Table of Contents

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

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 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: 001-36620

Tokai Pharmaceuticals, Inc.
(Exact name of registrant as specified in its charter)

Delaware	**20-1000967**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification Number)**
One Broadway, 14th floor Cambridge, MA	**02142**
(Address of principal executive offices)	**(Zip Code)**

(617) 225-4305
(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 ☐ 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 ☒

As of November 10, 2014 there were 22,381,742 shares of Common Stock, $0.001 par value per share, outstanding.

Tokai Pharmaceuticals, Inc.

INDEX

FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Quarterly Report on Form 10-Q, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "would" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

These forward-looking statements include, among other things, statements about:

- the anticipated timing, cost and conduct of our ongoing ARMOR2 trial and our planned pivotal Phase 3 clinical trial and our efforts to complete the clinical development of galeterone for CRPC patients with AR-V7;
- the outcome of regulatory review of galeterone for the treatment of prostate cancer in CRPC patients with AR-V7 or other indications or patient populations and any other future product candidates;
- the development of future product candidates, including compounds that are designed to disrupt androgen receptor signaling through enhanced androgen receptor degradation;
- our plans to enter into collaborations for the commercialization of galeterone and any other future product candidates;
- the potential benefits of any future collaboration;
- the rate and degree of market acceptance and clinical utility of our products;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our intellectual property position and strategy;
- our ability to identify additional products or product candidates with significant commercial potential;
- our estimates regarding expenses, future revenue, capital requirements and needs for additional financing;
- developments relating to our competitors and our industry; and
- the impact of government laws and regulations.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this Quarterly Report on Form 10-Q, particularly in the "Risk Factors" section, that could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, collaborations, joint ventures or investments that we may make or enter into.

You should read this Quarterly Report on Form 10-Q and the documents that we have filed as exhibits to this Quarterly Report on Form 10-Q completely and with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

Tokai Pharmaceuticals, Inc.

Consolidated Balance Sheets

(In thousands, except share and per share data)
(Unaudited)

	September 30, 2014	December 31, 2013
Assets		
Current assets:		
Cash and cash equivalents	$ 105,464	$ 31,753
Prepaid expenses and other current assets	1,260	425
Total current assets	106,724	32,178
Property and equipment, net	38	29
Deferred offering costs	—	30
Restricted cash	200	50
Other assets	21	—
Total assets	$ 106,983	$ 32,287
Liabilities, Redeemable Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Current liabilities:		
Accounts payable	$ 2,889	$ 5
Accrued expenses	2,483	2,204
Total current liabilities	5,372	2,209
Total liabilities	5,372	2,209
Commitments and contingencies (Note 7)		
Redeemable convertible preferred stock (Series A, B-1, B-2, C, D-1, D-2, D-3 and E), $0.001 par value; no shares and 155,586,141 shares authorized at September 30, 2014 and December 31, 2013, respectively; no shares and 155,586,141 shares issued and outstanding at September 30, 2014 and December 31, 2013; aggregate liquidation preference of $0 and $83,528 at September 30, 2014 and December 31, 2013, respectively	—	85,345
Stockholders' equity (deficit):		
Preferred stock, $0.001 par value, 5,000,000 and no shares authorized at September 30, 2014 and December 31, 2013, respectively; no shares issued or outstanding at September 30, 2014 and December 31, 2013, respectively	—	—
Common stock, $0.001 par value; 200,000,000 and 173,018,331 shares authorized at September 30, 2014 and December 31, 2013, respectively; 21,841,742 and 493,292 shares issued and outstanding at September 30, 2014 and December 31, 2013, respectively	22	—
Additional paid-in capital	181,732	7,788
Accumulated deficit	(80,143)	(63,055)
Total stockholders' equity (deficit)	101,611	(55,267)
Total liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)	$ 106,983	$ 32,287

The accompanying notes are an integral part of these consolidated financial statements.

Tokai Pharmaceuticals, Inc.

Consolidated Statements of Operations and Comprehensive Loss

(In thousands, except share and per share data)
(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2013	2014	2013
Revenue	$ —	$ —	$ —	$ —
Operating expenses:				
Research and development	2,825	2,704	10,773	7,852
General and administrative	3,599	661	6,428	2,348
Total operating expenses	6,424	3,365	17,201	10,200
Loss from operations	(6,424)	(3,365)	(17,201)	(10,200)
Other income (expense), net	34	—	113	—
Net loss and comprehensive loss	(6,390)	(3,365)	(17,088)	(10,200)
Accretion of redeemable convertible preferred stock to redemption value	—	—	—	(79)
Net loss attributable to common stockholders	$ (6,390)	$ (3,365)	$ (17,088)	$ (10,279)
Net loss per share attributable to common stockholders, basic and diluted	$ (2.71)	$ (6.82)	$ (15.19)	$ (26.36)
Weighted average common shares outstanding, basic and diluted	2,357,236	493,299	1,124,627	390,001

The accompanying notes are an integral part of these consolidated financial statements.

Tokai Pharmaceuticals, Inc.

Consolidated Statements of Cash Flows

(In thousands)
(Unaudited)

	Nine Months Ended September 30,	
	2014	2013
Cash flows from operating activities:		
Net loss	$ (17,088)	$(10,200)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	1,519	137
Depreciation expense	16	7
Release of reserve for loan to stockholder	(113)	—
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(835)	(180)
Accounts payable	1,884	167
Accrued expenses	(23)	1,414
Other assets	(21)	—
Net cash used in operating activities	(14,661)	(8,655)
Cash flows from investing activities:		
Purchases of property and equipment	(25)	(19)
Change in restricted cash	(150)	(30)
Net cash used in investing activities	(175)	(49)
Cash flows from financing activities:		
Proceeds from initial public offering, net of underwriting discounts and commissions	90,396	—
Payments of initial public offering costs	(1,974)	(10)
Proceeds from issuance of redeemable convertible preferred stock, net of issuance costs	—	19,921
Proceeds from exercise of common stock options	12	215
Repayment of notes receivable	113	—
Net cash provided by financing activities	88,547	20,126
Net increase in cash and cash equivalents	73,711	11,422
Cash and cash equivalents at beginning of period	31,753	11,691
Cash and cash equivalents at end of period	$105,464	$ 23,113
Supplemental disclosure of non-cash investing and financing activities:		
Accretion of redeemable convertible preferred stock to redemption values	$ —	$ 79
Conversion of redeemable convertible preferred stock to common stock	$ 85,345	$ —
Deferred offering costs included in accounts payable and accrued expenses	$ 1,302	$ —

The accompanying notes are an integral part of these consolidated financial statements.

Tokai Pharmaceuticals, Inc.

Notes to the Consolidated Financial Statements
(Amounts in thousands, except share and per share data)
(Unaudited)

1. Nature of the Business and Basis of Presentation

Tokai Pharmaceuticals, Inc. (the "Company") was incorporated on March 26, 2004 under the laws of the State of Delaware. The Company is a clinical-stage biopharmaceutical company focused on developing novel proprietary therapies for the treatment of prostate cancer and other hormonally-driven diseases. The Company's lead drug candidate, galeterone, is a highly selective, multi-targeted, oral small molecule drug candidate. Since its inception, the Company has devoted substantially all of its efforts to research and development, recruiting management, in-licensing technology and raising capital.

The Company was previously classified as a "development stage entity" in the Accounting Standards Codification and, as such, was required to present inception-to-date information in the Company's statements of operations and comprehensive loss, redeemable convertible preferred stock and stockholders' deficit, and cash flows. In June 2014, the Financial Accounting Standards Board ("FASB") issued an accounting standards update that eliminates the concept of a development stage entity from U.S. generally accepted accounting principles and removes the related incremental reporting requirements. See Note 2 below for additional information on this new standard. The Company elected to early adopt the new standard in the three months ended September 30, 2014. Accordingly, in contrast to the Company's financial statements and the notes thereto for the year ended December 31, 2013 included in Company's Registration Statement on Form S-1 on file with the Securities and Exchange Commission ("SEC"), the financial statements contained in this report do not include inception-to-date information.

The Company is subject to risks common to companies in the biotechnology industry including, but not limited to, new technological innovations, protection of proprietary technology, dependence on key personnel, compliance with government regulations and the need to obtain additional financing. Galeterone, which is currently under development, and any product candidates that the Company may seek to develop in the future will require significant additional research and development efforts, including extensive preclinical and clinical testing and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel infrastructure, and extensive compliance-reporting capabilities.

There can be no assurance that the Company's research and development will be successfully completed, that adequate protection for the Company's intellectual property will be obtained, that any products developed will obtain necessary government regulatory approval or that any approved products will be commercially viable. Even if the Company's drug development efforts are successful, it is uncertain when, if ever, the Company will generate significant revenue from product sales. The Company operates in an environment of rapid change in technology and substantial competition from pharmaceutical and biotechnology companies. In addition, the Company is dependent upon the services of its employees and consultants.

The accompanying consolidated financial statements and footnotes include Diotima Pharmaceuticals, Inc. ("Diotima"), a variable interest entity in which the Company had a variable financial interest and was the primary beneficiary but had no ownership interest. In 2010, the Company formed and incorporated Diotima. Diotima operated as a stand-alone company with limited activity through April 2014. In early 2014, the license agreements relating to the Diotima compounds were terminated. Additionally, in April 2014, the board of directors and stockholders of Diotima approved the dissolution of Diotima, and Diotima was dissolved. Expenses incurred by Diotima for the three and nine months ended September 30, 2014 were $0 and $8, respectively, and for the three and nine months ended September 30, 2013, were $1 and $59, respectively. All significant intercompany balances and transactions between the Company and Diotima have been eliminated in consolidation.

Tokai Pharmaceuticals, Inc.

Notes to the Consolidated Financial Statements—(Continued)
(Amounts in thousands, except share and per share data)
(Unaudited)

On September 22, 2014, the Company completed an initial public offering ("IPO") of its common stock, and issued and sold 6,480,000 shares of common stock at a public offering price of $15.00 per share, resulting in net proceeds of approximately $90,396 after deducting underwriting discounts and commissions but before the payment of estimated offering expenses of $3,306. Upon the closing of the IPO, all outstanding shares of the Company's redeemable convertible preferred stock automatically converted into 14,860,173 shares of the Company's common stock. On October 9, 2014, the Company issued and sold an additional 540,000 shares of its common stock at the IPO price of $15.00 per share, less underwriting discounts and commissions, pursuant to the underwriters' partial exercise of their option to purchase additional shares of common stock, resulting in additional net proceeds to the Company of approximately $7,533 after deducting underwriting discounts and commissions (Note 10).

Unaudited Interim Financial Information

The consolidated balance sheet at December 31, 2013 was derived from audited financial statements, but does not include all disclosures required by U.S. generally accepted accounting principles ("GAAP"). The accompanying unaudited consolidated financial statements as of September 30, 2014 and for the three months and nine months ended September 30, 2014 and 2013 have been prepared by the Company, pursuant to the rules and regulations of the SEC for interim financial statements. Certain information and footnote disclosures normally included in financial statements prepared in accordance with GAAP have been condensed or omitted pursuant to such rules and regulations. However, the Company believes that the disclosures are adequate to make the information presented not misleading. These consolidated financial statements should be read in conjunction with the Company's audited consolidated financial statements and the notes thereto for the year ended December 31, 2013 included in the Company's Registration Statement on Form S-1, File Number 333-198052 on file with the SEC. In the opinion of management, all adjustments, consisting only of normal recurring adjustments necessary for a fair statement of the Company's financial position as of September 30, 2014 and consolidated results of operations for the three and nine months ended September 30, 2014 and 2013 and cash flows for the nine months ended September 30, 2014 and 2013 have been made. The results of operations for the three and nine months ended September 30, 2014 are not necessarily indicative of the results of operations that may be expected for the year ending December 31, 2014.

2. Summary of Significant Accounting Policies

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the consolidated financial statements, and the reported amounts of expenses during the reporting periods. Significant estimates and assumptions reflected in these consolidated financial statements include, but are not limited to, the accrual of research and development expenses and the valuation of common stock, redeemable convertible preferred stock and stock-based awards. Estimates are periodically reviewed in light of changes in circumstances, facts and experience. Actual results could differ from the Company's estimates.

Cash Equivalents

The Company considers all short-term, highly liquid investments with original maturities of ninety days or less at date of purchase to be cash equivalents. Cash equivalents, which consist of money market accounts, are stated at fair value.

Concentration of Credit Risk and of Significant Suppliers

Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash and cash equivalents. The Company has all cash and cash equivalents balances at one accredited financial institution, in amounts that exceed federally insured limits. The Company does not believe that it is subject to unusual credit risk beyond the normal credit risk associated with commercial banking relationships.

The Company is dependent on third-party manufacturers to supply products for research and development activities in its programs. In particular, the Company relies and expects to continue to rely on a small number of manufacturers to supply it with its requirements for the active pharmaceutical ingredients and formulated drugs related to these programs. These programs could be adversely affected by a significant interruption in the supply of active pharmaceutical ingredients and formulated drugs.

Tokai Pharmaceuticals, Inc.

Notes to the Consolidated Financial Statements—(Continued)
(Amounts in thousands, except share and per share data)
(Unaudited)

Fair Value Measurements

Certain assets and liabilities are carried at fair value under GAAP. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

- Level 1—Quoted prices in active markets for identical assets or liabilities.
- Level 2—Observable inputs (other than Level 1 quoted prices) such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities, or other inputs that are observable or can be corroborated by observable market data.
- Level 3—Unobservable inputs that are supported by little or no market activity and that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

The Company's cash equivalents of $91,311 and $1,311 as of September 30, 2014 and December 31, 2013, respectively, were carried at fair value based on Level 2 inputs. The carrying values of accounts payable and accrued expenses approximate their fair value due to the short-term nature of these liabilities.

Deferred Offering Costs

The Company capitalizes certain legal, accounting and other third-party fees that are directly associated with in-process equity financings as other assets until such financings are consummated. After consummation of the equity financing, these costs are recorded in stockholders' equity (deficit) as a reduction of additional paid-in capital generated as a result of the offering or as a reduction to the carrying value of preferred stock issued. As of December 31, 2013, the Company had recorded $30 of deferred offering costs, included in other assets in the accompanying consolidated balance sheet in contemplation of the Company's IPO, which closed in September 2014. The Company has no deferred offering costs as of September 30, 2014.

Property and Equipment

Property and equipment are stated at cost less accumulated depreciation. Depreciation expense is recognized using the straight-line method over a three-year estimated useful life for computer equipment, which is the only type of property and equipment the Company holds. Expenditures for repairs and maintenance of assets are charged to expense as incurred. Upon retirement or sale, the cost and related accumulated depreciation of assets disposed of are removed from the accounts and any resulting gain or loss is included in loss from operations.

Impairment of Long-Lived Assets

Long-lived assets consist of property and equipment. Long-lived assets to be held and used are tested for recoverability whenever events or changes in business circumstances indicate that the carrying amount of the assets may not be fully recoverable. Factors that the Company considers in deciding when to perform an impairment review include significant underperformance of the business in relation to expectations, significant negative industry or economic trends, and significant changes or planned changes in the use of the assets. If an impairment review is performed to evaluate a long-lived asset for recoverability, the Company compares forecasts of undiscounted cash flows expected to result from the use and eventual disposition of the long-lived asset to its carrying value. An impairment loss would be recognized when estimated undiscounted future cash flows expected to result from the use of an asset are less than its carrying amount. The impairment loss would be based on the excess of the carrying value of the impaired asset over its fair value, determined based on discounted cash flows. To date, the Company has not recorded any impairment losses on long-lived assets.

Research and Development Costs

Research and development costs are expensed as incurred. Included in research and development expenses are salaries, stock-based compensation and benefits of employees, third-party license fees and other operational costs related to the Company's research and development activities, including manufacturing expenses and external costs of outside vendors engaged to conduct both preclinical studies and clinical trials.

Tokai Pharmaceuticals, Inc.

Notes to the Consolidated Financial Statements—(Continued)
(Amounts in thousands, except share and per share data)
(Unaudited)

The Company has entered into various research and development contracts with research institutions and other companies both inside and outside of the United States. These agreements are generally cancelable, and related payments are recorded as research and development expenses as incurred. The Company records accruals for estimated ongoing costs. This process involves reviewing open contracts and purchase orders, communicating with personnel to identify services that have been performed and estimating the level of service performed and the associated costs incurred for the services for which the Company has not yet been invoiced. Significant judgments and estimates are made in determining the accrued balances at the end of any reporting period. Actual results could differ from the Company's estimates. The Company's historical accrual estimates have not been materially different from the actual costs.

Patent Costs

All patent-related costs incurred in connection with filing and prosecuting patent applications are recorded as general and administrative expenses as incurred, as recoverability of such expenditures is uncertain.

Accounting for Stock-Based Compensation

The Company measures all stock options and other stock-based awards granted to employees and directors at the fair value on the date of the grant using the Black-Scholes option-pricing model. The fair value of the awards is recognized as expense, net of estimated forfeitures, over the requisite service period, which is generally the vesting period of the respective award. The straight-line method of expense recognition is applied to all awards with service-only conditions, while the graded vesting method is applied to all grants with both service and performance conditions.

For stock-based awards granted to non-employees, compensation expense is recognized over the period during which services are rendered by such non-employees until completed. At the end of each financial reporting period prior to completion of the service, the fair value of these awards is remeasured using the then-current fair value of the Company's common stock and updated assumption inputs in the Black-Scholes option-pricing model.

The Company classifies stock-based compensation expense in its consolidated statement of operations and comprehensive loss in the same manner in which the award recipient's payroll costs are classified or in which the award recipient's service payments are classified.

The Company recognizes compensation expense for only the portion of awards that are expected to vest. In developing a forfeiture rate estimate, the Company has considered its historical experience to estimate pre-vesting forfeitures for service-based awards. The impact of a forfeiture rate adjustment will be recognized in full in the period of adjustment, and if the actual forfeiture rate is materially different from the Company's estimate, the Company may be required to record adjustments to stock-based compensation expense in future periods.

Income Taxes

The Company accounts for income taxes using the asset and liability method, which requires the recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been recognized in the consolidated financial statements or in the Company's tax returns. Deferred taxes are determined based on the difference between the financial statement and tax basis of assets and liabilities using enacted tax rates in effect in the years in which the differences are expected to reverse. Changes in deferred tax assets and liabilities are recorded in the provision for income taxes. The Company assesses the likelihood that its deferred tax assets will be recovered from future taxable income and, to the extent it believes, based upon the weight of available evidence, that it is more likely than not that all or a portion of deferred tax assets will not be realized, a valuation allowance is established through a charge to income tax expense. Potential for recovery of deferred tax assets is evaluated by estimating the future taxable profits expected and considering prudent and feasible tax planning strategies.

The Company accounts for uncertainty in income taxes recognized in the consolidated financial statements by applying a two-step process to determine the amount of tax benefit to be recognized. First, the tax position must be evaluated to determine the likelihood that it will be sustained upon external examination by the taxing authorities. If the tax position is deemed more-likely-than-not to be sustained, the tax position is then assessed to determine the amount of benefit to recognize in the consolidated financial statements. The amount of the benefit that may be recognized is the largest amount that has a greater than 50% likelihood of being realized upon ultimate settlement. The provision for income taxes includes the effects of any resulting tax reserves, or unrecognized tax benefits, that are considered appropriate as well as the related net interest and penalties.

Tokai Pharmaceuticals, Inc.

Notes to the Consolidated Financial Statements—(Continued)
(Amounts in thousands, except share and per share data)
(Unaudited)

Segment Data

The Company manages its operations as a single segment for the purposes of assessing performance and making operating decisions. The Company's singular focus is on developing novel proprietary therapies for the treatment of prostate cancer and other hormonally-driven diseases. No revenue has been generated since inception, and all tangible assets are held in the United States.

Comprehensive Loss

Comprehensive loss includes net loss as well as other changes in stockholders' equity (deficit) that result from transactions and economic events other than those with stockholders. For the three and nine months ended September 30, 2014 and 2013, there was no difference between net loss and comprehensive loss.

Net Income (Loss) Per Share

In September 2014, upon the closing of the IPO, all of the outstanding shares of the Company's redeemable convertible preferred stock automatically converted into 14,860,173 shares of the Company's common stock. Prior to this conversion, the Company followed the two-class method when computing net income (loss) per share as the Company had issued shares that met the definition of participating securities. The two-class method determines net income (loss) per share for each class of common and participating securities according to dividends declared or accumulated and participation rights in undistributed earnings. The two-class method requires income available to common stockholders for the period to be allocated between common and participating securities based upon their respective rights to receive dividends as if all income for the period had been distributed. The Company's redeemable convertible preferred stock contractually entitled the holders of such shares to participate in dividends, but did not contractually require the holders of such shares to participate in losses of the Company. Accordingly, the two-class method did not apply for periods in which the Company reported a net loss or a net loss attributable to common stockholders resulting from dividends or accretion related to its redeemable convertible preferred stock.

Basic net income (loss) per share attributable to common stockholders is computed by dividing the net income (loss) attributable to common stockholders by the weighted average number of common shares outstanding for the period. Diluted net income (loss) per share attributable to common stockholders is computed by dividing the diluted net income (loss) attributable to common stockholders by the weighted average number of common shares, including potential dilutive common shares assuming the dilutive effect of outstanding stock options and unvested restricted common shares, as determined using the treasury stock method. For periods in which the Company has reported net losses, diluted net loss per common share attributable to common stockholders is the same as basic net loss per common share attributable to common stockholders, since dilutive common shares are not assumed to have been issued if their effect is anti-dilutive.

The Company reported a net loss attributable to common stockholders for the three and nine months ended September 30, 2014 and 2013.

The following common stock equivalents outstanding as of September 30, 2014 and 2013 were excluded from the computation of diluted net loss per share for the three and nine months ended September 30, 2014 and 2013, because they had an anti-dilutive impact:

	As of September 30,	
	2014	2013
Options to purchase common stock	1,852,709	1,062,423
Restricted common stock units	54,604	—
Redeemable convertible preferred stock	—	12,487,660
Option to purchase additional shares of common stock granted to the underwriters in connection with the IPO	972,000	—
Total options, restricted stock units and redeemable convertible preferred stock exercisable or convertible into common stock	2,879,313	13,550,083

In connection with the IPO, the Company granted the underwriters an option to purchase up to 972,000 additional shares of the Company's common stock for a period of 30 days from September 16, 2014, which is reflected in the table above. As noted in Note 10, the Company issued and sold 540,000 shares of its common stock in October 2014 pursuant to the underwriters' partial exercise of their option to purchase additional shares of common stock.

Tokai Pharmaceuticals, Inc.

Notes to the Consolidated Financial Statements—(Continued)
(Amounts in thousands, except share and per share data)
(Unaudited)

Recently Issued and Adopted Accounting Pronouncements

In June 2014, the FASB issued Accounting Standards Update No. 2014-10, *Development Stage Entities (Topic 915): Elimination of Certain Financial Reporting Requirements, Including an Amendment to Variable Interest Entities Guidance in Topic 810, Consolidation*. The amendments in this guidance remove all incremental financial reporting requirements for development stage entities. Among other changes, this guidance will no longer require development stage entities to present inception-to-date information about income statement line items, cash flows, and equity transactions. This guidance is effective for public companies in the first annual period beginning after December 15, 2014 with early adoption permitted. The Company elected to apply this disclosure guidance to its financial statements for the three months ended September 30, 2014 and as a result, no longer discloses inception-to-date information in its statements of operations and comprehensive loss, cash flows and stockholders' deficit and the related notes thereto.

In August 2014, the FASB issued ASU 2014-15, *Presentation of Financial Statements — Going Concern* (*Subtopic 205-40).* The new guidance addresses management's responsibility to evaluate whether there is substantial doubt about an entity's ability to continue as a going concern and to provide related footnote disclosures. Management's evaluation should be based on relevant conditions and events that are known and reasonably knowable at the date that the financial statements are issued. The standard will be effective for the first interim period within annual reporting periods beginning after December 15, 2016. Early adoption is permitted. The Company is evaluating the effect that this guidance will have on its consolidated financial statements.

3. Accrued Expenses

Accrued expenses consisted of the following:

	September 30, 2014	December 31, 2013
Accrued research and development expenses	$ 1,015	$ 1,370
Accrued payroll and related expenses	571	436
Accrued professional fees	710	338
Accrued other	187	60
	$ 2,483	$ 2,204

4. Redeemable Convertible Preferred Stock and Stockholders' Equity (Deficit)

On August 29, 2014, the Company effected a 1-for-10.47 reverse stock split of its issued and outstanding shares of common stock and a proportional adjustment to the existing conversion ratios for each series of redeemable convertible preferred stock. Accordingly, all share and per share amounts for all periods presented in these consolidated financial statements and notes thereto have been adjusted retroactively, where applicable, to reflect this reverse stock split and adjustment of the redeemable convertible preferred stock conversion ratios.

On September 22, 2014, the Company completed its IPO and issued and sold 6,480,000 shares of common stock at a public offering price of $15.00 per share, resulting in net proceeds of approximately $90,396 after deducting underwriting discounts and commissions but before the payment of estimated offering expenses of $3,306.

Upon the closing of the IPO, all outstanding shares of the Company's redeemable convertible preferred stock automatically converted into 14,860,173 shares of the Company's common stock.

As of September 30, 2014 the Company's Certificate of Incorporation, as amended and restated, authorizes the Company to issue 5,000,000 shares of preferred stock, $0.001 par value.

As of September 30, 2014 and December 31, 2013, the Company's Certificate of Incorporation, as amended and restated, authorizes the Company to issue 200,000,000 and 173,018,331 shares, respectively, of common stock, $0.001 par value.

Tokai Pharmaceuticals, Inc.

Notes to the Consolidated Financial Statements—(Continued)
(Amounts in thousands, except share and per share data)
(Unaudited)

5. Income Taxes

The Company did not provide for any income taxes in any of the three or nine month periods ended September 30, 2014 or 2013. The Company had gross deferred tax assets of $24,402 at December 31, 2013 which increased by approximately $6,800 at September 30, 2014. The Company has provided a valuation allowance for the full amount of its net deferred tax assets because, at September 30, 2014 and December 31, 2013, it was more likely than not that any future benefit from deductible temporary differences and net operating loss and tax credit carryforwards would not be realized.

The Company has not recorded any amounts for unrecognized tax benefits as of September 30, 2014 or December 31, 2013. As of September 30, 2014 and December 31, 2013, the Company had no accrued interest or tax penalties recorded. The Company's income tax return reporting periods since December 31, 2011 are open to income tax audit examination by the federal and state tax authorities. In addition, because the Company has net operating loss carryforwards, the Internal Revenue Service is permitted to audit earlier years and propose adjustments up to the amount of net operating losses generated in those years.

6. Stock-Based Awards

2007 Stock Incentive Plan

Under the Company's 2007 Stock Incentive Plan, as amended (the "2007 Plan"), the Company was authorized to grant options, restricted stock, restricted stock units and other stock-based awards to the Company's employees, officers, directors, consultants and advisors. As of December 31, 2013, a total of 1,590,580 shares of common stock were reserved for issuance under the 2007 Plan. The Company's 2014 Stock Incentive Plan (the "2014 Plan") became effective upon effectiveness of the registration statement for the IPO on September 12, 2014. Upon the effectiveness of the 2014 Plan, 43,923 shares of common stock that remained available for grant under the 2007 Plan became available for grant under the 2014 Plan, and no further awards were available to be issued under the 2007 Plan.

2014 Stock Incentive Plan

In August 2014, the Company's board of directors and stockholders approved the 2014 Plan, which became effective upon effectiveness of the registration statement for the IPO on September 12, 2014. Under the 2014 Plan, the Company may grant incentive stock options, nonstatutory stock options, restricted stock, restricted stock units, stock appreciation rights and other stock-based awards to the Company's employees, officers, directors, consultants and advisors; however, incentive stock options may only be granted to the Company's employees. The number of shares reserved for issuance under the 2014 Plan is equal to the sum of (1) 1,700,000 shares of common stock, plus (2) the number of shares (up to 1,678,220 shares) equal to the sum of (i) 43,923 shares of common stock, which is the number of shares reserved for issuance under the 2007 Plan that remained available for grant under the 2007 Plan immediately prior to the effectiveness of the 2014 Plan, and (ii) the number of shares of common stock subject to outstanding awards under the 2007 Plan that expire, terminate or are otherwise surrendered, cancelled, forfeited or repurchased by the Company at their original issuance price pursuant to a contractual repurchase right, plus (3) an annual increase, to be added on the first day of each fiscal year, beginning with the fiscal year ending December 31, 2015 and continuing for each fiscal year until, and including, the fiscal year ending December 31, 2024, equal to the least of 1,800,000 shares of the Company's common stock, 4% of the number of shares of the Company's common stock outstanding on the first day of the applicable fiscal year and an amount determined by the Company's board of directors.

As required by the 2007 Plan and 2014 Plan, the exercise price for stock options granted is not to be less than the fair value of common stock as of the date of grant. Prior to the IPO, the value of common stock was determined by the Company's board of directors by taking into consideration its most recently available valuation of common stock performed by management and the board of directors as well as additional factors which might have changed since the date of the most recent contemporaneous valuation through the date of grant.

For the three months ended September 30, 2014, the Company granted an employee a stock option to purchase 218,417 shares of its common stock and a restricted stock unit for 54,604 shares of common stock. The Company did not grant any other equity awards during the three months ended September 30, 2014. As of September 30, 2014, 1,470,902 shares remained available for issuance under the 2014 Plan.

Tokai Pharmaceuticals, Inc.

Notes to the Consolidated Financial Statements—(Continued)
(Amounts in thousands, except share and per share data)
(Unaudited)

2014 Employee Stock Purchase Plan

In August 2014, the Company's board of directors and stockholders approved the 2014 Employee Stock Purchase Plan (the "ESPP"), which became effective upon effectiveness of the registration statement for the IPO on September 12, 2014. An aggregate of 225,000 shares of the Company's common stock are reserved for issuance under the ESPP. The number of shares of the Company's common stock reserved for issuance under the ESPP will automatically increase on the first day of each fiscal year, commencing on January 1, 2015 and ending on December 31, 2024, in an amount equal to the least of (1) 450,000 shares of the Company's common stock, (2) 1% of the total number of shares of the Company's common stock outstanding on the first day of the applicable fiscal year and (3) an amount determined by the Company's board of directors.

Stock Option Valuation

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model. Prior to its IPO, the Company was a private company and lacked company-specific historical and implied volatility information. Therefore, the Company estimated its expected stock volatility based on the historical volatility of a publicly traded group of peer companies. The Company expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded stock price. The expected term of the Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The expected term of stock options granted to non-employees is equal to the contractual term of the option award. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield is based on the fact that the Company has never paid cash dividends and does not expect to pay any cash dividends in the foreseeable future.

Stock-based Compensation

The Company recorded stock-based compensation expense related to stock options and restricted common stock in the following expense categories of its statements of operations:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2013	2014	2013
Research and development	$ 186	$ 14	$ 322	$ 55
General and administrative	993	33	1,197	82
	$1,179	$ 47	$1,519	$137

Stock-based compensation expense for the three and nine months ended September 30, 2014 includes $880 of stock-based compensation expense related to a performance-based option grant which vested during the three months ended September 30, 2014. As of September 30, 2014, the Company had an aggregate of $5,073 of unrecognized stock-based compensation cost, which is expected to be recognized over a weighted average period of 3.5 years.

Tokai Pharmaceuticals, Inc.

Notes to the Consolidated Financial Statements—(Continued)
(Amounts in thousands, except share and per share data)
(Unaudited)

7. Commitments and Contingencies

Leases

The Company leases its office space and obtains certain office-related services on a month-to-month basis under a 30-day cancelable operating service agreement. Payments under this service agreement include monthly rent and certain fee-for-service charges.

During the three months ended September 30, 2014 and 2013, the Company recognized $152 and $92, respectively, of rental expense related to office space. During the nine months ended September 30, 2014 and 2013, the Company recognized $361 and $265, respectively, of rental expense related to office space.

Intellectual Property Licenses

The Company has a master license agreement, dated May 19, 2006, with the University of Maryland, Baltimore ("UMB"), as amended by First Amendment, dated as of March 3, 2009, Second Amendment, dated as of April 10, 2012, and Third Amendment, dated as of October 28, 2013 (the "license agreement"). Pursuant to the license agreement, UMB granted an exclusive worldwide license, with the right to sublicense, under certain patents and patent applications to make, have made, use, sell, offer to sell and import certain anti-androgen steroids including galeterone for the prevention, diagnosis, treatment or control of any human or animal disease. In addition, UMB granted the Company a first option to receive an exclusive license to UMB's rights in certain improvements to the licensed products. The Company has exercised the option and acquired exclusive rights to licensed improvements under three amendments to the license agreement.

The Company is obligated to pay UMB an annual maintenance fee of $10 each year until the first commercial sale of a product developed using the licensed technology. The Company is also obligated to make an additional $50 milestone payment to UMB for each additional investigational new drug application filed for a licensed product and a $100 milestone payment upon the approval of each new drug application for a licensed product by the U.S. Food and Drug Administration. Because the achievement of these milestones has not occurred as of December 31, 2013 or September 30, 2014, no liabilities for such milestone payments have been recorded in the Company's consolidated financial statements.

The Company must also pay UMB low single-digit percentage royalties on aggregate worldwide net sales of licensed products, including sales by sublicensees, on a licensed product-by-licensed product and country-by-country basis until the later of the expiration of the last-to-expire applicable licensed patent or ten years after first commercial sale of the applicable licensed product, in each case in the applicable country. The royalty obligations are subject to specified reductions in the event that additional licenses need to be obtained from third parties or in the event of specified competition from third-party products licensed by UMB. Minimum annual royalty payments to UMB are $50 beginning in the year following the year in which the first commercial sale occurs. The Company must also pay UMB 10% of all non-royalty sublicense income received from sublicensees. Finally, the Company is responsible for all patent expenses related to the prosecution and maintenance of the licensed patents. As of December 31, 2013 and September 30, 2014, the Company has not yet developed a commercial product using the licensed technologies, and it has not entered into any sublicense agreements for the technologies. In connection with this license agreement, the Company incurred no license, milestone or maintenance fees for the three months ended September 30, 2014 or 2013. In connection with this license agreement, the Company incurred license, milestone and maintenance fees of $10 in each of the nine month periods ended September 30, 2014 and 2013.

Advisor Agreement

The Company was obligated to pay a fee to a financial advisor of $1,053 upon the closing of the IPO of the Company's shares of common stock in connection with strategic and financial advisory services unrelated to the IPO. As of September 30, 2014, the Company had recorded general and administrative expense of $1,053 related to this payment.

Tokai Pharmaceuticals, Inc.

Notes to the Consolidated Financial Statements—(Continued)
(Amounts in thousands, except share and per share data)
(Unaudited)

Indemnification Agreements

In the ordinary course of business, the Company may provide indemnification of varying scope and terms to vendors, lessors, business partners, and other parties with respect to certain matters including, but not limited to, losses arising out of breach of such agreements or from intellectual property infringement claims made by third parties.

In addition, the Company has entered into indemnification agreements with each of its directors and executive officers, which provide, among other things, that the Company will indemnify such directors and executive officers to the fullest extent permitted by law for claims arising in his or her capacity as a director or officer. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited

To date, the Company has not incurred any material costs as a result of the indemnification agreements described above. The Company does not believe that the outcome of any claims under indemnification arrangements will have a material effect on its financial position, results of operations or cash flows, and it has not accrued any liabilities related to such obligations in its financial statements as of December 31, 2013 or September 30, 2014.

8. 401(k) Plan

The Company has a 401(k) plan available for participating employees who meet certain eligibility requirements. Eligible employees may defer a portion of their salary as defined by the plan. Company contributions to the plan may be made at the discretion of the Board of Directors. To date, the Company has not made any contributions to the plan.

9. Related Party Transactions

The Company has an outstanding loan to a former advisor and stockholder that has been fully reserved for by the Company. As of December 31, 2013, no payments had been received by the Company, and the unpaid principal and interest balance remained fully reserved. Subsequent to December 31, 2013, the Company started to receive repayment of this note. The Company is recording payments received as other income in 2014 as cash is received. As a result, the Company recorded other income of $34 and $113 for the three and nine months ended September 30, 2014, representing cash collected by the Company during those periods.

10. Subsequent Events

In connection with the IPO, on October 9, 2014, the Company issued and sold an additional 540,000 shares of its common stock at the IPO price of $15.00 per share, less underwriting discounts and commissions, pursuant to the underwriters' partial exercise of their option to purchase additional shares of common stock, resulting in additional net proceeds to the Company of approximately $7,533 after deducting underwriting discounts and commissions.

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

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q and our final prospectus for our initial public offering filed pursuant to Rule 424(b)(4) under the Securities Act of 1933, as amended, or the Securities Act, with the Securities and Exchange Commission, or SEC, on September 17, 2014. Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report on Form 10-Q, including information with respect to our plans and strategy for our business, includes forward looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of this Quarterly Report on Form 10-Q, our actual results could differ materially from the results described, in or implied, by these forward-looking statements.

We are a clinical-stage biopharmaceutical company focused on developing novel proprietary therapies for the treatment of prostate cancer and other hormonally-driven diseases. Our lead drug candidate, galeterone, is a highly selective, multi-targeted, oral small molecule drug candidate that we believe has advantages over existing prostate cancer therapies. As of October 31, 2014, we have administered galeterone to over 250 prostate cancer patients and healthy volunteers in Phase 1 and Phase 2 clinical trials. In these trials, galeterone was well tolerated and, in multiple prostate cancer populations, showed clinically meaningful reductions in levels of prostate specific antigen, a biochemical marker used to evaluate prostate cancer patients for signs of response to therapy. We are currently finalizing our plans for our pivotal Phase 3 clinical trial of galeterone based on discussions with the U.S. Food and Drug Administration, or FDA. We anticipate initiating the trial in the first half of 2015.

We are focusing our initial development of galeterone on the treatment of patients with castration resistant prostate cancer, or CRPC, whose prostate tumor cells express an altered androgen receptor that is truncated. These truncated androgen receptors are missing the end of the receptor that contains the ligand binding domain. We describe patients with these truncated androgen receptors as having C-terminal loss. An example of one such truncated androgen receptor with C-terminal loss is the splice variant AR-V7, which is the most prevalent of the splice variants that cause C-terminal loss. We intend to conduct our planned pivotal Phase 3 clinical trial of galeterone in CRPC patients with AR-V7.

We are currently conducting a Phase 2 clinical trial of galeterone for the treatment of multiple CRPC populations, which we refer to as our ARMOR2 trial. Subject to the results of the ARMOR2 trial and to the availability of resources, we anticipate continuing the clinical development of galeterone in multiple CRPC populations. In June 2012, the FDA designated galeterone for fast track review. We have exclusive worldwide development and commercialization rights to galeterone.

Since our inception in March 2004, we have devoted substantially all of our resources to developing our product candidates, building our intellectual property portfolio, business planning, raising capital and providing general and administrative support for these operations. Prior to 2007, we focused our efforts on the development of women's health products. In 2007, we changed our focus and began developing novel proprietary therapies for the treatment of prostate cancer and other hormonally-driven diseases, including our lead drug candidate, galeterone. Through September 30, 2014, we have funded our operations primarily through our initial public offering of our common stock, and prior to our initial public offering, private placements of our redeemable convertible preferred stock and convertible promissory notes. In September 2014, we completed the initial public offering of our common stock and issued and sold 6,480,000 shares of our common stock at a public offering price of $15.00 per share, resulting in net proceeds of approximately $90.4 million after deducting underwriting discounts and commissions but before the payment of estimated offering expenses of $3.3 million. From our inception through September 30, 2014, we received aggregate net proceeds of $91.6 million from the sale of our redeemable convertible preferred stock and convertible promissory notes. In connection with our initial public offering, we issued and sold an additional 540,000 shares of our common stock in October 2014 pursuant to the underwriters' partial exercise of their option to purchase additional shares of common stock at the public offering price of $15.00 and received additional net proceeds of $7.5 million after deducting underwriting discounts and commissions.

We have never generated any revenue and have incurred net losses in each year since our inception. We have an accumulated deficit of $80.1 million as of September 30, 2014. Our net loss was $17.1 million for the nine months ended September 30, 2014. These losses have resulted principally from costs incurred in connection with research and development activities, general and administrative costs associated with our operations and in-licensing our product candidates. 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, if and as we:

- conduct our planned pivotal Phase 3 clinical trial of galeterone for metastatic CRPC treatment-naïve patients whose prostate tumors express the splice variant AR-V7 and conduct other clinical trials and non-clinical studies to support the submission of a new drug application, or NDA, to the FDA for galeterone for this indication;
- continue to conduct our ongoing ARMOR2 trial for the treatment of multiple CRPC patient populations;
- develop galeterone for the treatment of other indications and patient populations in prostate cancer;
- explore the use of galeterone for the treatment of other hormonally-driven diseases that are associated with the androgen receptor signaling pathway;
- identify and develop compounds that are designed to disrupt androgen receptor signaling through enhanced androgen receptor degradation;
- enter into agreements with third parties to develop an in vitro companion diagnostic test to identify CRPC patients with AR-V7;
- enter into agreements with third parties to manufacture galeterone;
- establish a sales, marketing and distribution infrastructure to support the commercialization of galeterone in the United States;
- maintain, expand and protect our intellectual property portfolio;
- continue our other research and development efforts;
- acquire or in-license additional compounds or technologies; and
- operate as a public company.

Our ability to generate product revenue, which we do not expect will occur for at least the next several years, if ever, will depend heavily on the successful development and eventual commercialization of galeterone and other product candidates that we may develop in the future. As a result, we will need additional financing to support our continuing operations. Until such time that we can generate significant revenue from product sales, if ever, we expect to finance our operations through a combination of equity offerings, debt financings, collaborations, strategic alliances, licensing arrangements and other marketing and distribution arrangements. We may be unable to raise capital when needed or on acceptable terms, which would force us to delay, limit, reduce or terminate our research and development programs or commercialization efforts. We will need to generate significant revenue to achieve profitability, and we may never do so.

As of September 30, 2014, we had cash and cash equivalents of $105.5 million. We expect that our existing cash and cash equivalents, together with the net proceeds from the additional shares of common stock sold in October 2014 in connection with our initial public offering, will be sufficient to enable us to continue our ongoing ARMOR2 trial, conduct our planned pivotal Phase 3 clinical trial of galeterone for CRPC patients with AR-V7 and conduct other clinical trials and non-clinical studies to support the submission of an NDA to the FDA for galeterone for this indication, as well as to continue to fund our operating expenses and capital expenditure requirements into the first half of 2017. See "—Liquidity and Capital Resources."

Financial Operations Overview

Revenue

To date, we have not generated any revenue from product sales and do not expect to generate any revenue from the sale of products in the near future. If our development efforts for galeterone or other product candidates that we may develop in the future are successful and result in regulatory approval or license or collaboration agreements with third parties, we may generate revenue in the future from a combination of product sales or payments from collaboration or license agreements that we may enter into with third parties.

Operating Expenses

The majority of our operating expenses consist of research and development activities and general and administrative costs.

Research and Development Expenses

Research and development expenses, which consist primarily of costs associated with our product research and development efforts, are expensed as incurred. Research and development expenses consist primarily of:

- third-party contract costs relating to research, formulation and manufacturing, preclinical studies and clinical trial activities;
- personnel costs, including salaries, related benefits and stock-based compensation for personnel engaged in research and development functions;
- consulting fees paid to third parties;
- costs related to compliance with regulatory requirements; and
- payments made under our third-party licensing agreements.

We typically use our employee and infrastructure resources across our development programs. We track outsourced development costs by product candidate or development program, but we do not allocate personnel costs, payments made under our licensing agreements or other internal costs to specific development programs or product candidates. These costs are included in unallocated research and development expenses in the table below.

The following table summarizes our research and development expenses by program:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2013	2014	2013
	(in thousands)			
Galeterone for prostate cancer	$1,884	$2,276	$ 8,365	$6,461
Other early-stage development programs and additional indications for galeterone	—	—	49	17
Unallocated research and development expenses	941	428	2,359	1,374
Total research and development expenses	$2,825	$2,704	$10,773	$7,852

Research and development activities are central to our business. 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 that our research and development expenses will continue to increase in the foreseeable future as we pursue later stages of clinical development of galeterone and other product candidates that we may develop in the future.

We cannot determine with certainty the duration and completion costs of the current or future clinical trials of our product candidates or if, when, or to what extent we will generate revenue from the commercialization and sale of any of our product candidates that obtain regulatory approval. We may never succeed in achieving regulatory approval for any of our product candidates. The duration, costs and timing of clinical studies and development of our product candidates will depend on a variety of factors, including:

- the scope, rate of progress, expense and results of our ongoing clinical trials as well as any additional clinical trials and other research and development activities that we may conduct;
- future clinical trial results;
- uncertainties in clinical trial design and patient enrollment rate;
- significant and changing government regulation and regulatory guidance;
- the timing and receipt of any regulatory approvals; and
- the expense 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 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 patient 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.

We are currently conducting a Phase 2 clinical trial of galeterone for the treatment of CRPC, which we refer to as our ARMOR2 trial. We anticipate initiating a pivotal Phase 3 clinical trial of galeterone in the first half of 2015. Our current estimate for the costs associated with funding our ongoing ARMOR2 trial, conducting our planned pivotal Phase 3 clinical trial of galeterone for CRPC patients whose prostate tumors express the splice variant AR-V7 and conducting other clinical trials and non-clinical studies to support the submission of an NDA to the FDA for galeterone for this indication is approximately $49.5 million.

General and Administrative Expenses

General and administrative expenses consist primarily of personnel costs, including salaries, related benefits and stock-based compensation expense, of our executive, finance, business and corporate development and other administrative functions. General and administrative expenses also include professional fees for auditing, tax and legal services, including legal expenses to pursue patent protection of our intellectual property, travel expenses and facility-related costs.

We expect that our general and administrative expenses will increase in future periods as we continue the development and potential commercialization of galeterone for the treatment of CRPC and any future product candidates and as a result of increased payroll, expanded infrastructure, increased consulting, legal, accounting and investor relations expenses associated with being a public company and costs incurred to seek collaborations with respect to galeterone and any other product candidates that we may develop in the future.

Other Income (Expense)

Interest Income. Interest income consists of interest earned on our cash and cash equivalents. Our interest income has not been significant due to nominal investment balances and low interest earned on those balances. We expect that our interest income will increase in the future due to the cash proceeds received from our initial public offering, including the cash proceeds received from the underwriters' partial exercise of their option to purchase additional shares of common stock.

Other Income (Expense), Net. Other income (expense), net also consists of small amounts of miscellaneous income and expense unrelated to our core operations.

Income Taxes

Since our inception in 2004, we have not recorded any U.S. federal or state income tax benefits for the net losses we have incurred in each year or our earned research and development tax credits, due to our uncertainty of realizing a benefit from those items. As of December 31, 2013, we had federal and state net operating loss carryforwards of $10.5 million and $8.1 million respectively. Our federal and state net operating loss carryforwards begin to expire in 2024 and 2014, respectively. We also had federal and state research and development tax credit carryforwards of $0.6 million and $0.4 million, respectively, as of December 31, 2013, which begin to expire in 2025 and 2023, respectively. Our federal and state net operating loss carryforwards do not yet include the effect of research and development expenses of $49.0 million that we have capitalized for income tax purposes as of December 31, 2013.

Critical Accounting Policies and Significant Judgments and Estimates

Our consolidated financial statements are prepared in accordance with U.S. generally accepted accounting principles. The preparation of our financial statements and related disclosures requires us to make estimates, assumptions and judgments that affect the reported amount of assets, liabilities, revenue, costs and expenses, and related disclosures. During the three months ended September 30, 2014, there were no material changes to our critical accounting policies. Our critical accounting policies are described under the heading "Management's Discussion and Analysis of Financial Condition and Results of Operations— Critical Accounting Policies and Significant Judgments and Estimates" in our final prospectus for our initial public offering filed pursuant to Rule 424(b)(4) under the Securities Act with the SEC on September 17, 2014 and the notes to the consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q. We believe that of our critical accounting policies, the following accounting policies involve the most judgment and complexity:

- accrued research and development costs; and
- stock-based compensation.

Accordingly, we believe the policies set forth above are critical to fully understanding and evaluating our financial condition and results of operations. If actual results or events differ materially from the estimates, judgments and assumptions used by us in applying these policies, our reported financial condition and results of operations could be materially affected.

Results of Operations

Comparison of the Three Months Ended September 30, 2014 and 2013

The following table summarizes our results of operations for the three months ended September 30, 2014 and 2013:

	Three Months Ended September 30,		Increase (Decrease)
	2014	2013	
	(in thousands)		
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development	2,825	2,704	121
General and administrative	3,599	661	2,938
Total operating expenses	6,424	3,365	3,059
Loss from operations	(6,424)	(3,365)	(3,059)
Other income (expense), net	34	—	34
Net loss	$(6,390)	$(3,365)	$ (3,025)

Research and Development Expenses

	Three Months Ended September 30,		Increase (Decrease)
	2014	2013	
	(in thousands)		
Galeterone for prostate cancer	$1,884	$2,276	$ (392)
Other early-stage development programs and additional indications for galeterone	—	—	—
Unallocated research and development expenses	941	428	513
Total research and development expenses	$2,825	$2,704	$ 121

Research and development expenses for the three months ended September 30, 2014 were $2.8 million, compared to $2.7 million for the three months ended September 30, 2013. The increase of $0.1 million was primarily due to an increase of $0.5 million in unallocated research and development expenses partially offset by decreased costs of $0.4 million associated with our galeterone for prostate cancer program. The increase in unallocated research and development costs of $0.5 million for the three months ended September 30, 2014 from the three months ended September 30, 2013 was primarily due to increased personnel related costs, including stock-based compensation expense, as a result of increased headcount in our research and development function. The decrease in costs of our galeterone for prostate cancer program of $0.4 million consisted primarily of decreased manufacturing costs of $0.8 million, partially offset by increased costs of clinical trials of $0.4 million. The decrease in manufacturing costs was due to decreased costs related to manufacturing galeterone for use in our ARMOR2 trial and in anticipation of our planned pivotal Phase 3 clinical trial of galeterone and the technology transfer of our manufacturing process to a new vendor in the three months ended September 30, 2013, that we did not incur in the three months ended September 30, 2014. The increase in clinical trial costs was due to an increased number of patients and sites in our ARMOR2 trial in the three months ended September 30, 2014 as compared to the three months ended September 30, 2013.

General and Administrative Expenses

	Three Months Ended September 30,		Increase (Decrease)
	2014	2013	
	(in thousands)		
Personnel related (including stock-based compensation)	$1,642	$288	$ 1,354
Professional and consultant fees	1,677	268	1,409
Facility related and other	280	105	175
Total general and administrative expenses	$3,599	$661	$ 2,938

General and administrative expenses for the three months ended September 30, 2014 were $3.6 million, compared to $0.7 million for the three months ended September 30, 2013. The increase of $2.9 million was primarily due to an increase in professional and consultant fees of $1.4 million as well as an increase in personnel related costs of $1.4 million. The increase in professional and consultant fees primarily consisted of a $1.1 million fee payable to a financial advisor upon the closing of our initial public offering in connection with strategic and financial advisory services unrelated to the offering, and an increase in accounting, public relations and patent fees of $0.3 million associated with ongoing business activities and our preparations to operate as a public company. Personnel related costs increased by $1.4 million primarily due to stock-based compensation expense of $0.9 million recorded in the three months ended September 30, 2014 that was related to the vesting of a performance-based option grant upon the closing of our initial public offering in September 2014. Personnel related costs, including stock-based compensation expense, also increased as a result of increased headcount in our general and administrative function and an increase in overall compensation.

Comparison of the Nine Months Ended September 30, 2014 and 2013

The following table summarizes our results of operations for the nine months ended September 30, 2014 and 2013:

	Nine Months Ended September 30,		Increase (Decrease)
	2014	2013	
	(in thousands)		
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development	10,773	7,852	2,921
General and administrative	6,428	2,348	4,080
Total operating expenses	17,201	10,200	7,001
Loss from operations	(17,201)	(10,200)	(7,001)
Other income (expense):			
Other income	113	—	113
Total other income, net	113	—	113
Net loss	$(17,088)	$(10,200)	$ (6,888)

Research and Development Expenses

	Nine Months Ended September 30,		Increase (Decrease)
	2014	2013	
	(in thousands)		
Galeterone for prostate cancer	$ 8,365	$6,461	$ 1,904
Other early-stage development programs and additional indications for galeterone	49	17	32
Unallocated research and development expenses	2,359	1,374	985
Total research and development expenses	$10,773	$7,852	$ 2,921

Research and development expenses for the nine months ended September 30, 2014 were $10.8 million, compared to $7.9 million for the nine months ended September 30, 2013. The increase of $2.9 million was primarily due to increased costs of $1.9 million associated with our galeterone for prostate cancer program and an increase in unallocated research and development expenses of $1.0 million. The increase in costs of our galeterone for prostate cancer program consisted primarily of increased costs of clinical trials of $2.9 million and an increase of $0.4 million in costs related to non-clinical studies to support our galeterone for prostate cancer program, partially offset by decreased manufacturing costs of $1.3 million. The increase in clinical trial costs was due to an increased number of patients and sites in our ARMOR2 trial in the nine months ended September 30, 2014 as compared to the nine months ended September 30, 2013. The decrease in manufacturing costs was due to decreased costs related to manufacturing galeterone for use in our ARMOR2 trial and in anticipation of our planned pivotal Phase 3 clinical trial of galeterone, including the purchase of raw materials, and the technology transfer of our manufacturing process to a new vendor in the nine months ended September 30, 2013. The increase in unallocated research and development costs of $1.0 million for the nine months ended September 30, 2014 from the nine months ended September 30 2013 was due to increased personnel related costs, including stock-based compensation expense, as a result of increased headcount in our research and development function.

General and Administrative Expenses

	Nine Months Ended September 30,		Increase (Decrease)
	2014	2013	
	(in thousands)		
Personnel related (including stock-based compensation)	$2,815	$1,300	$ 1,515
Professional and consultant fees	3,012	760	2,252
Facility related and other	601	288	313
Total general and administrative expenses	$6,428	$2,348	$ 4,080

General and administrative expenses for the nine months ended September 30, 2014 were $6.4 million, compared to $2.3 million for the nine months ended September 30, 2013. The increase of $4.1 million was primarily due to an increase in professional and consultant fees of $2.3 million, an increase in personnel related costs of $1.5 million, and an increase in facility related and other costs of $0.3 million. The increase in professional and consultant fees primarily consisted of a $1.1 million fee payable to a financial advisor upon the closing of our initial public offering in connection with strategic and financial advisory services unrelated to the offering and an increase in accounting, public relations and patent fees associated with ongoing business activities and our preparations to operate as a public company as well as consulting fees for an external market research study that was conducted in 2014. Personnel related costs increased by $1.5 million primarily due to stock-based compensation expense of $0.9 million recorded in the three months ended September 30, 2014 that was related to the vesting of a performance-based option grant upon the closing of our initial public offering in September 2014. Personnel related costs, including stock-based compensation expense, also increased as a result of increased headcount in our general and administrative function and an increase in overall compensation, partially offset by a decrease in personnel related costs due to severance paid to our former Chief Executive Officer in the nine months ended September 30, 2013.

Liquidity and Capital Resources

Since our inception in March 2004, we have not generated any revenue and have incurred recurring net losses. 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 obtain from additional financings, research funding, collaborations, contract and grant revenue or other sources.

To date, we have funded our operations primarily through our initial public offering of our common stock, and prior to our initial public offering, private placements of our redeemable convertible preferred stock and convertible promissory notes. In September 2014, we completed the initial public offering of our common stock and issued and sold 6,480,000 shares of our common stock at a public offering price of $15.00 per share, resulting in net proceeds of approximately $90.4 million after deducting underwriting discounts and commissions but before the payment of estimated offering expenses of $3.3 million. From our inception through September 30, 2014, we have received aggregate net proceeds of $91.6 million from the sale of our redeemable convertible preferred stock and convertible promissory notes.

As of September 30, 2014 and December 31, 2013, we had cash and cash equivalents of $105.5 million and $31.8 million, respectively.

In addition, in connection with our initial public offering, we issued and sold an additional 540,000 shares of common stock in October 2014 at the initial public offering price of $15.00 per share pursuant to the underwriters' partial exercise of their option to purchase additional shares of common stock, resulting in additional net proceeds of approximately $7.5 million after deducting underwriting discounts and commissions.

Cash Flows

As of September 30, 2014, we had cash and cash equivalents of $105.5 million. We invest our cash equivalents in money market accounts in order to preserve principal.

The following table summarizes our sources and uses of cash for each of the periods presented:

	Nine Months Ended September 30,	
	2014	2013
	(in thousands)	
Cash used in operating activities	$(14,661)	$ (8,655)
Cash used in investing activities	(175)	(49)
Cash provided by financing activities	88,547	20,126
Net increase in cash and cash equivalents	$ 73,711	$11,422

Operating activities. During the nine months ended September 30, 2014, cash used in operating activities was $14.7 million, resulting from our net loss of $17.1 million, partially offset by net non-cash charges of $1.4 million and by net cash provided by changes in our operating assets and liabilities of $1.0 million. Our net loss was primarily attributable to research and development activities related to galeterone and our general and administrative expenses, as we had no revenue in the period. Our net non-cash charges during the period consisted primarily of stock-based compensation expense of $1.5 million. Cash provided by changes in our operating assets and liabilities consisted primarily of a net increase in accounts payable and accrued expenses of $1.9 million, partially offset by an increase in prepaid expenses and other current assets of $0.9 million.

During the nine months ended September 30, 2013, cash used in operating activities was $8.7 million, resulting primarily from our net loss of $10.2 million, partially offset by net non-cash charges of $0.1 million and by net cash provided by changes in our operating assets and liabilities of $1.4 million. Our net loss was primarily attributable to research and development activities related to galeterone and our general and administrative expenses, as we had no revenue in the period. Net cash provided by changes in our operating assets and liabilities during the nine months ended September 30, 2013 consisted primarily of a $1.6 million increase in accounts payable and accrued expenses, partially offset by an increase in prepaid expenses and other current assets of $0.2 million.

Our prepaid expenses and other current assets and accounts payable and accrued expense balances were affected by the timing of vendor invoicing and payments.

Investing activities. We used a small amount of cash during the nine months ended September 30, 2014 and 2013 related to purchases of property and equipment and to increase restricted cash balance related to our corporate credit cards.

Financing activities. During the nine months ended September 30, 2014, net cash provided by financing activities was $88.5 million, primarily as a result of proceeds, net of underwriting discounts and commissions, of $90.4 million from our initial public offering, partially offset by payments of $2.0 million of deferred offering costs related to our initial public offering that were paid during the period.

During the nine months ended September 30, 2013, cash provided by financing activities was $20.1 million, consisting of the net proceeds from the sale and issuance of our Series E redeemable convertible preferred stock of $19.9 million and proceeds of $0.2 million from the exercise of stock options.

Funding Requirements

Galeterone is still in clinical development. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. We anticipate that our expenses will increase substantially if and as we:

- conduct our planned pivotal Phase 3 clinical trial of galeterone for metastatic CRPC treatment-naïve patients whose prostate tumors express the splice variant AR-V7 and conduct other clinical trials and non-clinical studies to support the submission of an NDA to the FDA for galeterone for this indication;
- continue to conduct our ongoing ARMOR2 trial for the treatment of multiple CRPC patient populations;
- develop galeterone for the treatment of other indications and patient populations in prostate cancer;
- explore the use of galeterone for the treatment of other hormonally-driven diseases that are associated with the androgen receptor signaling pathway;
- identify and develop compounds that are designed to disrupt androgen receptor signaling through enhanced androgen receptor degradation;
- enter into agreements with third parties to develop an in vitro companion diagnostic test to identify CRPC patients with AR-V7;
- enter into agreements with third parties to manufacture galeterone;
- establish a sales, marketing and distribution infrastructure to support the commercialization of galeterone in the United States;
- maintain, expand and protect our intellectual property portfolio;
- continue our other research and development efforts;
- acquire or in-license additional compounds or technologies; and
- operate as a public company.

As of September 30, 2014, we had cash and cash equivalents of $105.5 million. We expect that our existing cash and cash equivalents, together with the net proceeds from the additional shares of common stock sold in October 2014 in connection with our initial public offering, will be sufficient to enable us to continue our ongoing ARMOR2 trial, conduct our planned pivotal Phase 3 clinical trial of galeterone for metastatic CRPC treatment-naïve patients with AR-V7 and conduct other clinical trials and non-clinical studies to support the submission of an NDA to the FDA for galeterone for this indication, as well as to continue to fund our operating expenses and capital expenditure requirements into the first half of 2017. We have based this estimate on assumptions that may prove to be wrong, as we may use our available capital resources sooner than we currently expect or our clinical trials may take longer than we anticipate. Because of the numerous risks and uncertainties associated with the development of galeterone and because the extent to which we may enter into collaborations with third parties for development of this product candidate is unknown, we are unable to estimate the amounts of increased capital outlays and operating expenses associated with completing the research and development of our product candidate. Our future capital requirements for galeterone will depend on many factors, including:

- the progress and results of our planned pivotal Phase 3 clinical trial of galeterone for the treatment of prostate cancer in metastatic CRPC treatment-naïve patients with AR-V7 and the completion of the clinical development of galeterone for this indication;
- the progress and results of our ongoing ARMOR2 trial and any additional clinical trials of galeterone that we decide to conduct for the treatment of other indications and patient populations in prostate cancer, including early-stage prostate cancer, and the treatment of prostate cancer in combination with currently marketed prostate cancer therapies and novel targeted agents;
- the timing and outcome of regulatory review of galeterone for the treatment of prostate cancer in CRPC patients with AR-V7 and other indications and patient populations, and of any other future product candidates;
- the costs under agreements with third parties to develop an in vitro companion diagnostic test for identifying CRPC patients with AR-V7;
- the cost of commercialization activities, including product sales, marketing, manufacturing and distribution, for galeterone and our future product candidates for which we receive regulatory approval;
- revenue, if any, received from commercial sales of galeterone and any future product candidates, should any of our product candidates be approved by the FDA or a similar regulatory authority outside the United States;
- our ability to establish collaborations on favorable terms, if at all, particularly arrangements to develop, market and distribute galeterone and any future product candidates outside the United States;
- the cost of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims; and
- the extent to which we expand our product pipeline by acquiring or in-licensing additional compounds or technologies for the treatment of hormonally-driven diseases.

Until such time, if ever, that we can generate substantial product revenue, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances, licensing arrangements and other marketing and distribution arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that could adversely affect your rights as a common stockholder. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends and may require the issuance of warrants, which could potentially dilute your ownership interest. If we raise additional funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams or research programs of galeterone or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market galeterone that we would otherwise prefer to develop and market ourselves.

Contractual Obligations and Commitments

During the three months ended September 30, 2014, there have been no material changes to our contractual obligations and commitments outside the ordinary course of business from those described under the heading "Management's Discussion and Analysis of Financial Condition and Results of Operations—Contractual Obligations and Commitments" in our final prospectus for our initial public offering filed pursuant to Rule 424(b)(4) under the Securities Act with the SEC on September 17, 2014.

Off-Balance Sheet Arrangements

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

Recently Issued Accounting Pronouncements

In August 2014, the Financial Accounting Standards Board, or FASB, issued Accounting Standards Update 2014-15, "*Presentation of Financial Statements — Going Concern* (*Subtopic 205-40).*" The new guidance addresses management's responsibility to evaluate whether there is substantial doubt about an entity's ability to continue as a going concern and to provide related footnote disclosures. Management's evaluation should be based on relevant conditions and events that are known and reasonably knowable at the date that the financial statements are issued. The standard will be effective for the first interim period within annual reporting periods beginning after December 15, 2016. Early adoption is permitted. We are evaluating the effect that this guidance will have on our consolidated financial statements.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

Interest Rate Fluctuation Risk

Our cash and cash equivalents as of September 30, 2014 consisted of cash and money market accounts. The primary objectives of our investment activities are to preserve principal, provide liquidity and maximize income without significantly increasing risk. Our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates. However, because of the short-term nature of the instruments in our portfolio, a sudden change in market interest rates would not be expected to have a material impact on our financial condition or results of operations.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer (our principal executive officer and principal financial officer, respectively), evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2014. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of September 30, 2014, our Chief Executive Officer and Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) occurred during the three months ended September 30, 2014 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

We are not currently subject to any material legal proceedings.

Item 1A. Risk Factors.

Investing in our common stock involves a high degree of risk. You should consider carefully the risks described below, together with the other information included or incorporated by reference in this quarterly report on Form 10-Q. If any of the following risks occur, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected. In these circumstances, the market price of our common stock could decline.

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred significant losses since our inception. We expect to incur losses for at least the next several years and may never achieve or maintain profitability.

Since inception, we have incurred significant operating losses. Our net loss was $9.6 million for the year ended December 31, 2012, $15.7 million for the year ended December 31, 2013 and $17.1 million for the nine months ended September 30, 2014. As of September 30, 2014, we had an accumulated deficit of $80.1 million. To date, we have financed our operations primarily through our initial public offering of our common stock, and prior to our initial public offering, through private placements of our redeemable convertible preferred stock and convertible promissory notes. We have devoted substantially all of our efforts to research and development, including clinical trials. We have not completed development of any product candidate and it may be several years, if ever, before we have a product candidate ready for commercialization. We expect to continue to incur significant expenses and increasing operating losses for at least the next several years. We anticipate that our expenses will increase substantially if and as we:

- conduct our planned pivotal Phase 3 clinical trial of galeterone for metastatic castration resistant prostate cancer, or CRPC, treatment-naïve patients whose prostate tumors express the splice variant AR-V7 and conduct other clinical trials and non-clinical studies to support the submission of a new drug application, or NDA, to the U.S. Food and Drug Administration, or FDA, for galeterone for this indication;
- continue to conduct our ongoing ARMOR2 trial for the treatment of multiple CRPC patient populations;
- develop galeterone for the treatment of other indications and patient populations in prostate cancer, including early-stage prostate cancer, and the treatment of prostate cancer in combination with currently marketed prostate cancer therapies and novel targeted agents;
- explore the use of galeterone for the treatment of other hormonally-driven diseases that are associated with the androgen receptor signaling pathway;
- identify and develop compounds that are designed to disrupt androgen receptor signaling through enhanced androgen receptor degradation;
- enter into agreements with third parties to develop an in vitro companion diagnostic test to identify CRPC patients with AR-V7;
- enter into agreements with third parties to manufacture galeterone;
- establish a sales, marketing and distribution infrastructure to support the commercialization of galeterone in the United States;
- maintain, expand and protect our intellectual property portfolio;
- continue our other research and development efforts;
- acquire or in-license additional compounds or technologies for the treatment of hormonally-driven diseases; and
- operate as a public company.

We have never generated any revenue from product sales and may never be profitable. To become and remain profitable, we must develop and eventually commercialize a product or products with significant market potential and market acceptance. This development and commercialization will require us to be successful in a range of challenging activities, including successfully completing preclinical testing and clinical trials of galeterone for the treatment of CRPC patients with truncated androgen receptors such as AR-V7 and other indications and patient populations, as well as preclinical testing and clinical trials of any of our future product candidates, obtaining marketing and regulatory approval for these product candidates, contracting with third parties to develop an *in vitro* companion diagnostic test to identify CRPC patients with AR-V7, partnering with third parties to manufacture our product candidates in commercial quantities, marketing and selling those products for which we may obtain regulatory approval and obtaining reimbursement from third-party payors. We may never succeed in these activities and may never generate revenues that are significant or large enough 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. The net losses we incur may fluctuate significantly from quarter-to-quarter and year-to-year, such that a period-to-period comparison of our results of operations may not be a good indication of our future performance. In any particular quarter or quarters, our operating results could be below the expectations of securities analysts or investors, which could cause our share price to decline. 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 or continue our operations. A decline in the value of our company could also cause our stockholders to lose all or part of their investment.

We will need substantial additional funding to complete our development of, and to commercialize, galeterone for the treatment of CRPC patients with AR-V7, which may not be available on acceptable terms, or at all. If we are unable to raise capital when needed, we may be forced to delay, reduce, terminate or eliminate product development programs, including our commercialization efforts for galeterone for the treatment of these patients and other indications and patient populations and for our future product candidates.

As of September 30, 2014, we had cash and cash equivalents of $105.5 million and working capital of $101.4 million. We expect that our existing cash and cash equivalents will only be sufficient to enable us to continue our ongoing ARMOR2 trial, conduct our planned pivotal Phase 3 clinical trial of galeterone for the treatment of prostate cancer in metastatic CRPC treatment-naïve patients with AR-V7, and conduct other clinical trials and non-clinical studies to support the submission of an NDA to the FDA for galeterone for these patients, as well as to continue to fund our operating expenses and capital expenditure requirements into the first half of 2017. We will need to obtain substantial additional funding in order to submit an NDA to the FDA for galeterone for the treatment of CRPC patients with AR-V7, complete the development of, and commercialize, galeterone for these patients and other indications and patient populations and develop or commercialize any future product candidates. If we are unable to raise capital when needed or on acceptable terms, we may be forced to delay, reduce, terminate or eliminate our product development programs and our commercialization efforts.

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

- the progress and results of our planned pivotal Phase 3 clinical trial of galeterone for the treatment of prostate cancer in metastatic CRPC treatment-naïve patients with AR-V7, and our efforts to complete the clinical development of galeterone and submit an NDA to the FDA for this indication;
- the progress and results of our ongoing ARMOR2 trial and any additional clinical trials of galeterone that we decide to conduct for the treatment of other indications and patient populations in prostate cancer, including for early-stage prostate cancer, and for the treatment of prostate cancer in combination with currently marketed prostate cancer therapies and novel targeted agents;
- the timing and outcome of regulatory review of galeterone for the treatment of prostate cancer in CRPC patients with AR-V7 and other indications and patient populations, and of any other future product candidates;
- the costs under agreements with third parties to develop an in vitro companion diagnostic test for identifying CRPC patients with AR-V7;
- the cost of commercialization activities, including product sales, marketing, manufacturing and distribution, for galeterone and our future product candidates for which we receive regulatory approval;
- the development of future product candidates, including our plans to seek to acquire or in-license additional compounds or technologies for the treatment of hormonally-driven diseases;
- revenue, if any, received from commercial sales of galeterone and any future product candidates, should any of our product candidates be approved by the FDA or a similar regulatory authority outside the United States;
- our ability to establish collaborations on favorable terms, if at all, particularly arrangements to develop, market and distribute galeterone and any future product candidates outside the United States; and
- the cost of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims.

Conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory approval and achieve product sales. In addition, galeterone and any future product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of products that we do not expect to be commercially available for several years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Additional financing may not be available to us on acceptable terms, or at all.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations, require us to relinquish rights to our technologies or product candidates or divert our management's attention from our operating activities.

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, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements. We will require substantial funding to fund our development and commercialization efforts, operating expenses and other activities. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our stockholders' ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our stockholders' rights as a stockholder. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through marketing and distribution arrangements or other collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or current or future product candidates or to grant licenses on terms that may not be favorable to us. Additional fundraising efforts may also divert our management from their day-to-day activities, which may compromise our ability to develop and commercialize our product candidates.

Risks Related to the Development and Regulatory Approval of Galeterone and Our Future Product Candidates

We depend heavily on the success of our lead product candidate, galeterone, which is in clinical development for the treatment of CRPC patients. Any failure to successfully develop galeterone for these patients or for other indications or patient populations, or any future product candidates, or significant delays in doing so, would compromise our ability to generate revenue and become profitable.

We currently have no products approved for sale and have only one product candidate, galeterone, in clinical development. We have invested substantially all of our efforts and financial resources in the development of galeterone for the treatment of CRPC. We anticipate initiating a pivotal Phase 3 clinical trial of galeterone in the first half of 2015. Our ability to generate product revenues, which we do not expect will occur for at least the next several years, if ever, will depend heavily on the successful development and commercialization of galeterone for CRPC patients with AR-V7. We also may develop galeterone for other indications or patient populations in prostate cancer or for the treatment of other hormonally-driven diseases that are associated with the androgen receptor signaling pathway and compounds that are designed to disrupt androgen receptor signaling through enhanced androgen receptor degradation. The success of galeterone or other product candidates will depend on several factors, including the following:

- successfully completing clinical trials, including obtaining clinical results that are statistically significant as well as clinically meaningful in the context of the indications for which we are developing galeterone and our future product candidates;
- receiving marketing approvals for our products from the FDA and similar regulatory authorities outside the United States;
- successfully developing an in vitro companion diagnostic test to identify CRPC patients with AR-V7;
- making arrangements with third-party manufacturers for, or establishing, commercial manufacturing capabilities;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity for galeterone or other product candidates, both in the United States and internationally;
- establishing successful sales and marketing arrangements and launching commercial sales of our products, if and when approved, whether alone or in collaboration with others;
- obtaining commercial acceptance of our products, if and when approved, by patients, the medical community and third-party payors;
- obtaining and maintaining adequate reimbursement;
- effectively competing with other therapies;
- protecting our rights in our intellectual property portfolio; and
- maintaining a continued acceptable safety profile of our products following regulatory approval.

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize galeterone and our future product candidates, which would materially harm our business.

If clinical trials of galeterone and our future product candidates, including our ongoing Phase 2 clinical trial and our planned pivotal Phase 3 clinical trial of galeterone, fail to demonstrate safety and efficacy to the satisfaction of the FDA or similar regulatory authorities outside the United States or are not otherwise successful, we may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of galeterone and our future product candidates.

Before obtaining regulatory approval for the sale of galeterone and our future product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. A failure of one or more of our clinical trials can occur at any stage of testing. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval for their product candidates.

We are currently finalizing our plans for our pivotal Phase 3 clinical trial of galeterone. We anticipate initiating our planned pivotal Phase 3 clinical trial in the first half of 2015 and having top-line data from the trial by the end of 2016. However, we may not initiate the trial unless and until we develop an analytically validated assay to detect AR-V7 and submit an investigational device exemption application, or IDE, for the assay to the FDA. In addition, our anticipated time to top-line data is subject to the rates of patient enrollment and disease progression in the trial. The rate of patient enrollment in the trial, however, is difficult to predict as we have no experience recruiting patients with AR-V7 for a clinical trial, and the percentage of CRPC patients with AR-V7 is subject to widely varying projections in published literature. Moreover, because we have not previously conducted a clinical trial of galeterone in patients with AR-V7 and clinical trials of Xtandi in AR-V7 have only been conducted in a limited number of patients, our assumption concerning rates of disease progression could be incorrect. As a result, there can be no assurance that we will initiate, have top-line data from or complete the trial when we anticipate.

For drug and biological products, the FDA typically requires the successful completion of two adequate and well-controlled clinical trials to support marketing approval. In the case of galeterone, we intend to seek approval based upon the results of a single pivotal clinical trial. If the results of the trial are not robust, are subject to confounding factors, or are not adequately supported by other study endpoints, the FDA may refuse to approve galeterone based upon a single clinical trial. Thus there can be no guarantee that the FDA will not require additional pivotal clinical trials as a condition for approving galeterone.

In August 2014, we met with the FDA to discuss our plans for a pivotal Phase 3 clinical trial to support initial new drug approval by the FDA. Based on these discussions, we expect that our ARMOR3-SV trial will be a randomized, open label clinical trial comparing galeterone to Xtandi in 170 or fewer metastatic CRPC treatment-naïve patients whose prostate tumors express the AR-V7 splice variant. The primary endpoint of the trial will be radiographic progression-free survival and the secondary endpoints of the trial will include reduction of prostate specific antigen, or PSA, levels, overall survival and safety. We have not conducted any clinical trials of galeterone for patients with AR-V7, comparing galeterone to a comparator drug or using a primary endpoint of radiographic progression-free survival. As a result, the results of the clinical trials that we have conducted may not be predictive of the outcome of our ARMOR3-SV trial.

Moreover, we are unaware of any completed or currently ongoing pivotal trials of treatments for prostate cancer for which the sole primary endpoint to support initial FDA drug approval was radiographic progression-free survival. As a result, we cannot be assured as to how the FDA will interpret any radiographic progression-free survival data that we generate in our ARMOR3-SV trial. In connection with our August 2014 meeting with the FDA, the FDA advised us that in its view radiographic progression-free survival and the use of radiographic progression-free survival in the metastatic CRPC context is limited by difficulties in bone scan interpretation and the complexity of the criteria used to define progression, each of which creates uncertainty as to the ability of radiographic progression-free survival to predict improvements in morbidity or mortality. The FDA also advised us that if we used radiographic progression-free survival as the sole primary endpoint, this uncertainty would need to be overcome by a statistically persuasive large relative and absolute magnitude of improvement in radiographic progression-free survival as well as internal consistency across secondary endpoints, including a supportive result in overall survival.

If we are required to conduct additional clinical trials or other testing of galeterone or of our future product candidates beyond those that we currently contemplate, if we are unable to successfully complete clinical trials of our product candidates or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- be delayed in obtaining marketing approval for galeterone or our future 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 restrictions or safety warnings, including boxed warnings;
- be subject to additional post-marketing testing requirements;
- be subject to restrictions on how the product is distributed or used; or
- have the product removed from the market after obtaining marketing approval.

If we experience any of a number of possible unforeseen events in connection with our preclinical studies or clinical trials, our ability to conduct further clinical trials of, obtain regulatory approval of or commercialize galeterone or our future product candidates could be delayed or prevented.

We may experience numerous unforeseen events during, or as a result of, preclinical studies or clinical trials that could delay or prevent our ability to conduct further clinical trials, obtain regulatory approval or commercialization of galeterone or our future product candidates. For instance, we experienced delays following our open label, dose escalation Phase 1 clinical trial of galeterone, which we refer to as our ARMOR1 trial, due to the exposure variability associated with the food effect of administering galeterone in capsule formulation and our efforts to reformulate galeterone, which resulted in the development of the spray dried dispersion formulation of galeterone and required us to conduct additional Phase 1 clinical trials. Unforeseen events that could delay or prevent our ability to conduct clinical trials, obtain regulatory approval or commercialize galeterone and our future product candidates include:

- regulators or institutional review boards may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- we may have delays in reaching or fail to reach agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites;
- preclinical studies and clinical trials of galeterone or our future product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional preclinical or clinical trials or abandon product development programs;
- the number of patients required for clinical trials of galeterone or our future product candidates may be larger than we anticipate, patient enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials at a higher rate than we anticipate;
- our failure to conduct our clinical trials in accordance with the FDA's good clinical practices or applicable regulatory requirements in other countries;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- we may decide, or regulators or institutional review boards may require us or our investigators to, suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements,
- a finding that the participants are being exposed to unacceptable health risks or the occurrence of serious adverse events associated with galeterone or our future product candidates;
- the cost of clinical trials of galeterone and our future product candidates may be greater than we anticipate; and
- the supply or quality of galeterone or our future product candidates or other materials necessary to conduct clinical trials of such product candidates may be insufficient or inadequate.

In addition, the patients recruited for clinical trials of our product candidates may have characteristics that are different than we expect and different than the clinical trials were designed for, which could adversely impact the results of the clinical trials. For example, our patients could develop genetic mutations that are not responsive or are otherwise resistant to galeterone.

Our product development costs will also increase if we experience delays in testing or obtaining marketing approvals. We do not know whether any clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. In addition to additional costs, significant clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do and impair our ability to commercialize our product candidates.

Galeterone could ultimately prove to be ineffective or unsafe.

As of October 31, 2014, we have administered galeterone to over 250 prostate cancer patients and healthy volunteers in Phase 1 and Phase 2 clinical trials. We are currently conducting our ARMOR2 trial. As of October 31, 2014, we had enrolled 121 patients in the trial. However, we have yet to fully explore the safety and efficacy of galeterone. Ultimately, the results of our clinical trials to date, in which galeterone has been well tolerated and showed clinically meaningful reductions in levels of PSA, a biochemical marker used to evaluate prostate cancer patients for signs of response to therapy, may prove to be incorrect. No assessment of the efficacy, safety or side effects of a product candidate can be considered complete until all clinical trials needed to support a submission for marketing approval are complete, and success in early-stage clinical trials does not mean that subsequent trials will confirm the earlier findings, or that experience with use of a product in large-scale commercial distribution will not identify additional safety or efficacy issues. If we find that galeterone is not safe, or if its efficacy cannot be consistently demonstrated, we may not be able to commercialize, or may be required to cease distribution of, the product. Galeterone may also prove to be substantially identical or inferior to drugs already available, in which case the market for galeterone would be reduced or eliminated.

We anticipate initiating a pivotal Phase 3 clinical trial of galeterone in metastatic CRPC treatment-naïve patients with AR-V7 in the first half of 2015. We believe that patients' prostate tumor cells may not be responsive to treatment with Zytiga and Xtandi in the presence of C-terminal loss, including AR-V7, but that galeterone, with its mechanism of androgen receptor degradation, may effectively treat these patients. There can be no assurance, however, that our beliefs and assumptions about the effectiveness of galeterone, Zytiga (abiraterone acetate) or Xtandi (enzalutamide) in the treatment of CRPC patients with C-terminal loss or AR-V7 are accurate. Our belief that patients' prostate tumor cells may not be responsive to treatment with Zytiga and Xtandi in the presence of C-terminal loss or AR-V7 is based on our understanding of the mechanisms of action of these products, data from clinical studies conducted by MD Anderson Cancer Center, or MD Anderson, Johns Hopkins University, or Johns Hopkins, and Memorial Sloan Kettering Cancer Center, or Sloan Kettering, and data from preclinical studies conducted by us and independent laboratories. However, the clinical studies conducted by MD Anderson, Johns Hopkins and Sloan Kettering only involved a limited number of patients with C-terminal loss or AR-V7 and were conducted in different patient populations, using different protocols and using different and unvalidated assays to identify patients with C-terminal loss or AR-V7. The patient populations, protocols and assays used in the MD Anderson, Johns Hopkins and Sloan Kettering studies may also differ from the patient populations, protocols and assays used in our planned pivotal Phase 3 clinical trial. In addition, it is possible that other factors were present that caused, or contributed to, the poor responsiveness of Zytiga and Xtandi in the presence of C-terminal loss and AR-V7 in the clinical studies. The outcome of preclinical testing and clinical studies may not be predictive of the success of later clinical trials and is often susceptible to varying interpretations and analyses. If Zytiga and Xtandi are found to be more responsive to C-terminal loss or AR-V7 than we anticipate, any clinical trial designed to compare galeterone to Zytiga and Xtandi for this patient population would be less likely to succeed.

Our belief that galeterone may be effective in CRPC patients with C-terminal loss, including AR-V7, is based on data from preclinical studies and a retrospective subset analysis in which seven treatment-naïve CRPC patients in our ARMOR2 trial were identified as having truncated androgen receptors with C-terminal loss pursuant to an unvalidated assay. We believe that these data support our view that galeterone may be effective in patients without an intact ligand binding domain. However, there can be no assurance that these data will be predictive of the success of our planned pivotal Phase 3 clinical trial of galeterone. While we are still finalizing the design of the planned pivotal Phase 3 clinical trial, the trial will be the first clinical trial to evaluate galeterone in prospectively identified patients with AR-V7 and will have a design that is different than the design of our ARMOR2 trial, including primary endpoints that, unlike our ARMOR2 trial, are not based on PSA. The failure of our planned pivotal Phase 3 clinical trial of galeterone in this patient population would have a material adverse impact on our ability to obtain approval for galeterone and on our business, financial condition and prospects.

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

We may not be able to continue our ARMOR2 trial or conduct our planned pivotal Phase 3 clinical trial, or any other clinical trials, if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the FDA or similar regulatory authorities outside the United States.

Our competitors may have ongoing clinical trials for product candidates that could be competitive with galeterone and our future product candidates, and patients who would otherwise be eligible for our clinical trials may instead enroll in clinical trials of our competitors' product candidates.

We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics to achieve diversity in a trial, to complete our clinical trials in a timely manner. Patient enrollment is affected by other factors including:

- severity of the disease under investigation;
- design of the trial protocol;
- eligibility criteria for the study in question;
- perceived risks and benefits of the product candidate under study;
- trials of other products for similar indications;
- efforts to facilitate timely patient enrollment in clinical trials;
- patient referral practices of physicians;
- alternative products for similar indications;
- the ability to monitor patients adequately during and after treatment; and
- proximity and availability of clinical trial sites for prospective patients.

In particular, because we expect that our planned pivotal Phase 3 clinical trial of galeterone will be focused on CRPC patients with AR-V7, which we expect represents a small percentage of CRPC patients, our ability to enroll eligible patients may be limited or may result in slower enrollment than we anticipate. We expect that we may need to screen more than 1,000 patients to identify and enroll the target AR-V7 positive patients. However, because we have no experience recruiting patients with AR-V7 for a clinical trial and the percentage of CRPC patients with AR-V7 is subject to widely varying projections in published literature, we cannot be assured our projections for enrollment are accurate. Patient enrollment in our planned pivotal Phase 3 clinical trial may also be adversely affected by data that show little or no activity of Xtandi in patients with AR-V7 as patients in the trial will be randomized to the Xtandi arm and the trial will not provide for crossover to galeterone. Patient enrollment delays in our planned pivotal Phase 3 clinical trial or any of our other future clinical trials may result in increased development costs for our product candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing. Our inability to enroll a sufficient number of patients for our planned pivotal Phase 3 clinical trial would result in significant delays. Any significant delays or increases in costs of our planned pivotal Phase 3 clinical trial could result in the need for us to obtain additional funding to complete the trial.

In addition, patients enrolled in our clinical trials may discontinue their participation at any time during the trial as a result of a number of factors, including experiencing adverse clinical events that may or may not be associated with our product candidates under evaluation. We are aware that other late stage trials in CRPC have been adversely affected by discontinuations by patients who prematurely leave the trial in response to an increase in their PSA levels during the trial. The discontinuation of patients in any one of our trials may cause us to delay or abandon our clinical trial or may lead to negative or insufficient results to support a filing for marketing and regulatory approval of the applicable product candidate.

If serious adverse or unforeseen side effects are identified during the development of galeterone or our future product candidates, we may need to abandon or limit our development of some or all of our product candidates.

If galeterone or our future product candidates are associated with undesirable side effects or have characteristics that are unexpected, we may need to abandon their development or limit development to certain indications or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. Adverse or unexpected side effects or characteristics of galeterone, whether discovered by us or independently publicized by third parties during clinical trials, could cause an institutional review board or regulatory authorities to interrupt, delay or halt clinical trials of galeterone or our future product candidates, require us to conduct additional clinical trials or other tests or studies, and could result in a more restrictive label, or the delay or denial of marketing approval by the FDA or comparable foreign regulatory authorities.

In our ARMOR2 trial, there were three unexpected serious adverse events that were assessed by the investigators as possibly related to treatment with galeterone. These treatment-related serious adverse events involved a case of angioedema in a patient who was taking a medication associated with angioedema, an episode of dizziness and fainting in a patient with a history of nausea, diabetes and hypertension who had discontinued treatment with galeterone four days prior to the episode, and a case of hypocalcemia and hyperparathyroidism in a patient with a history of hypocalcemia and hyperparathyroidism. In our ARMOR1 trial, there was only one unexpected serious adverse event assessed by the investigator as possibly related to treatment with galeterone. This treatment-related serious adverse event involved a 77-year old patient who developed rhabdomyolysis, an acute disintegration of muscle tissue, and acute renal failure that occurred while receiving simvastatin, a statin known to be associated with rhabdomyolysis. In addition, the patient had underlying chronic renal insufficiency, renal artery stenosis and hydronephrosis requiring stents and presented after a fall, all of which are risk factors for either acute renal failure or rhabdomyolysis. To date, none of these events resulted in interruptions or delays of our clinical trials.

In order to develop and commercialize galeterone for the treatment of CRPC patients with AR-V7, we will need to develop and commercialize as an in vitro companion diagnostic test an analytically validated assay that can be used to identify CRPC patients with AR-V7. If this assay is unable to be developed, or if there are significant delays in doing so, our planned pivotal Phase 3 clinical trial and the development of galeterone may be delayed, and we may not achieve marketing approval or realize the full commercial potential of galeterone.

We will need to develop an analytically validated assay that sensitively detects AR-V7 in order to proceed with our planned pivotal Phase 3 clinical trial and seek approval of, and commercialize, galeterone for patients with these types of truncated androgen receptors. We plan to contract with third parties to develop the assay for the trial and as an *in vitro* companion diagnostic test and to use widely available methodologies and technologies, if possible, in order to minimize development and regulatory risks. We are currently finalizing our strategy for developing this assay. We have discussed with the FDA our development strategy and plans for identifying AR-V7 in our pivotal Phase 3 clinical trial, including our plans to develop the assay as an *in vitro* companion diagnostic test. Based on our discussions with the FDA, we will need to develop the assay and submit an IDE for the assay to the FDA before we screen patients in the trial.

We do not have experience or capabilities in developing, obtaining regulatory approval, or commercializing companion diagnostic tests and would need to rely in large part on third parties to perform these functions. Companion diagnostic tests are subject to regulation by the FDA and similar regulatory authorities outside of the United States as medical devices and require separate regulatory approval prior to commercialization. We and these third parties may encounter difficulties in developing and obtaining approval for the *in vitro* companion diagnostic test, including issues relating to selectivity/specificity, analytical validation, reproducibility or clinical validation.

If we or any of the third parties we engage to assist us are unable to successfully develop and obtain approval of an *in vitro* companion diagnostic test, or experience delays in doing so:

- the development of galeterone for use by CRPC patients with AR-V7 will be adversely affected if we are unable to appropriately select patients for enrollment in our clinical trials;
- galeterone may not receive marketing approval on a timely basis or at all; and
- we will not realize the full commercial potential of galeterone if, among other reasons, we are unable to appropriately identify patients with AR-V7.

If any of these events were to occur, our business would be materially harmed.

If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we will not be able to commercialize galeterone, and our ability to generate revenue will be materially impaired.

Failure to obtain regulatory approval for galeterone for CRPC patients with AR-V7 or other indications and patient populations will prevent us from commercializing galeterone for those indications. Although our management team has experience filing and supporting applications necessary to gain regulatory approvals, we have yet to file for or obtain regulatory approval to market galeterone in any jurisdiction. Securing FDA approval requires the submission of extensive preclinical and clinical data and supporting information to the FDA for each therapeutic indication to establish galeterone's safety and efficacy. Securing FDA approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the FDA. Galeterone may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining regulatory approval or prevent or limit commercial use.

The process of obtaining regulatory approvals, both in the United States and abroad, is expensive, may take many years if additional clinical trials are required, 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 candidate involved. Changes in regulatory approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. The FDA has substantial discretion in the approval process and may refuse to accept any application or may decide that our data is 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 regulatory approval of galeterone. Any regulatory approval we ultimately obtain may be limited or subject to restrictions or post-approval commitments that render galeterone commercially unviable.

If we experience delays in obtaining approval or if we fail to obtain approval of galeterone, the commercial prospects for galeterone may be harmed and our ability to generate revenues will be materially impaired.

Even if we complete the necessary clinical trials, we cannot predict when or if we will obtain regulatory approval to commercialize galeterone or our future product candidates or the approval may be for a more narrow indication than we expect.

Even if galeterone or our future product candidates demonstrate safety and efficacy in clinical trials, regulatory agencies may not complete their review processes in a timely manner or grant regulatory approval at all. Additional delays may result if a regulatory authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory agency policy during the period of product development, clinical trials and the review process. Regulatory agencies also may approve a treatment candidate for fewer or more limited indications than requested or may grant approval subject to the performance of post-marketing studies. In addition, regulatory agencies may not approve the labeling claims that are necessary or desirable for the successful commercialization of our treatment candidates.

We have obtained fast track designation from the FDA for galeterone for the treatment of metastatic CRPC. However, fast track designation may not actually lead to a faster development, regulatory review or approval process.

If a product is intended for the treatment of a serious or life-threatening condition and the product candidate demonstrates the potential to address unmet needs for this condition, the treatment sponsor may apply for FDA fast track designation. If the fast track designation is obtained, the FDA may initiate review of sections of an NDA, before the application is complete. This "rolling review" is available if the applicant provides, and the FDA approves, a schedule for submission of the individual sections of the application. In June 2012, the FDA notified us that we had obtained fast track designation for galeterone for the treatment of metastatic CRPC. Fast track designation does not ensure that we will experience a faster development, regulatory review or approval process compared to conventional FDA procedures or that we will ultimately obtain regulatory approval of galeterone. Additionally, the FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from our clinical development program.

In the event we receive FDA approval for galeterone for CRPC patients with AR-V7, we will not be able to expand the indications for which galeterone is approved unless we receive FDA approval for each additional indication. Failure to expand these indications will limit the size of the commercial market for galeterone.

We are focusing our initial development of galeterone on the treatment of CRPC patients whose prostate tumor cells express an altered androgen receptor and plan to seek marketing and regulatory approvals for galeterone for this patient population. We also plan to develop galeterone for the treatment of other indications and patient populations in prostate cancer, including early-stage prostate cancer, and the treatment of prostate cancer in combination with currently marketed prostate cancer therapies and novel targeted agents. In addition, we plan to explore the use of galeterone for the treatment of other hormonally-driven diseases that are associated with the androgen receptor signaling pathway. In order to market and sell galeterone in the U.S. for these additional indications, we will need to conduct additional clinical trials and obtain FDA approval for each proposed indication. There can be no assurance that we will be successful in obtaining FDA approval for additional indications for the use of galeterone. If we are unsuccessful in expanding the approved indications for the use of galeterone, the size of the commercial market for galeterone will be limited.

Failure to obtain regulatory approval in international jurisdictions would prevent galeterone or our future product candidates from being marketed abroad.

In order to market and sell our products in jurisdictions outside the United States, we or third parties must obtain separate regulatory approvals and comply with numerous and varying regulatory requirements. The approval process varies among countries and can involve additional testing. The time required to obtain foreign approval may differ substantially from that required to obtain FDA approval. The regulatory 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 separately approved for reimbursement before the product can be approved for sale in that country. We intend to enter into arrangements with third parties under which they would market our products outside the United States. We or these third parties 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. We may not be able to file for regulatory approvals and may not receive necessary approvals to commercialize our products in any market.

Risks Related to the Commercialization of Our Product Candidates

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

We have never commercialized a product candidate. Our operations to date have been limited to financing and staffing our company, developing our product candidates and conducting our preclinical studies and clinical trials. We have not completed a pivotal clinical trial, obtained marketing approvals or conducted 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 history of successfully developing and commercializing pharmaceutical products.

We may also encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. In the future, we will need to transition from a company with a preclinical and clinical development focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

Even if galeterone receives regulatory approval, it may fail to achieve the degree of market acceptance by physicians, patients, healthcare payors and others in the medical community necessary for commercial success.

Even if galeterone receives marketing approval, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, healthcare payors and others in the medical community. If galeterone does not achieve an adequate level of acceptance, we may not generate significant product revenues and we may not become profitable. The degree of market acceptance of galeterone or any of our future product candidates, if approved for commercial sale, will depend on a number of factors, including:

- efficacy and potential advantages compared to alternative treatments;
- the ability to offer galeterone and our future product candidates for sale at competitive prices;
- convenience and ease of administration compared to alternative treatments;
- the strength of sales, marketing and distribution support;
- the approval of other products for the same indications;
- combinations of existing or newly approved products that alter the standard of care;
- 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;
- clinical indications for which the product is approved; and
- the prevalence and severity of any side effects.

Even if a potential product candidate displays a favorable efficacy and safety profile in preclinical studies and clinical trials, market acceptance of the product will not be known until after it is launched. Our efforts to educate the medical community, patients and third-party payors on the benefits of galeterone or our other future product candidates may require significant resources and may never be successful.

If galeterone or any of our future product candidates receives marketing approval and we, or others, later discover that the product is less effective than previously believed or causes undesirable side effects that were not previously identified, our ability to market the product could be compromised.

Clinical trials 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 in a broader patient population or alternatively fail to identify undesirable side effects. If, following approval of a product candidate, we, or others, discover that the product 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 product or seize the product;
- we may be required to recall the product or change the way the product is administered;
- additional restrictions may be imposed on the marketing of, or the manufacturing processes for, the particular product;
- regulatory authorities may require the addition of labeling statements, such as a "black box" warning or a contraindication;
- we may be required to create a Medication Guide outlining the risks of the previously unidentified side effects for distribution to patients;
- additional restrictions may be imposed on the distribution or use of the product via a Risk Evaluation and Mitigation Strategy, or REMS;
- we could be sued and held liable for harm caused to patients;
- the product 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 and marketing capabilities or enter into agreements with third parties to sell and market our product candidates, we may not be successful in commercializing galeterone or any of our future product candidates if they are approved.

We do not have a sales or marketing 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 outsource these functions to third parties or develop an internal sales and marketing organization. If galeterone is approved in the United States, we intend to build a urology and oncology focused, specialty sales organization in the United States to support the commercialization of galeterone. We intend to commercialize galeterone outside the United States through collaborations with third parties. Such reliance on third parties to market our products, if approved, is risky as these parties may not perform satisfactorily or at all.

There are risks involved with both entering into arrangements with third parties to perform these services and establishing our own sales and marketing capabilities, neither of which we have pursued previously. For example, recruiting and training a sales force is expensive and 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 capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retrain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize our products on our own include:

- our inability to recruit and retain an adequate number of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to or persuade adequate numbers of physicians to prescribe any future products;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we enter into arrangements with third parties to perform sales, marketing and distribution services, our product revenues or the profitability of these products are likely to be lower than if we were to market and sell any products that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell and market galeterone or our future product candidates or doing so on terms that are favorable to us. We likely will have little 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 successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing galeterone or our future product candidates.

We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do.

The development and commercialization of new drug products is highly competitive. Research and discoveries by others may result in breakthroughs which may render our products obsolete even before they generate any revenue. We face competition with respect to our lead product candidate, and will face competition with respect to any products that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. There are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of the disease indications for which we are developing galeterone. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

Some of these competitive products and therapies are based on scientific approaches that are the same as or similar to our approach, and others are based on entirely different approaches. Many marketed therapies for the indications that we are currently pursuing, or indications that we may in the future seek to address, are widely accepted by physicians, patients and payors, which may make it difficult for us to replace them with any products that we successfully develop and are permitted to market.

We are focusing our initial development of galeterone on the treatment of CRPC patients whose prostate tumor cells express an altered androgen receptor that is truncated. Based on their mechanisms of action, preclinical data and the data from the MD Anderson, Johns Hopkins and Sloan Kettering trials, we believe that Zytiga and Xtandi may be less responsive in this patient population and do not expect that other drugs in development with similar mechanisms of action will be responsive in this patient population. However, we expect that other drugs with alternative mechanisms of action may be developed for the treatment of this patient population.

We believe that galeterone may be well suited to treat other prostate cancer patient populations. If galeterone is approved for additional indications, it may compete with other secondary hormonal treatments currently being marketed, such as Zytiga and Xtandi, or with secondary hormonal treatment drug candidates currently in development, such as ARN-509 and ODM-201. Galeterone could compete in the future with products, including secondary hormonal treatments, some of which are marketed by several of the world's largest and most experienced pharmaceutical companies, who have substantially more financial resources than us and greater flexibility to engage in aggressive price competition to gain revenues and market share. Approved secondary hormonal treatments in the United States for CRPC include Zytiga, marketed by Janssen Biotech, Inc. and Xtandi, marketed by Astellas Pharma US, Inc. and Medivation, Inc. Approved non-hormonal agents for CRPC include Taxotere® (docetaxel) and Jevtana® (cabazitaxel), marketed by sanofi-aventis U.S. LLC; Provenge® (sipuleucel-T), marketed by Dendreon Corporation; and Xofigo® (radium-223), marketed by Bayer HealthCare Pharmaceuticals, Inc. It is uncertain whether we could compete with such products, and our failure to compete or decision to reduce the price of galeterone or other future products we may develop in order to compete could severely impact our business.

In addition, there are numerous prostate cancer products in clinical development by many public and private biotechnology and pharmaceutical companies targeting numerous different cancer types. A number of these are in late stage development. These include secondary hormonal treatments such as Johnson & Johnson's ARN-509 and Orion Corporation's ODM-201. Other compounds that are not secondary hormonal treatments in clinical development include Exelixis, Inc.'s Cometriq and Bavarian Nordic A/S's Prostvac. If a therapy for prostate cancer were developed that targeted the C-terminal loss or AR-V7 patient populations or altered the standard of care for the treatment of CRPC, such therapy could render galeterone irrelevant.

Our competitors may develop products that are more effective, safer, more convenient or less costly than any that we are developing or that would render galeterone or any future product candidates obsolete or non-competitive. Our competitors may also obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours.

Many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific, medical and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

Even if we are able to commercialize galeterone or any other future product candidates, the products may become subject to unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives.

The regulations that govern marketing approvals, pricing and reimbursement for new drug products vary widely from country to country. In the United States, recently passed legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. 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 might obtain regulatory approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and 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 to recoup our investment in galeterone or our future product candidates, even if our product candidates obtain regulatory approval.

Our ability to commercialize any products successfully also will depend in part on the extent to which 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 pay for and establish reimbursement levels. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that reimbursement will be available for any product that we commercialize and, if reimbursement is available, the level of reimbursement. Reimbursement may impact the demand for, or the price of, any product candidate for which we receive marketing approval. If reimbursement is not available or is available only to limited levels, we may not be able to successfully commercialize any product candidate for which we receive marketing approval.

There may be significant delays in obtaining reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the drug is approved by the FDA or similar regulatory authorities outside the United States. 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. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs, and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. Third- party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. Our inability to promptly obtain coverage and profitable payment rates from both government-funded and private payors for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

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

We face an inherent risk of product liability exposure related to the testing of galeterone and our future product candidates in human clinical trials and will face an even greater risk if we commercially sell any products that we may develop. Galeterone has not been widely used over an extended period of time, and therefore our safety data are limited.

If we cannot successfully defend ourselves against claims that galeterone or future product candidates or products we may develop caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any 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 the related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue; and
- the inability to commercialize any products that we may develop.

We currently hold $5 million in product liability insurance coverage, which may not be adequate to cover all liabilities that we may incur. We will need to increase our insurance coverage when we begin commercializing galeterone and our future product candidates, if ever. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

Risks Related to Our Dependence on Third Parties

We rely on third parties to conduct our clinical trials, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials.

We do not independently conduct clinical trials of our product candidates. We rely on third parties, such as contract research organizations, clinical data management organizations, medical institutions and clinical investigators, to perform this function. Our reliance on these third parties for clinical development activities reduces our control over these activities but does not relieve us of our responsibilities. 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 Good Clinical Practices, 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. 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 will not be able to obtain, or may be delayed in obtaining, regulatory approvals for galeterone or other product candidates we may develop in the future and will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates.

We may enter into collaborations with third parties for the development and commercialization of galeterone and future product candidates we may develop. If those collaborations are not successful, we may not be able to capitalize on the market potential of these product candidates.

We may enter into collaborations with third parties for the development and commercialization of galeterone and future product candidates we may develop. Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner, or at all. We will likely have limited control under any additional arrangements we may enter into with third parties 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.

Collaborations involving our product candidates would pose the following risks to us:

- collaborators may have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- 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 collaborator's strategic focus or available funding, or external factors such as an acquisition that diverts resources or creates competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products or product candidates if the collaborators believe that competitive products
- are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- collaborators 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;
- collaborators may have the right to conduct clinical trials of our product candidates without our consent and could conduct trials with flawed designs that result in data that adversely affect our clinical trials, our ability to obtain marketing approval for our product candidates or market acceptance of our product candidates;
- 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 proprietary information or expose us to potential litigation;
- disputes may arise between the collaborators and us that result in the delay or termination of the research, development or commercialization of our products or product candidates or that result in costly litigation or arbitration that diverts management attention and resources; 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.

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. If a present or future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program could be delayed, diminished or terminated.

If we are not able to establish collaborations, we may have to alter our development and commercialization plans.

We will face significant competition in seeking appropriate collaborators if we determine to do so. Whether we reach a definitive agreement for a collaboration will depend upon, among other things, 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. Such factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge, and industry and market conditions generally. 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 galeterone. We may also be restricted under existing license agreements from entering into agreements on certain terms with potential collaborators. Collaborations are complex and time-consuming to negotiate and document. We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all.

If we are not able to obtain such funding or enter into collaborations for galeterone, we may have to curtail the development of galeterone, reduce or delay our 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 galeterone or other future candidates or bring these product candidates to market and generate product revenue.

Failure of third parties to successfully develop or commercialize an in vitro companion diagnostic test to prospectively identify prostate cancer patients with AR-V7 could harm our ability to commercialize galeterone.

We do not plan to internally develop an in vitro companion diagnostic test to prospectively identify prostate cancer patients with AR-V7 and, as a result, we will be dependent on the efforts of the third parties that we engage to successfully develop and commercialize these tests. The third parties:

- may not perform their obligations as expected;
- may encounter production difficulties that could constrain the supply of the in vitro companion diagnostic test;
- may have difficulties gaining acceptance of the use of the in vitro companion diagnostic test in the clinical community;
- may not pursue commercialization of the in vitro companion diagnostic test even if they receive any required regulatory approvals;
- may elect not to continue the development of the in vitro companion diagnostic test based on changes in the third parties' strategic focus or available funding, or external factors such as an acquisition, that divert resources or create competing priorities;
- may not commit sufficient resources to the marketing and distribution of the in vitro companion diagnostic test; and
- may terminate their relationship with us.

If the *in vitro* companion diagnostic test that is developed to prospectively identify prostate cancer patients with AR-V7 fails to gain market acceptance, our ability to derive revenues from sales from galeterone would be harmed. If the third parties we engage fail to commercialize the *in vitro* companion diagnostic test, we may not be able to enter into arrangements with another diagnostic company to obtain supplies of an alternative test for use in connection with galeterone or do so on commercially reasonable terms, which could adversely affect and delay the development or commercialization of galeterone.

If galeterone is approved, we intend to rely on third parties to perform many necessary services related to the sale and distribution of galeterone, and expect to do so for any future product candidates.

If galeterone is approved, we intend to retain third-party service providers to perform a variety of functions related to the sale and distribution of galeterone, key aspects of which are out of our direct control. For example, we intend to rely on third parties to provide key services related to logistics, warehousing and inventory management, distribution, contract administration and chargeback processing, accounts receivable management, and storage, including entrusting our inventories of galeterone to their care and handling. If these third-party service providers fail to comply with applicable laws and regulations, fail to meet expected deadlines, or otherwise do not carry out their contractual duties to us, or encounter physical damage or natural disaster at their facilities, our ability to deliver galeterone to meet commercial demand would be significantly impaired. In addition, we intend to utilize third parties to perform various other services for us relating to sample accountability and regulatory monitoring, including adverse event reporting, safety database management and other product maintenance services. If the quality or accuracy of the data maintained by these service providers is insufficient, our ability to market galeterone could be jeopardized or we could be subject to regulatory sanctions. We do not currently have the internal capacity to perform these important commercial functions, and we may not be able to maintain commercial arrangements for these services on reasonable terms.

Risks Related to the Manufacturing of Galeterone and Our Future Product Candidates

We contract with third parties for the manufacture of galeterone for clinical trials and expect to continue to do so in connection with the commercialization of galeterone and for clinical trials and commercialization of any other product candidates that we develop. 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 do not currently have nor do we plan to build the internal infrastructure or capability to manufacture galeterone. We currently rely on and expect to continue to rely on third-party contract manufacturers to manufacture clinical supplies of galeterone and any other product candidates we may develop. We expect to continue to rely upon third-party contract manufacturers to manufacture commercial quantities of galeterone and any other product candidates that we commercialize following approval for marketing by applicable regulatory authorities. Reliance on third-party manufacturers entails risks, including:

- manufacturing delays if our third-party manufacturers 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 agreement between us;
- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us;
- the possible breach of the manufacturing agreement by the third party;
- the failure of the third-party manufacturer to comply with applicable regulatory requirements; 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 for all of our required raw materials, drug substance and finished product for our 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 our clinical trials as we identify or qualify replacements.

We currently rely on a single third-party contract manufacturer, with which we do not have a long-term agreement, to supply us with the spray dried dispersion formulation of galeterone. If this third-party manufacturer fails to fulfill orders or should become unavailable to us for any reason, we likely would incur some delay in our clinical trials for galeterone and added costs and delays in identifying or qualifying such replacements. In addition, we may be unable to establish any agreements with such a replacement manufacturers or to do so on acceptable terms or at all. Even if we could transfer manufacturing to a different third party, the shift would likely be expensive and time-consuming.

If galeterone or any other product candidate that we may develop in the future is approved by any regulatory agency, we intend to enter into agreements with third-party contract manufacturers for the commercial production of those products. This process is difficult and time-consuming and 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. As a result, we may be unable to reach agreement with third-party manufacturers on satisfactory terms or at all, which could delay our commercialization.

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

If our third-party manufacturing facilities are damaged or destroyed, or production at one of these facilities is otherwise interrupted, our business and prospects would be negatively affected.

If any manufacturing facilities owned by third parties who manufacture galeterone or any of our future product candidates are damaged or destroyed, we likely would not be able to quickly or inexpensively replace our manufacturing capacity and possibly would not be able to replace it at all. Any new facility needed to replace these facilities would need to comply with the necessary regulatory requirements and need to be tailored to our specialized manufacturing requirements. We would need FDA approval before selling any products manufactured at a new facility. Such an event could delay our clinical trials or, if any of our product candidates are approved by the FDA, reduce or eliminate our product sales.

While we maintain insurance coverage to cover damage to our property and equipment and to cover business interruption and research and development restoration expenses, if we have underestimated our insurance needs with respect to an interruption in our clinical manufacturing of our product candidates, we may not be able to adequately cover our losses.

We rely on our third-party manufacturers for compliance with applicable regulatory requirements. This may increase the risk of sanctions being imposed on us or on a manufacturer of our products or product candidates, which could result in our inability to obtain sufficient quantities of these products or product candidates.

Our manufacturers may not be able to comply with cGMPs, regulations or other regulatory requirements or similar regulatory requirements outside the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including:

- fines;
- injunctions;
- civil penalties;
- failure of regulatory authorities to grant marketing approval of our product candidates;
- delays, suspension or withdrawal of approvals;
- suspension of manufacturing operations;
- license revocation;
- seizures or recalls of products or product candidates;
- operating restrictions; and
- criminal prosecutions.

Any of these sanctions could significantly and adversely affect supplies of our products and product candidates.

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

Because we rely on third parties to manufacture our product candidates, and because we collaborate with various organizations and academic institutions on the advancement of our technology, we must, at times, share trade secrets with them. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements or other similar agreements with our collaborators, advisors, employees and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, such as trade secrets. Despite these contractual provisions, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by potential competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, discovery by a third party of our trade secrets or other unauthorized use or disclosure would impair our intellectual property rights and protections in our product candidates.

In addition, these agreements typically restrict the ability of our collaborators, advisors, employees and consultants to publish data potentially relating to our trade secrets. Our academic collaborators typically have rights to publish data, provided that we are notified in advance and may delay publication for a specified time in order to secure our intellectual property rights arising from the collaboration. In other cases, publication rights are controlled exclusively by us, although in some cases we may share these rights with other parties. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of these agreements, independent development or publication of information including our trade secrets in cases where we do not have proprietary or otherwise protected rights at the time of publication.

Risks Related to Our Intellectual Property

If we fail to comply with our obligations under our intellectual property licenses with third parties, we could lose license rights that are important to our business.

We are a party to a Master License Agreement with UMB under which we license certain patents and patent applications to make, have made, use, sell, offer to sell and import certain anti-androgen compounds, including galeterone. We may enter into additional license agreements in the future. Our license agreement with UMB imposes, and we expect that future license agreements will impose, various diligence, milestone payment, royalty, insurance and other obligations on us. If we fail to comply with our obligations under these licenses, our licensors may have the right to terminate these license agreements, in which event we might not be able to market any product that is covered by these agreements, or our licensors may convert the license to a non-exclusive license, which could materially adversely affect the value of the product candidate being developed under the license agreement. Termination of these license agreements or reduction or elimination of our licensed rights may result in our having to negotiate new or reinstated licenses with less favorable terms.

Restrictions on our patent rights relating to our drug candidates may limit our ability to prevent third parties from competing against us.

As of October 31, 2014, we owned two issued U.S. patents, ten U.S. provisional and non-provisional patent applications, one issued foreign patent and 35 foreign applications in our galeterone patent portfolio]. We also had exclusive rights under our license agreement with UMB to five issued U.S. patents and 44 issued foreign patents as well as three U.S. patent applications and nine foreign applications. Our success will depend, in part, on our ability to obtain and maintain patent protection for galeterone and other product candidates, preserve our trade secrets, prevent third parties from infringing upon our proprietary rights and operate without infringing upon the proprietary rights of others.

Patent applications in the United States and most other countries are confidential for a period of time until they are published, and publication of discoveries in scientific or patent literature typically lags actual discoveries by several months or more. As a result, we cannot be certain that we and the inventors of the intellectual property for which we have submitted patent applications or in-license issued patents and applications, were the first to conceive of the inventions covered by such patents and pending patent applications or that we and those inventors were the first to file patent applications covering such inventions. Also, the patent protection of our numerous issued and pending patent applications may lapse before we manage to obtain commercial value from them, which might result in increased competition and materially affect our position in the market.

We have an exclusive license from UMB for a U.S. patent covering compositions and methods of use of a class of compounds encompassing galeterone, which expires in 2017. Given its expiration date and the anticipated timing of development and commercialization of galeterone, we do not believe this patent will provide significant protection for galeterone. We have no patent protection specifically covering the chemical structure of galeterone. As a result, a third party that obtains regulatory approval of a product with the same active ingredient as galeterone may be able to market such product so long as the third party does not infringe any other patents owned or licensed by us with respect to galeterone. For this reason, we have filed for or licensed patents and patent applications relating to galeterone covering methods of use, pharmaceutical compositions, combination treatments, prodrugs, metabolites and analogs of galeterone and their use.

Our owned and licensed patents and patent applications, if issued, are expected to expire on various dates from 2017 through 2034. Upon the expiration of these patents, we and UMB will lose the right to exclude others from practicing the inventions claimed by such patents. As a result, the expiration of these patents could have a material adverse effect on our business, results of operations, financial condition and prospects.

If we are unable to obtain and maintain patent protection for our technology and products, or if our licensors are unable to obtain and maintain patent protection for the technology or products that we license from them, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize technology and products similar or identical to ours, and our ability to successfully commercialize our technology and products may be adversely affected.

Our success depends in large part on our and UMB's ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary technology and products. We and UMB have sought to protect our proprietary position by filing patent applications in the United States and abroad related to our novel technologies and products that are important to our business. This process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development efforts before it is too late to obtain patent protection. Moreover, prior to April 10, 2012, we did not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology or products that we license from UMB, and we were and still are reliant on UMB. Therefore, we cannot be certain that these patents and applications were prosecuted in a manner consistent with the best interests of our business. If we or UMB fail to maintain such patents, or lose rights to those patents, the rights we have licensed may be reduced or eliminated.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our and UMB's patent rights are highly uncertain. Our and UMB's pending and future patent applications may not result in patents being issued which protect our technology or products or which effectively prevent others from commercializing competitive technologies and 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.

Third parties could practice our inventions in territories where we do not have patent protection. Furthermore, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States. A U.S. patent may be infringed by anyone who, without authorization, practices a patented process in the United States or imports a product made by a process covered by the U.S. patent. In foreign countries, however, importation of a product made by a process patented in that country may not constitute an infringing activity, which would limit our ability to enforce process patents against importers in that country. Furthermore, the legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents and other intellectual property protection. This could make it difficult for us to stop the infringement or misappropriation of our intellectual property rights. If competitors are able to use our technologies, our ability to compete effectively could be harmed.

Assuming the other requirements for patentability are met, in the United States, the first to invent the claimed invention is entitled to the patent, while outside the United States, the first to file a patent application is generally entitled to the patent. Under the America Invents Act, or AIA, enacted in September 2011, the United States moved to a first inventor to file system in March 2013. The United States Patent and Trademark Office recently finalized the rules relating to these changes and courts have yet to address the new provisions. These changes could increase the costs and uncertainties surrounding the prosecution of patent applications and the enforcement or defense of patent rights. Furthermore, we may become involved in interference proceedings, opposition proceedings, or other post-grant proceedings, such as reexamination or *inter partes* review proceedings, challenging our patent rights or the patent rights of others. An adverse determination in any such proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products 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.

Even if our owned and licensed 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 owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner. The issuance of a patent is not conclusive as to its 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. Such challenges, including through opposition or other post-grant proceedings, may result in patent claims being narrowed, invalidated or held unenforceable, which could limit our ability to or stop or prevent us from stopping others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. 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. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

The degree of future protection for our proprietary rights is uncertain because legal means afford only limited protection and may not adequately protect our rights or permit us to gain or keep our competitive advantage. For example:

- others may be able to make or use compounds that are similar to galeterone but that are not covered by the claims of our patents;
- the galeterone compound may become generic, and no patent protection will be available without regard to formulation or method of use;
- we or our licensors, as the case may be, may not be able to detect infringement against our owned or in-licensed patents, which may be especially difficult for manufacturing processes or formulations;
- we or our licensors, as the case may be, might not have been the first to make the inventions covered by our owned or in-licensed issued patents or pending patent applications;
- we or our licensors, as the case may be, might not have been the first to file patent applications for these inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies;
- this may be especially likely for manufacturing processes or formulations;
- it is possible that our pending patent applications will not result in issued patents;
- it is possible that our owned or in-licensed issued patents or pending patent applications are not Orange-Book eligible;
- it is possible that there are dominating patents to galeterone of which we are not aware;
- it is possible that there are prior public disclosures that could invalidate our or our licensors' inventions, as the case may be, or parts of our or their inventions of which we or they are not aware;
- it is possible that others may circumvent our owned or in-licensed patents;
- it is possible that there are unpublished applications or patent applications maintained in secrecy that may later issue with claims covering our products or technology similar to ours;
- it is possible that the U.S. government may exercise any of its statutory rights to our owned or in-licensed patents or patent applications that was developed with government funding;
- the laws of foreign countries may not protect our or our licensors', as the case may be, proprietary rights to the same extent as the laws of the United States;
- the claims of our owned or in-licensed issued patents or patent applications, if and when issued, may not cover our system or product candidates;
- our owned or in-licensed issued patents may not provide us with any competitive advantages, or may be narrowed in scope, be held invalid or unenforceable as a result of legal challenges by third parties; or
- we may not develop additional proprietary technologies for which we can obtain patent protection.

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

Competitors may infringe our patents. To counter such infringement or unauthorized use, we may be required to file infringement claims against third parties, which can be expensive and time-consuming. In addition, during an infringement proceeding, a court may decide that the patent rights we are asserting are invalid or unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation proceeding could put one or more of our patents at risk of being invalidated or interpreted narrowly. 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 this type of litigation. In addition, our licensors may have rights to file and prosecute such claims, and we are reliant on them.

Claims that galeterone or the manufacture, use or sale of galeterone infringe the patent rights of third parties could result in costly litigation or could require substantial time and money to resolve, even if litigation is avoided.

We cannot guarantee that galeterone, its manufacture, use or sale, does not and will not infringe third-party patents. Third parties might allege that we are infringing their patent rights or that we have misappropriated their trade secrets. Such third parties might resort to litigation against us. The basis of such litigation could be existing patents or patents that issue in the future.

It is also possible that we failed to identify relevant third-party patents or applications. For example, certain U.S. patent applications that will not be filed outside the United States may remain confidential until patents issue. Furthermore, patent applications in the United States and elsewhere are published approximately 18 months after the earliest filing, which is referred to as the priority date. Therefore, patent applications covering galeterone, its manufacture, use or sale, could have been filed by others without our knowledge. Additionally, pending patent applications which have been published can, subject to certain limitations, be later amended in a manner that could cover galeterone or its use.

We are aware of two issued U.S. patents having broad claims relating to a composition of matter or its use in regulating cellular differentiation or proliferation. We are also aware of certain third-party pending U.S. patent applications that have broad generic disclosures and disclosure of certain compounds possessing structural similarities to galeterone. Although we believe that it is unlikely that such applications will lead to issued claims that would cover galeterone and its use and still be valid, patent prosecution is inherently unpredictable and an application could be allowed. Based on our analyses, if any of the above third-party patents or patent applications, if issued, were asserted against us, we do not believe our proposed products or activities would be found to infringe any valid claims of these patents. If we were to challenge the validity of an issued U.S. patent in court, we would need to overcome a statutory presumption of validity that attaches to every U.S. patent. This means that in order to prevail, we would have to present clear and convincing evidence as to the invalidity of the patent's claims. There is no assurance that a court would find in our favor on questions of infringement or validity.

In order to avoid or settle potential claims with respect to any patent rights of third parties, we may choose or be required to seek a license from a third party and be required to pay license fees or royalties or both. These licenses may not be available on acceptable terms, or at all. Even if we were able to obtain a license, the rights may be non-exclusive, which could result in our competitors gaining access to the same intellectual property. Ultimately, we could be prevented from commercializing galeterone, or be forced to cease some aspect of our business operations, if, as a result of actual or threatened patent infringement claims, we are unable to enter into licenses on acceptable terms. This could harm our business significantly.

Defending against claims of patent infringement or misappropriation of trade secrets could be costly and time-consuming, regardless of the outcome. Most of our competitors are larger than we are and have substantially greater resources. They are, therefore, likely to be able to sustain the costs of complex patent or trade secret litigation longer than we could. Thus, even if we were to ultimately prevail, or to settle at an early stage, such litigation could burden us with substantial unanticipated costs. In addition, litigation or threatened litigation could result in significant demands on the time and attention of our management team, distracting them from the pursuit of other company business. In addition, the uncertainties associated with litigation could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our internal research programs, in-license needed technology, or enter into strategic partnerships that would help us bring our product candidates to market.

We may be subject to claims that our employees have wrongfully used or disclosed alleged trade secrets of their former employers.

Many of our employees were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these employees have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such employee's former employer. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

Intellectual property litigation could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities. We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to seeking patents for some of our technology and products, we also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. 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 employees, corporate collaborators, outside scientific collaborators, sponsored researchers, contract manufacturers, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants. Despite these efforts, any of these parties may breach the agreements 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, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. Moreover, our competitors may independently develop equivalent knowledge, methods and know-how. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them 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 competitive position would be harmed.

Risks Related to Legal Compliance Matters

Any product candidate for which we receive marketing approval could be subject to restrictions or withdrawal from the market and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our products, if any of them are approved.

Any product candidate for which we receive marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, advertising and promotional activities for such product, 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, cGMP requirements relating to quality control, quality assurance and corresponding maintenance of records and documents. Even if regulatory 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, or contain requirements for costly post-marketing testing and surveillance to monitor the safety or efficacy of the product. The FDA closely regulates the post-approval marketing and promotion of drugs to ensure drugs are marketed only for the approved indications and in accordance with the provisions of the approved label. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we do not market our products for their approved indications, we may be subject to enforcement action for off-label marketing.

In addition, later discovery of previously unknown problems with our products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may have adverse consequences, including:

- restrictions on such products, manufacturers or manufacturing processes;
- restrictions on the marketing of a product;
- restrictions on product distribution;
- requirements to conduct post-marketing clinical trials;
- warning 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 revenue;
- suspension or withdrawal of regulatory approvals;
- refusal to permit the import or export of our products;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

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, program exclusion, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and third-party payors play a primary role in the recommendation and prescription of any product candidates for which we receive marketing approval. Our future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute our products for which we receive marketing approval. Restrictions under applicable federal and state healthcare laws and regulations, include the following:

- 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 either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under federal and state healthcare programs such as Medicare and Medicaid;
- the federal False Claims Act imposes civil penalties, including civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;
- the federal Health Insurance Portability and Accountability Act of 1996, as amended by the Health Information Technology for Economic and Clinical Health Act, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program and also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- the federal false statements statute prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services;
- the federal transparency requirements under the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively the PPACA, require manufacturers of covered drugs, devices, biologics and medical supplies to report to the Department of Health and Human Services information related to physician payments and other transfers of value and physician ownership and investment interests; and
- analogous state laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, and some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other health care providers or marketing expenditures.

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 may 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 preempted by the federal Health Insurance Portability and Accountability Act of 1996, or 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, exclusion 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 providers or entities with whom we expect to do business are 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.

Recently enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize galeterone or other future products candidates and affect the prices we 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 galeterone or other future products candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any product candidates for which we receive marketing approval.

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or Medicare Modernization Act, changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for drug purchases by the elderly and introduced a new reimbursement methodology based on average sales prices for physician administered drugs. In addition, this legislation provided authority for limiting the number of drugs that will be covered in any therapeutic class in certain cases. Cost reduction initiatives and other provisions of this and other more recent legislation could decrease the coverage and reimbursement that is provided for any approved products. While the Medicare Modernization Act applies only to 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 Medicare Modernization Act or other more recent legislation may result in a similar reduction in payments from private payors.

In March 2010, President Obama signed into law the Health Care Reform Law, a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for health care and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. Effective October 1, 2010, the Health Care Reform Law revises the definition of "average manufacturer price" for reporting purposes, which could increase the amount of Medicaid drug rebates to states. Further, the new law imposes a significant annual fee on companies that manufacture or import branded prescription drug products. Substantial new provisions affecting compliance have also been enacted, which may affect our business practices with health care practitioners. We will not know the full effects of the Health Care Reform Law until applicable federal and state agencies issue regulations or guidance under the new law. Although it is too early to determine the effect of the Health Care Reform Law, the new law appears likely to continue the pressure on pharmaceutical pricing, especially under the Medicare program, and may also increase our regulatory burdens and operating costs.

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 galeterone or our other future products candidates, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

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 the success of 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. Our operations involve the use of hazardous and flammable materials, including chemicals and radioactive and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of 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.

Although 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, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological, hazardous or radioactive materials.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

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.

We are highly dependent on Jodie Morrison, our President and Chief Executive Officer, John McBride, our Chief Operating Officer, Karen Ferrante, our Chief Medical Officer and Head of Research and Development, and Lee Kalowski, our Chief Financial Officer, as well as the other principal members of our management and scientific teams. Although we have formal employment agreements with each of our executive officers, these agreements do not prevent our executives from terminating their employment with us at any time. We do not maintain "key person" insurance for any of our executives or other employees. The loss of the services of any of these persons could impede the achievement of our research, development and commercialization objectives.

Recruiting and retaining qualified scientific, clinical and sales and marketing personnel will also be critical to our success. We may not be able to attract and retain these personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us.

We expect to expand our research and development, manufacturing and sales and marketing capabilities, 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 research and development, manufacturing and sales and marketing. To manage our anticipated future growth, 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. The physical expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

Risks Related to Our Common Stock

Our executive officers, directors and principal stockholders maintain the ability to control all matters submitted to stockholders for approval.

Our executive officers, directors and stockholders who own more than 5% of our outstanding common stock, together with their affiliates and related persons, in the aggregate, beneficially own shares representing more than 70% of our common stock, based on the number of shares of our common stock outstanding as of October 31, 2014. As a result, if these stockholders were to choose to act together, they would be able to control 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 control the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of voting power could delay or prevent an acquisition of our company on terms that other stockholders may desire.

We believe our two largest stockholders, Apple Tree Partners and Novartis BioVentures, Ltd., in the aggregate, beneficially own shares representing more than 55% of our common stock in the aggregate, based on the number of shares of our common stock outstanding as of October 31, 2014. As a result, each of these stockholders acting individually, as well as together, may exercise significant control over our management and affairs.

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 not all members of the board are 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 stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- 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 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.

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.

An active trading market for our common stock may not be sustained, and investors may not be able to resell their shares at or above the price they paid.

Although we have listed our common stock on The NASDAQ Global Market, an active trading market for our shares 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 prices at which they acquired their shares or at the time 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.

If our stock price is volatile, purchasers of our common stock could incur substantial losses.

Our stock price is likely to be volatile. The stock market in general and the market for biotechnology companies in particular has experienced extreme volatility that has often been unrelated to the operating performance of particular companies. For example, our stock traded within a range of a high price of $30.00 and a low price of $9.67 per share for the period September 17, 2014, our first day of trading on The NASDAQ Global Market, through October 31, 2014. As a result of this volatility, our stockholders could incur substantial losses. The market price for our common stock may be influenced by many factors, including:

- results of clinical trials of galeterone and our future product candidates or those of our competitors;
- the success of competitive products or technologies;
- potential approvals of galeterone or other future product candidates for marketing by the FDA or equivalent foreign regulatory authorities or our failure to obtain such approvals;
- regulatory or legal developments in the United States and other countries;
- the results of our efforts to commercialize galeterone or other future products candidates;
- developments or disputes concerning patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to galeterone or any of our future product candidates or clinical development programs;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates or products;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors and issuance of new or changed securities analysts' reports or recommendations;
- general economic, industry and market conditions; and
- the other factors described in this "Risk Factors" section.

In addition, pharmaceutical companies have experienced significant share price volatility in recent years, and securities class action litigation often follows a decline in the market price of a company's securities. If we face such litigation, it could result in substantial costs and a diversion of management's attention and resources.

We must maintain effective internal control over financial reporting, and if we are unable to do so, the accuracy and timeliness of our financial reporting may be adversely affected, which could have a material adverse effect on our business and stock price.

We must maintain effective internal control over financial reporting in order to accurately and timely report our results of operations and financial condition. In addition, as a public company, the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, will require, among other things, that we assess the effectiveness of our disclosure controls and procedures quarterly and the effectiveness of our internal control over financial reporting at the end of each fiscal year. We anticipate being first required to issue management's annual report on internal control over financial reporting, pursuant to Section 404 of the Sarbanes-Oxley Act, in connection with issuing our consolidated financial statements as of and for the year ending December 31, 2015.

In connection with the preparation of our consolidated financial statements as of and for the year December 31, 2012 and with the audit of those financial statements, a material weakness in internal control was identified relating to our accounting for complex stockholders' equity transactions. During 2013, we engaged resources with significant financial and accounting technical experience. These additional resources have enabled us to remediate the material weakness. Based on our assessment of the impact of the additional resources, our management concluded that, as of December 31, 2013, we had remediated the material weakness in our internal control over financial reporting described above.

The rules governing the standards that must be met for our management to assess our internal control over financial reporting pursuant to Section 404 of the Sarbanes-Oxley Act are complex and require significant documentation, testing and possible remediation. These standards require that our audit committee be advised and regularly updated on management's review of internal control over financial reporting. Our management may not be able to effectively and timely implement controls and procedures that adequately respond to the increased regulatory compliance and reporting requirements that will be applicable to us as a public company. If we fail to staff our accounting and finance function adequately or maintain internal control over financial reporting adequate to meet the demands that will be placed upon us as a public company, including the requirements of the Sarbanes-Oxley Act, our business and reputation may be harmed and our stock price may decline. Furthermore, investor perceptions of us may be adversely affected, which could cause a decline in the market price of our common stock.

We will 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 public company, and particularly after we are no longer an "emerging growth company," we will incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of the Nasdaq Stock 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 in connection with our becoming, and our efforts 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. Overall, we estimate that our incremental costs resulting from operating as a public company may be between $2.0 million and $4.0 million per year. The rules and regulations associated with being a public company 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.

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, although circumstances could cause us to lose that status earlier. 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, 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 stockholder approval of any golden parachute payments not previously approved. In this prospectus, we have not included all of the executive compensation related information that would be required if we were not an emerging growth company. 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 will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be the sole source of gain for our stockholders.

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

A significant portion of our total outstanding shares are 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 drop 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 intend to sell shares, could reduce the market price of our common stock. As of October 31, 2014, we had outstanding 22,381,742 shares of common stock, including the shares that we sold in our initial public offering. Of these shares of our common stock, 7,020,000 shares are freely tradable, without restriction, in the public market, unless purchased by our affiliates. Subject to restrictions under applicable securities laws, all of the remaining 15,361,742 shares will become freely tradable on March 16, 2015 upon expiration of the lock-up agreements entered into in connection with our initial public offering. Moreover, holders of an aggregate of 14,764,034 shares of our common stock will have rights, subject to some conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. We also intend to register all shares of our common stock that we may issue under our equity compensation plans. Once we register these shares, they can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates and applicable lock-up agreements.

If equity research analysts do not publish research reports about our business or if they issue unfavorable commentary or downgrade our common stock, the price of our common stock could decline.

The trading market for our common stock will rely in part on the research and reports that equity research analysts publish about us and our business. We do not have any control over these analysts. The price of our common stock could decline if we do not obtain research analyst coverage, or one or more securities analysts downgrade our common stock or if those analysts issue other unfavorable commentary or cease publishing reports about us or our business.

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

Use of Proceeds from Registered Securities

On September 22, 2014, we completed the initial public offering of our common stock and issued and sold 6,480,000 shares of our common stock at a public offering price of $15.00 per share. In addition, on October 9, 2014, we issued and sold an additional 540,000 shares of common stock at the initial public offering price of $15.00 per share pursuant to the underwriters' partial exercise of their option to purchase additional shares of common stock.

The offer and sale of all of the shares in the offering was registered under the Securities Act pursuant to a registration statement on Form S-1 (File No. 333-198052), which was declared effective by the SEC on September 16, 2014, and a registration statement on Form S-1MEF (File No. 333-198792), which was automatically effective upon filing with the SEC on September 16, 2014. Following the sale of the shares in connection with the closing of our initial public offering, the offering terminated. The offering commenced on September 16, 2014 and did not terminate until the sale of all of the shares offered. BMO Capital Markets Corp., Stifel, Nicolaus & Company, Incorporated and William Blair & Company, L.L.C. acted as joint book-running managers of the offering, and Janney Montgomery Scott LLC acted as co-manager of the offering.

We received aggregate gross proceeds from the offering of approximately $105.3 million, or aggregate net proceeds of approximately $97.9 million after deducting underwriting discounts and commissions but before the payment of estimated offering expenses of $3.3 million. None of the underwriting discounts and commissions or other offering expenses were incurred or 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.

We have not used any of the net proceeds from the offering to make payments, directly or indirectly, to any director or officer of ours, or any of their associates, to any person owning 10 percent or more of our common stock or to any affiliate of ours. We have invested the net proceeds from the offering in money market accounts. There has been no material change in our planned use of the net proceeds from the offering as described in our final prospectus filed pursuant to Rule 424(b)(4) under the Securities Act with the SEC on September 17, 2014. There has been no material change in the planned use of the proceeds from the offering as described in our final prospectus filed pursuant to Rule 424(b)(4) under the Securities Act with the SEC on September 17, 2014.

Item 6. Exhibits.

The exhibits filed as part of this Quarterly Report on Form 10-Q are set forth on the Exhibit Index, which Exhibit Index is 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.

TOKAI PHARMACEUTICALS, INC.

Date: November 13, 2014

By: /s/ Lee H. Kalowski
Lee H. Kalowski
Chief Financial Officer
(Principal Financial and Accounting Officer)

EXHIBIT INDEX

Exhibit Number	Description of Exhibit
31.1	Certification of Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2	Certification of Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1*	Certification of Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
32.2*	Certification of Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Database
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document

* This certification will not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liability of that section. Such certification will not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act, except to the extent specifically incorporated by reference into such filing.

Exhibit 31.1

CERTIFICATIONS

I, Jodie P. Morrison, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Tokai 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 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 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: November 13, 2014

By: /s/ Jodie P. Morrison
Jodie P. Morrison
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATIONS

I, Lee H. Kalowski, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Tokai 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 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 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: November 13, 2014

By: /s/ Lee H. Kalowski
Lee H. Kalowski
Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,

AS ADOPTED PURSUANT TO

SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report on Form 10-Q of Tokai Pharmaceuticals, Inc. (the "Company") for the period ended September 30, 2014 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Jodie P. Morrison, President and Chief Executive Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that to her knowledge:

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

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

Date: November 13, 2014

By: /s/ Jodie P. Morrison
Jodie P. Morrison
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,

AS ADOPTED PURSUANT TO

SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report on Form 10-Q of Tokai Pharmaceuticals, Inc. (the "Company") for the period ended September 30, 2014 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Lee H. Kalowski, Chief Financial Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that to his knowledge:

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

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

Date: November 13, 2014

By: /s/ Lee H. Kalowski
Lee H. Kalowski
Chief Financial Officer
(Principal Financial and Accounting Officer)