CONCERT PHARMACEUTICALS, INC.
Form 10-Q
May 05, 2016

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

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FORM 10-Q

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(Mark One)
ý QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the quarterly period ended March 31, 2016
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

_____

CONCERT PHARMACEUTICALS, INC.
(Exact name of registrant as specified in its charter)

_____

Delaware (State or other jurisdiction of incorporation or organization)	20-4839882 (I.R.S. Employer Identification No.)
99 Hayden Avenue, Suite 500 Lexington, Massachusetts (Address of principal executive offices)	02421 (Zip Code)

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

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 ý 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 ý 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 x
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 ý

The number of shares outstanding of the registrant's common stock as of April 29, 2016: 22,216,718

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PART I. FINANCIAL INFORMATION

Item 1. Financial Statements.

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

	March 31, 2016	December 31, 2015
Assets		
Current assets:		
Cash and cash equivalents	$37,821	$ 92,510
Investments, available for sale	89,898	49,680
Interest receivable	379	181
Accounts receivable	152	70
Prepaid expenses and other current assets	2,305	1,667
Total current assets	130,555	144,108
Property and equipment, net	2,248	2,346
Restricted cash	400	400
Other assets	92	78
Total assets	$133,295	$ 146,932
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$771	$ 501
Accrued expenses and other liabilities	3,450	4,772
Income taxes payable	—	75
Deferred revenue, current portion	1,250	1,279
Total current liabilities	5,471	6,627
Deferred revenue, net of current portion	8,885	8,891
Deferred lease incentive, net of current portion	493	573
Deferred rent, net of current portion	184	206
Total liabilities	15,033	16,297
Commitments		
Stockholders' equity:		
Preferred stock, $0.001 par value per share; 5,000,000 shares authorized; no shares issued and outstanding in 2016 and 2015, respectively		
Common stock, $0.001 par value per share; 100,000,000 shares authorized; 22,210,797 and 22,166,803 shares issued and 22,209,160 and 22,165,166 outstanding in 2016 and 2015, respectively	22	22
Additional paid-in capital	253,251	251,793
Accumulated other comprehensive income (loss)	31	(18)
Accumulated deficit	(135,042)	(121,162)
Total stockholders' equity	118,262	130,635
Total liabilities and stockholders' equity	$133,295	$ 146,932

See accompanying notes.

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

	Three Months Ended March 31,	
	2016	2015
Revenue:		
License and research and development revenue	$56	$1,306
Total revenue	56	1,306
Operating expenses:		
Research and development	10,453	6,944
General and administrative	3,577	3,233
Total operating expenses	14,030	10,177
Loss from operations	(13,974)	(8,871)
Investment income	94	17
Interest and other expense	—	(148)
Net loss	$(13,880)	$(9,002)
Other comprehensive income (loss):		
Unrealized gain on investments	49	20
Comprehensive loss	$(13,831)	$(8,982)
Net loss per share applicable to common stockholders — basic and diluted	$(0.63)	$(0.48)
Weighted-average number of common shares used in net loss per share applicable to common stockholders— basic and diluted	22,198	18,726

See accompanying notes.

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

	Three Months Ended March 31,	
	2016	2015
Operating activities		
Net loss	$(13,880)	$(9,002)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	210	183
Stock-based compensation expense	1,282	646
Accretion of premiums and discounts on investments	134	181
Amortization of discount on loan payable	—	25
Amortization of deferred financing costs	—	9
Amortization of deferred lease incentive	(78)	(76)
Loss on disposal of asset	2	—
Changes in operating assets and liabilities:		
Accounts receivable	(82)	898
Interest receivable	(198)	64
Prepaid expenses and other current assets	(638)	(1,045)
Other assets	(14)	8
Accounts payable	270	444
Accrued expenses and other liabilities	(1,359)	(2,598)
Income taxes payable	(75)	—
Deferred rent	(9)	(20)
Deferred revenue	(35)	(753)
Net cash used in operating activities	(14,470)	(11,036)
Investing activities		
Purchases of property and equipment	(92)	(150)
Purchases of investments	(71,898)	(5,576)
Maturities of investments	31,595	22,000
Net cash (used in) provided by investing activities	(40,395)	16,274
Financing activities		
Principal payments on loan payable	—	(2,089)
Proceeds from sale of common stock, net of underwriting discounts and commissions	—	46,995
Proceeds from exercise of stock options	176	310
Payment of public offering costs	—	(34)
Net cash provided by financing activities	176	45,182
Net (decrease) increase in cash and cash equivalents	(54,689)	50,420
Cash and cash equivalents at beginning of period	92,510	13,396
Cash and cash equivalents at end of period	$37,821	$63,816
Supplemental cash flow information:		
Cash paid for interest	$—	$138
Cash paid for income taxes	$85	$—
Purchases of property and equipment unpaid at period end	$64	$92
Public offering costs incurred but unpaid at period end	$—	$276

See accompanying notes.

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

1. Nature of Business

Concert Pharmaceuticals, Inc., or Concert or the Company, was incorporated on April 12, 2006 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, enhancing 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 research and development. The Company's pipeline includes multiple clinical-stage candidates and a number of preclinical compounds that it is currently assessing.

In March 2015, the Company sold 3,300,000 shares of common stock in a public offering at a price to the public of $15.15 per share, resulting in net proceeds to the Company of approximately $46.7 million after deducting underwriting discounts and commissions and offering expenses. In June 2015, the Company received a one-time payment of $50.2 million from Auspex Pharmaceuticals, Inc. pursuant to a patent assignment agreement between Concert and Auspex. Concert became eligible to receive the payment due to a change of control of Auspex, which was acquired by Teva Pharmaceuticals Industries Ltd. in May 2015. For additional details regarding the one-time payment received from Auspex, refer to the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2015, which was filed with the Securities and Exchange Commission on March 1, 2016.

The Company had cash and cash equivalents and investments of $127.7 million at March 31, 2016. The Company believes that its cash and cash equivalents and investments at March 31, 2016 will be sufficient to allow the Company to fund its current operating plan for at least the next twelve months. The Company may pursue additional cash resources through public or private financings and by establishing collaborations with or licensing its technology to other companies.

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

2. Basis of Presentation and Significant Accounting Policies

Basis of Presentation

The accompanying condensed consolidated financial statements have been prepared in accordance with generally accepted accounting principles for interim financial information and with the instructions to Form 10-Q and Article 10 of Regulation S-X. Accordingly, they do not include all of the information and footnotes required by generally accepted accounting principles for complete financial statements. In the opinion of management, all adjustments, consisting of normal recurring accruals and revisions of estimates, considered necessary for a fair presentation of the condensed consolidated financial statements have been included. Interim results for the three months ended March 31, 2016 are not necessarily indicative of the results that may be expected for the fiscal year ending December 31, 2016 or any other future period.

The accompanying condensed consolidated financial statements reflect the accounts of Concert and its subsidiaries. All intercompany transactions between the Company and its subsidiary have been eliminated. Management has determined that the Company operates in one segment: the development of pharmaceutical products on its own behalf or in collaboration with others. The information included in this quarterly report on Form 10-Q should be read in conjunction with the Company's consolidated financial statements and the accompanying notes included in the Company's Annual Report on Form 10-K for the year ended December 31, 2015 filed with the Securities and Exchange Commission on March 1, 2016.

Use of Estimates and Summary of Significant Accounting Policies

The preparation of condensed consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that can affect the reported amounts of assets, liabilities, equity, revenue and expenses and the disclosure of contingent assets and liabilities. In preparing the condensed consolidated financial

statements, management used estimates in the following areas, among others: revenue recognition for multiple-element revenue arrangements, stock-based compensation expense; and accrued expenses. Actual results could differ from those estimates.

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, 2015.

Recent Accounting Pronouncements

In May 2014, the Financial Accounting Standard Board, or FASB, issued Accounting Standards Update, or ASU, No. 2014-09, Revenue from Contracts with Customers (Topic 606), or ASU 2014-09, which stipulates that an entity should recognize revenue to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. To achieve this core principle, ASU 2014-09 provides that an entity should apply the following steps: (1) identify the contract(s) with a customer, (2) identify the performance obligations in the contract, (3) determine the transaction price, (4) allocate the transaction price to the performance obligations in the contract and (5) recognize revenue when (or as) the entity satisfies a performance obligation. This update will be effective for the Company beginning in the first quarter of fiscal 2018 as a result of the FASB's one year deferral of the effective date for this standard. Early adoption is permitted, however not before the original effective date of annual periods beginning on or after December 15, 2016. The Company is currently assessing the impact of this ASU on its financial statements.

In August 2014, the FASB issued ASU No. 2014-15, Disclosure of Uncertainties About an Entity's Ability to Continue as a Going Concern, or ASU 2014-15 ASU 2014-15 amends FASB Accounting Standards Codification, or ASC, 205-40, Presentation of Financial Statements – Going Concern, by providing guidance on determining when and how reporting entities must disclose going-concern uncertainties in their financial statements, including requiring management to perform interim and annual assessments of an entity's ability to continue as a going concern within one year of the date of issuance of the entity's financial statements and providing certain disclosures if there is substantial doubt about the entity's ability to continue as a going concern. ASU 2014-15 will be effective for the Company's fiscal year 2016 and for interim periods beginning in the first quarter of fiscal 2017. If this standard had been adopted as of March 31, 2016, the Company believes that it would have concluded there was not substantial doubt about its ability to continue as a going concern. However, the Company's disclosures in future periods may be affected by the adoption of this accounting standard.

In February 2016, the FASB issued ASU No. 2016-02, Leases (Topic 842), or ASU 2016-02. ASU 2016-02 requires lessees to recognize assets and liabilities on the balance sheet for the rights and obligations created by all leases with terms of more than 12 months. ASU 2016-02 also will require certain qualitative and quantitative disclosures designed to give financial statement users information on the amount, timing, and uncertainty of cash flows arising from leases. ASU 2016-02 will be effective for the Company on January 1, 2019, with early adoption permitted. The Company is currently evaluating the impact ASU 2016-02 will have on its financial statements.

In March 2016, the FASB issued ASU No. 2016-09, Compensation-Stock Compensation-Improvements to Employee Share-Based Payment Accounting, or ASU 2016-09. This update simplifies several aspects of the accounting for share-based compensation arrangements, including accounting for income taxes, forfeitures and statutory tax withholding requirements as well as classification of related amounts on the statement of cash flows. The ASU is effective for fiscal years beginning after December 15, 2016, with early adoption permitted. The Company is currently evaluating the effect that the updated standard will have on its financial statements and related disclosures.

3. Fair Value Measurements

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

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

The tables below present information about the Company's financial assets and liabilities that are measured and carried at fair value as of March 31, 2016 and December 31, 2015 (in thousands) and indicate the level within the fair value hierarchy where each measurement is classified.

	Level 1	Level 2	Level 3	Total
	(in thousands)			
March 31, 2016				
Cash equivalents:				
Money market funds	$24,831	$—	$ —	$24,831
U.S. Treasury obligations	5,000	—	—	5,000
Government agency securities	6,008	—	—	6,008
Investments, available for sale:				
U.S. Treasury obligations	32,159	—	—	32,159
Government agency securities	37,915	19,824	—	57,739
Total	$105,913	$19,824	$ —	$125,737

	Level 1	Level 2	Level 3	Total
December 31, 2015				
Cash equivalents:				
Money market funds	$52,221	$—	$ —	$52,221
U.S. Treasury obligations	5,001	—	—	5,001
Government agency securities	—	34,390	—	34,390
Investments, available for sale:				
U.S. Treasury obligations	9,781	—	—	9,781
Government agency securities	19,578	20,321	—	39,899
Total	$86,581	$54,711	$ —	$141,292

4. Cash, Cash Equivalents and Investments

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 (loss). 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, 2016 and December 31, 2015:

	Average maturity	Amortized cost	Unrealized gains	Unrealized losses	Fair value
		(in thousands)			
March 31, 2016					
Cash		$1,982	$ —	$ —	$1,982
Money market funds		24,831	—	—	24,831
U.S. Treasury obligations	30 days	5,000	—	—	5,000
Government agency securities	15 days	6,008	—	—	6,008
Cash and cash equivalents		$37,821	$ —	$ —	$37,821
U.S. Treasury obligations	204 days	$32,145	15	(1)	$32,159
Government agency securities	108 days	57,722	18	(1)	57,739
Investments, available for sale		$89,867	$ 33	$ (2)	$89,898

	Average maturity	Amortized cost	Unrealized gains	Unrealized losses	Fair value
December 31, 2015					
Cash		$ 898	$ —	$ —	$ 898
Money market funds		52,221	—	—	52,221
U.S. Treasury obligations	31 days	5,002	—	(1)	5,001
Government agency securities	41 days	34,389	1	—	34,390
Cash and cash equivalents		$ 92,510	$ 1	$ (1)	$ 92,510
U.S. Treasury obligations	42 days	$ 9,785	$ —	$ (4)	$ 9,781
Government agency securities	104 days	39,913	1	(15)	39,899
Investments, available for sale		$ 49,698	$ 1	$ (19)	$ 49,680

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 2016 and 2015, there were no realized gains or losses on sales of investments, and no investments were adjusted for other than temporary declines in fair value.

5. Accrued Expenses and Other Liabilities

Accrued expenses and other liabilities consisted of the following:

	March 31, 2016	December 31, 2015
Accrued professional fees and other	$ 573	$ 732
Employee compensation and benefits	631	2,503
Research and development expenses	1,865	1,171
Deferred lease incentive, current portion	317	315
Deferred rent, current portion	64	51
	$ 3,450	$ 4,772

6. Collaborations

Celgene

In April 2013, the Company entered into a master development and license agreement with Celgene Corporation and Celgene International Sàrl, referred to together as Celgene, which is primarily focused on the research, development and commercialization of specified deuterated compounds targeting inflammation or cancer.

The initial program in the collaboration is CTP-730, a deuterium-modified analog of apremilast. Celgene has an exclusive worldwide license to develop, manufacture and commercialize deuterated analogs of apremilast and certain 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 was responsible for conducting and funding research and early development activities for the CTP-730 program at its own expense pursuant to mutually agreed upon development plans. This included the completion of single and multiple ascending dose Phase 1 clinical trials in 2015.

Under the terms of the agreement, the Company received a non-refundable upfront payment of $35.0 million. In October 2015, the Company achieved an $8.0 million development milestone upon completion of Phase 1 clinical evaluation of CTP-730. In addition, the Company is eligible to earn an additional $15.0 million development milestone payment, 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 CTP-730 program. The next milestone payment the Company may be entitled to achieve under the CTP-730 program is $15.0

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million related to the first dosing in a Phase 3 clinical trial or, if earlier, acceptance for filing of a NDA. If Celgene exercises its rights with respect to either of the two additional license programs, the Company 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, the Company is eligible to receive up to $100.0 million in 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 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, 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 worldwide net sales of each licensed product at defined percentages ranging from the mid-single digits to low double digits below 20%. 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 CTP-730 program, or the License Deliverable, (ii) obligations to perform research and development services associated with the CTP-730 program, or the R&D Services Deliverable, (iii) obligation to supply preclinical and clinical trial material related to the CTP-730 program, or the Supply Deliverable, (iv) participation on the JSC during the term of the CTP-730 program, or the JSC Deliverable, (v) significant and incremental discount related to the first additional license program for which the non-deuterated compound has been selected, or 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, or the Second Discount Deliverable.

Allocable arrangement consideration at inception was limited to the $35.0 million non-refundable upfront payment. The Company allocated the arrangement consideration for the collaboration among the separate units of accounting using the relative selling price method. 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 45 months and the amount allocated to the JSC Deliverable is recognized ratably over the expected period of performance, or 45 months.

During the three months ended March 31, 2016 and 2015, the Company recognized revenue of $8 thousand and $0.6 million for the R&D Services Deliverable and $20 thousand and $0.1 million for the Supply Deliverable, respectively. 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, 2016, there was $7.3 million of deferred revenue related to the Company's collaboration with Celgene, $1.2 million of which relates to the Supply Deliverable and R&D Services Deliverable and was classified as a current liability and $6.1 million of which relates to the First and Second Discount Deliverables and was classified as a noncurrent liability, in the accompanying condensed consolidated balance sheet.

Jazz Pharmaceuticals

In February 2013, the Company entered into a development and license agreement with Jazz Pharmaceuticals, Inc., or Jazz Pharmaceuticals, to research, develop and commercialize deuterated sodium oxybate analogs, or D-SXB. Jazz Pharmaceuticals is focusing on one analog, designated as JZP-386. Under the terms of the agreement, the Company granted Jazz Pharmaceuticals an exclusive, worldwide, royalty-bearing license under intellectual property controlled by the Company to develop, manufacture and commercialize D-SXB products including, but not limited to, JZP-386. The Company, together with Jazz Pharmaceuticals, has conducted certain development activities for Phase 1 clinical trials with respect to JZP-386 pursuant to an agreed upon development plan. The Company was responsible under the development plan for conducting the Phase 1 clinical trials with respect to JZP-386. The Company's obligations to conduct further development activities are subject to mutual agreement. Jazz Pharmaceuticals has assumed all manufacturing and development responsibilities relating to JZP-386. Pursuant to the agreement, the Company's costs for activities under the development plan were reimbursed by Jazz Pharmaceuticals, except for the costs of a Phase 1

clinical trial that was conducted in the first half of 2015, which was shared between Jazz Pharmaceuticals and the Company.

Under the agreement, the Company received a non-refundable upfront payment of $4.0 million and is eligible to earn an aggregate of 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 based on net product sales of licensed products. The next milestone payment that the Company may be entitled to receive is $4.0 million related to initiation of the first Phase 2 clinical trial of JZP-386.

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 worldwide net sales of licensed products. The royalty rate is lowered, on a country-by-country basis, under certain circumstances as specified in the agreement.

For the three months ended March 31, 2016 and 2015, the Company recognized revenue of $34 thousand and $0.4 million related to the performance of development support services, respectively.

Avanir

In February 2012, the Company entered into a development and license agreement with Avanir Pharmaceuticals, Inc., or Avanir, under which the Company granted Avanir an exclusive worldwide license to develop, manufacture and commercialize deuterated dextromethorphan containing products. Avanir is currently focused on developing AVP-786, which is a combination of a deuterated analog of dextromethorphan and a low dose of quinidine. Subsequent to the Company's development and license agreement, Avanir was acquired by Otsuka Pharmaceutical Co., Ltd. and it is now a wholly owned subsidiary of Otsuka America, Inc.

Since June 2012, Avanir has elected to conduct all research and development activities, including manufacturing activities; however, the Company has received intellectual property cost reimbursements.

Under the agreement, the Company received a non-refundable upfront payment of $2.0 million and has received milestone payments of $6.0 million. The Company is also eligible to earn, with respect to licensed products comprising a combination of deuterated dextromethorphan and quinidine, up to $37.0 million in regulatory and commercial launch milestone payments, of which $21.5 million in development and regulatory milestone payments are associated with the first indication, and up to $125.0 million in sales-based milestone payments. The next milestone payments that the Company may be entitled to receive are $5.0 million upon acceptance for filing of a New Drug Application, or NDA, and $3.0 million upon acceptance for filing of a Marketing Authorization Application, or MAA, related to AVP-786. In addition, the Company is 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 net sales of licensed products on a country-by-country basis. 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.

7. Stock-Based Compensation

The Company's equity incentive plans provide for the issuance of a variety of stock-based awards, including incentive stock options, nonstatutory stock options and awards of stock, to directors, officers and employees of the Company, as well as consultants and advisors to the Company. To date, the Company has granted awards solely in the form of stock options, which have generally been granted with an exercise price equal to the fair value of the underlying common stock on the date of grant, expire no later than ten years from the date of grant and generally vest over three or four years.

Effective January 1, 2016, an additional 886,606 shares were added to the Company's 2014 Stock Incentive Plan, or the 2014 Plan, for future issuance pursuant to the terms of the 2014 Plan. As of March 31, 2016, there were 1,805,286 shares of common stock available for future award grants under the 2014 Plan.

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

	Three Months Ended March 31,	
	2016	2015
Research and development	$ 553	$ 325
General and administrative	729	321
Total stock-based compensation expense	$ 1,282	$ 646

Stock Options

Stock options are valued using the Black-Scholes-Merton option valuation model and compensation cost is recognized based on such fair value over the period of vesting. The weighted average fair value of options granted in the three months ended March 31, 2016 and 2015 reflect the following weighted-average assumptions:

	Three Months Ended March 31,			
	2016		2015	
Expected volatility	78.34	%	73.11	%
Expected term	6.0 years		6.0 years	
Risk-free interest rate	1.38	%	1.54	%
Expected dividend yield	—	%	—	%

For the three months ended March 31, 2016 and 2015, expected volatility was estimated using the historical volatility of the common stock of a group of similar companies that were publicly traded. The Company will continue to apply this process until a sufficient amount of historical information regarding the volatility of its own stock price becomes available.

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

	Three Months Ended March 31,	
	2016	2015
	(in thousands, except per share data)	
Weighted average fair value of options granted, per option	$11.19	$9.34
Aggregate grant date fair value of options vested during the year	$1,032	$423
Total cash received from exercises of stock options	$176	$310
Total intrinsic value of stock options exercised	$496	$2,086

The following is a summary of stock option activity for the three months ended March 31, 2016:

	Number of Option Shares	Weighted Average Exercise Price per Share	Weighted Average Remaining Contractual Term (In years)	Aggregate Intrinsic Value (In thousands)
Outstanding at December 31, 2015	2,244,177	$ 8.10		
Granted	759,500	$ 16.55		
Exercised	(43,994)	$ 4.00		
Forfeited or expired	(838)	$ 13.57		
Outstanding at March 31, 2016	2,958,845	$ 10.33	7.35	$ 12,758

Exercisable at March 31, 2016	1,401,787	$ 6.45	5.61	$ 10,323
Vested and expected to vest at March 31, 2016 (1)	2,819,857	$ 10.12	7.26	$ 12,591

(1) This represents the number of vested stock option shares as of March 31, 2016, plus the number of unvested stock option shares that the Company estimated as of March 31, 2016 would vest, based on the unvested stock option shares at March 31, 2016 and an estimated forfeiture rate of 6%.

As of March 31, 2016, there was $13.8 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 2.9 years.

8. Earnings (Loss) Per Share

Basic net earnings (loss) per common share is calculated by dividing net earnings (loss) allocable to common stockholders by the weighted-average common shares outstanding during the period, without consideration of common stock equivalents. Diluted net earnings per share is calculated by adjusting the weighted-average shares outstanding for the dilutive effect of common stock equivalents, including stock options and warrants, outstanding for the period as determined using the treasury stock method. For purposes of the diluted net loss per share calculation, common stock equivalents are excluded from the calculation because their effect would be anti-dilutive. Therefore, basic and diluted net loss per share applicable to common stockholders is the same for periods with a net loss.

	Three Months Ended March 31,	
	2016	2015
	(in thousands, expect per share amounts)	
Numerator:		
Net loss applicable to common stockholders -basic and diluted	$(13,880)	$(9,002)
Denominator:		
Weighted average shares outstanding - basic	22,198	18,726
Dilutive stock options	—	—
Dilutive warrants	—	—
Weighted average shares outstanding - diluted	22,198	18,726
Net loss per share applicable to common stockholders - basic and diluted	$(0.63)	$(0.48)
Anti-dilutive potential common stock equivalents excluded from the calculation of net loss per share:		
Stock options	767	1,116
Warrants	71	71

9. Income Taxes

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

The Company records a provision or benefit for income taxes on ordinary pre-tax income or loss based on its estimated effective tax rate for the year. As of March 31, 2016, the Company forecast an ordinary pre-tax loss for the year ended December 31, 2016 and, since it maintains a full valuation allowance on its deferred tax assets, the

Company did not record an income tax benefit for the three months ended March 31, 2016.

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. Statements contained or incorporated by reference in this Quarterly Report on Form 10-Q that are not based on historical fact are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. These forward-looking statements regarding future events and our future results are based on current expectations, estimates, projections, intentions, goals, strategies, plans, prospects and the beliefs and assumptions of our management including, without limitation, our expectations regarding results of operations, general and administrative expenses, research and development expenses, current and future development and manufacturing efforts, regulatory filings, nonclinical and clinical trial results, and the sufficiency of our cash for future operations. 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. Incorporation of deuterium into known molecules has the potential, on a case-by-case basis, to provide better pharmacokinetic or metabolic properties, thereby enhancing their clinical safety, tolerability or efficacy. Our approach starts with approved drugs, advanced clinical candidates or previously studied compounds that may be improved with deuterium substitution. Our technology provides the opportunity to develop products that may compete with the non-deuterated drug in existing markets or to leverage the known activity of approved drugs to expand into new indications. Our deuterated chemical entity platform, or DCE Platform®, has broad potential across numerous therapeutic areas. The following table summarizes our diverse clinical pipeline of product candidates. All of these candidates are small molecules designed for oral administration.

Product Candidate	Lead Indication	Status	Worldwide rights
AVP-786 Deuterated dextromethorphan	Alzheimer's Agitation	Phase 3	Avanir Pharmaceuticals
	Major Depressive Disorder	Phase 2	
	Residual Schizophrenia	Phase 2	
CTP-656 Deuterated ivacaftor	Cystic Fibrosis	Phase 1	Concert
CTP-543 Deuterated ruxolitinib	Alopecia Areata	Preclinical	Concert
CTP-730 Deuterated apremilast	Inflammation	Phase 1	Celgene
JZP-386 Deuterated sodium-oxybate	Narcolepsy	Phase 1	Jazz Pharmaceuticals

CTP-656

CTP-656 is a novel, next generation potentiator that we are initially developing for the treatment of cystic fibrosis in patients who have gating mutations, including the G551D mutation. CTP-656 was discovered by applying our deuterium chemistry technology to modify ivacaftor, which is the current standard-of-care for this population. Ivacaftor is marketed by Vertex Pharmaceuticals, Inc., or Vertex, under the name Kalydeco®. Due to its differentiated pharmacokinetic profile, CTP-656 has the potential to offer a greater therapeutic benefit than ivacaftor for this patient population. CTP-656 may provide improved efficacy as a result of 1) a once-daily dosing regimen which could enable better treatment adherence, and 2) enhanced exposure to the parent drug, which is more active than the metabolites. In addition, the increased metabolic stability of CTP-656 may ameliorate certain drug-drug interactions.

CTP-656 also has the potential to be a key component of combination therapies that enable the treatment of patients having other cystic fibrosis transmembrane conductance regulator protein, or CFTR, mutations. To advance

combination therapies of CTP-656, we intend to collaborate with companies who are focused on developing drugs that target other mechanisms of modulating CFTR that we believe may be suitable to combine with CTP-656.

We intend to follow a Section 505(b)(2) regulatory pathway for the development of CTP-656. Under a 505(b)(2) pathway, we would support our NDA by our own safety and efficacy studies as well as rely on certain of FDA's findings for the non-deuterated product. This approach may allow us to reduce the time and expense required to develop CTP-656 relative to the regulatory pathway for a traditional NDA.

Clinical Development of CTP-656

In the third quarter of 2015, we completed a single ascending dose Phase 1 trial in healthy volunteers which included a comparison of CTP-656 with a single dose of Kalydeco. The single ascending dose Phase 1 clinical trial was conducted in 10 healthy volunteers and evaluated three doses (75, 150 and 300 mg) of CTP-656, each as an aqueous suspension, and a single dose 150 mg tablet of Kalydeco in a crossover design with the 150 mg dose of CTP-656. The single ascending dose findings support once-daily administration of CTP-656 based on a half-life in the range of 14 to 17 hours. In this trial, CTP-656 also demonstrated a linear dose response. CTP-656 was well-tolerated across all dose groups. There were no serious adverse events reported in subjects who received CTP-656.

Nine subjects completed the single dose crossover comparison of the aqueous suspension of 150 mg of CTP-656 to a 150 mg solid dose of Kalydeco. In the trial, CTP-656 demonstrated a pharmacokinetic profile that was superior to Kalydeco. In particular, it demonstrated a reduced rate of clearance, longer half-life, and substantially increased exposure with greater plasma levels at 12 and 24 hours versus Kalydeco. For CTP-656, there was greater plasma exposure of the parent drug relative to its less active metabolites. With Kalydeco, the less active metabolites were more prominent than the parent drug.

In November 2015, we initiated a multiple ascending dose Phase 1 clinical trial in healthy volunteers. The trial was conducted in two parts and enrolled 38 healthy volunteers to assess safety, tolerability and pharmacokinetics of CTP-656 in a tablet formulation. The first part assessed the pharmacokinetic properties of a single dose tablet formulation of 150 mg of CTP-656 versus the 150 mg commercial tablet formulation of Kalydeco in 8 healthy volunteers following a high-fat meal. The second part assessed placebo and three doses of CTP-656, including 75 mg, 150 mg, and 225 mg, in each case given once-daily for seven days following a high-fat meal.

In February 2016, we announced results from Part 1 of the multiple ascending dose trial with a tablet formulation of CTP-656. The data from this trial were consistent with results from the previously completed single ascending dose trial, in which an aqueous suspension of CTP-656 demonstrated superior pharmacokinetic properties compared to Kalydeco. In this trial, CTP-656 tablets demonstrated a similarly reduced rate of clearance, longer half-life, similarly increased exposure and greater plasma levels at 24 hours compared to Kalydeco. With both the solid dose and aqueous suspension formulations, the overall metabolite exposure profile of CTP-656 differed from that of Kalydeco. After administration of CTP-656, there was greater plasma exposure of the more active parent drug relative to less-active metabolites, whereas with Kalydeco there was greater plasma exposure of the less-active metabolites.

In April 2016, we announced results from Part 2 of the CTP-656 multiple ascending dose trial. CTP-656 demonstrated enhanced exposure to the parent drug and less exposure to metabolites after repeat dosing, confirming the differentiated metabolic profile of CTP-656 relative to Kalydeco. Results indicated that the average plasma half-life of CTP-656 across all doses was approximately 18 hours at steady state. CTP-656 showed a dose-proportional increase in exposure with repeated dosing for the 75 mg and 150 mg doses. The 225 mg dose group showed higher than dose-proportional exposure. Results of the Phase 1 trial also showed that CTP-656 was well tolerated with a safety profile comparable to that of Kalydeco.

Also in April 2016, we announced initial results from a food effect trial with CTP-656. In the trial, we found that exposures to CTP-656 dosed with a medium fat-containing meal and with a low fat-containing meal were similar, and both were consistent with the exposure previously observed following dosing CTP-656 with a high-fat meal. We

expect to open an IND to commence our Phase 2 clinical trial in the second half of 2016.

CTP-543

CTP-543 is an oral selective inhibitor of certain Janus kinases, known as JAK1 and JAK2, that we are developing for the treatment of alopecia areata, an autoimmune disease that results in partial or complete loss of hair on the scalp and body. CTP-543 was discovered by applying our deuterium chemistry technology to modify ruxolitinib, which is commercially available under the name Jakafi® in the U.S. for the treatment of myelofibrosis and polycythemia vera. Ruxolitinib has been used in an academic investigator-sponsored clinical trial and has been shown to promote hair growth in individuals with alopecia areata. We conducted preclinical studies demonstrating that CTP-543 retains ruxolitinib's JAK1and JAK2 inhibition

profile and showing improved metabolic stability relative to ruxolitinib. We expect to initiate Phase 1 clinical evaluation of CTP-543 in the second quarter of 2016 with efficacy studies commencing in 2017.

COLLABORATION PRODUCT CANDIDATES

We have entered into several collaborative arrangements with companies to develop deuterium-modified versions of their marketed products. In each of these collaborations, the deuterium-modified compound was independently discovered at Concert. Our collaborators are responsible for any future clinical development activities and disclosures associated with the following programs.

- AVP-786 is a combination of a deuterium-substituted dextromethorphan analog and a low dose of quinidine being investigated for the treatment of neurologic and psychiatric disorders. Avanir is conducting several Phase 2 and Phase 3 clinical trials to evaluate AVP-786 for the treatment of neurologic and psychiatric disorders, the most advanced of which are Phase 3 clinical trials for the treatment of agitation associated with Alzheimer's disease.
- CTP-730 is a deuterated analog of apremilast that is being developed under a collaboration with Celgene. Apremilast is a selective phosphodiesterase 4 (PDE4) inhibitor approved for the treatment of psoriasis and psoriatic arthritis. While the collaboration with Celgene has the potential to encompass multiple programs for the treatment of inflammation or cancer, it is currently focused on one program, CTP-730. We have completed the Phase 1 clinical evaluation of CTP-730. Once-daily dosing of 50 mg of CTP-730 administered for seven days in the Phase 1 clinical trial demonstrated similar steady state exposure to historical data for 30 mg of apremilast twice daily. Treatment with CTP-730 was generally well-tolerated and no serious adverse events were observed. Celgene is responsible for any development of CTP-730 beyond the completed Phase 1 clinical trials. Celgene is assessing the path forward for CTP-730, however, CTP-730 has not advanced into new trials at this time.
- JZP-386 is a product candidate containing a deuterated sodium oxybate analog for potential use in patients with narcolepsy. We have granted Jazz Pharmaceuticals worldwide rights to develop and commercialize deuterated sodium oxybate analogs, including JZP-386. JZP-386 is being developed for the potential treatment of patients with narcolepsy. In May 2015, we and Jazz Pharmaceuticals announced the completion of a Phase 1 clinical study. Clinical data from this Phase 1 study demonstrated that JZP-386 provided favorable deuterium-related effects, including higher serum concentrations and correspondingly increased pharmacodynamic effects at clinically relevant time points compared to Xyrem® (sodium oxybate) oral solution. The safety profile of JZP-386 was similar to that observed with Xyrem®. Jazz Pharmaceuticals is responsible for any further development of JZP-386.

Since our inception in 2006, we have devoted substantially all of our resources to our research and development efforts, including activities to develop our deuterated chemical entity platform, or DCE Platform, and our core capabilities in deuterium chemistry, identify potential product candidates, undertake non-clinical studies and clinical trials, manufacture clinical trial material 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 $135.0 million since inception through March 31, 2016 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 and patent assignments. In March 2015, we sold 3,300,000 shares of common stock at a price to the public of $15.15 per share, resulting in net proceeds to us of $46.7 million, after deducting the underwriting discounts, commissions and offering-related transaction costs.

We have incurred net losses in each year from our inception in 2006, except for fiscal year 2015. We incurred a net loss of $13.9 million during the three months ended March 31, 2016. We generated net income of $24.2 million during the year ended December 31, 2015, which was primarily the result of a $50.2 million one-time payment from Auspex Pharmaceuticals, Inc., or Auspex, as discussed further in our Annual Report on Form 10-K for the year ended December 31, 2015, which was filed with the Securities and Exchange Commission on March 1, 2016. Our operating results may fluctuate significantly from year to year, depending on the timing and magnitude of clinical trial and other development activities under our current development programs. Substantially all of our net losses have resulted from costs incurred in connection with our research and development programs and from general and administrative costs

associated with our operations.

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

We do not expect to generate revenue from product sales unless and until we, or our collaborators, obtain marketing approval for one or more of our product candidates, which we expect will take a number of years and is subject to significant uncertainty. If we obtain, or believe that we are likely to obtain, marketing approval for any product candidates for which we retain commercialization rights, and intend to commercialize a product, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution. We expect to seek to fund our operations through a combination of equity offerings, debt financings 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. Our collaborators also have applied their considerable scientific, development, regulatory and commercial capabilities to the development of our compounds. 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 for an expedited development or regulatory pathway by relying on previous clinical data regarding their corresponding non-deuterated compound. 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, which are discussed further in Note 6 in the condensed consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q.

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 analogs. Avanir is currently focused on developing AVP-786, which is a combination of a deuterated analog of dextromethorphan and a low dose of quinidine, for the treatment of neurologic and psychiatric disorders. In January 2015, Avanir was acquired by Otsuka Pharmaceutical Co., Ltd. and it is now a wholly owned subsidiary of Otsuka America, Inc.

Under the agreement, we received a non-refundable upfront payment of $2.0 million and have received milestone payments of $6.0 million. We have the potential to earn up to $162.0 million in additional development, regulatory and sales-based milestone payments, of which $21.5 million in development and regulatory milestone payments are associated with the first indication. The next anticipated milestone payments that we may be entitled to receive are $5.0 million upon acceptance for filing of a New Drug Application, or NDA, and $3.0 million upon acceptance for filing of a Marketing Authorization Application, or MAA, related to AVP-786. Avanir also is required to pay us royalties at defined percentages ranging from the mid-single digits to low double digits below 20% on net sales of licensed products on a country-by-country basis.

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 inflammation or cancer. While the collaboration has the potential to encompass multiple programs, it is initially focused on one program, CTP-730, which is deuterated apremilast.

We were responsible for conducting and funding research and early development activities for the CTP-730 program pursuant to mutually agreed upon development plans. This included the completion of single and multiple ascending dose Phase 1 clinical trials. Celgene is responsible for any development of CTP-730 beyond the completed 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 mutually 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, discussed further in Note 6 in the condensed consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q. 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.

Under the agreement, we received a non-refundable upfront payment of $35.0 million and received an $8.0 million development milestone in October 2015 upon completion of clinical evaluation for CTP-730. In addition, we have the potential to earn up to $312.5 million in additional development, regulatory and sales-based milestone payments with respect to CTP-730. The next milestone that we may be entitled to receive is $15.0 million upon the first dosing in a Phase 3 clinical trial or, if earlier, acceptance for filing a new drug application, or NDA, related to CTP-730. If Celgene exercises its rights under any additional program, we may be eligible for milestone payments for each additional program. In addition, with respect to each program, Celgene is required to pay us royalties on worldwide net sales of each licensed product at defined percentages ranging from the mid-single digits to low double digits below 20%.

Jazz Pharmaceuticals

In February 2013, we entered into a development and license agreement with Jazz Pharmaceuticals to research, develop and commercialize products containing a deuterated sodium oxybate analog, or D-SXB. Jazz Pharmaceuticals is initially focused on developing one analog, designated as JZP-386 for the treatment of narcolepsy. 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, but not limited to, JZP-386.

We, together with Jazz Pharmaceuticals, have conducted certain development activities for Phase 1 clinical trials with respect to JZP-386 pursuant to an agreed upon development plan. We were responsible under the development plan for conducting the Phase 1 clinical trials with respect to JZP-386. Thereafter, our obligations to conduct further development activities are subject to mutual agreement. Jazz Pharmaceuticals has assumed all manufacturing and development responsibilities relating to JZP-386.

Under the agreement, we received a non-refundable upfront payment of $4.0 million and are eligible to earn an aggregate of up to $113.0 million in development, regulatory and sales-based milestone payments. The next milestone payment that we may be entitled to receive is $4.0 million related to initiation of the first Phase 2 clinical trial of JZP-386. 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 worldwide net sales of licensed products.

For further discussion regarding our collaboration agreements, refer to Note 6 in the condensed consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q.

FINANCIAL OPERATIONS OVERVIEW

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 and a patent assignment agreement.

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, payments for research and development services provided to our collaborators and a change in control payment pursuant to a patent assignment agreement. However, we have not yet earned any license exercise or option fees, sales-based milestone payments or royalty revenue as a result of product sales.

In the future, we will seek to generate revenue from a combination of product sales and milestone payments and royalties on product sales in connection with our current collaborations with Avanir, Celgene, 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 includes costs related to synthesis, analysis and in vitro and in vivo characterization of deuterated compounds to support the selection and progression of potential product candidates;
- expenses related to consultants and advisors; and
- costs associated with non-clinical activities and regulatory operations.

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

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

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

- the scope and rate of progress of our ongoing as well as any additional clinical trials and other research and development activities;
- conduct of and results from ongoing as well as any additional clinical trials and research and development activities;
- significant and changing government regulation;
- the terms and timing and receipt of any regulatory approvals;
- the performance of our collaborators;
- our ability to manufacture any of our product candidates that we are developing or may develop in the future; and
- the expense and success of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights.

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

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

General and administrative expenses

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

We anticipate that our general and administrative expenses will increase in the future as our pipeline grows and matures. Additionally, if and when we believe 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, marketing and distribution of our product candidates.

Investment income

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

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, and amortization of debt discount. On October 1, 2015, we made a final payment to Hercules, thereby fulfilling all obligations under our debt facility.

Critical Accounting Policies and Significant Judgments and Estimates

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

During the three months ended March 31, 2016, there were no material changes to our critical accounting policies as detailed in our Annual Report on Form 10-K for the fiscal year ended December 31, 2015, which was filed with the Securities and Exchange Commission on March 1, 2016.

Pending Accounting Pronouncements

In May 2014, the FASB issued Accounting Standards Update, or ASU, No. 2014-09, Revenue from Contracts with Customers (Topic 606), or ASU 2014-09, which stipulates that an entity should recognize revenue to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. To achieve this core principle, ASU 2014-09 provides that an entity should apply the following steps: (1) identify the contract(s) with a customer, (2) identify the performance obligations in the contract, (3) determine the transaction price, (4) allocate the transaction price to the performance obligations in the contract and (5) recognize revenue when (or as) the entity satisfies a performance obligation. This update will be effective for us beginning in the first quarter of fiscal 2018 as a result of the FASB's one year deferral of the effective date for this standard. Early adoption is permitted, however not before the original effective date of annual periods beginning on or after December 15, 2016. We are currently assessing the impact of this ASU on our financial statements.

In August 2014, the FASB issued ASU No. 2014-15, Disclosure of Uncertainties About an Entity's Ability to Continue as a Going Concern, or ASU 2014-15. ASU 2014-15 amends FASB Accounting Standards Codification 205-40 Presentation of Financial Statements – Going Concern, by providing guidance on determining when and how reporting entities must disclose going-concern uncertainties in their financial statements, including requiring management to perform interim and annual assessments of an entity's ability to continue as a going concern within one year of the date of issuance of the entity's financial statements and providing certain disclosures if there is substantial doubt about the entity's ability to continue as a going concern. ASU 2014-15 will be effective for our fiscal 2016 and annual financial statements and for interim periods beginning in the first quarter of fiscal 2017. If this standard had been adopted as of March 31, 2016, we believe that we would have concluded there was not substantial doubt about our ability to continue as a going concern. However, our disclosures in future periods may be affected by the adoption of this accounting standard.

In February 2016, the FASB issued ASU No. 2016-02, Leases (Topic 842), or ASU 2016-02. ASU 2016-02 requires lessees to recognize assets and liabilities on the balance sheet for the rights and obligations created by all leases with terms of more than 12 months. ASU 2016-02 also will require certain qualitative and quantitative disclosures designed to give financial statement users information on the amount, timing, and uncertainty of cash flows arising from leases. ASU 2016-02 will be effective for us on January 1, 2019, with early adoption permitted. We are currently evaluating the impact ASU 2016-02 will have on our financial statements.

In March 2016, the FASB issued ASU No. 2016-09, Compensation-Stock Compensation-Improvements to Employee Share-Based Payment Accounting, or ASU 2016-09. This update simplifies several aspects of the accounting for share-based compensation arrangements, including accounting for income taxes, forfeitures and statutory tax withholding requirements as well as classification of related amounts on the statement of cash flows. The ASU is effective for fiscal years beginning after December 15, 2016, with early adoption permitted. We are currently evaluating the effect that the updated standard will have on our financial statements and related disclosures.

RESULTS OF OPERATIONS

Comparison of the three months ended March 31, 2016 and 2015

The following table summarizes our results of operations for the three months ended March 31, 2016 and 2015, together with the changes in those items in dollars.

	Three months ended March 31,		
(in thousands)	2016	2015	Change
Revenue:			
License and research and development revenue	$56	$1,306	$(1,250)
Total revenue	56	1,306	(1,250)
Operating expenses:			
Research and development	10,453	6,944	3,509
General and administrative	3,577	3,233	344
Total operating expenses	14,030	10,177	3,853
Loss from operations	(13,974)	(8,871)	(5,103)
Investment income	94	17	77
Interest and other expense	—	(148)	148
Net loss	$(13,880)	$(9,002)	