds		7,450	—	—	7,450
Cash and cash equivalents		<u>\$ 9,638</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 9,638</u>
U.S. Treasury obligation	301 days	\$ 500	\$ —	\$ —	\$ 500
Government agency securities	324 days	22,535	4	—	22,539
Investments		<u>\$ 23,035</u>	<u>\$ 4</u>	<u>\$ —</u>	<u>\$23,039</u>

Although available to be sold to meet operating needs or otherwise, securities are generally held through maturity. The cost of securities sold is determined based on the specific identification method for purposes of recording realized gains and losses. During 2014 and 2013, there were no realized gains or losses on sales of investments, and no investments were adjusted for other than temporary declines in fair value.



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### 5. Accrued expenses and other liabilities

Accrued expenses and other liabilities consisted of the following (in thousands):

	<u>March 31,</u> <u>2014</u>	<u>December 31,</u> <u>2013</u>
Accrued professional fees and other	\$ 410	\$ 569
Employee compensation and benefits	566	290
Research and development expenses	785	860
Deferred lease incentive, current portion	513	513
Deferred rent, current portion	254	243
	<u>\$ 2,528</u>	<u>\$ 2,475</u>

### 6. 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. There were no significant income tax provisions or benefits for the three months ended March 31, 2014 and 2013. Due to the uncertainty surrounding the realization of its favorable tax attributes in future tax returns, the Company has recorded a full valuation allowance against the Company's otherwise recognizable net deferred tax assets.

### 7. Collaborations

#### *Celgene*

In April 2013, the Company entered into a master development and license agreement (the Celgene Agreement) with Celgene Corporation and Celgene International Sàrl (Celgene), which is primarily focused on the research, development and commercialization of deuterated compounds that are deuterated analogs of certain non-deuterated compounds targeting cancer or inflammation. The collaboration will initially focus on one program, CTP-730 targeting inflammatory disease, but has the potential to encompass up to four programs.

For the initial program, the Company granted Celgene an exclusive worldwide license to develop, manufacture and commercialize products that contain deuterated analogs of a selected non-deuterated compound and several close chemical derivatives thereof. The Company further granted Celgene licenses with respect to two additional programs and an option with respect to a third additional program. The Company and Celgene have agreed on the non-deuterated compounds for each of the two additional license programs. For the option program, Celgene may select the non-deuterated compound at a later time, which, unless otherwise agreed by the Company, will be limited to a compound for which Celgene possesses exclusive rights. With respect to the two additional license programs, on the effective date of the Celgene Agreement the Company granted Celgene an exclusive worldwide license to develop, manufacture and commercialize products that contain deuterated analogs of the agreed upon non-deuterated compounds. Celgene is restricted from utilizing their research, development and commercialization rights under each of the upfront licenses unless, within seven years of the effective date of the Celgene Agreement, Celgene pays the Company a license exercise fee. If Celgene does not elect to pay the license exercise fee during the seven year period, the license will expire. With respect to the option program, once a compound is selected, Celgene may exercise its option by paying the Company an option exercise fee within seven years of the effective date of the Celgene Agreement, and upon Celgene's exercise of the option the Company will grant to Celgene an exclusive worldwide license to develop, manufacture and commercialize deuterated products that contain deuterated analogs of the selected non-deuterated compound.

The Company is responsible, at its own expense, for conducting research and early development activities for CTP-730, the initial program pursuant to agreed upon development plans. This includes the completion of single and multiple ascending dose Phase 1 clinical trials and any mutually agreed upon additional Phase 1 clinical trials, as set forth in the development plan and approved by the joint steering committee (JSC) for the collaboration.

Under the terms of the Celgene Agreement, the Company received a \$35.0 million non-refundable upfront payment from Celgene. In addition, the Company is eligible to earn up to \$23.0 million in development milestone payments, up to \$247.5 million in regulatory milestone payments and up to \$50.0 million in sales-based milestone payments

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related to products within the initial program. The next milestone payment the Company might be entitled to receive under the initial program is \$8.0 million related to the completion of a Phase 1 clinical trial of CTP-730 for inflammatory diseases. If Celgene exercises its rights with respect to either of the two additional license programs, the Company will receive a license exercise fee of \$30.0 million and will also be eligible to receive up to \$23.0 million in development milestone payments and up to \$247.5 million in regulatory milestone payments for such program. With respect to one of the additional license programs, the Company is eligible to receive up to \$100.0 million in sales-based milestone payments based on net sales of products, and with respect to the other additional license program, the Company is eligible to receive up to \$50.0 million in sales-based milestone payments based on net sales of products. If Celgene exercises its option with respect to the option program, the Company will receive an option exercise fee of \$10.0 million and will be eligible to earn up to \$23.0 million in development milestone payments and up to \$247.5 million in regulatory milestone payments.

In addition, with respect to each program, Celgene is required to pay the Company royalties on net sales of each licensed product at defined percentages ranging from the mid-single digits to low double digits below 20%, on worldwide net product sales of licensed products. The royalty term for each licensed product in each country is the period commencing with first commercial sale of the applicable licensed product in the applicable country and ending on the latest of expiration of specified patent coverage, expiration of regulatory exclusivity or 10 years following commercial launch. The royalty rate is reduced on a country-by-country basis during any period within the royalty term when there is no patent claim or regulatory exclusivity covering the licensed product in the particular country.

The Company's arrangement with Celgene contains the following deliverables: (i) an exclusive worldwide license to develop, manufacture and commercialize deuterated analogs of a selected compound related to the initial program (the License Deliverable), (ii) obligations to perform research and development services associated with the initial program (the R&D Services Deliverable), (iii) obligation to supply preclinical and clinical trial material related to the initial program (the Supply Deliverable), (iv) participation on the JSC during the term of the initial program (the JSC Deliverable), (v) significant and incremental discount related to the first additional license program for which the non-deuterated compound has been selected (the First Discount Deliverable) and (vi) significant and incremental discount related to the second additional license program for which the non-deuterated compound has been selected (the Second Discount Deliverable).

Allocable arrangement consideration at inception was limited to the \$35.0 million non-refundable upfront payment. Total allocable arrangement consideration was allocated among the separate units of accounting using the relative selling price method as follows: (i) \$17.0 million to the License Deliverable; (ii) \$8.7 million to the R&D Services Deliverable for the initial program; (iii) \$3.2 million to the Supply Deliverable for the initial program; (iv) \$0.1 million to the JSC Deliverable for the initial program; (v) \$3.0 million to the First Discount Deliverable for the first additional program; and (vi) \$3.0 million to the Second Discount Deliverable for the second additional program.

The arrangement consideration allocated to the License Deliverable was recognized upon delivery. Amounts allocated to the R&D Services Deliverable and Supply Deliverable are recognized under the proportional performance method over the expected period of performance, or 39 months. The amount allocated to the JSC Deliverable is recognized ratably over the expected period of performance, or 39 months. Amounts allocated to the First Discount Deliverable and the Second Discount Deliverable are deferred and will be recognized at the earlier of when the associated license rights are exercised and licenses are delivered or upon lapsing of the underlying right, if the respective right expires unexercised. The Company reassesses the estimated periods of performance for each unit of accounting at the end of each reporting period.

During the three months ended March 31, 2014, the Company recognized revenue of \$0.4 million for the R&D Services Deliverable and \$0.5 million for the Supply Deliverable. The revenue was classified as license and research and development revenue in the accompanying condensed consolidated statement of operations and comprehensive loss. As of March 31, 2014, there was \$15.7 million of deferred revenue related to the Company's collaboration with Celgene, \$4.6 million of which was classified as current and \$11.1 million of which was classified as noncurrent, in the accompanying condensed consolidated balance sheet.

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### *Jazz Pharmaceuticals*

In February 2013, the Company signed a development and license agreement with Jazz Pharmaceuticals, Inc. (Jazz Pharmaceuticals) that provides Jazz Pharmaceuticals worldwide rights to develop and commercialize the Company's deuterated sodium oxybate (D-SXB) compounds (the Jazz Pharmaceuticals Agreement). Jazz Pharmaceuticals has principal responsibility for ongoing development activities. Pursuant to the terms of the license agreement, Jazz Pharmaceuticals has the option to require the Company to provide development support services through a single Phase 1 clinical trial. If Jazz Pharmaceuticals exercises its option, the Company will receive payment for any development support services provided and will be reimbursed for all external costs related to the development support services including preclinical, manufacturing, regulatory and clinical costs.

Under the terms of the Jazz Pharmaceuticals Agreement, the Company received a \$4.0 million non-refundable upfront payment. In addition, the Company is eligible to earn up to \$8.0 million in development milestone payments, up to \$35.0 million in regulatory milestone payments and up to \$70.0 million in sales-based milestone payments. The next milestone payment that the Company might be entitled to receive under this agreement is \$4.0 million related to the completion of a Phase 1 clinical trial of JZP-386, a deuterated analog of sodium oxybate.

In addition, Jazz Pharmaceuticals is required to pay the Company royalties at defined percentages ranging from the mid-single digits to low double digits below 20%, on a country-by-country and licensed product-by-licensed product basis, on worldwide net product sales of licensed products. The royalty term for each licensed product in each country is the period commencing with first commercial sale of the applicable licensed product in the applicable country and ending on the later of the expiration of specified patent coverage or 10 years following commercial launch. The royalty rate is lowered on a country-by-country basis, under certain circumstances as specified in the agreement.

The Company determined that there were three deliverables under the Jazz Pharmaceuticals Agreement: (i) an exclusive, royalty-bearing sub-licensable worldwide license to develop and commercialize D-SXB compounds (the License Deliverable), (ii) participation on a joint steering committee (the JSC Deliverable) and (iii) a deliverable to direct external patent activities and bear a portion of the external patent fees (the Patent Support Deliverable).

The Company allocated arrangement consideration of \$3.7 million to the License Deliverable, \$0.1 million to the JSC Deliverable and \$0.2 million to the Patent Support Deliverable. The Company recognized the arrangement consideration allocated to the License Deliverable upon delivery and will recognize revenue related to the JSC Deliverable and the Patent Support Deliverable over the respective periods of performance which is estimated to be 46 months.

For the three months ended March 31, 2014, the Company recognized revenue of \$0.6 million related to the performance of development support services and revenue of approximately \$16 thousand for reimbursements of travel and intellectual property expenses, the cost of which was recorded within general and administrative expenses.

For the three months ended March 31, 2013, the Company recognized revenue of \$3.7 million upon delivery of the License Deliverable, approximately \$3 thousand related to the JSC Deliverable and approximately \$2 thousand related to the Patent Support Deliverable. In addition, during the three months ended March 31, 2013, the Company recognized revenue of \$48 thousand related to the performance of development support services and revenue of approximately \$4 thousand for reimbursements of intellectual property expenses, the cost of which was recorded within general and administrative expenses.

### *Avanir*

In February 2012, the Company signed a license agreement (the Avanir Agreement) with Avanir Pharmaceuticals, Inc. (Avanir) that provides Avanir worldwide rights to develop and commercialize Concert's deuterated dextromethorphan (D-DM). The agreement includes the rights to multiple D-DM compounds. Avanir will have overall responsibility for research, development and commercialization of D-DM. Avanir has the option to

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require the Company to provide manufacturing services through a first IND filing. If Avanir exercises its option, the Company will receive payment for any manufacturing services provided and will be reimbursed for all external costs related to the manufacturing services.

Under the terms of the Avanir Agreement, the Company received a \$2.0 million non-refundable upfront payment in March 2012. In addition, the Company is eligible to earn up to \$4.0 million in development milestone payments, up to \$37.0 million in regulatory milestone payments and up to \$125.0 million in sales-based milestone payments. The next potential milestone the Company might be entitled to receive under the Avanir Agreement is \$2.0 million for initiation of dosing in a Phase 2 or Phase 3 clinical trial for AVP-786, one of the D-DM analogs licensed to Avanir. In addition, we are eligible for higher development milestones, up to an additional \$43.0 million, for licensed products that do not require quinidine. Avanir is currently developing deuterated dextromethorphan only in combination with quinidine.

Avanir also is required to pay the Company royalties at defined percentages ranging from the mid-single digits to low double digits below 20% on worldwide net product sales of licensed products. The royalty term for each licensed product in each country is the period commencing with first commercial sale of the applicable licensed product in the applicable country and ending on the later of expiration of specified patent coverage or 10 years following commercial launch. The royalty rate is reduced, on a country-by-country basis, during any period within the royalty term when there is no patent claim, covering the licensed product in the particular country.

The Company determined that the deliverables under the Avanir Agreement were the exclusive, royalty-bearing sub-licensable license to D-DM delivered at the inception of the arrangement as well as participation on a joint steering committee through a first IND filing. The Company allocated arrangement consideration of \$2.0 million to the license and an insignificant amount to the Company's participation on the joint steering committee. Accordingly, the Company recognized the \$2.0 million non-refundable upfront fee as revenue upon delivery of the D-DM license in March 2012.

During the three months ended March 31, 2013, the Company recognized as revenue a \$2.0 million milestone payment received from Avanir based on positive data from Avanir's Phase 1 clinical trial of AVP-786. Since June 2012, Avanir has elected to conduct all research and development activities, including manufacturing activities; however, the Company has continued to receive intellectual property cost reimbursements. The Company recognized \$61 thousand for the three months ended March 31, 2014 and \$51 thousand for the three months ended March 31, 2013 within license and research and development revenue for intellectual property cost reimbursements, the cost of which was recorded within general and administrative expense.

## GSK

In May 2009, we entered into a research and development collaboration and license agreement with Glaxo Group Limited, or GSK to research, develop and commercialize multiple products containing deuterated compounds, including CTP-499 and, ultimately, CTP-298, which was developed pursuant to the agreement for the treatment of HIV. In March 2011, the Company and GSK signed an amendment to the GSK Agreement. Under this amendment GSK paid the Company a \$2.75 million payment and returned all rights in the second identified program to the Company. This \$2.75 million amount is subject to repayment to GSK in the event that the Company commercializes CTP-499 or if, at any time during the seven year period from the date of the amendment, the Company re-licenses or otherwise transfers the rights to the Company's CTP-499 program to a third party. The payment was classified as deferred revenue and will not be recognized as revenue until all repayment obligations lapse.

## 8. Stock-Based Compensation

Total stock-based compensation expense related to stock options was \$0.2 million and \$0.2 million for the three months ended March 31, 2014 and 2013, respectively.

Total compensation cost recognized for all stock-based compensation awards in the condensed consolidated statements of operations and comprehensive loss consisted of (in thousands):

	<u>Three Months Ended March 31,</u>	
	<u>2014</u>	<u>2013</u>
Research and development	\$ 90	\$ 121
General and administrative	120	105
Total stock-based compensation expense	<u>\$ 210</u>	<u>\$ 226</u>

### Stock Options

The weighted-average fair value of options granted during the three months ended March 31, 2014, based on fair values estimated as of the applicable grant dates using the Black-Scholes option pricing model, was \$8.79 per option share. There were no stock options granted during the three months ended March 31, 2013. Key assumptions used to apply this pricing model were as follows:

	<u>Three Months Ended</u> <u>March 31, 2014</u>
Risk-free interest rate	2.02%
Expected life of options	6.0 years
Expected volatility of underlying stock	68.95%
Expected dividend yield	0.0%

The following is a summary of stock option activity for the three months ended March 31, 2014:

	<u>Number of</u> <u>Option Shares</u>	<u>Weighted</u> <u>Average</u> <u>Exercise</u> <u>Price per</u> <u>Share</u>	<u>Weighted</u> <u>Average</u> <u>Remaining</u> <u>Contractual</u> <u>Term</u> <u>(In years)</u>	<u>Aggregate</u> <u>Intrinsic</u> <u>Value</u> <u>(In thousands)</u>
Outstanding at December 31, 2013	1,952,578	\$ 3.14		
Granted	148,893	14.06		
Exercised	(31,775)	3.30		
Forfeited or expired	(33,131)	3.95		
Outstanding at March 31, 2014	<u>2,036,565</u>	\$ 3.92	5.5	\$ 19,492
Exercisable at March 31, 2014	<u>1,670,622</u>	\$ 3.07	4.8	\$ 17,349
Vested and expected to vest at March 31, 2014 (1)	<u>2,017,046</u>	\$ 3.85	5.5	\$ 19,437

- (1) This represents the number of vested stock option shares as of March 31, 2014, plus the number of unvested stock option shares that the Company estimated as of March 31, 2014 would vest, based on the unvested stock option shares at March 31, 2014 and an estimated forfeiture rate of 5%.

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As of March 31, 2014, there was \$2.6 million of unrecognized compensation cost related to stock options that are expected to vest. These costs are expected to be recognized over a weighted average remaining vesting period of 2.5 years.

### **9. Loan payable**

On December 22, 2011, the Company entered into a Loan and Security Agreement (the Loan and Security Agreement) with Hercules Technology Growth Capital, Inc. (Hercules). The Loan and Security Agreement provides for aggregate advances of up to \$20 million. The advances under the Loan and Security Agreement were to be made in two tranches: (i) \$7.5 million funded at closing, and (ii) up to an additional \$12.5 million through March 31, 2012. The maximum amount of principal outstanding allowable under the Loan and Security Agreement is \$20 million. Under the first tranche of the Loan and Security Agreement, the Company obtained an advance on December 22, 2011 totaling \$7.5 million (the December 2011 Advance). Under the second tranche of the Loan and Security Agreement, the Company obtained an advance on March 29, 2012 totaling \$12.5 million (the March 2012 Advance). The Company incurred \$0.2 million in loan issuance costs paid directly to the lenders, which have been offset against the loan proceeds as a loan discount.

Each advance made under the Loan and Security Agreement bears interest at a variable rate of the greater of 8.5% and an amount equal to 8.5% plus the prime rate of interest minus 5.25%, provided however, that the per annum interest rate shall not exceed 11%. Through March 31, 2014, the December 2011 Advance and the March 2012 Advance had an interest rate of 8.5%. Interest-only payments were due monthly on the first day of each month beginning the month after the date of the respective advance until April 30, 2013. Thereafter the aggregate principal balance outstanding became payable in 30 equal monthly installments of principal and interest beginning May 1, 2013 and continuing through the maturity date of October 1, 2015.

Future minimum payments, which include principal and interest due under the Loan and Security Agreement, are \$6.7 million for the remainder of 2014 and \$7.5 million for the year ended December 31, 2015.

### **10. (Loss) Earnings Per Share**

Basic (loss) earnings per share is computed by dividing (loss) income allocable to common stockholders by the weighted average number of common shares outstanding. During periods of income, participating securities are allocated a proportional share of income determined by dividing total weighted-average participating securities by the sum of the total weighted-average common shares and participating securities (the "two-class method"). Prior to its automatic conversion into common stock upon the closing of the IPO, the Company's redeemable convertible preferred stock was entitled to participate in any dividends declared by the Company and was therefore considered to constitute participating securities. Participating securities have the effect of diluting both basic and diluted earnings per share during periods of income. During periods of loss, no loss is allocated to participating securities since they have no contractual obligation to share in the losses of the Company. Diluted (loss) earnings per share is computed after giving consideration to the dilutive effect of stock options that are outstanding during the period, except where such non-participating securities would be anti-dilutive.

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Basic and diluted (loss) earnings per share allocable to common stockholders were computed as follows:

	Three Months Ended	
	March 31,	
	2014	2013
	(in thousands, except per share data)	
Net loss	\$ (6,950)	\$ (1,779)
Less: accretion of redeemable convertible preferred stock to redemption value	55	98
Loss allocable to common stockholders	<u>\$ (7,005)</u>	<u>\$ (1,877)</u>
Weighted-average shares outstanding	9,188	1,290
Basic and diluted (loss) earnings per share allocable to common stockholders	<u>\$ (0.76)</u>	<u>\$ (1.46)</u>

In the first quarter of 2014, the Company issued an additional 6,649,690 shares of common stock in connection with its IPO and 9,919,821 shares of common stock in connection with the automatic conversion of its redeemable convertible preferred stock upon the closing of the IPO. The issuance of these shares resulted in a significant increase in the Company's weighted-average shares outstanding for the three months ended March 31, 2014 when compared to the comparable prior year period and is expected to continue to impact the year-over-year comparability of the Company's (loss) earnings per share calculations for the next twelve months.

The following common stock equivalents were excluded from the calculation of diluted loss per share allocable to common stockholders because their inclusion would have been anti-dilutive:

	Three Months Ended March 31,	
	2014	2013
	(in thousands)	
Redeemable convertible preferred stock	—	9,920
Warrant	71	71
Outstanding stock options	<u>2,037</u>	<u>1,956</u>
	2,108	11,947

**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. Some of the information contained in this discussion and analysis or set forth elsewhere in this report, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. You should read the “Risk Factors” section in Part II—Item 1A. of this report for a discussion of important factors that could cause 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 applying our extensive knowledge of deuterium chemistry to discover and develop novel small molecule drugs. Our approach starts with approved drugs, advanced clinical candidates or previously studied compounds that we believe can be improved with deuterium substitution, a process we refer to as deuteration, to provide better pharmacokinetic or metabolic properties and thereby enhance clinical safety, tolerability or efficacy. We believe this approach may enable drug discovery and clinical development that is more efficient and less expensive than conventional small molecule drug research and development.

We are utilizing our DCE Platform to discover and develop product candidates for a variety of indications. We expect CTP-354, CTP-499 and AVP-786 to advance into or through Phase 2 clinical trials in 2014 and we have multiple preclinical candidates, two of which we expect to move into clinical trials in 2014. Our priority programs include:

- CTP-354 for spasticity associated with multiple sclerosis and spinal cord injury, which is in Phase 1 clinical trials;
- CTP-499 for type 2 diabetic kidney disease, which is in the final, open-label portion of the three-part Phase 2 clinical trial;
- AVP-786 for neurologic and psychiatric disorders, which has completed a Phase 1 clinical trial under our collaboration with Avanir;
- CTP-730 for inflammatory diseases, which is in preclinical development under our collaboration with Celgene; and
- JZP-386 for narcolepsy, which is in preclinical development under our collaboration with Jazz Pharmaceuticals.

Since our inception in 2006, we have devoted substantially all of our resources to our research and development efforts relating to our product candidates, including activities to: develop our DCE Platform and our core capabilities in deuterium chemistry, identify potential product candidates, undertake preclinical studies and clinical trials, manufacture product in compliance with current good manufacturing practices, provide general and administrative support for these operations and establish our intellectual property. We have generated an accumulated deficit of \$120.6 million since inception through March 31, 2014 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 and funding from collaborations. On February 19, 2014, we completed the sale of 6,000,000 shares of common stock in our IPO, at a price to the public of \$14.00 per share, resulting in net proceeds to us of \$74.6 million after deducting underwriting discounts and commissions of \$5.9 million and offering costs of \$3.5 million. On March 3, 2014, we completed the sale of an additional 649,690 shares of common stock at a price to the public of \$14.00 per share under the underwriters’ over-allotment option to purchase additional shares of common stock, resulting in net proceeds to us of \$8.5 million after deducting underwriting discounts and commissions of \$0.6 million.

We have incurred net losses in each year from our inception in 2006. We incurred a net loss of \$7.0 million for the three months ended March 31, 2014. We do not expect to be profitable for the year ending December 31, 2014. 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.



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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 to develop and conduct clinical trials and additional preclinical studies with respect to CTP-354;
- initiate and continue research, preclinical and clinical development efforts for our other product candidates and potential product candidates;
- seek to identify 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;
- hire additional personnel, such as clinical, quality control and scientific personnel;
- add equipment and physical infrastructure to support our research and development; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and personnel and infrastructure necessary to help us comply with our obligations as a public company.

We do not expect to generate revenue from product sales unless and until we, or our collaborators, successfully complete development and 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. We have developed the internal capability to manufacture up to low kilogram quantities of deuterated active pharmaceutical ingredients for use in Phase 1 clinical trials. However, to date, almost all of our manufacturing activities have been performed by third parties. Additionally, we currently utilize third-party contract research organizations to carry out our clinical development activities and we do not yet have a sales organization. If we obtain or believe that we are likely to obtain, marketing approval for any of our product candidates for which we retain commercialization rights, 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 and additional collaborations and licensing arrangements 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.

## COLLABORATIONS

We have entered into a number of collaborations for the research, development and commercialization of deuterated compounds. To date, our collaborations have provided us with significant funding for both our specific development programs and our DCE Platform. They also have provided us with access to the considerable scientific, development, regulatory and commercial capabilities of our collaborators. In addition, in some instances, where we develop and seek to collaborate with respect to deuterated analogs of marketed drugs or of drug candidates that are more advanced in clinical trials, our collaborators may be eligible to seek an expedited development or regulatory pathway by relying on previous clinical data regarding their corresponding non-deuterated compound. For example, our collaborator Avanir reported agreeing with the FDA to an expedited development pathway for AVP-786. We believe that our collaborations have contributed to our ability to progress our product candidates and build our DCE Platform. We have established the following key collaborations:

- *Celgene*. In April 2013, we entered into a master development and license agreement with Celgene, which is primarily focused on the research, development and commercialization of specified deuterated compounds targeting cancer or inflammation. The collaboration is initially focused on CTP-730, targeting inflammatory disease, but has the potential to encompass up to four programs. For the initial program, we granted Celgene an exclusive worldwide license to develop, manufacture and commercialize deuterated analogs of a selected non-deuterated compound and several close chemical derivatives thereof. We further granted Celgene licenses with respect to two additional programs and an option with respect to a third additional program. We and Celgene have agreed on the non-deuterated compound for each of the two additional license programs. For the option program, Celgene may select the non-deuterated compound at a later time, which, unless otherwise agreed by us, will be limited to a compound for which Celgene possesses exclusive rights. With respect to the two additional license programs, we granted Celgene an upfront exclusive worldwide license to develop, manufacture and commercialize deuterated products that contain deuterated analogs of the agreed upon non-deuterated compounds. Celgene is restricted from utilizing their research, development and commercialization rights under each of the upfront licenses, unless, within seven years after the effective date of the agreement, Celgene pays us a license exercise fee. If Celgene does not elect to pay the license exercise fee during the seven year period, the license will expire. With respect to the option program, once a compound is selected, Celgene may exercise its option by paying us an option exercise fee within seven years of the effective date of the agreement, and upon Celgene's exercise of the option we will grant to Celgene an exclusive worldwide license to develop, manufacture and commercialize deuterated products that contain deuterated analogs of the selected non-deuterated compound.

Under the Celgene agreement, we received a non-refundable upfront payment of \$35.0 million in April 2013. In addition, we are eligible to earn up to \$23.0 million in development milestone payments, including \$8.0 million related to the completion of a Phase 1 clinical trial, up to \$247.5 million in regulatory milestone payments and up to \$50.0 million in sales-based milestone payments related to products within the initial program. If Celgene exercises its rights with respect to either of the two additional license programs, we will receive a license exercise fee for the applicable program of \$30.0 million and will also be eligible to earn up to \$23.0 million in development milestone payments and up to \$247.5 million in regulatory milestone payments for that program. Additionally, with respect to one of the additional license programs we are eligible to receive up to \$100.0 million in sales-based milestone payments based on net sales of products, and with respect to the other additional license program we are eligible to receive up to \$50.0 million in sales-based milestone payments based on net sales of products. If Celgene exercises its option with respect to the option program in respect of a compound to be identified at a later time, we will receive an option exercise fee of \$10.0 million and will be eligible to earn up to \$23.0 million in development milestone payments and up to \$247.5 million in regulatory milestone payments. In addition, with respect to each program, Celgene is required to pay us royalties on net sales of each licensed product at defined percentages ranging from the mid-single digits to low double digits below 20%, on worldwide net product sales of licensed products. The royalty rate is reduced on a country-by-country basis during any period within the royalty term when there is no patent claim or regulatory exclusivity covering the licensed product in the particular country.

Under the Celgene agreement, we are responsible for conducting and funding research and development activities for the initial program at our own expense pursuant to agreed upon development plans. These activities consist of the completion of single and multiple ascending dose Phase 1 clinical trials and any mutually agreed upon additional Phase 1 clinical trials. If Celgene exercises its rights with respect to any additional program and pays us the applicable exercise fee, we are responsible for conducting research and development activities at our own expense pursuant to agreed upon development plans until the completion of the first Phase 1 clinical trial, which will be defined in each development plan on a program-by-program basis. In addition, if Celgene exercises its rights with respect to the option program and pays us the applicable exercise fee, we are responsible for seeking to generate a deuterated compound for clinical development in the selected option program at our own expense.

- *Avanir*. In February 2012, we entered into a development and license agreement with Avanir under which we granted Avanir an exclusive worldwide license to develop, manufacture and commercialize deuterated dextromethorphan containing products. Avanir is initially focused on developing AVP-786, which is a combination of a deuterated dextromethorphan analog and an ultra-low dose of quinidine, for the treatment of neurologic and psychiatric disorders.

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Under the Avanir agreement, we received a non-refundable upfront payment of \$2.0 million in February 2012 and a milestone payment of \$2.0 million in April 2013. We are also eligible to receive, with respect to licensed products comprising a combination of deuterated dextromethorphan and quinidine, up to \$4.0 million in development milestone payments, including \$2.0 million related to initiation of dosing in a Phase 2 or Phase 3 clinical trial for AVP-786, up to \$37.0 million in regulatory and commercial launch milestone payments and up to \$125.0 million in sales-based milestone payments based on net product sales of licensed products. In addition, we are eligible for higher development milestones, up to an additional \$43.0 million, for licensed products that do not require quinidine. Avanir is currently developing deuterated dextromethorphan only in combination with quinidine. Avanir also is required to pay us royalties at defined percentages ranging from the mid-single digits to low double digits below 20% on worldwide net product sales of licensed products. The royalty rate is reduced, on a country-by-country basis, during any period within the royalty term when there is no patent claim covering the licensed product in the particular country.

Avanir is responsible for funding 100% of our research and development costs incurred under the development plan or for activities conducted at Avanir's request, subject to limitations specified in the agreement. However, Avanir is currently conducting all research and development activities without our services.

- *Jazz Pharmaceuticals.* In February 2013, we entered into a development and license agreement with Jazz Pharmaceuticals to research, develop and commercialize products containing deuterated sodium oxybate, or D-SXB. We are initially focusing on one analog, designated as JZP-386. Under the terms of the agreement, we granted Jazz Pharmaceuticals an exclusive, worldwide, royalty-bearing license under intellectual property controlled by us to develop, manufacture and commercialize D-SXB products including JZP-386.

Under the Jazz Pharmaceuticals agreement, we received a non-refundable upfront payment of \$4.0 million in February 2013. We are also eligible to receive up to \$8.0 million in development milestone payments, up to \$35.0 million in regulatory milestone payments and up to \$70.0 million in sales milestone payments based on net product sales of licensed products. In addition, Jazz Pharmaceuticals is required to pay us royalties at defined percentages ranging from the mid-single digits to low double digits below 20%, on a country-by-country and licensed product-by-licensed product basis, on worldwide net product sales of licensed products. The royalty rate is lowered, on a country-by-country basis, under certain circumstances as specified in the agreement.

We are currently conducting certain development activities for a Phase 1 clinical trial with respect to JZP-386 pursuant to an agreed upon development plan, and we have supplied a deuterated intermediate for making clinical trial material for a Phase 1 clinical trial. Thereafter, our obligations to conduct further development activities are subject to mutual agreement. Jazz Pharmaceuticals has assumed all manufacturing responsibilities. Pursuant to the agreement, our costs for activities under the development plan, including pass-through costs and the costs of our employees' time at an annual rate per full-time equivalent, which we mutually agreed to, are reimbursed by Jazz Pharmaceuticals. This reimbursement is subject to limitations in the agreement, including adherence within a particular percentage to the development budget.

Following termination of the agreement with respect to a country or countries, but not in its entirety, by Jazz Pharmaceuticals for Jazz Pharmaceuticals' convenience, Jazz Pharmaceuticals may provide us written notice that it desires to continue or recommence development and commercialization of licensed products in such country or countries, in which event Jazz Pharmaceuticals' license with respect to D-SXB products in such country or countries and corresponding payment obligations under the agreement will be reinstated except in specified circumstances in which we have previously notified Jazz Pharmaceuticals of our intent to develop or commercialize licensed products in such country or countries either directly or through a third party licensee.

In May 2009, we entered into a research and development collaboration and license agreement with Glaxo Group Limited, or GSK to research, develop and commercialize multiple products containing deuterated compounds, including CTP-499 and, ultimately, CTP-298, which was developed pursuant to the agreement for the treatment of HIV. Our agreement with GSK, as subsequently amended, expired in May 2012 after GSK opted out of further development under the agreement. The rights to the product candidates developed under the agreement have reverted to us and we are free to pursue them without further obligation to GSK other than to repay GSK an amount of up to \$2.75 million, if we commercialize CTP-499 or if, prior to a specified date in 2018, we re-license or transfer rights to CTP-499.

## FINANCIAL OPERATIONS OVERVIEW

### 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.

The terms of these agreements 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 and certain research and development service revenues. 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, milestone payments and royalties on future product sales in connection with our current collaborations with Celgene, Avanir and Jazz Pharmaceuticals, 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 consist of 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 preclinical 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.

The following summarizes our development programs.

- *CTP-354*, a novel, potentially first-in-class, non-sedating treatment for spasticity that we are initially developing for use in patients with multiple sclerosis and patients with spinal cord injury to address a significant unmet medical need in these markets. In 2013, we completed a 71-subject Phase 1 single ascending dose clinical trial of *CTP-354* and the nine-subject first part of a related Phase 1 imaging study. In January 2014, we initiated a multiple ascending dose Phase 1 clinical trial evaluating daily doses of 2 mg and 6 mg of *CTP-354* in healthy volunteers. Assuming successful completion of the Phase 1 clinical program, we plan to initiate a Phase 2 clinical program for *CTP-354* in the fourth quarter of 2014. We expect that the Phase 2 clinical program will include one clinical trial for the treatment of spasticity

associated with multiple sclerosis and one clinical trial for the treatment of spasticity associated with spinal cord injury. Due to the fact that we did not determine a maximum tolerated dose in our preclinical testing, the FDA has informed us that we may not administer multiple doses of CTP-354 in excess of 6 mg per day in clinical trials without first conducting an additional higher dose preclinical toxicology study. We believe that multiple doses of 6 mg per day may be sufficient for the treatment of spasticity; however, we have conducted an additional preclinical toxicology study and plan to submit the results to the FDA which may enable us to evaluate higher doses of CTP-354, if needed, in our spasticity trials, as well as to support clinical development in other disease indications.

- CTP-499, a novel, potentially first-in-class, treatment for type 2 diabetic kidney disease that we are developing as an additive treatment to the current standard of care. We are currently conducting the final, open-label portion of a three-part Phase 2 clinical trial of CTP-499 in which we have enrolled patients with type 2 diabetic kidney disease and macroalbuminuria. In December 2013, we completed 48 weeks of dosing for the second part of the trial in 123 patients. While we did not achieve statistical significance in the primary 24-week efficacy endpoint of this trial, at 48 weeks fewer patients on CTP-499 compared to placebo had large increases in serum creatinine, a marker of impaired kidney function and a key secondary endpoint. These data may indicate a slower decline of kidney function in patients treated with CTP-499 compared to those who received placebo. In addition, we observed at 48-weeks a statistically significant reduction of certain fibrotic biomarkers suggesting that CTP-499 may act as an anti-fibrotic agent. We believe that these results indicate that CTP-499 has the potential to help protect kidney function in patients with rapidly progressing type 2 diabetic kidney disease. We expect the ongoing open-label extension study to conclude by the end of 2014.
- AVP-786, a combination of a deuterium-substituted dextromethorphan analog and an ultra-low dose of quinidine. We have granted Avanir an exclusive license to develop and commercialize deuterated dextromethorphan analogs, including the analog in AVP-786. Avanir is developing AVP-786 for the treatment of neurologic and psychiatric disorders. In February 2013, Avanir reported positive results from a Phase 1 clinical trial of AVP-786. In October 2013, Avanir reported plans to advance AVP-786 into a Phase 2 clinical trial in the second half of 2014 for treatment-resistant major depressive disorder in patients with insufficient response to conventional anti-depressants.
- A collaboration with Celgene to research, develop and commercialize certain deuterated compounds for the treatment of cancer or inflammation, with an initial focus on a single program. In the initial program, we have selected CTP-730, a product candidate for the treatment of inflammatory diseases, and expect to begin clinical trials in 2014.
- A collaboration with Jazz Pharmaceuticals to research, develop and commercialize JZP-386, a product candidate containing a deuterated analog of sodium oxybate for potential use in patients with narcolepsy. Sodium oxybate is the active ingredient in the marketed drug Xyrem. In December 2013, an investigational medicinal product dossier, or IMPD, the basis for initiating clinical trials in the European Union, was filed for JZP-386. The IMPD received approval in January 2014. Jazz Pharmaceuticals expects a Phase 1 clinical trial of JZP-386 to be conducted in 2014, following manufacturing of clinical material.

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We are also conducting a number of other preclinical programs, including deuterated ivacaftor for the potential treatment of cystic fibrosis and chronic obstructive pulmonary disease and C-10068, a novel oral deuterium-substituted analog of dextroethorphan for the potential treatment of pain and seizures.

We plan to continue to seek to identify compounds that can be improved through selective deuterium substitution and believe we are capable of identifying one to two novel deuterated compounds per year that we can advance into preclinical development while concurrently progressing our existing pipeline.

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. However, product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of 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.

We anticipate that our general and administrative expenses will increase in the future as we increase our headcount to support our continued research and development of our product candidates. We also anticipate increased expenses associated with being a public company, including costs for audit, legal, regulatory and tax-related services, director and officer insurance premiums, and investor relations costs. Additionally, if and when we believe a regulatory 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 and marketing of our product candidates.

### **Investment income**

Investment income consists of interest income earned on cash equivalents and investments.

### **Interest and other expense**

Interest and other expense consists primarily of interest expense on amounts outstanding under our debt facility with Hercules Technology Growth Capital, Inc., or Hercules, amortization of debt discount and the re-measurement gain or loss associated with the change in the fair value of the preferred stock warrant liability for the periods prior to the Company's IPO.

**Critical Accounting Policies and Significant Judgments and Estimates**

During the three months ended March 31, 2014, there were no material changes to our critical accounting policies. Our critical accounting policies are described under *Management's Discussion and Analysis of Financial Condition and Results of Operations* in our Annual Report on Form 10-K for the fiscal year ended December 31, 2013, which was filed with the Securities and Exchange Commission on March 31, 2014.

**RESULTS OF OPERATIONS****Comparison of the three months ended March 31, 2014 and 2013**

The following table summarizes our results of operations for the three months ended March 31, 2014 and 2013, together with the changes in those items in dollars.

<u>(in thousands)</u>	<u>Three months ended</u> <u>March 31,</u>		<u>Increase</u> <u>(Decrease)</u>
	<u>2014</u>	<u>2013</u>	
Revenue:			
License and research and development revenue	\$ 1,613	\$ 3,873	\$ (2,260)
Milestone revenue	—	2,000	(2,000)
Total revenue	1,613	5,873	(4,260)
Operating expenses:			
Research and development	5,594	5,039	555
General and administrative	2,538	1,964	574
Total operating expenses	8,132	7,003	1,129
Loss from operations	(6,519)	(1,130)	5,389
Investment income	4	11	(7)
Interest and other expense	(435)	(660)	(225)
Net loss	<u>\$(6,950)</u>	<u>\$(1,779)</u>	<u>\$ 5,171</u>

*Revenue*

Revenue was \$1.6 million for the three months ended March 31, 2014, compared to \$5.9 million for the three months ended March 31, 2013, a decrease of \$4.3 million. The decrease in revenue was primarily due to license revenue recognized for the three months ended March 31, 2013 of \$3.7 million in connection with our grant of a license under our collaboration with Jazz Pharmaceuticals as well as \$2.0 million of milestone revenue recognized for the three months ended March 31, 2013 based on positive data from Avanir's Phase 1 clinical trial of AVP-786. These decreases were partially offset by an increase of \$1.4 million in revenue recognized for services performed under our collaborations during the three months ended March 31, 2014.

As of March 31, 2014, we had deferred revenue of:

- \$15.7 million related to our collaboration with Celgene, \$4.6 million of which is classified as current, on our condensed consolidated balance sheet;
- \$0.2 million related to our collaboration with Jazz Pharmaceuticals and associated with research and development services to be performed and recognized as revenue over the estimated remaining performance period of 36 months; and
- \$2.8 million related to a payment received from GSK that we will not recognize as revenue until all repayment obligations lapse.

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### *Research and development expenses*

The following table summarizes our external research and development expenses, by program, for the three months ended March 31, 2014 and 2013, with our internal research expenses separately classified by category. Because Avanim is conducting the clinical development of AVP-786 at its expense, we made minimal investment in the program during these periods.

(in thousands)	Three months ended March 31,	
	2014	2013
Direct research and development expenses:		
CTP-499	\$ 347	\$ 1,275
CTP-354	1,267	38
CTP-730	369	104
JZP-386	111	15
Total direct research and development expenses	2,094	1,432
Employee and contractor-related expenses	2,367	2,483
Platform-related lab expenses	362	319
Facility expenses	675	662
Other expenses	96	143
Personnel and other expenses	3,500	3,607
Total research and development expenses	\$ 5,594	\$ 5,039

Research and development expenses were \$5.6 million for the three months ended March 31, 2014, compared to \$5.0 million for the three months ended March 31, 2013, an increase of \$0.6 million. The increase was primarily due to a \$1.2 million increase in CTP-354 expenses due to the conduct during the three months ended March 31, 2014 of our CTP-354 multiple ascending dose Phase 1 clinical trial, our higher dose preclinical CTP-354 toxicology study and increased manufacturing costs. The increase was partially offset by a \$0.9 million decrease in CTP-499 expense due to the completion of dosing for the second part of our Phase 2 clinical trial in December 2013.

### *General and administrative expenses*

General and administrative expenses were \$2.5 million for the three months ended March 31, 2014, compared to general and administrative expenses of \$2.0 million for the three months ended March 31, 2013. The increase was primarily due to a \$0.4 million increase in compensation expense and \$0.2 million of expenses incurred in connection with our becoming a public company, including directors & officers insurance and professional fees.

We expect that our general and administrative expenses will increase in future periods as we expand our operations and incur additional costs in connection with being a public company. We anticipate that these increases will likely include legal, auditing and filing fees, additional insurance premiums and general compliance and consulting expenses.

### *Interest and other expense*

Interest and other expense was an expense of \$0.4 million for the three months ended March 31, 2014, compared to an expense of \$0.6 million for the three months ended March 31, 2013. The decrease was attributable in part to a decrease of \$0.1 million in expense recognized in connection with the re-measurement of the fair value of the redeemable convertible preferred stock warrant that we issued to Hercules in connection with draws under our debt facility. Upon completion of our IPO in February 2014, the warrant became exercisable for an aggregate of 70,796 shares of our common stock at an exercise price of \$14.13 per share and the related warrant liability was reclassified to additional paid-in capital and will not be subject to re-measurement in future periods. In addition, interest expense associated with our debt facility with Hercules decreased by \$0.1 million for the three months ended March 31, 2014 compared to the prior year period due to a lower principal balance outstanding.



## LIQUIDITY AND CAPITAL RESOURCES

We have incurred cumulative losses and negative cash flows from operations since our inception in April 2006, and as of March 31, 2014, we had an accumulated deficit of \$120.6 million. 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 and additional collaborations and licensing arrangements.

We have financed our operations to date primarily through the public offering and private placement of our equity, debt financing and funding from collaborations. As of March 31, 2014 we had cash and cash equivalents and investments of \$108.0 million.

### Cash flows

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

(in thousands)	Three months ended	
	March 31,	
	2014	2013
Net cash provided by (used in):		
Operating activities	\$ (8,095)	\$(2,089)
Investing activities	(45,532)	8,148
Financing activities	83,640	(83)
Net increase in cash and cash equivalents	<u>\$ 30,013</u>	<u>\$ 5,976</u>

*Operating activities.* Net cash used in operating activities was \$8.1 million during the three months ended March 31, 2014 compared to net cash used in operating activities of \$2.1 million during the three months ended March 31, 2013. The increase in cash used in operating activities was primarily due to receipt of a non-refundable upfront payment of \$4.0 million related to our collaboration with Jazz Pharmaceuticals in the three months ended March 31, 2013.

*Investing activities.* Net cash provided by (used in) investing activities consisted of purchases of fixed assets, purchases of investments and proceeds from the maturity of investments. Net cash used in investing activities for the three months ended March 31, 2014 was \$45.5 million compared to net cash provided by investing activities of \$8.1 million for the three months ended March 31, 2013. The increase in net cash used in investing activities was primarily due to an increase in purchases of investments of \$48.9 million related to the investment of our IPO proceeds and a decrease in maturities of investments of \$4.7 million.

*Financing activities.* Net cash provided by financing activities for the three months ended March 31, 2014 was \$83.6 million compared to net cash used in financing activities of \$0.1 million for the three months ended March 31, 2013. The increase in net cash provided by financing activities was primarily due to the receipt of IPO proceeds (net of underwriting discounts and commissions but prior to deducting other transaction expenses) of \$86.6 million during the three months ended March 31, 2014, partially offset by an increase in principal payments under our debt facility with Hercules of \$1.9 million as compared to the prior year and \$1.0 million in costs incurred related to our IPO during the three months ended March 31, 2014.

### Credit Facilities

In December 2011, we executed a Loan and Security Agreement with Hercules, which provided for up to \$20.0 million in funding, to be made available in two tranches. We borrowed the first tranche of \$7.5 million in December 2011 and the second tranche of \$12.5 million in March 2012. As of March 31, 2014, an aggregate of \$13.2 million of principal and accrued interest remained outstanding under the Loan and Security Agreement.

Each advance under the Loan and Security Agreement bears interest at a variable rate equal to the greater of 8.5% and an amount equal to 8.5% plus the prime rate of interest minus 5.25%, provided however that the per

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annum rate of interest rate shall not exceed 11%. We were required to pay interest only on the indebtedness through April 30, 2013. We are now repaying our remaining indebtedness under the Loan and Security Agreement in 19 equal monthly payments of principal and interest of \$0.7 million through October 1, 2015.

The loan is collateralized by a blanket lien on all of our corporate assets, excluding intellectual property, and by a negative pledge on our intellectual property. The loan and security agreement contains default provisions that include the occurrence of a material adverse effect, as defined therein, that would entitle the lender to declare all principal, interest and other amounts owed by us under the loan and security agreement immediately due and payable.

In connection with the December 2011 borrowing under the Loan and Security Agreement, we issued to Hercules a warrant to purchase an aggregate of 200,000 shares of Series C preferred stock with an exercise price of \$2.50 per share. In connection with the March 2012 borrowing under the Loan and Security Agreement, the warrant we issued to Hercules automatically became exercisable for an additional 200,000 shares of Series C preferred stock. Upon completion of our IPO in February 2014 the warrant became exercisable for an aggregate of 70,796 shares of our common stock at an exercise price of \$14.13 per share and the related warrant liability was reclassified to additional paid-in capital.

### **Operating capital requirements**

We do not anticipate commercializing any of our product candidates for several years. 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 regulatory 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.

We believe our existing cash and cash equivalents and investments as of March 31, 2014, will enable us to fund our operating expenses, debt service and capital expenditure requirements into the first half of 2016, without giving effect to potential milestone payments that we may receive under existing collaboration agreements. This estimate assumes we either enter into a collaboration agreement pursuant to which a partner funds further development of CTP-499 or we do not otherwise expend significant funds for further development of this product candidate. However, we may 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. 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.

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. 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 debt or equity securities, it could result in dilution to our existing stockholders, increased fixed payment obligations and these securities may have rights senior to those of our common stock. We are subject

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to covenants under our existing loan and security agreement with Hercules, and 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. In addition, the pledge of substantially all of our assets with the exception of our intellectual property as collateral, and the negative pledge with respect to our intellectual property, under our debt facility with Hercules limit our ability to obtain additional debt financing.

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.

### **Contractual obligations**

During the three months ended March 31, 2014, there were no material changes to our contractual obligations and commitments described under Management’s Discussion and Analysis of Financial Condition and Results of Operations in our Annual Report on Form 10-K for the fiscal year ended December 31, 2013.

### **OFF-BALANCE SHEET ARRANGEMENTS**

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

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

We are exposed to market risk related to changes in interest rates. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments are in short-term available-for-sale securities and interest on our debt facility accrues at a variable rate that references the prime rate.

We had cash and cash equivalents and investments of \$108.0 million as of March 31, 2014 and \$32.7 million as of December 31, 2013, in each case primarily money market mutual funds consisting of U.S. government-backed and agency securities. The increase in cash and cash equivalents and investments during the three months ended March 31, 2014 was primarily the result of our receipt of IPO proceeds of \$86.6 million (net of underwriting discount and commissions but prior to deducting other transaction expenses) in February 2014. Our available-for-sale securities are subject to interest rate risk and will fall in value if market interest rates increase. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, an immediate 10% change in interest rates would not have a material effect on the fair market value of our portfolio.

We had outstanding borrowings under our debt facility with Hercules of \$13.2 million as of March 31, 2014 and \$15.1 million as of December 31, 2013. Interest is payable at a variable rate of the greater of 8.5% and an amount equal to 8.5% plus the prime rate of interest minus 5.25%, provided however, that the per annum interest rate shall not exceed 11%. As a result of the 11% maximum annual interest rate and interest rate protection until prime exceeds 5.25%, we have limited exposure to changes in interest rates on borrowings under this facility. An immediate 10% change in the prime rate as of March 31, 2014 would have no effect on the amount of our required interest payments under the debt facility over the next twelve-month period.

We contract with suppliers of raw materials and contract manufacturers 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 three months ended March 31, 2014 or 2013.

**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 Securities and Exchange Commission’s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company’s management, including its principal executive and principal financial officers, 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 Chief Executive Officer and Chief Financial Officer, has evaluated the effectiveness of our disclosure controls and procedures as of March 31, 2014, the end of the period covered by this Quarterly Report on Form 10-Q. Based upon such evaluation, our Chief Executive Officer and Chief Financial Officer have 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 was no change in our internal control over financial reporting that occurred during the period covered by this Quarterly Report on Form 10-Q 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 Securities and Exchange Commission, or 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 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.**

We have incurred significant annual net operating losses in every year since our inception. Our net loss was \$7.0 million for the three months ended March 31, 2014. As of March 31, 2014, we had an accumulated deficit of \$120.6 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. 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 preclinical 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 (deficit) and working capital.

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

- continue to develop and conduct clinical trials with respect to CTP-354;
- initiate and continue research, preclinical and clinical development efforts for our other product candidates and potential product candidates;
- seek to identify 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;
- hire additional personnel, such as clinical, quality control and scientific personnel;
- add operational, financial and management information systems and personnel, including personnel to support our product development and personnel and infrastructure necessary to help us comply with our obligations as a public company; and
- add equipment and physical infrastructure to support our research and development.

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 obtain marketing approval for, and successfully commercialize, one or more of our product candidates. This 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

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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 the second quarter of 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 a multi-center international clinical trial, conduct a large-scale pivotal clinical trial, obtain marketing approvals, manufacture a commercial scale product, 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 preclinical 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 preclinical development efforts for and seek marketing approval for, our 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. To our knowledge, no deuterated drug has ever been successfully commercialized. Manufacturing a deuterated drug at commercial scale may require expensive and specialized facilities, processes and materials. In addition, relative to previous years when we operated as a private company, we expect to incur significant additional costs in 2014 and future years 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.

We plan to use our current cash and cash equivalents and investments, including the net proceeds of our recent initial public offering, primarily to fund our ongoing research and development efforts. We will be required to expend significant funds in order to advance the development of CTP-354 and our other product candidates. In addition, while we may seek one or more collaborators for future development of CTP-499 and expect that we would conduct any large Phase 3 clinical trial of CTP-499 in type 2 diabetic kidney disease in collaboration with one or more partners that would pay most of the associated costs, we may not be able to enter into a collaboration for CTP-499 on suitable terms or at all. 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 public or private equity offerings, debt financings, collaborations and licensing arrangements or other sources. Adequate additional financing may not be available to us on acceptable terms, or at all. Our ability to obtain debt financing may be limited by covenants we have made under our loan and security agreement with Hercules Technology Growth Capital, Inc., or Hercules, and our pledge to Hercules of substantially all of our assets, other than our intellectual property, as collateral. The negative pledge in favor of Hercules with respect to our intellectual property under the loan and security agreement could further limit our ability to obtain additional debt financing. Our failure to raise capital as and when needed would have a negative impact on our financial condition and our ability to pursue our business strategy.

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We believe our existing cash and cash equivalents and investments as of March 31, 2014 will enable us to fund our operating expenses, debt service and capital expenditure requirements into the first half of 2016, without giving effect to potential milestone payments that we may receive under existing collaboration agreements. This estimate assumes we either enter into a collaboration agreement pursuant to which a partner funds further development of CTP-499 or we do not otherwise expend significant funds for further development of this product candidate. Our estimate as to how long we expect our existing 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 preclinical development efforts for, our product candidates and potential product candidates, including current and future clinical trials;
- our ability to identify a collaborator for CTP-499 and the terms and timing of any collaboration agreement that we may establish for the development and commercialization of CTP-499;
- our current collaboration agreements remaining in effect and achievement of milestones under these agreements;
- our ability to enter into and the terms and timing of any additional collaborations, licensing 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 regulatory approvals;
- the costs of commercialization activities for any of our product candidates that receive marketing approval, to the extent such costs are not the responsibility of one of our collaborators, including the costs and timing of establishing product sales, marketing, distribution and manufacturing capabilities;
- subject to receipt of marketing approval, revenue, if any, received from commercial sales of our product candidates;
- 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; and
- the costs of operating as a public company.

### **Raising additional capital may cause dilution to our stockholders, restrict our operations 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 public or private equity offerings, debt financings and additional collaborations and licensing arrangements. We do not have any committed external source of funds, other than potential milestone payments and royalties under our collaborations with Celgene, Avanir and Jazz Pharmaceuticals, each of which is subject to the achievement of development, regulatory 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. For example, our debt facility with Hercules contains restrictive covenants that, among other things and subject to certain exceptions, prohibit us from transferring any of our material assets, merging with or acquiring another entity, entering into a transaction that would result in a change of



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control, incurring additional indebtedness, creating any lien on our property, making investments in third parties or redeeming stock or paying dividends. Future debt securities or other financing arrangements could contain similar or more restrictive negative covenants. In addition, securing additional financing could require a substantial amount of time and attention from our management and may divert a disproportionate amount of their attention away from day-to-day activities, which may adversely affect our management's ability to oversee the development of our product candidates.

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.

### **Our existing and any future indebtedness could adversely affect our ability to operate our business.**

As of March 31, 2014, we had \$13.2 million of outstanding borrowings under our loan and security agreement with Hercules, which we are required to repay in monthly installments through October 2015. We could in the future incur additional indebtedness beyond our borrowings from Hercules.

Our outstanding indebtedness combined with our other financial obligations and contractual commitments, including any additional indebtedness beyond our borrowings from Hercules, 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.

In addition, although the rate of interest that we are required to pay under the loan and security agreement is capped, our indebtedness under the loan and security agreement bears interest at a variable rate below that cap, making us vulnerable to increases in the market rate of interest. If the market rate of interest increases substantially, we will have to pay additional interest on this indebtedness, which would reduce cash available for our other business needs.

We intend to satisfy our current and future debt service obligations with our existing cash and cash equivalents and investments and funds from external sources. However, we may not have sufficient funds, and may be unable to arrange for additional financing, to pay the amounts due under our existing debt instruments. Failure to make payments or comply with other covenants under our existing debt instruments could result in an event of default and acceleration of amounts due. Under our loan and security agreement with Hercules, the occurrence of an event that would reasonably be expected to have a material adverse effect on our business, operations, assets or condition is an event of default. If an event of default occurs and the lender accelerates the amounts due, we may not be able to make accelerated payments, and the lender could seek to enforce security interests in the collateral securing such indebtedness, which includes substantially all of our assets other than our intellectual property. In addition, the covenants under our existing debt instruments, the pledge of our assets as collateral and the negative pledge with respect to our intellectual property could limit our ability to obtain additional debt financing.

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

**Our approach to the discovery and development of product candidates based on selective deuteration is unproven, and we do not know whether we will be able to develop any products of commercial value.**

We are focused on discovering and developing novel small molecule drugs that have improved metabolic or pharmacokinetic characteristics as a result of our selective substitution of deuterium for hydrogen. We apply our proprietary platform to systematically identify approved drugs, advanced clinical candidates or previously studied compounds that we believe can be improved with deuterium substitution to provide better pharmacokinetic or metabolic properties and thereby enhance clinical safety, tolerability or efficacy. To our knowledge, no deuterated drug has ever been approved for sale in the United States. While we believe that selective deuteration can produce compounds that possess favorable pharmaceutical properties, and preclinical studies and early-stage clinical trials have indicated that certain of our product candidates may possess these properties, we have not yet succeeded and may not succeed in demonstrating efficacy and safety for any of our product candidates in later stage clinical trials or in obtaining marketing approval thereafter. For example, although we have discovered and evaluated numerous deuterated compounds, we have not yet advanced a compound beyond Phase 2 clinical development.

**We are particularly dependent on the success of our product candidate, CTP-354, and our ability to develop, obtain marketing approval for and successfully commercialize CTP-354. CTP-354 is currently subject to a partial clinical hold that prevents us from administering doses in excess of 60 mg per day in single dose clinical trials and 6 mg per day in multiple dose clinical trials without an additional preclinical study. If we are unable to develop, obtain marketing approval for or successfully commercialize CTP-354, either alone 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 and are investing a significant portion of our efforts and financial resources in the development of CTP-354 for the treatment of spasticity. Our prospects are substantially dependent on our ability, or that of any future partner, to develop, obtain marketing approval for and successfully commercialize CTP-354.

In November 2013, we received notice from the FDA of a partial clinical hold on CTP-354 that prevents us from administering single doses in excess of 60 mg per day and multiple doses in excess of 6 mg per day and the FDA subsequently informed us that we may not administer multiple doses of CTP-354 in excess of 6 mg per day in clinical trials without first conducting an additional higher dose preclinical study. We do not intend to conduct single dose clinical trials of CTP-354 with doses in excess of 60 mg. While we believe that multiple doses of 6 mg per day would be sufficient for the treatment of spasticity, we have conducted an additional preclinical toxicology study to potentially enable us to evaluate higher doses of CTP-354, if needed in our spasticity trials, as well as to support clinical development in other disease indications. If we are required to perform additional preclinical studies to support the lifting of the partial clinical hold, it will increase our expected development costs and could delay the clinical development of CTP-354. If we are delayed in addressing, or unable to address, the FDA's concerns, we could be delayed, or prevented, from studying higher doses of CTP-354, which higher doses may be necessary to show efficacy. If these higher doses are necessary to show efficacy, we could be delayed or prevented from obtaining marketing approval of CTP-354.

The success of CTP-354 will depend on several factors, including the following:

- successful completion of clinical trials, which could require lifting of the partial clinical hold on CTP-354 or agreement by the FDA that the dosing protocols necessary to support successful completion of clinical trials are not subject to the partial clinical hold;
- receipt of marketing approvals from applicable regulatory authorities;
- our ability to develop a solid dose formulation of CTP-354;
- the performance of our future collaborators for CTP-354, if any;
- 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;
- establishment of arrangements with third party manufacturers to obtain finished drug products that are appropriately packaged for sale;

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- obtaining and maintaining patent, trade secret protection and regulatory exclusivity, both in the United States and internationally;
- protection of our rights in our intellectual property portfolio;
- launch of commercial sales if and when approved;
- a continued acceptable safety profile of CTP-354 following any marketing approval;
- commercial acceptance, if and when approved, by patients, the medical community and third party payors; and
- competition with other therapies, including baclofen, tizanidine, benzodiazepines and injected botulinum toxin.

If we are unable to develop, receive marketing approval for, or successfully commercialize CTP-354, or experience delays as a result of any of these factors or otherwise, our business could be materially harmed.

### **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 studies 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 population of patients, the occurrence of severe or medically or commercially unacceptable adverse events, failure to comply with protocols or applicable regulatory requirements and determination 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 or intolerability 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.

While we believe that our DCE Platform may enable drug discovery and clinical development that is more efficient and less expensive than conventional small molecule drug research and development, we may not be able to realize the advantages that we expect. In addition, while a key element of our drug discovery and development strategy involves utilizing existing information regarding non-deuterated compounds to assist the discovery and development of deuterated analogs of those compounds, not all of the product candidates that we develop are based on drugs or drug candidates that progressed into advanced clinical development. Particularly in these situations, existing information regarding the corresponding non-deuterated compound may not be sufficient to mitigate drug development risks. For example, we have pursued clinical development of CTP-499 for the potential treatment of type 2 diabetic kidney disease in patients with macroalbuminuria. CTP-499 is a deuterated analog of a metabolite of a drug that was not approved for this indication. CTP-499 failed to achieve statistical significance in the primary efficacy endpoint of urinary albumin to creatinine ratio at 24 weeks for a Phase 2 clinical trial. CTP-354 is subject to development risks normally inherent in clinical development because no corresponding non-deuterated compound has been clinically evaluated. While Merck & Co. reported that the non-deuterated analog of CTP-354 activated the 2, 3 and 5 GABA<sub>A</sub> receptors, which are associated with anti-spasticity, muscle relaxation, anti-anxiety, anti-seizure and, potentially, anti-pain activities, with approximately 40% of the *in vitro* activity of a benzodiazepine, we do not know if the pharmacological profile of CTP-354 will be clinically effective for treating spasticity at doses of CTP-354 that are well tolerated.

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.

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The outcome of preclinical 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. For example, although Phase 1 clinical trials of CTP-499 supported advancement into Phase 2 clinical trials, CTP-499 failed to achieve statistical significance in the primary efficacy endpoint of urinary albumin to creatinine ratio at 24 weeks in the Phase 2 clinical trial. However, we believe that the incidence of large declines in kidney function, measured as decreases in eGFR or increases in serum creatinine in drug-treated versus placebo-treated patients, could become the primary efficacy endpoint required by the FDA for Phase 3 clinical development of a drug candidate for the treatment of type 2 diabetic kidney disease. While incidence of 30% and 40% declines in eGFR was a secondary endpoint of our ongoing Phase 2 clinical trial, and the CTP-499 results with respect to eGFR declines and serum creatinine increases were encouraging at 48 weeks, the data was collected from a smaller number of patients and over a shorter duration than would be required for a Phase 3 clinical trial and these results may not be indicative of the results that we may achieve in Phase 3 clinical trials. Furthermore, the FDA may not accept incidence of large declines in eGFR or large increases in serum creatinine as acceptable endpoints for a Phase 3 clinical trial of CTP-499 for the treatment of type 2 diabetic kidney disease. If the endpoints that are acceptable to the FDA for our Phase 3 clinical trials in this indication differ from the endpoints of the clinical trials we have conducted of CTP-499, we may need to conduct additional clinical trials of CTP-499 to support entry into Phase 3 clinical evaluation, or we may be required to conduct Phase 3 trials that are longer or require more patients than we currently anticipate.

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. The design of a clinical trial can determine whether its results will support approval of a product and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced. We have limited experience in designing clinical trials and may be unable to design and execute a clinical trial to support marketing approval. In addition, preclinical and clinical data are often susceptible to varying interpretations and analyses. Many companies that believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval for the product candidates. 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.

In some instances, there can be significant variability in safety or efficacy results between different clinical trials of the same product candidate due to numerous factors, including changes in trial procedures set forth in protocols, differences in the size and type of the patient populations, changes in and adherence to the dosing regimen and other clinical trial protocols and the rate of dropout among clinical trial participants. For example, while we are conducting Phase 1 clinical trials to evaluate the safety and tolerability CTP-354 in healthy volunteers, we have not yet evaluated the safety or efficacy of CTP-354 administered in the intended patient population, which will be required for FDA approval, and the FDA has placed a partial clinical hold on CTP-354 that prevents us from administering doses in excess of 60 mg per day in single dose clinical trials and 6 mg per day in multiple dose clinical trials. Any Phase 2, Phase 3 or other clinical trials that we, or our collaborators, may conduct may not demonstrate the efficacy and safety necessary to obtain regulatory approval to market our product candidates.

**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, and our collaborators, are not permitted to commercialize, market, promote or sell any product candidate in the United States without obtaining marketing approval from the FDA. Comparable foreign regulatory authorities, such as the European Medicines Agency, or EMA, impose similar restrictions. We, and our collaborators, may never receive such approvals. We, and our collaborators, must complete extensive preclinical development and clinical trials to demonstrate the safety and efficacy of our product candidates in humans before we, or they, will be able to obtain these approvals. For example, as described above, the FDA has placed a partial clinical hold on CTP-354 that prevents us from administering doses in excess of 60 mg per day in single dose clinical trials and 6 mg per day in multiple dose clinical trials. If we are delayed in addressing, or unable to address, the FDA's concerns, we could be delayed, or prevented, from studying higher doses of CTP-354, which higher doses may be necessary to show efficacy. If these higher doses are necessary to show efficacy, we could be delayed or prevented from obtaining marketing approval of CTP-354.

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Clinical testing is expensive, difficult to design and implement, can take many years to complete and is inherently uncertain as to outcome. We, and our collaborators, have not previously submitted an NDA to the FDA or similar drug approval filings to comparable foreign regulatory authorities for any of our product candidates.

Any inability to successfully complete preclinical 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 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.

**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:

- clinical trials of our product candidates may produce unfavorable or inconclusive results, such as with Part 1 of our Phase 2 clinical trial for CTP-499;
- we, or our collaborators, may decide, or regulators may require us or them, to conduct additional 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;
- 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 from the clinical trial, increase the needed enrollment size for the clinical trial or extend the clinical trial's duration;
- we, or our collaborators, may have to suspend or terminate clinical trials of our product candidates for various reasons, including a finding that the participants are being exposed to unacceptable health risks, undesirable side effects or other unexpected characteristics of the product candidate;

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- regulators or institutional review boards 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 preclinical studies and clinical trials;
- the FDA or comparable foreign regulatory authorities may fail to approve or subsequently find fault with the manufacturing processes or facilities of third party manufacturers with which we, or our collaborators, enter into agreements for clinical and commercial supplies;
- 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; 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 preclinical tests or clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant preclinical 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.

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

We, or our collaborators, may not be able to initiate or continue clinical trials for CTP-354 or any of our other 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 EMA. 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 proximity of patients to clinical sites;
- the eligibility criteria for the trial;
- the design of the clinical trial;
- efforts to facilitate timely enrollment;
- competing clinical trials; 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 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

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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.

**We believe we, or our collaborators, may in some instances be able to secure clearances from the FDA or comparable foreign regulatory authorities to use expedited development pathways. If unable to obtain such clearances, we, or they, may be required to conduct additional preclinical studies or clinical trials beyond those that we, or they, contemplate, which could increase the expense of obtaining, and delay the receipt of, necessary marketing approvals.**

The deuterated compounds that we produce and seek to develop can have similar pharmacological properties as their corresponding non-deuterated compounds. Therefore, we believe that we, or our collaborators, may, in some instances, be able to obtain clearance from the FDA or comparable foreign regulatory authorities to follow expedited development programs for some deuterated compounds that reference and rely on findings previously obtained from prior preclinical studies or clinical trials of the corresponding non-deuterated compounds. For example, our collaborator Avanir reported in June 2013 that the FDA has agreed to an expedited development pathway for AVP-786, a product candidate Avanir is developing that includes our licensed deuterated dextromethorphan compound, permitting Avanir to reference data from its development of dextromethorphan and quinidine in its IND, and any future NDA, for AVP-786.

While we anticipate that following an expedited development pathway may be possible for some of our current and future product candidates, we cannot be certain that we, or our collaborators, will be able to secure clearance to follow such expedited development pathways from the FDA or comparable foreign regulatory authorities. In addition, if we follow, or one of our collaborators follows, such an expedited regulatory pathway and the FDA or comparable foreign regulatory authorities are not satisfied with the results of our having done so, such as might be the case if a deuterated compound is found to have undesirable side effects or other undesirable properties that were not anticipated based on the corresponding non-deuterated compound, the FDA or foreign regulatory authorities may be unwilling to grant clearance to follow expedited development pathways for other deuterated compounds.

Consequently, we, or our collaborators, may be required to pursue full development programs with respect to any product candidates that we, or they, previously anticipated would be able to follow an expedited development pathway, including conducting a full range of preclinical and clinical studies to attempt to establish the safety and efficacy of these product candidates. A need to conduct a full range of development activities would significantly increase the costs of development and length of time required before we, or our collaborators, could seek marketing approval of such a product candidate as compared to the costs and timing that we or they anticipate. While we have been able to reference, for purposes of some of our IND-enabling studies, data generated during development of the corresponding non-deuterated compound, we have not ourselves obtained clearance from the FDA or any comparable foreign regulatory authority to reference such data in connection with more advanced stages of development.

**Serious adverse events or undesirable side effects or other unexpected properties of CTP-354 or any of our other 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.**

Serious adverse events or undesirable side effects caused by, or other unexpected properties of, our product candidates could cause us, one of our collaborators, an institutional review board or regulatory authorities to interrupt, 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 increased 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 other 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.



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For CTP-354, we are seeking to achieve GABA<sub>A</sub> receptor occupancy levels that are well above those attained by other GABA<sub>A</sub> modulators, such as benzodiazepines, and we do not know what adverse effects may be associated with such high GABA<sub>A</sub> receptor occupancy. In our clinical trials of CTP-354, moderate adverse events have been reported including dizziness, drowsiness and nausea at single doses. Additional or more serious adverse events, undesirable side effects or other unexpected properties of CTP-354 or any of our other product candidates could arise or become known either during further clinical development or, if approved, after the approved product has been marketed. If such an event occurs during development, clinical trials for our product candidates could be suspended or terminated and the FDA or comparable foreign regulatory authorities could order us or our collaborators to cease further development, require us to conduct additional clinical trials or other tests or studies or deny approval of the applicable product candidate.

**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.**

We have never commercialized a product. Even if CTP-354 or any of our other 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 and others in the medical community. 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.

Efforts to educate the medical community 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 CTP-354 or any of our other 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, contained in the product's approved labeling;
- our ability, or the ability of our collaborators, to offer the product for sale at competitive 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;
- 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;
- availability and amount of reimbursement from government payors, managed care plans and other third party payors;
- adverse publicity about the product or favorable publicity about competitive products; and
- potential product liability claims.



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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 effective than previously believed or causes undesirable side effects that were not previously identified, 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 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 or seize the drug;
- we, or our collaborators, may be required 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;
- we may be subject to fines, injunctions or the imposition of civil or criminal penalties;
- regulatory authorities may require the addition of labeling statements, such as a “black box” warning or a contraindication;
- we, or our collaborators, may be required to create a Medication Guide outlining the risks of the previously unidentified side effects for distribution to patients;
- we, or our collaborators, could be sued and held liable for harm caused to patients;
- the drug may become less competitive; and
- our reputation may suffer.

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 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 plan 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.

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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 plan 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 plan to commercialize our product candidates outside the United States through collaboration, licensing and distribution arrangements with third parties. 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.

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 CTP-354 and any other of 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 the key indications of our priority programs, including spasticity, neurologic disorders, cancer and inflammation. Our competitors may succeed in developing, acquiring or licensing technologies and drug products that are more effective, 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, which could render our product candidates obsolete and noncompetitive.

We are initially developing CTP-354 for the treatment of spasticity in multiple sclerosis and spinal cord injury. Current first-line treatment for spasticity includes oral and local agents and physical and occupational therapy. Four oral drugs have been approved in the United States for the treatment of spasticity: baclofen (Lioresal®), tizanidine (Zanaflex®), diazepam (Valium) and dantrolene (Dantrium®), each of which is available on a generic basis. Spasticity is also treated through localized injections of botulinum toxin. In addition, there are several potentially competitive product candidates in Phase 3 clinical development being pursued by pharmaceutical and biotechnology companies, including GW Pharmaceuticals plc and Osmotica Pharmaceuticals Corp.

We are developing CTP-499 for the treatment of type 2 diabetic kidney disease in patients with macroalbuminuria. The current standard of care in this indication is angiotensin modulation, which is treatment with an angiotensin converting enzyme inhibitor, which we refer to as an ACEi, or an angiotensin receptor blocker, which we refer to as an ARB. Both of these types of drugs are available on a generic basis. We are developing CTP-499 for administration in combination with these drugs. These drugs are well established therapies that are widely accepted by physicians, patients and third party payors. Physicians, patients and third party payors may not accept the addition of CTP-499 to their current treatment regimens for a variety of potential reasons, including a desire not to

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incur the additional cost of CTP-499 or a perception that the addition of CTP-499 would be poorly tolerated or of limited benefit. If CTP-499 receives marketing approval, it may also face competition from a number of product candidates that are currently in clinical development including potentially competitive product candidates in Phase 3 clinical development being pursued by AbbVie Inc., Janssen Research & Development LLC and NephroGenex, Inc.

Avanir has reported that it plans to develop AVP-786 for the treatment of neurologic and psychiatric disorders. There are a number of marketed drugs and product candidates in clinical development for these indications.

JZP-386 is in preclinical development for the treatment of narcolepsy, with a potential advantage over sodium oxybate, the current standard of care, of being able to be administered in a single dose before bedtime rather than in two nighttime doses. Flamel Technologies is currently developing a narcolepsy therapy involving once nightly dosing by using its Micropump® technology applied to sodium oxybate. In April 2014, Flamel reported conducting a clinical study in healthy volunteers that identified formulations with the potential to eliminate the second nighttime dose currently required in treating narcolepsy with sodium oxybate and JZP-386 may complete with this therapy if approved in this indication.

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 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, preclinical 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.

### **We also face competition in the development of deuterated compounds.**

Several large pharmaceutical and biotechnology companies have begun to cover deuterated analogs of their product candidates in patent applications and may choose to develop these deuterated compounds. These large pharmaceutical and biotechnology companies may have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining marketing approvals and marketing approved products than we do. In addition, we know of one biotechnology company, Auspex Pharmaceuticals, Inc., and possibly two others, DeutRx LLC and Berolina innovative Research and Development Services Pharma GmbH, that are developing product candidates based on deuterium substitution. These competitors may be more successful than us in developing deuterated compounds. In addition, these competitors may enter into collaborative arrangements or business combinations that result in their ability to research and develop deuterated compounds more effectively than us. Our potential competitors also include academic institutions, government agencies and other public and private research organizations.

If our competitors in the development of deuterated compounds are able to grow their intellectual property estates and create new and successful deuterated compounds more effectively than us, our ability to identify additional compounds for preclinical and clinical development and obtain product revenues in future periods could be compromised, which could result in significant harm to our operations and financial position.

**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 an 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 may be 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 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, our products will likely 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 in the same indications. 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.

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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. 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.

Our ability, and the ability of our collaborators, to commercialize CTP-354 or any other product candidate will depend in part on the extent to which coverage and reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. 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 the prices for our products, if any, decrease or if governmental and other third party payors do not provide adequate coverage or reimbursement, our prospects for revenue and profitability will suffer.

There may also be delays in obtaining coverage and reimbursement for newly approved drugs, and coverage may be more limited than the indications for which the drug is 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 and are 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

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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 our 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.**

A significant portion of the research that we are conducting involves the development of new deuterated compounds using our DCE Platform. The drug discovery that we are conducting using our DCE Platform may not be successful in creating compounds that have commercial value or therapeutic utility beyond the corresponding non-deuterated compound, or at all. Our research programs may initially show promise in creating potential product candidates, yet fail to yield viable product candidates for clinical development for a number of reasons, including:

- deuterated analogs of existing non-deuterated compounds or newly designed deuterated compounds may not demonstrate satisfactory efficacy or other benefits, such as convenience of dosing, increased tolerability, enhanced formation of desirable active metabolites or reduced formation of toxic metabolites;
- potential product candidates may, on further study, be shown to have harmful side effects or other characteristics that indicate that they are unlikely to be products that will receive marketing approval and achieve market acceptance; or
- pharmaceutical companies have begun to claim deuterated analogs of their compounds in patent filings, resulting in otherwise promising deuterated product candidates already being covered by patents or patent applications.

Our research programs to identify new product candidates will require substantial technical, financial and human resources. We may be unsuccessful in our efforts to identify new potential product candidates. In addition, we may focus our efforts and resources on one or more potential product candidates that ultimately prove to be unsuccessful.

If we are unable to identify suitable additional compounds for preclinical 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. For example, we may be sued if any product we develop allegedly causes injury or is found to be otherwise unsuitable during clinical testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability or a breach of warranties. Claims could also be asserted under state consumer protection acts. 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 resulting litigation;
- initiation of investigations by regulators;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;

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- substantial monetary awards to trial participants or patients;
- loss of revenue;
- reduced resources of our management to pursue our business strategy; and
- the inability to commercialize any products that we may develop.

Although we maintain general liability insurance of \$2 million in the aggregate and clinical trial liability insurance of \$5 million in the aggregate, this insurance 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.

**JZP-386 is a deuterated analog of a Schedule I controlled substance and will likely be classified as a Schedule I or Schedule III controlled substance, which could substantially limit our ability to obtain the quantities of JZP-386 needed to conduct clinical trials and the ability of our collaborator to market and sell JZP-386 if it receives marketing approval. We also expect CTP-354 to be classified as a Schedule IV controlled substance, which would result in restrictions on the sale and distribution of that product if it receives marketing approval.**

The placement of drugs or other substances into schedules under the Controlled Substances Act of 1970, the CSA, is based upon the substance's medical use, potential for abuse and safety or dependence liability. Under the CSA, every person who manufactures, distributes, dispenses, imports or exports any controlled substance must register with the U.S. Drug Enforcement Agency, or DEA, unless exempt. Our product candidate JZP-386, which we have licensed to Jazz Pharmaceuticals, is a deuterium-substituted analog of sodium oxybate. Sodium oxybate is regulated as a chemical by the DEA as a Schedule I controlled substance. Because of the Schedule I classification of sodium oxybate, JZP-386 is regulated by the DEA as a Schedule I controlled substance. As a result, we or Jazz Pharmaceuticals will be required to obtain a license to ship the chemical intermediate that we are using as the precursor to JZP-386, which may delay or prevent the manufacturing of JZP-386 for clinical trials.

Specifically, the DEA limits the quantity of certain Schedule I controlled substances that may be produced in the United States in any year through a quota system. If our contract manufacturers for JZP-386, or those for Jazz Pharmaceuticals, manufacture JZP-386 in the United States, they will be required to obtain separate DEA quotas to supply us or Jazz Pharmaceuticals with JZP-386 for the conduct of clinical trials. Different, but potentially no less burdensome regulations, may apply if we or Jazz Pharmaceuticals choose to contract for the manufacture of JZP-386 outside of the United States.

The process of obtaining the quotas needed to conduct the planned clinical trials of JZP-386 may involve lengthy legal and other efforts and we or Jazz Pharmaceuticals, or suppliers or manufacturers for us or Jazz Pharmaceuticals, may not be able to obtain sufficient quotas from the DEA. If we or Jazz Pharmaceuticals, or suppliers or manufacturers for us or Jazz Pharmaceuticals, cannot obtain the quotas that are needed on a timely basis, or at all, we and Jazz Pharmaceuticals may not be able to conduct, on a timely basis or at all, the clinical trials of JZP-386 that are planned, including the Phase 1 clinical trial that we will be responsible for conducting, and our business, financial condition, results of operations and growth prospects could be adversely affected.

If JZP-386 is approved for marketing in the United States, we believe that the commercial drug containing JZP-386 will remain subject to the CSA as a Schedule III controlled substance. Those restrictions could limit the marketing and distribution of the commercial drug containing JZP-386. We also expect our product candidate, CTP-354, to be classified as a Schedule IV controlled substance under the CSA. Although the CSA's restrictions governing substances in Schedule IV are not as stringent as those for substances in Schedule III, they too could limit our ability to market and sell CTP-354, if it is approved for marketing.

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In addition, failure to maintain compliance with applicable requirements under the CSA, particularly as manifested in loss or diversion of regulated substances, can result in enforcement action that could include civil penalties, refusal to renew registrations or quotas, revocation of registrations or quotas or criminal proceedings, any of which could have a material adverse effect on our business, results of operations and financial condition. Individual states also regulate controlled substances, and we and Jazz Pharmaceuticals, and contract manufacturers for us and Jazz Pharmaceuticals, will be subject to state regulation on distribution of these products.

### **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 with Celgene, Avanir and Jazz Pharmaceuticals 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. 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 the following:

- 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 such as occurred in a prior collaboration we had with Glaxo Group Limited;
- 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;
- 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 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.



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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. For example, conducting pivotal Phase 3 clinical trials of CTP-499 in patients with type 2 diabetic kidney disease with macroalbuminuria will likely involve significant cost and we expect that we would conduct any large Phase 3 clinical trial of CTP-499 in type 2 diabetic kidney disease in collaboration with one or more partners. A key element of our business strategy is the development of deuterated product candidates based on approved drugs, advanced clinical candidates or previously studied compounds. Our likely collaborators for these product candidates in many cases will include the pharmaceutical companies that developed the corresponding non-deuterated compounds. In addition, likely collaborators may include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a 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 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 for similar indications that may be available for collaboration and whether such a 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 on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail 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 preclinical or clinical studies of the corresponding non-deuterated compound.

**We rely on third parties to conduct our clinical trials. If they 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. Any of these third parties may terminate their engagements with us under certain circumstances. If we need to enter into alternative arrangements, it could delay our product development activities.

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

Furthermore, the third parties conducting clinical trials on our behalf 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, for whom they may also be conducting clinical trials or other drug development activities, 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 our clinical trials in accordance with 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 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.

**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 two suppliers, one located in the United States and one located in another country. We may establish a deuterium oxide supply arrangement with an additional supplier, which is located outside of the United States and is affiliated with a foreign government. It is also possible that our current U.S. supplier of deuterium oxide relies on one or more foreign suppliers for its supply of deuterium oxide, although we are not familiar with its procurement practices.

We estimate that our current source of deuterium oxide will be sufficient to meet our anticipated requirements through at least 2015. However, we do not have long-term agreements with our current suppliers. If we are not able

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to establish or maintain supply arrangements with the suppliers from which we have purchased and believe we may be able to purchase additional deuterium oxide, or the relevant foreign governments decide to withhold any authorization 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.

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 our current foreign supplier, our supplier will 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. Most 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 will depend on the ability of these manufacturers to obtain deuterium oxide from other countries. 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.

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, including those for CTP-499 and certain other of our product candidates, 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.

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**We contract with third parties for the manufacture and distribution of our product candidates for clinical trials 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, which could delay, prevent or impair our development or commercialization efforts.**

We currently have only very limited internal capabilities to manufacture our product candidates. We currently rely, and expect to continue to rely, on third party contractors to manufacture preclinical 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, 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 if our third party contractors give greater priority to the supply of other products over our product candidates or otherwise do not satisfactorily perform according to the terms of the agreements between us and them;
- the possible termination or nonrenewal of agreements by our third party contractors at a time that is costly or inconvenient for us;
- the possible breach by the third party contractors of our agreements with them;
- 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;
- 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.

We currently rely on a small number of third party contract manufacturers to supply the majority of our required finished product for our preclinical studies and clinical trials. We do not have long-term agreements with any of these third parties. If any of our existing manufacturers should become unavailable to us for any reason, we may incur some delay in identifying or qualifying replacements.

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 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 current good manufacturing practices, or 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 approved 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 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 cannot successfully manufacture material that conforms to the strict regulatory requirements of the FDA and any applicable foreign regulatory authority, they will not be able to secure the applicable approval for their manufacturing facilities. 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.

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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 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 compounds are 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.

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 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 limit, in part or in whole, our ability to seek and obtain patent protection for new product candidates based on deuterium modification of compounds. It may also limit in part or in whole, our ability to develop new product candidates based on deuterium modification of such compounds without obtaining a license from those patent holders.

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.

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Moreover, we may be subject to a third party preissuance submission of prior art to the U.S. Patent and Trademark Office, or become involved in opposition, derivation, reexamination, inter partes review or interference proceedings, in the United States or elsewhere, challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation 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 which 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 United States 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. Thus, even if we have valid and 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 U.S. Patent and Trademark Office post-grant review procedures. 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.

**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. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their patents, in addition to counterclaims asserting that our patents are invalid or unenforceable, or both. 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 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. Similarly, if we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks.

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 or 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.

**If we are sued for infringing intellectual property rights of third parties, 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 and use our DCE Platform without infringing the intellectual property and other proprietary rights of third parties. Numerous third party U.S. and non-U.S. issued patents and pending applications exist for compounds and methods of use for the treatment of spasticity, kidney disease, neurologic disorders, cancer and inflammation, the key indications for our priority programs. In addition, some of the non-deuterated compounds on which our product candidates are, or future product candidates may be, based 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. 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.

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 regarding intellectual property rights with respect to our products candidates, including interference proceedings before the U.S. Patent and Trademark Office. 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 pharmaceutical and biotechnology industries have produced a significant number of patents, and it may not always be clear to industry participants, including us, which patents cover various types of products or methods of use. 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 patent claims of the relevant patent or that the



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 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. However, we are not aware of any judicial proceedings addressing the question of whether our product would be outside the scope of such a patent claim. 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. 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.

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

**Even if we complete the necessary preclinical and clinical studies, the marketing approval process is expensive, time consuming and uncertain and may prevent us or our collaborators from obtaining 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. We, and our collaborators, are not permitted to market our product candidates in the United States or in other countries until we, or they, receive approval of an NDA from the FDA or marketing approval from applicable regulatory authorities outside the United States. 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 conducting and managing the clinical trials necessary to obtain marketing approvals, including FDA approval of an NDA.

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.

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 preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical 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.



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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, and 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 countries outside the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. 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.

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**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 Risk Evaluation and Mitigation Strategy, or 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 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;
- 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. We expect that current laws, as well as other healthcare reform measures that be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we, or our collaborators, may receive for any approved products.

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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 and could decrease the coverage and price that we, or our collaborators, may 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.

More recently, 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 United States 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 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. These include the following:

- *Anti-Kickback Statute.* The federal healthcare anti-kickback statute prohibits, among other things, persons 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 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, currently set at \$5,500 to \$11,000 per false claim;
- *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 making false statements relating to healthcare matters, 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 laws require applicable manufacturers of covered drugs to report payments and other transfers of value to physicians and teaching hospitals;
- *Controlled Substances Act.* The CSA regulates the handling of controlled substances such as JZP-386 and, potentially, CTP-354; and
- *Analogous State and Foreign Laws.* Analogous state and foreign fraud and abuse 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 and are generally broad and are enforced by many different federal and state agencies as well as through private actions.

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.

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### **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 and in the future, our operations may involve the use of hazardous and flammable 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. However, 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. 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 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 medical 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

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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.**

We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug manufacturing, regulatory affairs and sales, 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. Our inability to effectively manage the expansion of our operations may result in weaknesses in our infrastructure, give rise to operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our expected growth, our expenses may increase more than expected, our ability to generate revenues could be reduced and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize product candidates and compete effectively will depend, in part, on our ability to effectively manage any future growth.

## **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.**

Our stock price may be volatile. 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. As a result of this volatility, investors may not be able to sell their common stock at or above the prices they paid for it. The market price for our common stock may be influenced by many factors, including:

- the success of existing or new competitive products or technologies;
- the timing and results of clinical trials of CTP-354 and CTP-499 and any other product candidate;
- commencement or termination of collaborations for our development programs;
- failure or discontinuation of any of our development programs;
- results of clinical trials of product candidates of our competitors;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- 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;

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- sales of our common stock by us, our insiders or other stockholders;
- 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, if any, that cover our stock;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- 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 will have 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 an “emerging growth company,” and the reduced disclosure requirements applicable to emerging growth companies may make our common stock less attractive to investors.**

We are an “emerging growth company,” as defined in the JOBS Act, and may remain an emerging growth company for up to five years. For so long as we remain an emerging growth company, we are permitted and plan to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or SOX Section 404, not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor’s report providing additional information about the audit and the financial statements, reduced disclosure obligations regarding executive compensation and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved. 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.

In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, we are subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

**We are currently incurring and expect to continue to incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives and corporate governance practices.**

As a newly public company, we are incurring and expect to incur additional significant legal, accounting and other expenses that we did not incur as a private company. We expect that these expenses will further increase after we are no longer an “emerging growth company.” The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of The NASDAQ Global Market and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. We expect that we will need to hire additional accounting, finance and other personnel to comply with the requirements of being a public company, and our management and other personnel will need to devote a substantial amount of time towards maintaining compliance with these requirements. These requirements will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect that the rules and regulations applicable to us as a public company may make it more difficult and more expensive for us to obtain director and officer liability insurance, which could make it more difficult for us to attract and retain qualified members of our board of directors. We are currently evaluating these rules and regulations, and cannot predict or estimate the amount of additional costs we may incur or the timing of such costs. These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

Pursuant to SOX Section 404 we will be required to furnish a report by our management on our internal control over financial reporting beginning with our second filing of an Annual Report on Form 10-K with the SEC. However, while we remain an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with SOX Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude, within the prescribed timeframe or at all, 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 is restricted from immediate resale but 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. Of the 17,899,585 shares of our common stock outstanding as of April 30, 2014, 11,695,895 shares are currently subject to restrictions on transfer under 180-day lock-up arrangements with either the underwriters for our initial public offering or under stock option agreements entered into between us and the holders of those shares. These restrictions are due to expire on August 11, 2014, resulting in these shares becoming eligible for public sale on August 12, 2014 if they are registered under the Securities Act of 1933, as amended, which we refer to as the Securities Act, or if they qualify for an exemption from registration under the Securities Act, including under Rules 144 or 701.

Moreover, holders of an aggregate of 9,919,821 shares of our common stock, have rights, subject to conditions, to require us to file registration statements covering their shares or, along with the holder of a warrant to purchase 70,796 shares of common stock, to include their shares in registration statements that we may file for ourselves or other stockholders. In addition, we have registered 4,159,374 shares of common stock that we may issue under our equity compensation plans. These shares can be freely sold in the public market upon issuance and once vested, subject to volume limitations applicable to affiliates and the lock-up arrangements described above.



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**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, the terms of our debt facility with Hercules preclude us from paying dividends, and any future debt agreements may also 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.**

As of April 30, 2014, our executive officers and directors, combined with our stockholders who owned more than 5% of our outstanding common stock, and their affiliates, in the aggregate, beneficially owned shares representing approximately 48.2% 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.

**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 bylaws 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 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 stockholder meetings;
- 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 bylaws.

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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 would likely 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.

## **Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.**

### **Recent Sales of Unregistered Securities**

Set forth below is information regarding shares of common stock issued, and options granted, by us during the three months ended March 31, 2014 that were not registered under the Securities Act of 1933, as amended, or the Securities Act. Included is the consideration, if any, we received for such shares, options and warrants and information relating to the section of the Securities Act, or rule of the Securities and Exchange Commission, or the SEC, under which exemption from registration was claimed. No underwriters were involved in any such issuances.

During the three months ended March 31, 2014, we granted options to purchase an aggregate of 148,893 shares of common stock, at a weighted-average exercise price of \$14.06 per share, to an employee and a director pursuant to our 2014 Stock Incentive Plan. During the three months ended March 31, 2014, we issued an aggregate of 31,775 shares of common stock upon the exercise of options for aggregate consideration of \$104,930.

The stock options described above were issued in reliance on the exemption provided by Section 4(a)(2) of the Securities Act and the common stock issuable upon the exercise of such options described above were issued pursuant to written compensatory plans or arrangements with our employees, directors and consultants, in reliance on the exemption provided by Rule 701 promulgated under the Securities Act. All recipients either received adequate information about us or had access, through employment or other relationships, to such information.

### **Use of Proceeds**

We effected the initial public offering, or IPO, of our common stock through a Registration Statement on Form S-1 (File No. 333-193335) that was declared effective by the Securities and Exchange Commission on February 12, 2014, and a registration statement on Form S-1 (File No. 333-193920) filed pursuant to Rule 462(b) of the Securities Act that became effective on February 12, 2014. On February 19, 2014, we completed the sale of 6,000,000 shares of common stock in our IPO at a price to the public of \$14.00 per share, resulting in net proceeds to us of \$74.6 million after deducting underwriting discounts and commissions of \$5.9 million and offering costs of \$3.5 million. In addition, we granted the underwriters a 30-day option to purchase up to 900,000 additional shares of common stock at the IPO price to cover over allotments, if any. On March 3, 2014, we completed the additional sale of 649,690 shares of common stock under this option at a price to the public of \$14.00 per share, resulting in net proceeds to us of \$8.5 million after deducting underwriting discounts and commissions of \$0.6 million. The offering commenced on February 12, 2014 and terminated before the sale of all of the securities registered in the offering. None of the underwriting discounts and commissions or other offering expenses were paid to directors or officers of ours or their associates or to persons owning 10 percent or more of our common stock or to any affiliates of ours. UBS Securities LLC and Wells Fargo Securities, LLC acted as joint book-running managers of the offering and as representatives of the underwriters. JMP Securities LLC acted as lead manager and Roth Capital Partners, LLC acted as co-manager for the offering. There were no selling stockholders in the offering.

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As of May 10, 2014, we estimate that we have used approximately \$11.2 million of the net proceeds primarily to fund the development of CTP-354, to advance and expand the research and preclinical development of additional product candidates and for working capital, capital expenditures and other general corporate purposes. None of the net proceeds were paid directly or indirectly to directors or officers of ours or their associates or to persons owning 10 percent or more of our common stock or to any affiliates of ours, other than payments in the ordinary course of business to officers for salaries and to non-employee directors as compensation for board or board committee service. We have invested the balance of the net proceeds from the offering in a variety of capital preservation investments, including short-term, investment grade, interest bearing instruments and U.S. government securities. There has been no material change in our planned use of the balance of the net proceeds from the offering as described in our final prospectus filed with the SEC pursuant to Rule 424(b) under the Securities Act.

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**Item 6. Exhibits.**

The exhibits listed in the Exhibit Index to this Quarterly Report on Form 10-Q are incorporated herein by reference.

**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: May 14, 2014

By: /s/ Ryan Daws

Ryan Daws  
Chief Financial Officer

**Exhibit Index**

<b><u>Exhibit number</u></b>	<b><u>Description</u></b>
3.1	Restated Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.1 to the Registrant's current report on Form 8-K (File No. 001-36310) filed with the SEC on February 20, 2014)
3.2	Amended and Restated Bylaws of the Registrant (incorporated by reference to Exhibit 3.2 to the Registrant's current report on Form 8-K (File No. 001-36310) filed with the SEC on February 20, 2014)
10.1	2014 Stock Incentive Plan (incorporated by reference to Exhibit 10.6 to the Registrant's registration statement on Form S-1 (File No. 333-193335) filed with the SEC on February 3, 2014)
10.2	Form of Incentive Stock Option Agreement under 2014 Stock Incentive Plan (incorporated by reference to Exhibit 10.7 to the Registrant's registration statement on Form S-1 (File No. 333-193335) filed with the SEC on February 3, 2014)
10.3	Form of Nonstatutory Stock Option Agreement under 2014 Stock Incentive Plan (incorporated by reference to Exhibit 10.8 to the Registrant's registration statement on Form S-1 (File No. 333-193335) filed with the SEC on February 3, 2014)
10.4	Amended and Restated Employment Agreement, dated as of January 10, 2014, by and between the Registrant and Roger Tung, as amended (incorporated by reference to Exhibit 10.9 to the Registrant's registration statement on Form S-1 (File No. 333-193335) filed with the SEC on January 13, 2014)
10.5	Amended and Restated Employment Agreement, dated as of January 10, 2014, by and between the Registrant and Nancy Stuart, as amended (incorporated by reference to Exhibit 10.10 to the Registrant's registration statement on Form S-1 (File No. 333-193335) filed with the SEC on January 13, 2014)
10.6	Amended and Restated Employment Agreement, dated as of January 10, 2014, by and between the Registrant and I. Robert Silverman, as amended (incorporated by reference to Exhibit 10.12 to the Registrant's registration statement on Form S-1 (File No. 333-193335) filed with the SEC on January 13, 2014)
10.7	Employment Agreement, dated as of January 16, 2014, by and between the Registrant and D. Ryan Daws (incorporated by reference to Exhibit 10.21 to the Registrant's registration statement on Form S-1 (File No. 333-193335) filed with the SEC on February 3, 2014)
31.1*	Chief 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
31.2*	Chief 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
32.1*	Chief Executive Officer — Certification pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
32.2*	Chief Financial Officer — Certification pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
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.

† In accordance with Rule 406T of Regulation S-T, XBRL (Extensible Business Reporting Language) information is furnished and not filed or a part of a registration statement or prospectus for purposes of Sections 11 or 12 of the Securities Act of 1933, as amended, is deemed not filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and is not otherwise subject to liability under these sections.

**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)) 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) 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
  - (c) 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: May 14, 2014

/s/ Roger D. Tung

Roger D. Tung  
President and Chief 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, Ryan Daws, 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)) 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) 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
  - (c) 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: May 14, 2014

/s/ Ryan Daws

Ryan Daws  
Chief 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 March 31, 2014, 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: May 14, 2014

/s/ Roger D. Tung

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Roger D. Tung

President and Chief 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 March 31, 2014, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Ryan Daws, 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: May 14, 2014

/s/ Ryan Daws

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Ryan Daws  
Chief 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.*