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Registration No. 333-198052
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Prospectus

6,480,000 Shares

Tokai Pharmaceuticals, Inc.

Common Stock

$15.00 Per Share

This is the initial public offering of shares of common stock of Tokai Pharmaceuticals, Inc. The initial public offering price is $15.00 per share.

We are offering all of the 6,480,000 shares of common stock offered by this prospectus. No public market currently exists for our common stock.

Our common stock has been approved for listing on The NASDAQ Global Market under the symbol "TKAI."

We are an "emerging growth company" under applicable Securities and Exchange Commission rules and will be eligible for reduced public company disclosure requirements. See "Prospectus Summary—Implications of Being an Emerging Growth Company."

Investing in our common stock involves a high degree of risk. See "Risk Factors" beginning on page 12 of this prospectus.

	Per Share	Total
Initial public offering price	$ 15.00	$97,200,000
Underwriting discounts and commissions(1)	$ 1.05	$ 6,804,000
Proceeds to Tokai Pharmaceuticals, Inc. (before expenses)	$ 13.95	$90,396,000

(1) We refer you to "Underwriting" beginning on page 153 for additional information regarding underwriter compensation.

We have granted the underwriters the option to purchase up to an additional 972,000 shares of common stock on the same terms and conditions set forth above within 30 days from the date of this prospectus if the underwriters sell more than 6,480,000 shares of common stock in this offering.

Certain of our existing stockholders and their affiliates, including our existing principal stockholders, have agreed to purchase an aggregate of 1,105,000 shares of our common stock in this offering at the initial public offering price. In addition, Novo A/S has agreed to purchase 1,000,000 shares of our common stock in this offering at the initial public offering price. The underwriters will receive the same underwriting discounts and commissions on any shares purchased by these parties as they will on any other shares sold to the public in this offering.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or passed on the adequacy or accuracy of this prospectus. Any representation to the contrary is a criminal offense.

The underwriters expect to deliver the shares on or about September 22, 2014.

Joint Book-Running Managers

BMO Capital Markets **Stifel** **William Blair**

Co-Manager

Janney Montgomery Scott

Prospectus dated September 16, 2014.

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You should rely only on the information contained in this prospectus and any free writing prospectus prepared by or on behalf of us or to which we have referred you. We have not authorized anyone to provide you with information that is different. We are offering to sell shares of our common stock, and seeking offers to buy shares of our common stock, only in jurisdictions where offers and sales are permitted. The information in this prospectus is accurate only as of the date of this prospectus, regardless of the time of delivery of this prospectus or any sale of our common stock.

Until October 11, 2014, 25 days after the date of this prospectus, all dealers that buy, sell or trade our common stock, whether or not participating in this offering, may be required to deliver a prospectus. This is in addition to the dealer's obligation to deliver a prospectus when acting as an underwriter and with respect to unsold allotments or subscriptions.

For investors outside the United States: Neither we nor any of the underwriters have taken any action to permit a public offering of the shares of our common stock or the possession or distribution of this prospectus in any jurisdiction where action for that purpose is required, other than the United States. You are required to inform yourselves about and to observe any restrictions relating to this offering and the distribution of this prospectus.

Prospectus Summary

This summary highlights selected information included elsewhere in this prospectus. This summary does not contain all of the information you should consider before investing in our common stock. You should read this entire prospectus carefully, including the "Risk Factors" section beginning on page 12 and our consolidated financial statements and the related notes appearing at the end of this prospectus before making an investment decision. Unless the context otherwise requires, we use the terms "Tokai," "our company," "we," "us" and "our" in this prospectus to refer to Tokai Pharmaceuticals, Inc.

Overview

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. 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, or PSA, 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. We intend to conduct our pivotal Phase 3 clinical trial in these patients who we believe may not be effectively treated by the therapies approved by the FDA in recent years. We believe that one of galeterone's multiple mechanisms of action, androgen receptor degradation, provides an opportunity to treat this population of patients. In our ongoing Phase 2 clinical trial of galeterone, which we refer to as our ARMOR2 trial, we observed clinically meaningful PSA reductions in patients that were identified as having altered androgen receptors that were truncated in a retrospective subset analysis of seven patients. Although our initial development focus is on galeterone for the treatment of this population of patients, we are conducting our Phase 2 ARMOR2 trial of galeterone in multiple CRPC patient populations.

Galeterone acts by disrupting the androgen receptor signaling pathway, which is the primary pathway that drives prostate cancer growth. The pathway is ordinarily activated by the binding of male hormones, or androgens, such as testosterone and the more potent androgen dihydrotestosterone, or DHT, to the ligand binding domain of androgen receptors in prostate cancer cells. Galeterone disrupts the activation of the pathway through multiple mechanisms of action:

- inhibition of the enzyme CYP17, which blocks the synthesis of testosterone;
- androgen receptor antagonism, which blocks the binding of testosterone or DHT with the androgen receptor; and
- androgen receptor degradation, which reduces the amount of androgen receptor protein in the tumor cells.

We believe that, in comparison to therapies that act solely through CYP17 inhibition or androgen receptor antagonism, galeterone's unique combination of mechanisms of action may provide galeterone with advantages in efficacy in the treatment of CRPC and may reduce the risk of or delay the development of resistance to therapy and provide efficacy in patients with tumors resistant to other treatments.

The truncated androgen receptors for which we are developing galeterone 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 plan to conduct our pivotal Phase 3 clinical trial in patients with AR-V7. In patients with C-terminal loss, including AR-V7, the lack of a functional ligand binding domain causes the truncated androgen receptor to be constitutively active, or continuously signaling, meaning that activation of the androgen receptor pathway and tumor growth occurs even in the absence of androgens and androgen binding. In clinical studies conducted by researchers at MD Anderson Cancer Center and Johns Hopkins University, the presence in patients of truncated androgen receptors with C-terminal loss and AR-V7 was associated with poor responsiveness of patients' prostate tumors to treatment with Zytiga® (abiraterone acetate) and Xtandi® (enzalutamide), two of the highest selling therapies for CRPC with aggregate reported worldwide 2013 sales of more than $2.1 billion. We believe that these studies indicate that there is a need for effective treatments for CRPC patients with C-terminal loss, including AR-V7.

We are currently finalizing our plans for our pivotal Phase 3 clinical trial of galeterone, which we refer to as our ARMOR3-Splice Variant, or SV, trial. 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 up to 170 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 PSA levels, overall survival and safety. We expect to commence the trial in the first half of 2015 and, subject to patient enrollment rates and the rates of disease progression in patients in the trial, to have top-line data from the trial by the end of 2016.

In addition to our planned pivotal Phase 3 clinical trial, we are conducting our ARMOR2 trial of galeterone for the treatment of multiple CRPC populations. 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, as well as in hormone-sensitive prostate cancer. We have exclusive worldwide development and commercialization rights to galeterone.

Prostate Cancer. According to the American Cancer Society, in the United States, prostate cancer is the most frequently diagnosed cancer among men other than skin cancer. The American Cancer Society estimates that, in the United States during 2014, approximately 233,000 new cases of prostate cancer will be diagnosed, and approximately 29,000 men will die from the disease. Prostate cancer drugs represent a large and growing market. According to Decision Resources Group, an independent research firm, sales of prostate cancer drugs are expected to increase from $6.0 billion in 2013 to more than $9.0 billion in 2021, due to a growing aged population, a rising incidence of cancer and the introduction of new drugs for the treatment of prostate cancer. These new drugs include Zytiga and Xtandi, which are approved for the treatment of CRPC. Although Zytiga was only approved in 2011 and Xtandi in 2012, both of these drugs have experienced rapid sales growth, with reported worldwide 2013 sales of $1.7 billion for Zytiga and $445 million for Xtandi. Despite their success, the need for new treatment options remains as each of these drugs has treatment limitations in CRPC patients and may not be effective in CRPC patients with C-terminal loss, including AR-V7.

ARMOR2 Trial. In December 2012, we initiated a two-part Phase 2 open label clinical trial of galeterone for the treatment of CRPC, which we refer to as our ARMOR2 trial. Part 1 of the trial was a dose escalation phase designed to confirm the dose of galeterone to be evaluated in Part 2 of the trial. We are currently evaluating the 2550 mg/day dose for safety and efficacy in Part 2 of the trial in four distinct CRPC patient populations.

In May 2014, we announced interim data from our ARMOR2 trial at The American Society of Clinical Oncology 2014 Annual Meeting, or ASCO. The interim data included patients who had not previously undergone chemotherapy and had not received treatment with Zytiga or Xtandi, whom we refer to as CRPC treatment-naïve patients, and patients whose disease progressed during treatment with Zytiga, whom we refer to as Zytiga- refractory patients. We reported that, as of May 12, 2014, our cut-off date for our data presentation at ASCO, in

51 evaluable CRPC treatment-naïve patients, galeterone showed clinically meaningful reductions in levels of PSA. Specifically, we reported the following:

- *Non-metastatic and metastatic CRPC treatment-naïve patients at the selected Phase 2 dose of 2550 mg/day:* During the first 12 weeks of dosing, 82% of patients showed maximal reduction in PSA levels of at least 30%, and 75% of patients showed maximal reduction in PSA levels of at least 50%.
- *Metastatic CRPC treatment-naïve patients at the selected Phase 2 dose of 2550 mg/day:* During the first 12 weeks of dosing, 85% of patients showed maximal reduction in PSA levels of at least 30%, and 77% of patients showed maximal reduction in PSA levels of at least 50%.

We also reported 12-week data for 15 Zytiga-refractory patients, two of whom had a maximal reduction in PSA levels of at least 30%.

In addition, we presented data from a retrospective subset analysis in which four treatment-naïve CRPC patients in ARMOR2 were identified as having truncated androgen receptors with C-terminal loss. All four of these patients had maximal reductions in PSA levels of at least 50%. These data are consistent with galeterone's mechanism of action of androgen receptor degradation, which does not require a functional ligand binding domain.

We plan to announce additional interim data from our ARMOR2 trial in September 2014 at the European Society for Medical Oncology, or ESMO, 2014 Congress in Madrid, Spain. We believe these data are consistent with the efficacy and safety data that we reported at ASCO. Specifically, we expect to report, among other data, that as of August 15, 2014, our cut-off date for our data presentation at the ESMO 2014 Congress, seven treatment-naïve CRPC patients had been identified in the retrospective subset analysis as having truncated androgen receptors with C-terminal loss, and that six of these patients had maximal reductions in PSA levels of at least 50%. The seventh patient, who did not show any PSA reduction, discontinued therapy due to an adverse event unrelated to galeterone after approximately six weeks in the trial and did not receive the full treatment regimen.

In June 2012, the FDA designated galeterone for the treatment of CRPC for fast track review. The FDA may designate a product for fast track review if it is intended, whether alone or in combination with one or more other drugs, for the treatment of a serious or life-threatening disease or condition, and it demonstrates the potential to address unmet medical needs for such a disease or condition. For fast track products, sponsors may have more frequent interactions with the FDA, and the FDA may initiate review of sections of a fast track product's new drug application, or NDA, on a rolling basis before the application is complete. In addition, sponsors may request and be granted priority review of their application.

Mechanisms of Action. The growth and survival of prostate cancer tumor cells depend primarily on the functioning of the androgen receptor signaling pathway. Ordinarily, the pathway and tumor growth are activated by the binding of testosterone and DHT to the ligand binding domain of androgen receptors. As a result, therapies that block this binding can be effective in disrupting the pathway and tumor cell growth. Zytiga blocks this binding by reducing the synthesis of testosterone through the inhibition of the enzyme CYP17. Xtandi blocks the binding of testosterone or DHT with the androgen receptor through androgen receptor antagonism. However, the effectiveness of Zytiga, Xtandi and other therapies based solely on one of these mechanisms of action requires a functional ligand binding domain. In the case of prostate tumor cells that express truncated androgen receptors with C-terminal loss, including AR-V7, there is no functional ligand binding domain, which causes the truncated androgen receptor to be constitutively active. As a result, we believe that patients with truncated androgen receptors with C-terminal loss may not be effectively treated by these therapies.

In contrast, galeterone disrupts the androgen receptor signaling pathway at multiple points by combining the mechanisms of action of CYP17 inhibition and androgen receptor antagonism with the mechanism of androgen receptor degradation. Unlike CYP17 inhibition and androgen receptor antagonism, androgen receptor degradation does not require a functional ligand binding domain to disrupt the activation of the pathway and tumor growth. As a

result, we believe that, based on galeterone's multiple mechanisms of action, data from the subset of patients in our ARMOR2 trial and data from preclinical studies conducted by us and independent laboratories, galeterone may have the ability to treat both patients with full-length androgen receptors and patients with C-terminal loss, including AR-V7. To our knowledge, there are no approved drugs or drugs in clinical development, other than galeterone, that disrupt the androgen receptor signaling pathway through androgen receptor degradation.

The following figure shows a comparison of the mechanisms of action of Zytiga, Xtandi and galeterone:

	Mechanisms of Action		
	CYP17 Inhibition	Androgen Receptor Antagonism	Androgen Receptor Degradation
Zytiga (abiraterone acetate)	✓		
Xtandi (enzalutamide)		✓	
Galeterone	✓	✓	✓

Advantages of Galeterone. Although Zytiga and Xtandi have improved survival of CRPC patients, they have limitations in terms of safety, dosing, patient compliance and the development of resistance. In addition, Zytiga and Xtandi may not be effective in treating CRPC patients with prostate cancer tumors that express altered androgen receptors with C-terminal loss, including AR-V7. As a result, there remains an unmet medical need for therapies that address populations that are resistant to therapy and will further improve overall survival while providing a more favorable risk benefit profile.

We believe that galeterone has advantages over Zytiga and Xtandi because of the following key differentiating attributes:

- ***Potential for improved efficacy.*** We believe that galeterone, which combines the mechanism of action of androgen receptor degradation with the mechanisms of action of CYP17 inhibition and androgen receptor antagonism, may further improve progression-free survival and overall survival beyond that of products that depend on only a single mechanism of action.
- ***Potential for lower risk of resistance.*** We believe that galeterone may reduce the risk of or delay the development of resistance to therapy because galeterone addresses multiple mechanisms of action simultaneously.
- ***Potential for broad utility in prostate cancer.*** We believe that galeterone may be well suited to treat different prostate cancer patient populations, from early-stage prostate cancer patients to end-stage salvage metastatic CRPC patients, because of its efficacy, safety and tolerability.
- ***Potential for efficacy in CRPC patients with C-terminal loss, including AR-V7.*** Because galeterone's distinct mechanism of androgen receptor degradation does not require an intact ligand binding domain for efficacy, we believe galeterone may be effective in prostate cancer tumors that express altered androgen receptors with C-terminal loss, including AR-V7. In contrast, the mechanisms of action of Zytiga and Xtandi and other similar drugs in development all require the presence of a functional ligand binding domain in order to be effective.
- ***Favorable safety profile.*** 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 demonstrated a favorable safety and tolerability profile.

- ***No requirement for steroids.*** Zytiga must be co-administered with the steroid prednisone to minimize the risk of mineralocorticoid excess, a potentially fatal syndrome characterized by hypertension, hypokalemia, fluid retention and edema. Unlike Zytiga, galeterone has not been shown in clinical trials to cause mineralocorticoid excess and, as a result, does not require co-administration of steroids.
- ***No associated seizure risk.*** Xtandi has shown a risk of grand mal seizures in clinical trials. Unlike Xtandi, galeterone is not in a class of therapeutics that has shown a risk of seizures. We have not had any reports of seizures in clinical trials of galeterone.
- ***Ease of dosing.*** Galeterone is dosed orally once per day, does not require the co-administration of steroids and can be taken with or without food. We believe that this convenient dosing regimen will enhance patient compliance. In contrast, Zytiga must be taken in a fasted state to avoid large increases in absorption, which may cause side effects. Zytiga must also be co-administered with steroids. The steroid co-administered with Zytiga must be taken with food, resulting in a staggered dosing regimen of pills three times per day.
- ***Potential for use as part of combination therapy.*** We believe that galeterone may prove to be well suited for use in combination with other therapies used across all patient populations of prostate cancer because of its favorable safety profile, ease of administration and highly selective, multiple mechanisms of action.

Our Strategy

Our goal is to become a leading biopharmaceutical company that develops and commercializes products for the treatment of prostate cancer and other hormonally-driven diseases. Our strategy includes the following components:

- ***Complete the clinical development of and seek marketing approval for galeterone for the treatment of CRPC patients with prostate cancer tumors that express the AR-V7 splice variant.*** Based on discussions with the FDA, we expect that our ARMOR3-SV trial will be a randomized, open label clinical trial comparing galeterone to Xtandi in up to 170 metastatic CRPC treatment-naïve patients whose prostate tumors express the AR-V7 splice variant. We expect to commence the trial in the first half of 2015 and, subject to patient enrollment rates and the rates of disease progression in patients in the trial, to have top-line data from the trial by the end of 2016.
- ***Develop galeterone for other prostate cancer indications and patient populations***. Although we are focusing our initial development of galeterone on the treatment of patients with CRPC whose prostate tumor cells express an altered androgen receptor, we are conducting our ARMOR2 trial of galeterone for the treatment of multiple CRPC patient populations. 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, as well as in hormone-sensitive prostate cancer. We also plan to develop galeterone for the treatment of prostate cancer in combination with currently marketed prostate cancer therapies and novel targeted agents.
- ***Explore the use of galeterone for other hormonally-driven diseases***. 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.
- ***Maximize the commercial potential of galeterone.*** We have worldwide development and commercialization rights to galeterone. 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.
- ***Advance the development of our platform of androgen receptor degradation agents.*** We plan to identify and develop novel compounds that are designed to disrupt androgen receptor signaling through enhanced

androgen receptor degradation under an exclusive license from the University of Maryland, Baltimore. We believe that such compounds may have utility as monotherapies or in combination with existing therapies in treating patients with androgen receptor signaling diseases, including prostate cancer patients with primary or acquired resistance to existing therapies.

Risks Associated with Our Business

Our business is subject to a number of risks of which you should be aware before making an investment decision. These risks are discussed more fully in the "Risk Factors" section of this prospectus immediately following this prospectus summary. These risks include the following:

- 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.
- 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.
- 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 our future product candidates.
- We have no history of commercializing pharmaceutical products, which may make it difficult to evaluate the prospects for our future viability.
- 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 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. We will need to develop this assay and submit an investigational device exemption application for the assay before we can initiate our planned pivotal Phase 3 clinical trial of galeterone. 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.
- 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.
- 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 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.

Our Company

We were incorporated under the laws of the State of Delaware on March 26, 2004 under the name Tokai Pharmaceuticals, Inc. Our executive offices are located at One Broadway, 14th Floor, Cambridge, Massachusetts 02142 and our telephone number is (617) 225-4305. Our website address is www.tokaipharma.com. The information contained in, or accessible through, our website does not constitute part of this prospectus. We have included our website address in this prospectus solely as an inactive textual reference.

The Tokai logo is our trademark. The trademarks, trade names and service marks appearing in this prospectus are the property of their respective owners.

Implications of Being an Emerging Growth Company

As a company with less than $1 billion in revenue during our last fiscal year, we qualify as an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. As an emerging growth company, we are permitted and intend to rely on exemptions from specified disclosure and other requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include:

- being permitted to provide only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations" disclosure;
- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation; and
- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved.

We may take advantage of some or all these exemptions for up to five years or such earlier time that we are no longer an emerging growth company. We would cease to be an emerging growth company if we have more than $1 billion in annual revenue, we have more than $700 million in market value of our stock held by non-affiliates or we issue more than $1 billion of non-convertible debt over a three-year period. We have taken advantage of certain reduced reporting obligations in this prospectus. Accordingly, the information contained herein may be different than the information you receive from other public companies in which you hold stock.

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 provision 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.

The Offering

Common stock offered by us	6,480,000 shares
Common stock to be outstanding after this offering	21,841,742 shares (22,813,742 shares in the event the underwriters elect to exercise in full their over-allotment option to purchase additional shares from us)
Over-allotment option	The underwriters have an option for a period of 30 days to purchase up to 972,000 additional shares of our common stock to cover over-allotments.
Use of proceeds	We estimate that the net proceeds to us from this offering, after deducting underwriting discounts and commissions and estimated offering expenses payable by us, will be approximately $87.7 million, or approximately $101.2 million if the underwriters exercise their over-allotment option to purchase additional shares from us in full, based on the initial public offering price of $15.00 per share. We plan to use the net proceeds of this offering, together with our existing cash and cash equivalents, to fund our planned pivotal Phase 3 clinical trial of galeterone and conduct other clinical trials and non-clinical studies to support the submission of an NDA to the FDA for galeterone, to fund our ongoing ARMOR2 trial and to fund our initial research of other compounds that are designed to disrupt androgen receptor signaling through enhanced androgen receptor degradation and for working capital and other general corporate purposes. See "Use of Proceeds" for more information.
Risk factors	You should read the "Risk Factors" section starting on page 12 of this prospectus and other information included in this prospectus for a discussion of factors to consider carefully before deciding to invest in shares of our common stock.
NASDAQ Global Market symbol	"TKAI"

The number of shares of common stock to be outstanding after this offering is based on 501,569 shares of common stock outstanding as of July 31, 2014 and gives effect to the conversion of all outstanding shares of our redeemable convertible preferred stock into 14,860,173 shares of common stock upon the closing of this offering.

The number of shares of common stock to be outstanding after this offering excludes:

- 1,634,275 shares of common stock issuable upon the exercise of stock options outstanding as of July 31, 2014, at a weighted average exercise price of $2.86 per share;
- 43,945 shares of common stock available for future issuance under our 2007 Stock Incentive Plan, as amended, as of July 31, 2014;

- an additional 1,700,000 shares of common stock that will become available for future issuance under our 2014 Stock Incentive Plan upon the effectiveness of the registration statement of which this prospectus forms a part, which includes the grant of an option to purchase 218,417 shares of our common stock and 54,604 shares subject to a restricted stock unit award; and
- an additional 225,000 shares of common stock that will become available for future issuance under our 2014 Employee Stock Purchase Plan upon the effectiveness of the registration statement of which this prospectus forms a part.

Except as otherwise noted, all information in this prospectus:

- gives effect to a 1-for-10.47 reverse stock split of our common stock and a proportional adjustment to the existing conversion ratio of each series of our redeemable convertible preferred stock, which became effective on August 29, 2014;
- assumes no exercise of the outstanding options described above;
- assumes no exercise by the underwriters of their over-allotment option to purchase up to 972,000 additional shares of common stock from us; and
- gives effect to the restatement of our certificate of incorporation and bylaws upon the closing of this offering.

Certain of our existing stockholders and their affiliates, including our existing principal stockholders, have agreed to purchase an aggregate of 1,105,000 shares of our common stock in this offering at the initial public offering price. In addition, Novo A/S has agreed to purchase 1,000,000 shares of our common stock in this offering at the initial public offering price.

Summary Consolidated Financial Data

The following table summarizes our consolidated financial data. We have derived the consolidated statement of operations data for the years ended December 31, 2012 and 2013 from our audited consolidated financial statements included elsewhere in this prospectus. The consolidated statement of operations data for the six months ended June 30, 2013 and 2014 and for the cumulative period from inception (March 26, 2004) through June 30, 2014 and the consolidated balance sheet data as of June 30, 2014 have been derived from our unaudited consolidated financial statements included elsewhere in this prospectus and have been prepared on the same basis as the audited consolidated financial statements. In the opinion of management, the unaudited financial data reflect all adjustments, consisting only of normal recurring adjustments, necessary for a fair presentation of the financial information in those statements. You should read the following summary consolidated financial data together with our consolidated financial statements and the related notes appearing at the end of this prospectus and the "Selected Consolidated Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of this prospectus. Our historical results are not necessarily indicative of results that should be expected in the future, and results for the six months ended June 30, 2014 are not necessarily indicative of the results that should be expected for the full year ending December 31, 2014.

	Year Ended December 31,		Six Months Ended June 30,		Cumulative Period from Inception (March 26, 2004) to June 30, 2014
	2012	2013	2013	2014	
	(in thousands, except per share data)				
Consolidated Statement of Operations Data:					
Revenue	$ —	$ —	$ —	$ —	$ —
Operating expenses:					
Research and development	7,370	12,201	5,148	7,948	57,314
General and administrative	2,279	3,548	1,687	2,829	16,286
Total operating expenses	9,649	15,749	6,835	10,777	73,600
Loss from operations	(9,649)	(15,749)	(6,835)	(10,777)	(73,600)
Other income (expense):					
Interest income	—	—	—	—	216
Interest expense	—	—	—	—	(302)
Other income (expense), net	—	24	—	79	342
Total other income, net	—	24	—	79	256
Net loss	(9,649)	(15,725)	(6,835)	(10,698)	(73,344)
Accretion of redeemable convertible preferred stock to redemption value	(34)	(94)	(79)	—	(3,359)
Accrual of dividend on redeemable convertible preferred stock	—	—	—	—	(347)
Modifications of redeemable convertible preferred stock	—	—	—	—	9,925
Net loss attributable to common stockholders	$ (9,683)	$(15,819)	$(6,914)	$(10,698)	$ (67,125)
Net loss per share attributable to common stockholders, basic and diluted[1]	$ (31.09)	$ (38.02)	$(20.49)	$ (21.48)	
Weighted average common shares outstanding, basic and diluted[1]	311	416	337	498	
Pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)[1]		$ (1.29)		$ (0.70)	
Pro forma weighted average common shares outstanding, basic and diluted (unaudited)[1]		12,230		15,358	

	As of June 30, 2014		
	Actual	Pro Forma(2)	Pro Forma As Adjusted(3)
	(in thousands)		
Consolidated Balance Sheet Data:			
Cash and cash equivalents	$ 21,150	$ 21,150	$ 109,325
Working capital(4)	18,051	18,051	107,241
Total assets	23,420	23,420	110,071
Redeemable convertible preferred stock	85,345	—	—
Total stockholders' equity (deficit)	(65,613)	19,732	107,398

(1) See Note 10 to our consolidated financial statements for further details on the calculation of basic and diluted net loss per share attributable to common stockholders and the calculation of basic and diluted pro forma net loss per share attributable to common stockholders.

(2) Pro forma consolidated balance sheet data give effect to the automatic conversion of all outstanding shares of our redeemable convertible preferred stock into an aggregate of 14,860,173 shares of common stock upon the closing of this offering.

(3) Pro forma as adjusted consolidated balance sheet data give effect to the pro forma adjustment described in footnote 2 above as well as the sale by us of 6,480,000 shares of common stock in this offering at the initial public offering price of $15.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

(4) We define working capital as current assets less current liabilities.

Risk Factors

Investing in our common stock involves a high degree of risk. Before you decide to invest in our common stock, you should consider carefully the risks described below, together with the other information contained in this prospectus, including our financial statements and the related notes appearing at the end of this prospectus. We believe the risks described below are the risks that are material to us as of the date of this prospectus. 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, and you may lose all or part of your investment.

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 $10.7 million for the six months ended June 30, 2014. As of June 30, 2014, we had an accumulated deficit of $73.8 million. To date, we have financed our operations primarily through private placements of our redeemable convertible preferred stock and convertible promissory notes, none of which are currently outstanding. 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 following this offering.

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 you to lose all or part of your 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 June 30, 2014, we had cash and cash equivalents of $21.2 million and working capital of $18.1 million. We expect that the net proceeds from this offering, together with 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 in addition to the net proceeds of this offering 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, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your 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 up to 170 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 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.

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 August 15, 2014, we had enrolled 121 patients in the trial and expect to enroll a total of approximately 136 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 prostate specific antigen, 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, and Johns Hopkins University, or Johns Hopkins, and data from preclinical studies conducted by us and independent laboratories. However, the clinical studies conducted by MD Anderson and Johns Hopkins 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 and Johns Hopkins 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 and Johns Hopkins 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 July 31, 2014, we owned two issued U.S. patents, ten U.S. provisional and non-provisional patent applications, one issued foreign patent and 34 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 42 issued foreign patents as well as three U.S. patent applications and 11 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 and this Offering

After this offering, our executive officers, directors and principal stockholders will maintain the ability to control all matters submitted to stockholders for approval.

Upon the closing of this offering, our executive officers, directors and our existing stockholders who owned more than 5% of our outstanding common stock before this offering will, in the aggregate, beneficially own shares representing approximately 69.12% of our common stock (66.22% if the underwriters exercise in full their option to purchase additional shares), which includes an aggregate of 1,105,000 shares that certain of our existing stockholders and their affiliates, including our existing principal stockholders, have agreed to purchase in this offering at the initial public offering price. 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.

In addition, upon the closing of this offering, our two largest stockholders, Apple Tree Partners and Novartis BioVentures, will beneficially own shares representing approximately 36.68% and 21.26% of our common stock, respectively (35.12% and 20.36%, respectively, if the underwriters exercise in full their option to purchase additional shares). These shares include an aggregate of 787,500 shares that Apple Tree Partners and Novartis BioVentures have agreed to purchase in this offering at the initial public offering price. Each stockholder acting individually, as well as together, will 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 that will become effective upon the closing of this offering may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your 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.

If you purchase shares of our common stock in this offering, you will suffer immediate dilution of your investment.

The initial public offering price of our common stock is substantially higher than the net tangible book value per share of our common stock. Therefore, if you purchase shares of our common stock in this offering, you will pay a price per share that substantially exceeds our net tangible book value per share after this offering. To the extent outstanding options are exercised, you will incur further dilution. Based on the initial public offering price of $15.00 per share, you will experience immediate dilution of $10.08 per share, representing the difference between our pro forma as adjusted net tangible book value per share after giving effect to this offering and the initial public offering price. In addition, purchasers of common stock in this offering will have contributed approximately 51.2% of the aggregate price paid for all purchases of our stock but the shares purchased in this offering will represent an aggregate of only approximately 29.7% of our total common stock outstanding after this offering. In addition, future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.

An active trading market for our common stock may not develop.

Prior to this offering, there has been no public market for our common stock. The initial public offering price for our common stock was determined through negotiations with the underwriters. Although our common stock has been approved for listing on The NASDAQ Global Market, an active trading market for our shares may never develop or be sustained following this offering. If an active market for our common stock does not develop, it may be difficult for you to sell shares you purchase in this offering without depressing the market price for the shares or at all.

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. As a result of this volatility, you may not be able to sell your common stock at or above the initial public offering price. 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, once we are 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.

We have broad discretion in the use of the net proceeds from this offering and may not use them effectively.

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

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

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 your sole source of gain 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. After this offering, we will have outstanding 21,841,742 shares of common stock based on the number of shares outstanding as of July 31, 2014. Of these shares of our common stock, 5,375,000 shares to be sold in this offering, plus any shares sold upon exercise of the underwriters' over-allotment option, will be freely tradable, without restriction, in the public market immediately following this offering, unless purchased by our affiliates. All of the remaining 16,466,742 shares are currently restricted, including 1,105,000 shares which our existing stockholders and their affiliates have agreed to purchase in this offering, as a result of securities laws or lock-up agreements but will be able to be sold after the offering as described in the "Shares Eligible for Future Sale" section of this prospectus. Moreover, after this offering, holders of an aggregate of 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 the lock-up agreements described in the "Underwriting" section of this prospectus.

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. 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.

Cautionary Note Regarding Forward-Looking Statements and Industry Data

This prospectus contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this prospectus, including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans and objectives of management and expected market growth 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 C-terminal loss generally and AR-V7 specifically;
- 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 expectations related to the use of proceeds from this offering;
- 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 prospectus, 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 prospectus, the documents that we reference in this prospectus and the documents that we have filed as exhibits to the registration statement of which this prospectus is a part 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.

This prospectus includes statistical and other industry and market data that we obtained from industry publications and research, surveys and studies conducted by third parties. Industry publications and third-party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. While we believe these industry publications and third-party research, surveys and studies are reliable, we have not independently verified such data.

Use of Proceeds

We estimate that the net proceeds from our issuance and sale of 6,480,000 shares of our common stock in this offering will be approximately $87.7 million, based on the initial public offering price of $15.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us. If the underwriters exercise their over-allotment option in full, we estimate that the net proceeds from this offering will be approximately $101.2 million.

As of June 30, 2014, we had cash and cash equivalents of $21.2 million. We currently estimate that we will use the net proceeds from this offering, together with our existing cash and cash equivalents, as follows:

- approximately $45.0 million to fund 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;
- approximately $4.5 million to fund our ongoing ARMOR2 trial;
- approximately $2.0 million to fund our initial research of compounds that are designed to disrupt androgen receptor signaling through enhanced androgen receptor degradation;
- the greater of $0.5 million and 1% of the gross proceeds of this offering to pay a fee to a financial advisor in connection with strategic and financial advisory services unrelated to this offering; and
- the remainder for working capital and other general corporate purposes.

The expected use of net proceeds from this offering represents our intentions based upon our current plans and business conditions, which could change in the future as our plans and business conditions evolve. The amounts and timing of our actual expenditures may vary significantly depending on numerous factors, including the progress of our development, the status of and results from clinical trials, as well as any collaborations that we may enter into with third parties for our product candidates and any unforeseen cash needs. As a result, our management will retain broad discretion over the allocation of the net proceeds from this offering.

Based on our planned use of the net proceeds from this offering and our existing cash and cash equivalents described above, as well as the anticipated timing of our clinical trials, we estimate that such funds will be sufficient to enable us to complete 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 this indication, as well as to continue to fund our operating expenses and capital expenditure requirements into the first half of 2017. We do not expect that the net proceeds from this offering and our existing cash and cash equivalents will be sufficient to enable us to submit an NDA to the FDA for galeterone for this indication, complete the development of galeterone for this indication, commercialize galeterone for this indication and develop or commercialize any future product candidates.

Pending our use of the net proceeds from this offering, we intend to invest the net proceeds in a variety of capital preservation investments, including short-term, investment-grade, interest-bearing instruments and U.S. government securities.

Dividend Policy

We have never declared or paid any 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. We do not intend to pay any cash dividends to the holders of our common stock in the foreseeable future.

Capitalization

The following table sets forth our cash and cash equivalents and our capitalization as of June 30, 2014:

- on an actual basis;
- on a pro forma basis, after giving effect to the automatic conversion of all outstanding shares of our redeemable convertible preferred stock into an aggregate of 14,860,173 shares of common stock upon the closing of this offering; and
- on a pro forma as adjusted basis, after giving effect to the pro forma adjustment listed above as well as the sale by us of 6,480,000 shares of our common stock in this offering at the initial public offering price of $15.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us, and the filing and effectiveness of our restated certificate of incorporation upon the closing of this offering.

You should read the following table in conjunction with the sections of this prospectus entitled "Selected Consolidated Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our consolidated financial statements and related notes included elsewhere in this prospectus.

	As of June 30, 2014		
	Actual	Pro Forma	Pro Forma As Adjusted
	(in thousands, except share and per share data)		
Cash and cash equivalents	$ 21,150	$ 21,150	$ 109,325
Redeemable convertible preferred stock (Series A, B-1, B-2, C, D-1, D-2, D-3 and E), $0.001 par value; 155,586,141 shares authorized, issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	$ 85,345	$ —	$ —
Stockholders' equity (deficit):			
Preferred stock, $0.001 par value; no shares authorized, issued or outstanding, actual; 5,000,000 shares authorized and no shares issued or outstanding, pro forma and pro forma as adjusted	—	—	—
Common stock, $0.001 par value; 178,408,438 shares authorized, 501,569 shares issued and outstanding, actual; 200,000,000 shares authorized, 15,361,742 shares issued and outstanding, pro forma; 200,000,000 shares authorized, 21,841,742 shares issued and outstanding, pro forma as adjusted	1	15	22
Additional paid-in capital	8,139	93,470	181,129
Deficit accumulated during the development stage	(73,753)	(73,753)	(73,753)
Total stockholders' equity (deficit)	(65,613)	19,732	107,398
Total capitalization	$ 19,732	$ 19,732	$ 107,398

The number of shares of common stock shown as issued and outstanding on a pro forma as adjusted basis in the table above is based on 501,569 shares of common stock outstanding as of June 30, 2014 and excludes:

- 1,634,275 shares of common stock issuable upon the exercise of stock options outstanding as of June 30, 2014, at a weighted average exercise price of $2.86 per share;
- 43,945 shares of our common stock available for future issuance under our 2007 Stock Incentive Plan, as amended, or the 2007 Plan, as of June 30, 2014;
- an additional 1,700,000 shares of common stock that will become available for future issuance under our 2014 Stock Incentive Plan, or the 2014 Plan, upon the effectiveness of the registration statement of which

this prospectus forms a part, which includes the grant of an option to purchase 218,417 shares of our common stock and 54,604 shares subject to a restricted stock unit award; and

- an additional 225,000 shares of common stock that will become available for future issuance under our 2014 Employee Stock Purchase Plan, or the 2014 ESPP, upon the effectiveness of the registration statement of which this prospectus forms a part.

Dilution

If you invest in our common stock in this offering, your ownership interest will be immediately diluted to the extent of the difference between the initial public offering price per share and the pro forma as adjusted net tangible book value per share of our common stock immediately after this offering.

Our historical net tangible book value (deficit) as of June 30, 2014 was $(67.1) million, or $(133.85) per share of common stock. Our historical net tangible book value (deficit) is the amount of our total tangible assets less our total liabilities and redeemable convertible preferred stock, which is not included within stockholders' equity (deficit). Historical net tangible book value per share is our historical net tangible book value (deficit) divided by the number of shares of common stock outstanding as of June 30, 2014.

Our pro forma net tangible book value as of June 30, 2014 was $18.2 million, or $1.19 per share of common stock. Pro forma net tangible book value represents the amount of our total tangible assets less our total liabilities, after giving effect to the automatic conversion of all outstanding shares of our redeemable convertible preferred stock into an aggregate of 14,860,173 shares of common stock upon the closing of this offering. Pro forma net tangible book value per share represents pro forma net tangible book value divided by the pro forma number of shares of our common stock outstanding as of June 30, 2014, after giving effect to the automatic conversion of all outstanding shares of our redeemable convertible preferred stock into an aggregate of 14,860,173 shares of common stock upon the closing of this offering.

After giving effect to our issuance and sale of 6,480,000 shares of common stock in this offering at the initial public offering price of $15.00 per share, and after deducting underwriting discounts and commissions and estimated offering expenses payable by us, the pro forma as adjusted net tangible book value as of June 30, 2014 would have been $107.4 million, or $4.92 per share. This represents an immediate increase in pro forma as adjusted net tangible book value of $3.73 per share to existing stockholders. The initial public offering price per share significantly exceeds the pro forma as adjusted net tangible book value per share. Accordingly, new investors who purchase shares of common stock in this offering will suffer an immediate dilution of their investment of $10.08 per share. The following table illustrates this per share dilution to the new investors purchasing shares of common stock in this offering, without giving effect to the over-allotment option granted to the underwriters:

Initial public offering price per share		$15.00
Historical net tangible book value (deficit) per share as of June 30, 2014	$(133.85)	
Increase per share attributable to the conversion of all shares of redeemable convertible preferred stock outstanding	135.04	
Pro forma net tangible book value per share as of June 30, 2014	1.19	
Increase in pro forma as adjusted net tangible book value per share attributable to sale of shares of common stock in this offering	3.73	
Pro forma as adjusted net tangible book value per share after this offering		4.92
Dilution per share to new investors participating in this offering		$10.08

If the underwriters exercise their over-allotment option in full, the pro forma as adjusted net tangible book value per share after this offering will increase to $5.30 per share, representing an immediate increase in pro forma as adjusted net tangible book value per share to existing stockholders of $4.11 per share and an immediate dilution of $9.70 per share to new investors.

The following table summarizes, as of June 30, 2014, on a pro forma as adjusted basis as described above, the differences between the number of shares of common stock purchased from us, the total consideration paid to us and the average price per share paid to us by existing stockholders and by new investors purchasing shares of

common stock in this offering. The calculation below is based on the initial public offering price of $15.00 per share before deducting underwriting discounts and commissions and estimated offering expenses payable by us:

	Shares Purchased		Total Consideration		Average Price Per Share
	Number	Percent	Amount	Percent	
Existing stockholders	15,361,742	70.3%	$ 92,754,707	48.8%	$ 6.04
New investors	6,480,000	29.7	97,200,000	51.2	$15.00
Total	21,841,742	100.0%	$189,954,707	100.0%	

The table above assumes no exercise of the underwriters' over-allotment option to purchase additional shares in this offering. If the underwriters exercise their over-allotment option to purchase additional shares from us in full, the number of shares of our common stock held by new investors will increase to 7,452,000, or 32.7% of the total number of shares of common stock outstanding after this offering, and the percentage of shares held by existing stockholders will decrease to 67.3% of the total shares outstanding number of shares of common stock outstanding after this offering.

The above discussion and tables are based on 501,569 shares of common stock outstanding as of June 30, 2014, gives effect to the automatic conversion of all outstanding shares of our redeemable convertible preferred stock into an aggregate of 14,860,173 shares of common stock upon the closing of this offering, assumes no exercise of any outstanding stock options and excludes:

- 1,634,275 shares of common stock issuable upon the exercise of stock options outstanding as of June 30, 2014, at a weighted average exercise price of $2.86 per share;
- 43,945 shares of our common stock available for future issuance under our 2007 Plan as of June 30, 2014;
- an additional 1,700,000 shares of common stock that will become available for future issuance under our 2014 Plan upon the effectiveness of the registration statement of which this prospectus forms a part, which includes the grant of an option to purchase 218,417 shares of our common stock and 54,604 shares subject to a restricted stock unit award; and
- an additional 225,000 shares of common stock that will become available for future issuance under our 2014 ESPP upon the effectiveness of the registration statement of which this prospectus forms a part.

Certain of our existing stockholders and their affiliates, including our existing principal stockholders, have agreed to purchase an aggregate of 1,105,000 shares of our common stock in this offering at the initial public offering price. The foregoing discussion and tables do not reflect any purchases by these parties or their affiliates.

To the extent that outstanding options are exercised, you will experience further dilution. In addition, we may choose to raise additional capital due to market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. To the extent that additional capital is raised through the sale of equity or convertible debt securities, the issuance of these securities may result in further dilution to our stockholders.

Selected Consolidated Financial Data

You should read the following selected consolidated financial data together with our consolidated financial statements and the related notes appearing at the end of this prospectus and the "Management's Discussion and Analysis of Financial Condition and Results of Operations" section of this prospectus. We have derived the consolidated statement of operations data for the years ended December 31, 2012 and 2013 and the consolidated balance sheet data as of December 31, 2012 and 2013 from our audited consolidated financial statements included elsewhere in this prospectus. The consolidated statement of operations data for the six months ended June 30, 2013 and 2014 and for the cumulative period from inception (March 26, 2004) through June 30, 2014 and the consolidated balance sheet data as of June 30, 2014 have been derived from our unaudited consolidated financial statements and the related notes appearing at the end of this prospectus and have been prepared on the same basis as the audited consolidated financial statements. In the opinion of management, the unaudited financial data reflect all adjustments, consisting only of normal recurring adjustments, necessary for a fair presentation of the financial information in those statements. The selected consolidated financial data in this section are not intended to replace our consolidated financial statements and the related notes included elsewhere in this prospectus. Our historical results are not necessarily indicative of the results that should be expected in the future, and results for the six months ended June 30, 2014 are not necessarily indicative of the results that should be expected for the full year ending December 31, 2014.

	Year Ended December 31,		Six Months Ended June 30,		Cumulative Period from Inception (March 26, 2004) to June 30, 2014
	2012	2013	2013	2014	
	(in thousands, except per share data)				
Consolidated Statement of Operations Data:					
Revenue	$ —	$ —	$ —	$ —	$ —
Operating expenses:					
Research and development	7,370	12,201	5,148	7,948	57,314
General and administrative	2,279	3,548	1,687	2,829	16,286
Total operating expenses	9,649	15,749	6,835	10,777	73,600
Loss from operations	(9,649)	(15,749)	(6,835)	(10,777)	(73,600)
Other income (expense):					
Interest income	—	—	—	—	216
Interest expense	—	—	—	—	(302)
Other income (expense), net	—	24	—	79	342
Total other income, net	—	24	—	79	256
Net loss	(9,649)	(15,725)	(6,835)	(10,698)	(73,344)
Accretion of redeemable convertible preferred stock to redemption value	(34)	(94)	(79)	—	(3,359)
Accrual of dividend on redeemable convertible preferred stock	—	—	—	—	(347)
Modifications of redeemable convertible preferred stock	—	—	—	—	9,925
Net loss attributable to common stockholders	$(9,683)	$(15,819)	$(6,914)	$(10,698)	$ (67,125)
Net loss per share attributable to common stockholders, basic and diluted[1]	$(31.09)	$ (38.02)	$(20.49)	$ (21.48)	
Weighted average common shares outstanding, basic and diluted[1]	311	416	337	498	
Pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)[1]		$ (1.29)		$ (0.70)	
Pro forma weighted average common shares outstanding, basic and diluted (unaudited)[1]		12,230		15,358	

	As of December 31,		As of June 30,
	2012	2013	2014
	(in thousands)		
Consolidated Balance Sheet Data:			
Cash and cash equivalents	$ 11,691	$ 31,753	$ 21,150
Working capital(2)	9,908	29,969	18,051
Total assets	11,962	32,287	23,420
Redeemable convertible preferred stock	49,845	85,345	85,345
Total stockholders' deficit	(39,901)	(55,267)	(65,613)

(1) See Note 10 to our consolidated financial statements for further details on the calculation of basic and diluted net loss per share attributable to common stockholders and the calculation of basic and diluted pro forma net loss per share attributable to common stockholders.

(2) We define working capital as current assets less current liabilities.

Management's Discussion and Analysis of Financial Condition and Results of Operations

You should read the following discussion and analysis of financial condition and results of operations together with the section entitled "Selected Consolidated Financial Data" and our consolidated financial statements and related notes included elsewhere in this prospectus. The information contained in this discussion and analysis or set forth elsewhere in this prospectus contains forward-looking statements that involve risks and uncertainties, such as statements regarding our plans, objectives, expectations, intentions and projections. Our actual results could differ materially from the results described in or implied by these forward-looking statements as a result of many important factors, including those factors set forth in the "Risk Factors" section of this prospectus.

Overview

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. 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.

In addition to our planned pivotal Phase 3 clinical trial, we are 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, as well as in hormone-sensitive prostate cancer. 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. To date, we have funded our operations primarily through private placements of redeemable convertible preferred stock and, to a lesser extent, through the issuances of convertible promissory notes, none of which are currently outstanding. From our inception through June 30, 2014, we have received aggregate gross proceeds of $92.5 million from such transactions.

We are a development stage company and have not generated any revenue. We have incurred net losses in each year since our inception, and we have an accumulated deficit of $73.8 million as of June 30, 2014. 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 $10.7 million for the six months ended June 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, 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 following this offering.

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 June 30, 2014, we had cash and cash equivalents of $21.2 million. We expect that the net proceeds from this offering, together with our existing cash and cash equivalents, 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 since inception have consisted primarily 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:

	Year Ended December 31,		Six Months Ended June 30,		Cumulative Period from Inception (March 26, 2004) to June 30, 2014
	2012	2013	2013	2014	
			(in thousands)		
Galeterone for prostate cancer	$ 5,417	$10,257	$ 4,185	$ 6,481	$ 39,476
Other early-stage development programs and additional indications for galeterone	18	40	17	49	2,371
Unallocated research and development expenses	1,935	1,904	946	1,418	15,467
Total research and development expenses	$ 7,370	$12,201	$ 5,148	$ 7,948	$ 57,314

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 and, for years prior to 2012, marketable securities. Our interest income has not been significant due to nominal investment balances and low interest earned on those balances. We anticipate that our interest income will increase in the future due to anticipated cash proceeds from this offering.

Interest Expense. Interest expense consists of interest expense on our convertible promissory notes at the stated interest rates and interest expense related to the amortization of deferred financing costs associated with our issuances of the convertible promissory notes. Prior to 2012, all of our convertible promissory notes and

accrued interest had been converted into shares of our redeemable convertible preferred stock. As a result, we no longer incur interest expense related to this debt.

Other Income (Expense), Net. Other income (expense), net, primarily consists of other income for the year ended December 31, 2010 related to the Qualifying Therapeutic Discovery Project, or QTDP, reimbursement program of the United States government, which provided for reimbursement of certain qualifying costs. We do not anticipate any further income related to the QTDP program. 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. See "—Liquidity and Capital Resources—Net Operating Loss Carryforwards and Other Deferred Tax Assets."

Critical Accounting Policies and Significant Judgments and Estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements, which we have prepared in accordance with generally accepted accounting principles in the United States. The preparation of our consolidated financial statements and related disclosures requires us to make estimates and assumptions that affect the reported amount of assets, liabilities, revenue, costs and expenses and related disclosures. We believe that the estimates and assumptions involved in the accounting policies described below may have the greatest potential impact on our consolidated financial statements and, therefore, consider these to be our critical accounting policies. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions and conditions. See also Note 2 of our consolidated financial statements included elsewhere in this prospectus for information about these critical accounting policies as well as a description of our other significant accounting policies.

Accrued Research and Development Expenses

As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued research and development expenses. This process involves reviewing open contracts and purchase orders, communicating with our personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated costs incurred for the services when we have not yet been invoiced or otherwise notified of the actual costs. The majority of our service providers invoice us in arrears for services performed, on a pre-determined schedule or when contractual milestones are met; however, some require advanced payments. We make estimates of our accrued expenses as of each balance sheet date in our consolidated financial statements based on facts and circumstances known to us at that time. Examples of estimated accrued research and development expenses include fees paid to:

- clinical research organizations in connection with clinical trials;
- investigative sites or other providers in connection with clinical trials;
- vendors in connection with preclinical development activities; and
- vendors related to product manufacturing, development and distribution of clinical supplies.

We base our expenses related to clinical trials on our estimates of the services received and efforts expended pursuant to contracts with multiple clinical research organizations and investigative sites that manage and conduct clinical trials on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the clinical expense. Payments under some of these contracts depend on factors such as the successful enrollment of patients and the completion of clinical trial milestones. In accruing service fees, we estimate the time period over which services will be performed, enrollment of patients, number of sites activated and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual or amount of prepaid expense accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in us reporting amounts that are too high or too low in any particular period. To date, we have not made any material adjustments to our prior estimates of accrued research and development expenses.

Stock-Based Compensation

We measure the fair value of stock-based awards granted to employees and directors on the date of grant and recognize the corresponding compensation expense of those awards, net of estimated forfeitures, over the requisite service period, which is generally the vesting period of the respective award. Our stock-based awards typically vest over four years. Generally, we issue stock options and restricted stock awards with service-based vesting conditions and record the expense for these awards using the straight-line method. We have historically granted stock options with exercise prices equivalent to the fair value of our common stock as of the date of grant.

We measure the fair value of stock-based awards granted to non-employees on the date at which the related service is complete. Compensation expense is recognized over the period during which services are rendered by such non-employee consultants until completed. At the end of each financial reporting period prior to completion of the service, the fair value of these awards is re-measured using, for options, the then-current fair value of our common stock and updated assumption inputs in the Black-Scholes option-pricing model and using, for restricted stock, the then-current fair value of our common stock.

The fair value of each stock option grant is estimated using the Black-Scholes option-pricing model, which uses as inputs the fair value of our common stock and assumptions for volatility, expected term, risk-free interest rate and dividend yield, determined as follows:

- *Fair Value of Common Stock*. Because our common stock is not publicly traded, we must estimate its fair value, as discussed in "—Determination of the Fair Value of Common Stock" below.
- *Volatility*. We historically have been a private company and lack company-specific historical and implied volatility information. Therefore, we estimate our expected volatility based on the historical volatility of our publicly traded peer companies and expect to continue to do so until such time as we have adequate historical data regarding the volatility of our traded stock price.
- *Expected Term*. We use the simplified method prescribed by Securities and Exchange Commission Staff Accounting Bulletin No. 107, *Share-Based Payment*, to calculate the expected term of options granted to employees and directors, and we determine the expected term of options granted to non-employees based on the contractual term of the options.
- *Risk-Free Interest Rate*. 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.
- *Dividend Yield*. Expected dividend yield is based on the fact that we have never paid cash dividends and do not expect to pay any cash dividends in the foreseeable future.

The following table sets forth the assumptions we used to determine the fair value of stock options granted, presented on a weighted average basis:

	Year Ended December 31,		Six Months Ended June 30,	
	2012	2013	2013	2014
Risk-free interest rate	0.79%	1.72%	1.71%	1.87%
Expected term (in years)	6.07	5.98	5.99	5.89
Expected volatility	65.5%	79.7%	79.6%	79.2%
Expected dividend yield	0%	0%	0%	0%

The assumptions used in determining the fair value of stock-based awards represent our best estimates, which involve inherent uncertainties and the application of our judgment. As a result, if factors change and we use significantly different assumptions or estimates, our stock-based compensation expense could be materially different in the future.

We recognize compensation expense for only the portion of awards that are expected to vest. In developing a forfeiture rate estimate for pre-vesting forfeitures, we have considered our historical experience of actual forfeitures. If our future actual forfeiture rate is materially different from our estimate, our stock-based compensation expense could be significantly different from what we have recorded in the current period.

The following table summarizes the classification of our stock-based compensation expenses recognized in our consolidated statements of operations:

	Year Ended December 31,		Six Months Ended June 30,	
	2012	2013	2013	2014
	(in thousands)			
Research and development	$ 87	$ 91	$ 41	$ 136
General and administrative	123	147	49	204
	$ 210	$ 238	$ 90	$ 340

Determination of the Fair Value of Common Stock

We are a privately held company with no active public market for our common stock. Therefore, our board of directors has estimated the fair value of our common stock at various dates, with input from management, considering our most recently available third-party valuations of common stock and its assessment of additional objective and subjective factors that it believed were relevant and which may have changed from the date of the most recent valuation through the date of the grant. Once a public trading market for our common stock has been established in connection with the closing of this offering, it will no longer be necessary for our board of directors to estimate the fair value of our common stock in connection with our accounting for granted stock options.

In the absence of a public trading market for our common stock, our determination of the fair value of our common stock was performed using methodologies, approaches and assumptions consistent with the *American Institute of Certified Public Accountants Audit and Accounting Practice Aid Series: Valuation of Privately-Held-Company Equity Securities Issued as Compensation*, which we refer to as the Practice Aid. We performed these contemporaneous valuations, with the assistance of a third-party specialist, as of December 31, 2012, May 13, 2013, December 1, 2013, February 12, 2014 and April 3, 2014, which resulted in valuations of our common stock of $1.37, $1.58, $3.67, $4.19 and $6.50 per share, respectively, as of those dates. In addition, our board of directors considered various objective and subjective factors, along with input from management, to determine its best estimate of the fair value of our common stock as of each grant date, including the following:

- the prices of shares of our preferred stock that we had sold and the rights, preferences and privileges of that preferred stock relative to our common stock;

- the progress of our research and development programs, including the status of clinical trials for our product candidates;
- our stage of development and business strategy;
- our financial condition, including cash on hand;
- our historical and forecasted performance and operating results;
- the composition of, and changes to, our management team and board of directors;
- the lack of an active public market for our common stock as a private company;
- the likelihood of achieving a liquidity event, such as a sale of our company or an initial public offering, or IPO, given prevailing market conditions;
- the analysis of initial public offerings and the market performance of similar companies in the biopharmaceutical industry; and
- external market conditions affecting the biopharmaceutical industry.

There are significant judgments and estimates inherent in these valuations. These judgments and estimates include assumptions regarding our future operating performance, the stage of development of our product candidates, the timing of a potential IPO or other liquidity event and the determination of the appropriate valuation methodology at each valuation date. If we had made different assumptions, our stock-based compensation expense, net loss attributable to common stockholders and net loss per share attributable to common stockholders could have been significantly different.

Valuation Methodologies

Our common stock valuations as of December 31, 2012 and May 13, 2013 were prepared utilizing the option-pricing method, or OPM, as described in the Practice Aid, to determine the estimated fair value of our common stock. Our common stock valuations as of December 1, 2013, February 12, 2014 and April 3, 2014 were prepared utilizing the probability-weighted expected return method, or PWERM, as described in the Practice Aid, to determine the estimated fair value of our common stock. The method selected was based on availability and the quality of information to develop the assumptions for the methodology.

OPM. The OPM treats common stock and preferred stock as call options on the total equity value of a company, with exercise prices based on the value thresholds at which the allocation among the various holders of a company's securities changes. Under this method, the common stock has value only if the funds available for distribution to stockholders exceed the value of the preferred stock liquidation preference at the time of a liquidity event, such as a strategic sale or merger. The common stock is modeled as a call option on the underlying equity value at a predetermined exercise price. In the model, the exercise price is based on a comparison with the total equity value rather than, as in the case of a regular call option, a comparison with a per share stock price. Thus, common stock is considered to be a call option with a claim on the enterprise at an exercise price equal to the remaining value immediately after the preferred stock liquidation preference is paid.

The OPM uses the Black-Scholes option-pricing model to price the call options. This model defines the securities' fair values as functions of the current fair value of a company and uses assumptions such as the anticipated timing of a potential liquidity event and the estimated volatility of the equity securities.

We estimated enterprise value used in the OPM using either the guideline public company method and the guideline transaction method or the OPM backsolve method. The guideline public company method includes comparisons to companies with several years of trading history as well as recent IPOs. The guideline transaction method evaluates market multiples indicated by recent acquisitions of companies in the relevant industry. The OPM backsolve method uses the OPM to calculate the implied equity value for one type of security based on

recent sales transaction involving another type of the company's equity securities. In the OPM, the assumed volatility factor was based on the historical trading volatility of our publicly traded peer companies. At each valuation date, a determination was made by us as to the appropriate volatility to be used, considering such factors as the expected time to a liquidity event and our stage of development.

To derive the fair value of the common stock using the OPM, the proceeds to the common stockholders were calculated based on the preferences and priorities of the preferred and common stock, including the participation features of certain series of the preferred stock. We then applied a discount for lack of marketability to the common stock to account for the lack of access to an active public market. The aggregate value of the common stock derived from the OPM is then divided by the number of shares of common stock outstanding to arrive at a per-share value.

PWERM. The PWERM is a scenario-based methodology that estimates the fair value of common stock based upon an analysis of future values for the company, assuming various outcomes. We considered four scenarios for the valuation of our common stock determined using the PWERM methodology: an IPO scenario, a longer-term strategic sale scenario, a near-term sale scenario and a low-value sale scenario.

For the IPO, near-term and low-value sale scenarios, the enterprise value was determined using the guideline public company method, which considered the pricing of recent IPOs by comparable clinical-stage biopharmaceutical companies. In determining the enterprise value for these scenarios, we considered the multiples of paid-in capital indicated by these IPOs as well as the pricing of each IPO relative to the pricing of the most recent preferred round preceding comparable biopharmaceutical companies that were acquired from 2011 through January 2014.

The common stock value is based on the probability-weighted present value of expected future investment returns considering each of the possible outcomes available as well as the rights of each class of stock. The future value of the common stock under each outcome is discounted back to the valuation date at an appropriate risk-adjusted discount rate and probability weighted to arrive at an indication of value for the common stock. We applied a discount for lack of marketability to the common stock to account for the lack of access to an active public market.

Option Grants

The following table summarizes by grant date the number of shares subject to options granted between January 1, 2013 and July 31, 2014, the per share exercise price of the options, the fair value of common stock underlying the options on date of grant and the per share estimated fair value of the options:

Grant Date	Number of Shares Subject to Options Granted	Per Share Exercise Price of Options[1]	Fair Value of Common Stock on Date of Option Grant	Per Share Estimated Fair Value of Options[2]
June 26, 2013 (non-employee award)	8,262	$ 1.58	$ 1.58	$ 1.26[3]
June 26, 2013	716,577	$ 1.58	$ 1.58	$ 1.05
December 11, 2013	61,693	$ 3.67	$ 3.67	$ 2.51
February 25, 2014	209,178	$ 4.19	$ 4.19	$ 2.83
March 17, 2014	2,387	$ 4.19	$ 4.19	$ 2.83
April 8, 2014	312,568	$ 6.50	$ 6.50	$ 4.40

(1) The per share exercise price of options represents the fair value of our common stock on the date of grant, as determined by our board of directors after taking into account our most recently available contemporaneous valuations of our common stock as well as additional factors that may have changed since the date of such contemporaneous valuation through the date of grant.

(2) The per share estimated fair value of options reflects the weighted average fair value of options granted on each grant date as estimated at the date of grant using the Black-Scholes option-pricing model. This model

estimates the fair value using as inputs the exercise price of the option and assumptions of the risk-free interest rate, expected term of the option, expected share price volatility of the underlying common stock, expected dividends on the underlying common stock and the per share fair value of the underlying common stock.

(3) For the purposes of recording stock-based compensation, we measure the fair value of options on the service completion date (vesting date). At the end of each reporting period prior to completion of the services, we re-measure the value of the unvested portion of the outstanding options at the then-current fair value of our common stock and updated assumption inputs in the Black-Scholes option-pricing model.

JOBS Act

In April 2012, the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, was enacted. The JOBS Act contains provisions that, among other things, reduce certain reporting requirements for an "emerging growth company," as defined in the JOBS Act. As an emerging growth company, we have irrevocably elected not to avail ourselves of the extended transition period for complying with 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.

In addition, we are in the process of evaluating the benefits of relying on the other exemptions and reduced reporting requirements provided by the JOBS Act. Subject to certain conditions set forth in the JOBS Act, if we choose to rely on such exemptions, for so long as we remain an emerging growth company, we will not be required to, among other things:

- provide an auditor's attestation report on our system of internal controls over financial reporting pursuant to Section 404 of the Sarbanes-Oxley Act of 2002;
- provide all of the compensation disclosure that may be required of non-emerging growth companies under the Dodd-Frank Wall Street Reform and Consumer Protection Act;
- 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; and
- comply with certain disclosure obligations regarding executive compensation.

We may take advantage of some or all these exemptions for up to five years or such earlier time that we are no longer an emerging growth company. We would cease to be an emerging growth company if we have more than $1 billion in annual revenue, we have more than $700 million in market value of our stock held by non-affiliates or we issue more than $1 billion of non-convertible debt over a three-year period.

Results of Operations

Comparison of Six Months Ended June 30, 2013 and 2014

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

	Six Months Ended June 30,		Increase (Decrease)
	2013	2014	
	(in thousands)		
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development	5,148	7,948	2,800
General and administrative	1,687	2,829	1,142
Total operating expenses	6,835	10,777	3,942
Loss from operations	(6,835)	(10,777)	(3,942)
Other income (expense):			
Other income	—	79	79
Total other income, net	—	79	79
Net loss	$ (6,835)	$(10,698)	$(3,863)

Research and development expenses

	Six Months Ended June 30,		Increase (Decrease)
	2013	2014	
	(in thousands)		
Galeterone for prostate cancer	$ 4,185	$ 6,481	$ 2,296
Other early-stage development programs and additional indications for galeterone	17	49	32
Unallocated research and development expenses	946	1,418	472
Total research and development expenses	$ 5,148	$ 7,948	$ 2,800

Research and development expenses for the six months ended June 30, 2013 were $5.1 million, compared to $7.9 million for the six months ended June 30, 2014. The increase was primarily due to increased costs of $2.3 million associated with our galeterone for prostate cancer program and an increase in unallocated research and development expenses of $0.5 million. The increase in costs of our galeterone for prostate cancer program consisted primarily of increased costs of clinical trials of $2.6 million, partially offset by decreased manufacturing costs of $0.5 million. The increase in clinical trial costs was due to an increased number of patients and sites in our ARMOR2 trial in the six months ended June 30, 2014 as compared to the six months ended June 30, 2013. The decrease in manufacturing costs primarily consisted of decreased drug product cost, reflecting the purchase of raw materials in the three months ended June 30, 2013, partially offset by increased manufacturing costs as we increased our manufacturing of galeterone in the six months ended June 30, 2014 for our ARMOR2 trial and in anticipation of our planned pivotal Phase 3 clinical trial of galeterone. The increase in unallocated research and development costs of $0.5 million for the six months ended June 30, 2014 from the six months ended June 30 2013 was due to increased personnel related costs as a result of increased headcount in our research and development function, including the additions of our Chief Medical Officer and our Vice President of Medical Affairs in the three months ended June 30, 2014.

General and administrative expenses

General and administrative expenses for the six months ended June 30, 2013 were $1.7 million, compared to $2.8 million for the six months ended June 30, 2014. The increase of $1.1 million in general and administrative

expenses was primarily due to an increase in professional fees of $0.8 million as well as an increase in personnel related costs of $0.2 million. The increase in professional fees primarily consisted of a $0.8 million increase in accounting, audit and legal fees associated with ongoing business activities and our preparations to operate as a public company. Personnel related costs increased by $0.2 million primarily due to an increase in headcount in our general and administrative function in the six months ended June 30, 2014, including the addition of our Chief Operating Officer in the three months ended March 31, 2014, partially offset by a decrease in personnel related costs due to severance paid to our former Chief Executive Officer in the six months ended June 30, 2013.

Comparison of Year Ended December 31, 2012 and 2013

The following table summarizes our results of operations for the year ended December 31, 2012 and 2013:

	Year Ended December 31,		Increase (Decrease)
	2012	2013	
	(in thousands)		
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development	7,370	12,201	4,831
General and administrative	2,279	3,548	1,269
Total operating expenses	9,649	15,749	6,100
Loss from operations	(9,649)	(15,749)	(6,100)
Other income (expense):			
Other income	—	24	24
Total other income, net	—	24	24
Net loss	$ (9,649)	$(15,725)	$(6,076)

Research and development expenses

	Year Ended December 31,		Increase (Decrease)
	2012	2013	
	(in thousands)		
Galeterone for prostate cancer	$ 5,417	$10,257	$ 4,840
Other early-stage development programs and additional indications for galeterone	18	40	22
Unallocated research and development expenses	1,935	1,904	(31)
Total research and development expenses	$ 7,370	$12,201	$ 4,831

Research and development expenses for the year ended December 31, 2012 were $7.4 million, compared to $12.2 million for the year ended December 31, 2013. The increase was primarily due to increased costs of $4.8 million associated with our galeterone for prostate cancer program, consisting primarily of increased manufacturing costs of $2.8 million and increased costs of clinical trials of $2.0 million. These increases were due to the higher costs associated with our ARMOR2 trial of galeterone, manufacturing galeterone for use in our ARMOR2 trial and further developing the manufacturing process for our spray dried dispersion formulation. During 2012, we focused our research and development efforts on the reformulation of galeterone from our product in capsule formulation to our spray dried dispersion formulation and a bridging Phase 1 clinical trial.

General and administrative expenses

General and administrative expenses for the year ended December 31, 2012 were $2.3 million, compared to $3.5 million for the year ended December 31, 2013. The increase of $1.2 million in general and administrative expenses was primarily due to an increase in professional fees of $0.6 million and an increase in personnel related costs of $0.5 million year over year. The increase in professional fees consisted of a $0.4 million increase in accounting, audit and legal fees due to ongoing business activities and our preparations to operate as a public company as well as an increase of $0.2 million related to business development activities. Personnel related costs increased by $0.5 million year over year primarily due to severance costs of $0.4 million in 2013 paid to our former Chief Executive Officer.

Liquidity and Capital Resources

Since our inception, 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.

We have funded our operations since inception primarily through private placements of redeemable convertible preferred stock and, to a lesser extent, through the issuances of convertible promissory notes, none of which are currently outstanding. From our inception through June 30, 2014, we have received aggregate gross proceeds of $92.5 million from such transactions.

As of June 30, 2014, we had cash and cash equivalents of $21.2 million. We invest our cash equivalents in money market accounts in order to preserve principal.

Cash Flows

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

	Year Ended December 31,		Six Months Ended June 30,	
	2012	2013	2013	2014
	(in thousands)			
Cash used in operating activities	$ (9,333)	$(15,476)	$ (5,674)	$(10,197)
Cash used in investing activities	(8)	(53)	(33)	(18)
Cash provided by (used in) financing activities	18,779	35,591	20,136	(388)
Net increase (decrease) in cash and cash equivalents	$ 9,438	$ 20,062	$14,429	$(10,603)

Net cash used in operating activities. During the six months ended June 30, 2013, cash used in operating activities was $5.7 million, resulting primarily from our net loss of $6.8 million, partially offset by net cash provided by changes in our operating assets and liabilities of $1.1 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 six months ended June 30, 2013 consisted primarily of a $1.0 million increase in accounts payable and accrued expenses. Our accounts payable and accrued expenses balances were affected by the timing of vendor invoicing and payments.

During the six months ended June 30, 2014, cash used in operating activities was $10.2 million, resulting from our net loss of $10.7 million, partially offset by net non-cash charges of $0.3 million and by net cash provided by changes in our operating assets and liabilities of $0.2 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 $0.3 million. Cash provided by changes in our operating assets and liabilities consisted primarily of a net increase in accounts payable and accrued expenses of $0.5 million, 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.

During the year ended December 31, 2012, cash used in operating activities was $9.3 million, primarily resulting from our net loss of $9.6 million, partially offset by non-cash charges of $0.2 million and by cash provided from changes in our operating assets and liabilities of $0.1 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 year ended December 31, 2012 consisted primarily of stock-based compensation expense of $0.2 million.

During the year ended December 31, 2013, cash used in operating activities was $15.5 million, resulting from our net loss of $15.7 million, partially offset by non-cash charges of $0.2 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 year ended December 31, 2013 consisted primarily of stock-based compensation expense of $0.2 million.

Net cash used in investing activities. We used a small amount of cash during the years ended December 31, 2012 and 2013 and the six months ended June 30, 2014 related to purchases of property and equipment.

Net cash provided by (used in) financing activities. During the six months ended June 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.

During the six months ended June 30, 2014, cash used in financing activities was $0.4 million, consisting primarily of payments of deferred offering costs of $0.5 million related to our anticipated initial public offering of common stock, partially offset by the collection of cash related to our outstanding note receivable from a stockholder.

During the year ended December 31, 2012, net cash provided by financing activities was $18.8 million, resulting from net proceeds from the sale and issuance of our Series D-3 redeemable convertible preferred stock.

During the year ended December 31, 2013, net cash provided by financing activities was $35.6 million, resulting from net proceeds of $35.4 million from the sale and issuance of our Series E redeemable convertible preferred stock, as well as $0.2 million received 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, 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 following this offering.

As of June 30, 2014, we had cash and cash equivalents of $21.2 million. We expect that the net proceeds from this offering, together with our existing cash and cash equivalents, 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.

Net Operating Loss Carryforwards and Other Deferred Tax Assets

Since our inception, 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 net operating loss carryforwards of $10.5 million, which begin to expire in 2024. As of December 31, 2013, we had state net operating loss carryforwards of $8.1 million, which began to expire in 2014. As of December 31, 2013, we also had federal and state research and development tax credit carryforwards of $0.6 million and $0.4 million, 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. We expect to begin to amortize this capitalized asset for tax purposes, which will increase our net operating loss carryforwards, over a period of five years, commencing once we begin to recognize product or collaboration revenue. Ownership changes, as defined in Section 382 of the U.S. Internal Revenue Code of 1986, as amended, or the Code, limit the amount of net operating loss carryforwards and research and development credit carryforwards we can use each year to offset future taxable income and taxes payable. We have not completed a study to assess whether a change in ownership, as defined under Section 382 of the Code, has occurred or whether there have been multiple changes in ownership since our inception due to the significant costs and complexities associated with such studies. Accordingly, our ability to utilize our tax carryforwards may be limited. Additionally, U.S. federal and state tax laws limit the time during which these carryforwards may be utilized against future taxes. As a result, we may be unable to take full advantage of these carryforwards for U.S. federal and state tax purposes.

Contractual Obligations and Commitments

As of June 30, 2014, we did not have any significant contractual obligations or commitments.

We lease office space and obtain office support services in Cambridge, Massachusetts under a 30-day cancelable operating service agreement under which a small minimum monthly amount is required.

We are party to a license agreement with UMB. In consideration for the rights granted to us, we made an upfront payment to UMB of $20,000 following the execution of the license agreement and a payment of $10,000 following the execution of each of the March 2009, April 2012 and October 2013 amendments. We are obligated to pay UMB an annual maintenance fee of $10,000 each year until the first commercial sale. In addition, we paid UMB a $50,000 milestone payment upon the submission of our investigational new drug application, or IND, for galeterone and a $40,000 milestone payment upon the issuance of the first patent related to UMB's prodrug patent application. We are obligated to make an additional $50,000 milestone payment to UMB for each additional IND we file for a licensed product and a $100,000 milestone payment upon the approval of each NDA for a licensed product by the FDA. We must also pay UMB low-single digit percentage royalties on aggregate worldwide net sales of licensed products, including sales by our 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. Our royalty obligations are subject to specified reductions in the event that we are required to obtain additional licenses from third parties or in the event of specified competition from third-party products licensed by UMB. Our minimum annual royalty payment to UMB is $50,000 beginning in the year following the year in which the first commercial sale occurs. We must also pay UMB 10% of all non-royalty sublicense income received from sublicensees. Finally, as of April 10, 2012, we assumed responsibility for all patent expenses related to the prosecution and maintenance of the licensed patents. As of June 30, 2014, we have not yet developed a commercial product using the licensed technologies and we have not entered into any sublicense agreements for the technologies.

In November 2010, we assigned rights to develop and commercialize certain compounds that were unrelated to our core operations to Diotima Pharmaceuticals, Inc., or Diotima, which was then our wholly owned subsidiary, and then spun off Diotima to our existing stockholders. In connection with various agreements between us and Diotima, we funded certain license and license maintenance fees during the period from the spin-off in 2010 through June 30, 2014. In February and April 2014, we terminated the license agreements relating to these compounds on behalf of Diotima and believe that no further payments are due to these licensors. In April 2014, the board of directors and stockholders of Diotima approved the dissolution of Diotima, and Diotima was dissolved.

We enter into contracts in the normal course of business with contract research organizations for clinical trials, preclinical research studies and testing, manufacturing and other services and products for operating purposes. These contracts generally provide for termination upon notice, and therefore we believe that our non-cancelable obligations under these agreements are not material.

We previously agreed to pay a fee to a financial advisor upon the closing of this offering equal to the greater of $0.5 million and 1% of the gross proceeds of this offering for strategic and financial advisory services unrelated to this offering.

Off-Balance Sheet Arrangements

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

Recently Issued Accounting Pronouncements

In June 2014, the Financial Accounting Standards Board, or 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. These presentation and disclosure requirements will no longer be required for the first annual period beginning after

December 15, 2014 for public companies. Early application is permitted for interim and annual periods for which financial statements have not yet been issued or made available for issuance. Effective upon our adoption of this guidance, we will no longer disclose inception-to-date information currently included in our consolidated statements of operations and comprehensive loss, of cash flows, and of redeemable convertible preferred stock and stockholders' deficit and the related notes thereto.

Other accounting standards that have been issued or proposed by the FASB or other standards-setting bodies that do not require adoption until a future date are not expected to have a material impact on our consolidated financial statements upon adoption.

Quantitative and Qualitative Disclosure about Market Risk

Interest Rate Fluctuation Risk

Our cash and cash equivalents as of June 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.

Business

Overview

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. 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 patient populations showed clinically meaningful reductions in levels of prostate specific antigen, or PSA, 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. We intend to conduct our pivotal Phase 3 clinical trial in these patients who we believe may not be effectively treated by the therapies approved by the FDA in recent years. We believe that one of galeterone's multiple mechanisms of action, androgen receptor degradation, provides an opportunity to treat this population of patients. In our ongoing Phase 2 clinical trial of galeterone, which we refer to as our ARMOR2 trial, we observed clinically meaningful PSA reductions in patients that were identified as having altered androgen receptors that were truncated in a retrospective subset analysis of seven patients. Six of these patients showed clinically meaningful PSA reductions of at least 50%. Although our initial development focus is on galeterone for the treatment of this population of patients, we are conducting our Phase 2 ARMOR2 trial of galeterone in multiple CRPC patient populations.

Galeterone acts by disrupting the androgen receptor signaling pathway, which is the primary pathway that drives prostate cancer growth. The pathway is ordinarily activated by the binding of male hormones, or androgens, such as testosterone and the more potent androgen dihydrotestosterone, or DHT, to the ligand binding domain of androgen receptors in prostate cancer cells. Galeterone disrupts the activation of the pathway through multiple mechanisms of action:

- inhibition of the enzyme CYP17, which blocks the synthesis of testosterone;
- androgen receptor antagonism, which blocks the binding of testosterone or DHT with the androgen receptor; and
- androgen receptor degradation, which reduces the amount of androgen receptor protein in the tumor cells.

We believe that, in comparison to therapies that act solely through CYP17 inhibition or androgen receptor antagonism, galeterone's unique combination of mechanisms of action may provide galeterone with advantages in efficacy in the treatment of CRPC and may reduce the risk of or delay the development of resistance to therapy and provide efficacy in patients with tumors resistant to other treatments.

The truncated androgen receptors for which we are developing galeterone 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 plan to conduct our pivotal Phase 3 clinical trial in patients with AR-V7. In patients with C-terminal loss, including AR-V7, the lack of a functional ligand binding domain causes the truncated androgen receptor to be constitutively active, or continuously signaling, meaning that activation of the androgen receptor pathway and tumor growth occurs even in the absence of androgens and androgen binding. In clinical studies conducted by researchers at MD Anderson Cancer Center, or MD Anderson, and Johns Hopkins University, or Johns Hopkins, the presence in patients of truncated androgen receptors with C-terminal loss and AR-V7 was associated with poor responsiveness of patients' prostate tumors to treatment with Zytiga (abiraterone acetate) and Xtandi (enzalutamide), two of the highest selling therapies for

CRPC with aggregate reported worldwide 2013 sales of more than $2.1 billion. We believe that these studies indicate that there is a need for effective treatments for CRPC patients with C-terminal loss, including AR-V7.

We are currently finalizing our plans for our pivotal Phase 3 clinical trial of galeterone, which we refer to as our ARMOR3-Splice Variant, or SV, trial. 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 up to 170 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 PSA levels, overall survival and safety. We expect to commence the trial in the first half of 2015 and, subject to patient enrollment rates and the rates of disease progression in patients in the trial, to have top-line data from the trial by the end of 2016. We also plan to establish an independent data monitoring committee.

In addition to our planned pivotal Phase 3 clinical trial, we are conducting our ARMOR2 trial of galeterone for the treatment of multiple CRPC populations. 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, as well as in hormone-sensitive prostate cancer. We have exclusive worldwide development and commercialization rights to galeterone.

In June 2012, the FDA designated galeterone for the treatment of CRPC for fast track review. The FDA may designate a product for fast track review if it is intended, whether alone or in combination with one or more other drugs, for the treatment of a serious or life-threatening disease or condition, and it demonstrates the potential to address unmet medical needs for such a disease or condition. For fast track products, sponsors may have more frequent interactions with the FDA, and the FDA may initiate review of sections of a fast track product's new drug application, or NDA, on a rolling basis before the application is complete. In addition, sponsors may request and be granted priority review of their application.

According to the American Cancer Society, in the United States, prostate cancer is the most frequently diagnosed cancer among men other than skin cancer. The American Cancer Society estimates that, in the United States during 2014, approximately 233,000 new cases of prostate cancer will be diagnosed, and approximately 29,000 men will die from the disease. Prostate cancer drugs represent a large and growing market. According to Decision Resources Group, an independent research firm, sales of prostate cancer drugs are expected to increase from $6.0 billion in 2013 to more than $9.0 billion in 2021, due to a growing aged population, a rising incidence of cancer and the introduction of new drugs for the treatment of prostate cancer. These new drugs include Zytiga and Xtandi, which are approved for the treatment of CRPC. Although Zytiga was only approved in 2011 and Xtandi in 2012, both of these drugs have experienced rapid sales growth, with reported worldwide 2013 sales of $1.7 billion for Zytiga and $445 million for Xtandi. Despite their success, the need for new treatment options remains as each of these drugs has treatment limitations in CRPC patients and may not be effective in CRPC patients with C-terminal loss, including AR-V7.

The growth and survival of prostate cancer tumor cells depend primarily on the functioning of the androgen receptor signaling pathway. Ordinarily, the pathway and tumor growth are activated by the binding of testosterone and DHT to the ligand binding domain of androgen receptors. As a result, therapies that block this binding can be effective in disrupting the pathway and tumor cell growth. Zytiga blocks this binding by reducing the synthesis of testosterone through the inhibition of the enzyme CYP17. Xtandi blocks the binding of testosterone or DHT with the androgen receptor through androgen receptor antagonism. However, the effectiveness of Zytiga, Xtandi and other therapies based solely on one of these mechanisms of action requires a functional ligand binding domain. In the case of prostate tumor cells that express truncated androgen receptors with C-terminal loss, including AR-V7, there is no functional ligand binding domain, which causes the truncated androgen receptor to be constitutively active. As a result, we believe that patients with truncated androgen receptors with C-terminal loss may not be effectively treated by these therapies.

In contrast, galeterone disrupts the androgen receptor signaling pathway at multiple points by combining the mechanisms of action of CYP17 inhibition and androgen receptor antagonism with the mechanism of androgen

receptor degradation. Unlike CYP17 inhibition and androgen receptor antagonism, androgen receptor degradation does not require a functional ligand binding domain to disrupt the activation of the pathway and tumor growth. As a result, we believe that, based on galeterone's multiple mechanisms of action, data from a subset of patients in our ARMOR2 trial and data from preclinical studies conducted by us and independent laboratories, galeterone may have the ability to treat both patients with full-length androgen receptors and patients with C-terminal loss. To our knowledge, there are no approved drugs or drugs in clinical development, other than galeterone, that disrupt the androgen receptor signaling pathway through androgen receptor degradation.

Interim Clinical Trial Results. In May 2014, we announced interim data from our ARMOR2 trial at The American Society of Clinical Oncology 2014 Annual Meeting, or ASCO. The interim data included patients who had not previously undergone chemotherapy and had not received treatment with Zytiga or Xtandi, whom we refer to as CRPC treatment-naïve patients, and patients whose disease progressed during treatment with Zytiga, whom we refer to as Zytiga-refractory patients. We reported that, as of May 12, 2014, our cut-off date for our data presentation at ASCO, in 51 evaluable CRPC treatment-naïve patients, galeterone showed clinically meaningful reductions in levels of PSA. Specifically, we reported the following:

- *Non-metastatic and metastatic CRPC treatment-naïve patients at the selected Phase 2 dose of 2550 mg/day:* During the first 12 weeks of dosing, 82% of patients showed maximal reduction in PSA levels of at least 30%, and 75% of patients showed maximal reduction in PSA levels of at least 50%.
- *Metastatic CRPC treatment-naïve patients at the selected Phase 2 dose of 2550 mg/day:* During the first 12 weeks of dosing, 85% of patients showed maximal reduction in PSA levels of at least 30%, and 77% of patients showed maximal reduction in PSA levels of at least 50%.

We also reported 12-week data for 15 Zytiga-refractory patients, two of whom had a maximal reduction in PSA levels of at least 30%.

In addition, we presented data from a retrospective subset analysis in which four treatment-naïve CRPC patients in ARMOR2 were identified as having truncated androgen receptors with C-terminal loss. All four of these patients had maximal reductions in PSA levels of at least 50%. These data are consistent with galeterone's mechanism of action of androgen receptor degradation, which does not require a functional ligand binding domain.

We plan to announce additional interim data from our ARMOR2 trial in September 2014 at the European Society for Medical Oncology, or ESMO, 2014 Congress in Madrid, Spain. We believe these data are consistent with the efficacy and safety data that we reported at ASCO. Specifically, we expect to report, among other data, that as of August 15, 2014, our cut-off date for our data presentation at the ESMO 2014 Congress, seven treatment-naïve CRPC patients had been identified in the retrospective subset analysis as having truncated androgen receptors with C-terminal loss. Six of these patients had maximal reductions in PSA levels of at least 50%. The seventh patient, who did not show any PSA reduction, discontinued therapy due to an adverse event unrelated to galeterone after approximately six weeks in the trial and did not receive the full treatment regimen.

Advantages of Galeterone. Although Zytiga and Xtandi have improved survival of CRPC patients, they have limitations in terms of safety, dosing, patient compliance and the development of resistance. In addition, Zytiga and Xtandi may not be effective in treating CRPC patients with prostate tumors that express altered androgen receptors with C-terminal loss. As a result, there remains an unmet medical need for therapies that address populations that are resistant to therapy and will further improve overall survival while providing a more favorable risk benefit profile.

We believe that galeterone has advantages over Zytiga and Xtandi because of the following key differentiating attributes:

- ***Potential for improved efficacy.*** We believe that galeterone, which combines the mechanism of action of androgen receptor degradation with the mechanisms of action of CYP17 inhibition and androgen receptor antagonism, may further improve progression-free survival and overall survival beyond that of products that depend on only a single mechanism of action.

- ***Potential for lower risk of resistance.*** We believe that galeterone may reduce the risk of or delay the development of resistance to therapy because galeterone addresses multiple mechanisms of action simultaneously.
- ***Potential for broad utility in prostate cancer.*** We believe that galeterone may be well suited to treat different prostate cancer patient populations, from early-stage prostate cancer patients to end-stage salvage metastatic CRPC patients, because of its efficacy, safety and tolerability.
- ***Potential for efficacy in CRPC patients with C-terminal loss, including AR-V7.*** Because galeterone's distinct mechanism of androgen receptor degradation does not require an intact ligand binding domain for efficacy, we believe galeterone may be effective in prostate cancer tumors that express altered androgen receptors with C-terminal loss, including AR-V7. In contrast, the mechanisms of action of Zytiga and Xtandi and other similar drugs in development all require the presence of a functional ligand binding domain in order to be effective.
- ***Favorable safety profile.*** 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 demonstrated a favorable safety and tolerability profile.
- ***No requirement for steroids.*** Zytiga must be co-administered with the steroid prednisone to minimize the risk of mineralocorticoid excess, a potentially fatal syndrome characterized by hypertension, hypokalemia, fluid retention and edema. Unlike Zytiga, galeterone has not been shown in clinical trials to cause mineralocorticoid excess and, as a result, does not require co-administration of steroids.
- ***No associated seizure risk.*** Xtandi has shown a risk of grand mal seizures in clinical trials. Unlike Xtandi, galeterone is not in a class of therapeutics that has shown a risk of seizures. We have not had any reports of seizures in clinical trials of galeterone.
- ***Ease of dosing.*** Galeterone is dosed orally once per day, does not require the co-administration of steroids and can be taken with or without food. We believe that this convenient dosing regimen will enhance patient compliance. In contrast, Zytiga must be taken in a fasted state to avoid large increases in absorption, which may cause side effects. Zytiga must also be co-administered with steroids. The steroid co-administered with Zytiga must be taken with food, resulting in a staggered dosing regimen of pills three times per day.
- ***Potential for use as part of combination therapy.*** We believe that galeterone may prove to be well suited for use in combination with other therapies used across all patient populations of prostate cancer because of its favorable safety profile, ease of administration and highly selective, multiple mechanisms of action.

Our Strategy

Our goal is to become a leading biopharmaceutical company that develops and commercializes products for the treatment of prostate cancer and other hormonally-driven diseases. Our strategy includes the following components:

- ***Complete the clinical development of and seek marketing approval for galeterone for the treatment of CRPC patients with prostate cancer tumors that express the AR-V7 splice variant.*** Based on discussions with the FDA, we expect that our ARMOR3-SV trial will be a randomized, open label clinical trial comparing galeterone to Xtandi in up to 170 metastatic CRPC treatment-naïve patients whose prostate tumors express the AR-V7 splice variant. We expect to commence the trial in the first half of 2015 and, subject to patient enrollment rates and the rates of disease progression in patients in the trial, to have top-line data from the trial by the end of 2016.
- ***Develop galeterone for other prostate cancer indications and patient populations.*** Although we are focusing our initial development of galeterone on the treatment of patients with CRPC whose prostate tumor cells express an altered androgen receptor, we are conducting our ARMOR2 trial of galeterone for

the treatment of multiple CRPC patient populations. 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, as well as in hormone-sensitive prostate cancer. We also plan to develop galeterone for the treatment of prostate cancer in combination with currently marketed prostate cancer therapies and novel targeted agents.

- ***Explore the use of galeterone for other hormonally-driven diseases.*** 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.
- ***Maximize the commercial potential of galeterone.*** We have worldwide development and commercialization rights to galeterone. 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.
- ***Advance the development of our platform of androgen receptor degradation agents.*** We plan to identify and develop novel compounds that are designed to disrupt androgen receptor signaling through enhanced androgen receptor degradation under an exclusive license from the University of Maryland, Baltimore, or UMB. We believe that such compounds may have utility as monotherapies or in combination with existing therapies in treating patients with androgen receptor signaling diseases, including prostate cancer patients with primary or acquired resistance to existing therapies.

The Treatment of Prostate Cancer

Prostate Cancer Overview

According to the American Cancer Society, in the United States, prostate cancer is the most frequently diagnosed cancer among men other than skin cancer. The American Cancer Society estimates that, in the United States during 2014, approximately 233,000 new cases of prostate cancer will be diagnosed, and approximately 29,000 men will die from the disease. Overall, in the United States, about one in seven men will be diagnosed with prostate cancer during his lifetime, and about one in 36 men will die from the disease.

Prostate cancer is most frequently diagnosed at an early stage, when it is confined to the prostate gland and its immediate surroundings. Advances in screening and diagnosis, including the widespread use of PSA screening, have allowed detection of the disease in its early stages in approximately 85% of all cases diagnosed in the United States. Patients with early-stage disease are typically treated with surgery or radiation therapy, or in limited circumstances, with both. For the majority of men, these procedures are successful in curing the disease. However, for others, these procedures are not curative and their prostate cancer ultimately recurs. Men with recurrent prostate cancer are considered to have advanced prostate cancer. In addition, about 15% of men diagnosed with prostate cancer have metastatic disease at the time of diagnosis. Men with metastatic disease are also considered to have advanced prostate cancer. Men with advanced prostate cancer are most often treated with drug therapy. Decision Resources Group estimates that approximately 310,000 men in the United States currently have advanced prostate cancer and are eligible for treatment with drug therapy.

Treatment of Advanced Prostate Cancer

The growth and survival of prostate cancer tumor cells depend primarily on the functioning of the androgen receptor signaling pathway. The pathway is ordinarily activated by the binding of androgens, such as testosterone and DHT, to the ligand binding domain of androgen receptors in prostate cancer cells. Testosterone is primarily produced in the testes, adrenal glands and, to a lesser extent, in prostate cancer tumor cells. DHT is a product of enzymatic conversion of testosterone. Once binding has occurred, the bound androgen/androgen receptor complex passes into the nucleus of the tumor cell where it binds to DNA in the cancer cell, triggering abnormal cell growth and tumor progression.

Because testosterone fuels prostate cancer growth, first-line therapy for advanced prostate cancer typically entails androgen deprivation therapy, or ADT, with luteinizing hormone releasing hormone, or LHRH, analogs such as the drug Lupron (leuprolide). ADT reduces testosterone to levels that are commensurate with the levels of a male who has had surgical castration to minimize the testosterone that would otherwise fuel prostate cancer growth. Early-stage patients who receive and respond to this treatment are considered to have hormone-sensitive prostate cancer. ADT has been the principal option for the initial treatment of advanced prostate cancer for more than 50 years.

Most advanced prostate cancer patients initially respond to ADT. However, after initiation of ADT, almost all advanced prostate cancer patients experience a recurrence in tumor growth despite the reduction of testosterone to castrate levels. These patients are considered to be "castration resistant," and cancer that has reached this state is considered to be CRPC. The development of CRPC following initiation of ADT is due in part to tumor cells that have adapted to the hormone-deprived environment of the prostate and is generally diagnosed based on either rising levels of PSA or disease progression as evidenced by imaging tests or clinical symptoms. Decision Resources Group estimates that approximately 180,000 men in the United States currently have CRPC and are eligible for treatment with drug therapy. Patients treated with LHRH analogs typically remain on those drugs for the remainder of their lives in order to maintain castrate levels of testosterone.

During the course of ADT or following diagnosis of CRPC, most patients are treated with anti-androgens, which block the binding of androgens to the androgen receptor. An example of an anti-androgen marketed in the United States is the drug Casodex (bicalutamide). Like LHRH analogs, the anti-androgens suppress tumor growth for a period of time in many CRPC patients. However, almost all CRPC patients develop resistance to anti-androgen therapy. Unlike LHRH analogs, however, patients do not typically remain on these drugs because these drugs have been shown to cause tumor growth once the cancer becomes resistant to the treatment. We refer to initial hormonal treatments like LHRH analogs and Casodex as primary hormonal treatments.

Patients with CRPC may have metastatic or non-metastatic disease. Metastatic cancer is cancer that has spread from the organ of origin to one or more locations in the body. Approximately 90% of CRPC patients have radiologic evidence of metastases in the bone, which can cause pain, bone fracture, decreased quality of life and death. Approximately 30% of patients will develop metastases to solid organs, which can cause pain, decreased quality of life and potentially death. Metastases in the organs are referred to as visceral metastases. The liver and the lungs are the most common sites of visceral metastases.

Prior to 2010, the next line of treatment for patients who became resistant to primary hormonal treatment with LHRH analogs and anti-androgens was chemotherapy. At that time, the chemotherapy drug Taxotere (docetaxel) was the primary FDA-approved treatment used for CRPC patients who were resistant to primary hormonal treatments, and there were no effective FDA-approved treatments for CRPC patients following chemotherapy. Since 2010, the FDA has approved five new agents for the treatment of patients with CRPC. These new treatments have provided patients with alternatives to chemotherapy and have resulted in differentiation of disease stages and new patient populations for which treatments can be developed.

Of these new agents, the two with the highest worldwide sales in 2013 were Zytiga and Xtandi. Zytiga was reported to have worldwide 2013 sales of $1.7 billion, and Xtandi was reported to have worldwide 2013 sales of $445 million. Zytiga and Xtandi are members of a class of new drugs that act by disrupting the androgen receptor signaling pathway. We refer to this class of drugs as secondary hormonal treatments.

Zytiga is an oral secondary hormonal treatment approved by the FDA in April 2011 for use in combination with prednisone to treat men with post-chemotherapy metastatic CRPC. In December 2012, the FDA expanded the approval of Zytiga in combination with prednisone to include treatment of pre-chemotherapy metastatic CRPC patients. Zytiga disrupts the androgen receptor signaling pathway by inhibiting CYP17 and reducing production of testosterone in the testes, adrenal glands and prostate cancer tumor cells.

Xtandi is an oral secondary hormonal treatment approved by the FDA in August 2012 to treat men with post-chemotherapy metastatic CRPC. In March 2014, a supplemental new drug application was submitted to the FDA seeking to expand the label for Xtandi to include treatment of pre-chemotherapy metastatic CRPC patients. Xtandi is an androgen receptor antagonist that disrupts the androgen receptor signaling pathway by blocking the binding of testosterone or the androgen DHT with the androgen receptor.

Other new agents include Jevtana (cabazitaxel), a chemotherapeutic agent for use in combination with prednisone to treat men with metastatic CRPC following first-line chemotherapy, Provenge (sipuleucel-T), a prostate cancer immunotherapy to treat men with asymptomatic or minimally symptomatic metastatic CRPC, whether pre-chemotherapy or post-chemotherapy, and Xofigo (radium-223), a bone targeting radiopharmaceutical for the treatment of CRPC patients with symptomatic bone metastases and no visceral metastases that are detectable upon imaging.

Prior to the approval of the new agents, patients had no effective treatment alternatives following chemotherapy. Each of the new agents, however, has been approved for use following first-line chemotherapy. Patients who have undergone chemotherapy treatment and treatment with Zytiga or Xtandi and whose disease has progressed are referred to as salvage patients. There are only limited treatment options for salvage patients.

The treatment of patients with advanced prostate cancer varies depending on the status of the disease, including whether it is metastatic, and depending on the prior treatments that patients have undergone. Figure 1 below identifies the various patient populations within advanced prostate cancer and the treatments that are approved by the FDA for these populations.

Figure 1: Summary of FDA Approved Treatments for Advanced Prostate Cancer Populations

Patient Populations / Treatment Options		Non-Metastatic		Metastatic CRPC			
		Hormone-Sensitive	CRPC	Pre-Chemo	First-Line Chemo	First-Line Post-Chemo	Salvage
Primary Hormonal Treatment	LHRH	✓	✓	✓	✓	✓	✓
	Androgen Receptor Antagonists	✓	✓				
Secondary Hormonal Treatment	Zytiga			✓		✓	
	Xtandi					✓	
Chemotherapy	Taxotere				✓		
	Jevtana					✓	✓
Immunotherapy	Provenge			✓		✓	
Bone Targeting Agent	Xofigo			✓	✓	✓	✓

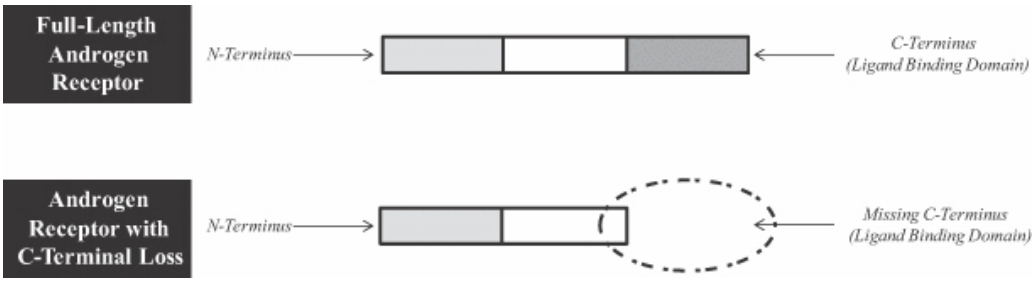
In addition to Zytiga and Xtandi, we are aware of a number of additional therapies that are in late stage clinical trials for prostate cancer, including additional secondary hormonal treatment candidates, which are designed to act by the same mechanisms of action of Zytiga and Xtandi.

Despite the new therapies, including Zytiga and Xtandi and the additional secondary hormonal treatment candidates, we believe that there continues to be an unmet need as there are patient populations that are not effectively addressed by these therapies, such as CRPC patients with C-terminal loss. Zytiga and Xtandi also have treatment limitations, including efficacy limitations, risk of resistance, risks associated with the co-administration of prednisone with Zytiga, a potential seizure risk observed with Xtandi and a complicated dosing regimen for Zytiga that may limit the ability to use it in combination therapies.

Unmet Need in Prostate Cancer Patients with C-Terminal Loss

The growth and survival of prostate cancer tumor cells depend primarily on the functioning of the androgen receptor signaling pathway. The pathway and tumor cell growth is ordinarily activated by the binding of androgens, such as testosterone and DHT, to the ligand binding domain of androgen receptors in prostate cancer cells. All proteins, including androgen receptors, are made up of a chain of amino acids that has an N-terminus at one end of the chain and a C-terminus at the other end of the chain as shown in the full-length androgen receptor depicted in Figure 2 below. In the case of androgen receptors, the C-terminus contains the ligand binding domain. The effectiveness of therapies like Zytiga and Xtandi, which act solely through CYP17 inhibition or androgen receptor antagonism, requires a functional ligand binding domain. As depicted in Figure 2 below, in the case of prostate tumor cells that express truncated androgen receptors with C-terminal loss, there is no functional ligand binding domain. This lack of a functional ligand binding domain causes the truncated androgen receptor to be constitutively active, or continuously signaling, meaning that activation of the androgen receptor pathway and tumor growth occurs even in the absence of androgens and androgen binding. As a result, we believe that patients with truncated androgen receptors with C-terminal loss may not be effectively treated by these therapies.

Figure 2: Full-Length Androgen Receptor and Androgen Receptor with C-Terminal Loss



These limitations of CYP17 inhibitors and androgen receptor antagonists have been supported by recent research from MD Anderson and Johns Hopkins, in which the presence of C-terminal loss and AR-V7 in patients was associated with poor responsiveness of patients' prostate tumors to Zytiga and Xtandi.

MD Anderson. At ASCO, researchers from MD Anderson presented data from a clinical study in which 60 CRPC patients with bone metastases were treated with a sequential combination regimen of Zytiga and Xtandi. In the study, the researchers defined primary resistance as discontinuation of therapy due to symptomatic and/or imaging evidence of disease progression within four months of initiating treatment and benefit as discontinuation of therapy due to symptomatic and/or imaging evidence of disease progression at least four months after initiating treatment. In a subset of 15 patients who were evaluable for C-terminal loss, four patients were identified as having C-terminal loss, including two who were identified as having AR-V7. In this study, the researchers used antibody-based assays to identify the presence of C-terminal loss and AR-V7. All four, or 100%, of these patients showed primary resistance. Of the 11 patients in the subset that did not have C-terminal loss or AR-V7, nine patients, or 82%, showed benefit. These data are set forth in Table 1 below.

Table 1: Summary of MD Anderson C-Terminal Loss and AR-V7 Findings (ASCO)

	N	Primary Resistance	Benefit
AR-V7 positive	2	100% (2/2)	0% (0/2)
C-terminal loss (excluding AR-V7)	2	100% (2/2)	0% (0/2)
Negative for AR-V7 and C-terminal loss	11	18% (2/11)	82% (9/11)

In addition, researchers from MD Anderson presented the results of a second study in an article in *European Urology* accepted for publication in May 2014. In the study, the researchers evaluated bone biopsy specimens from CRPC patients with bone metastases that had been treated with Xtandi to evaluate the effects of Xtandi on cancer and to associate these effects with clinical observations. In the study, the researchers defined resistance and benefit as follows:

- primary resistance, as discontinuation of therapy due to symptomatic and/or imaging evidence of disease progression within four months of initiating Xtandi treatment;
- moderate benefit, as discontinuation of therapy due to symptomatic and/or imaging evidence of disease progression within four to six months of initiating Xtandi treatment; and
- prolonged benefit, as discontinuation of therapy due to symptomatic and/or imaging evidence of disease progression at least six months after initiating Xtandi treatment.

The researchers evaluated a population of 23 patients who had two evaluable biopsies for AR-V7. As shown in Table 2 below, based on identification of AR-V7 at baseline, 86% of the patients with AR-V7 showed primary resistance, and 38% of the patients that did not have AR-V7 showed primary resistance.

Table 2: Summary of MD Anderson AR-V7 Baseline *(European Urology)*

Outcome	N	Primary Resistance	Moderate Benefit	Prolonged Benefit
AR-V7 positive	7	86% (6/7)	14% (1/7)	0% (0/7)
AR-V7 negative	16	38% (6/16)	31% (5/16)	31% (5/16)

Johns Hopkins. In a clinical trial conducted by Johns Hopkins, researchers prospectively evaluated the effect of AR-V7 in patients with metastatic CRPC on tumor responsiveness to treatment with Xtandi and Zytiga. In the trial, 31 patients received Xtandi, and 31 patients received Zytiga. In the trial, the presence of AR-V7 was determined by an analysis of circulating tumor cells isolated from the patient's blood. In the Xtandi-treated group, 12 of the 31 patients were identified as having AR-V7. None of these 12 patients with AR-V7 achieved the trial's primary endpoint of maximal PSA reduction of at least 50%. Eleven of the 12 patients with AR-V7 did not achieve any PSA reduction. Ten of the 19 patients who did not have AR-V7 achieved a maximal PSA reduction of at least 50%. In addition, the median radiographic progression-free survival of the patients with AR-V7 was 2.1 months, compared to 6.1 months in the patients without AR-V7. The differences between the AR-V7 and non-AR-V7 groups in terms of the number of patients achieving a maximal PSA reduction of 50% and the improvement in median radiographic progression-free survival were statistically significant.

In the Zytiga-treated group, six of the 31 patients were identified as having AR-V7. None of the six patients with AR-V7 achieved any PSA reduction during treatment. Seventeen of the 25 patients who did not have AR-V7 achieved a maximal PSA reduction of at least 50%. The median radiographic progression-free survival of the patients with AR-V7 was 2.3 months and had not yet been reached in the patients without AR-V7. The differences between the AR-V7 and non-AR-V7 groups in terms of the number of patients achieving a maximal PSA reduction of 50% and the improvement in median radiographic progression-free survival were statistically significant.

The data from the Johns Hopkins trial are summarized in Table 3 below.

Table 3: Summary of Johns Hopkins Data

			Results				
Treatment	*N*	*AR-V7+*	*AR-V7 Status*	*PSA50*	*P-value**	*rPFS*	*P-value**
Xtandi	31	38% (12/31)	+ -	0% 52%	0.004	2.1 months 6.1 months	<0.001
Zytiga	31	19% (6/31)	+ -	0% 68%	0.004	2.3 months Not Reached	<0.001

* Results are considered statistically significant if they have a p-value of 0.05 or less, meaning that there is less than a one-in-20 likelihood that the observed results occurred by chance.

The Johns Hopkins researchers also reported the prevalence of AR-V7 in different patient groups participating in the trial based on the prior treatment the patient had received. Table 4 below sets out the percentage of patients in each prior treatment group who had AR-V7.

Table 4: Prevalence of AR-V7 in CRPC in the Johns Hopkins Trial

Treatment Status Prior to Entry Into Johns Hopkins Trial	**Percentage of Patients in Pre-Treatment Group who had AR-V7**
Pre-enzalutamide *and* pre-abiraterone acetate	11.6%
Post-enzalutamide *only*	25.0%
Post-abiraterone acetate *only*	51.2%
Post-enzalutamide *and* post-abiraterone acetate	66.7%

Based on these data, we believe that treatment with Xtandi and Zytiga may be associated with an increase in the prevalence of AR-V7, causing cross-resistance to sequential therapy and leaving patients who are treated with either Xtandi or Zytiga with no currently available secondary hormonal treatment options. By contrast, we believe galeterone has the potential to reduce the prevalence of AR-V7 through its mechanism of androgen receptor degradation.

Galeterone

Overview

Our lead product candidate, galeterone, is a highly selective, multi-targeted, oral small molecule drug candidate that, like Zytiga and Xtandi, acts by disrupting the androgen receptor signaling pathway. Zytiga and Xtandi each disrupt the pathway at a single point using a single mechanism of action. In contrast, galeterone disrupts the pathway at multiple points by combining the mechanisms of action of Zytiga (CYP17 inhibition) and Xtandi (androgen receptor antagonism) with the additional mechanism of androgen receptor degradation. Unlike CYP17 inhibition and androgen receptor antagonism, the mechanism of action of androgen receptor degradation does not require a functional ligand binding domain. We believe that there are no approved drugs or drugs in clinical trials, other than galeterone, with the mechanism of action of androgen receptor degradation. We are currently finalizing our plans for our pivotal Phase 3 clinical trial of galeterone and expect to initiate the trial in the first half of 2015.

In addition to our planned pivotal Phase 3 clinical trial, we are conducting our ARMOR2 trial of galeterone for the treatment of multiple CRPC populations. 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, as well as in hormone-sensitive prostate cancer. We have exclusive worldwide development and commercialization rights to galeterone.

Our initial development strategy for galeterone focusing on the treatment of patients with CRPC whose prostate tumor cells express an altered androgen receptor that is truncated is consistent with the increasing focus in drug development of precision medicine therapies for the treatment of cancers caused by genetic and other alterations. We are not aware of any precision medicine therapies in clinical trials for the treatment of prostate cancer that are targeting C-terminal loss splice variants other than galeterone.

Key Differentiating Attributes of Galeterone

Based on preclinical and clinical data, we believe that galeterone has advantages over Zytiga and Xtandi because of the following key differentiating attributes:

- ***Potential for improved efficacy.*** We believe that galeterone, which combines the mechanism of action of androgen receptor degradation with the mechanisms of action of Zytiga (CYP17 inhibition) and Xtandi (androgen receptor antagonism), may further improve progression-free survival and overall survival beyond that of products that depend on only a single mechanism of action. At ASCO, we reported efficacy data from a total of 115 CRPC patients in our ARMOR1 and our ARMOR2 trials across a number of dose levels that showed meaningful reductions in maximal PSA in patients in the trials.
- ***Potential for lower risk of resistance.*** We believe that galeterone may reduce the risk of or delay the development of resistance to therapy because galeterone addresses multiple mechanisms of action simultaneously. We believe that reducing resistance may delay the development of disease progression.
- ***Potential for broad utility in prostate cancer.*** We believe that galeterone may be well suited to treat different prostate cancer patient populations, from early-stage prostate cancer patients to end-stage salvage metastatic CRPC patients, because of its efficacy, safety and tolerability.
- ***Potential for efficacy in CRPC patients with C-terminal loss, including AR-V7.*** Because galeterone's distinct mechanism of action of androgen receptor degradation does not require an intact ligand binding domain to be effective against prostate cancer tumors, we believe galeterone may be effective in prostate cancer tumors that express altered androgen receptors with C-terminal loss, including AR-V7. In contrast, the mechanisms of action of Zytiga and Xtandi and other similar drugs in development all require the presence of the ligand binding domain in order to be effective.
- ***Favorable safety profile.*** 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 demonstrated a favorable safety and tolerability profile. In our ARMOR2 trial, approximately 90% of all treatment-emergent adverse events reported as of August 15, 2014 were grade 1 or 2 in severity and were generally manageable and reversible.
- ***No requirements for steroids.*** Unlike Zytiga, galeterone has not been shown in clinical trials to cause mineralocorticoid excess and does not require co-administration of steroids. Because Zytiga has been shown in clinical trials to cause mineralocorticoid excess, a potentially fatal syndrome characterized by hypertension, hypokalemia, fluid retention and edema, Zytiga is required to be administered with prednisone to reduce the frequency of patients exhibiting mineralocorticoid excess. Despite the co-administration of prednisone, however, approximately 30% of patients treated with Zytiga in a pivotal Phase 3 trial developed symptoms of mineralocorticoid excess. In addition, the chronic use of prednisone poses other safety concerns. Side effects associated with chronic use of prednisone include muscle weakness, osteoporosis, diabetes and increased risk of infection.
- ***No associated seizure risk.*** Unlike Xtandi, we have not had any reports of seizures in clinical trials of galeterone. A 0.9% risk of grand mal seizures was reported in the Xtandi pivotal Phase 3 trial in post-chemotherapy CRPC patients. These seizures have been linked to the inhibition or antagonism by Xtandi of $GABA_A$, a receptor associated with the nervous system. Galeterone is not a $GABA_A$ antagonist.

- ***Ease of dosing.*** Unlike the complicated dosing regimen for Zytiga, galeterone is dosed orally once per day, does not require the co-administration of steroids and can be taken with or without food. We believe that this convenient dosing regimen will enhance patient compliance. In contrast, Zytiga must be taken in a fasted state to avoid large increases in absorption, which may cause side effects. Zytiga also must be co-administered with steroids. Prednisone, the steroid co-administered with Zytiga, must be taken with food in order to avoid potential development of gastric ulcers. As a result, Zytiga and prednisone cannot be taken together and dosing must be carefully coordinated with food intake, resulting in a staggered dosing regimen of pills three times per day.
- ***Potential for use as part of combination therapy.*** Combination therapy using drugs with different mechanisms of action has been an important component of cancer treatment. Combination therapy makes it possible to simultaneously attack different mechanisms responsible for the replication, progression and survival of tumor cells. This is important because of the genetic diversity within a tumor population and because not all cells are equally sensitive to a particular mechanism of action or drug. Because of galeterone's multiple mechanisms of action, galeterone acts as if it were a combination therapy. Moreover, because of galeterone's favorable safety profile, ease of administration and highly selective, multiple mechanisms of action, we believe that it may prove to be well suited for use in combination with other therapies.

Galeterone Clinical Development

In August 2009, we submitted an investigational new drug application, or IND, to the FDA for galeterone for the treatment of CRPC, and in November 2009, we commenced clinical trials of galeterone. As of August 15, 2014, our cut-off date for our data presentation at the ESMO 2014 Congress, we had administered galeterone to a total of 254 prostate cancer patients and healthy volunteers in Phase 1 and Phase 2 clinical trials. In the Androgen Receptor Modulation Optimized for Response, or ARMOR, program, we had treated 121 CRPC patients in our ongoing ARMOR2 trial and 49 CRPC patients in our ARMOR1 trial. In four additional Phase 1 clinical trials, we also administered galeterone to 84 healthy volunteers.

ARMOR2 Trial

In December 2012, we initiated our ARMOR2 trial, an open label Phase 2 clinical trial of galeterone. The trial is designed as a two-part trial. Part 1 of the trial is a dose escalation phase designed to confirm the dose of galeterone to be evaluated in Part 2 of the trial. Part 2 of the trial is designed to evaluate the efficacy and safety of galeterone at the dose selected in Part 1 in distinct CRPC patient populations. The trial is being conducted at 28 sites in the United States and Canada. In addition, galeterone had been well tolerated and had shown clinically meaningful reductions in levels of PSA.

The primary efficacy endpoints of our ARMOR2 trial are based on a decrease in PSA levels. In setting the primary endpoints of the trial, we considered the standard, accepted use of monitoring PSA levels to determine if a patient's prostate cancer is responding to therapy as well as the use of reductions in PSA levels as a key efficacy endpoint in Phase 2 clinical trials of other prostate cancer agents, as set forth in guidelines developed by the Prostate Cancer Working Group 2, or PCWG2. PCWG2 is an international group of prostate cancer investigators who published guidelines for the design and evaluation of prostate cancer trials.

Part 1 of ARMOR2 Trial. In Part 1 of the trial, we enrolled 25 CRPC treatment-naïve patients with progressive disease and three patients whose disease progressed during treatment with Zytiga, whom we refer to as Zytiga-refractory patients. The CRPC treatment-naïve patients were enrolled in one of three escalating dose cohorts: 1700 mg/day, 2550 mg/day or 3400 mg/day. The Zytiga-refractory patients all received doses of 2550 mg/day. All patients in Part 1 of the trial received treatment for up to an initial period of 12 weeks followed by optional continued dosing for those patients who tolerated treatment and did not show signs of disease progression. Treatment was continued until disease progression or patient withdrawal due to adverse events or other reasons.

At least 50% of patients at all dose levels achieved a 30% or greater decrease in PSA. Based on the recommendation of the monitoring committee for the trial following review of safety, efficacy and pharmacokinetic results of the three dose groups, we chose the 2550 mg/day dose for further study in Part 2 of the ARMOR2 trial.

Part 2 of ARMOR2 Trial. The protocol for Part 2 of the trial provides for a 2550 mg/day dose of galeterone to be evaluated in a total of up to 108 patients in the following advanced prostate cancer populations:

- non-metastatic and metastatic CRPC treatment-naïve patients in a combined cohort of up to 48 patients;
- patients whose disease progressed during treatment with Zytiga, whom we refer to as Zytiga-refractory patients, in a cohort of up to 30 patients; and
- patients whose disease progressed during treatment with Xtandi, whom we refer to as Xtandi-refractory patients, in a cohort of up to 30 patients.

Table 5 below summarizes the patient populations and primary endpoints for Part 2 of the ARMOR2 trial:

Table 5: Patient Populations and Primary Endpoints for Part 2 of the ARMOR2 Trial

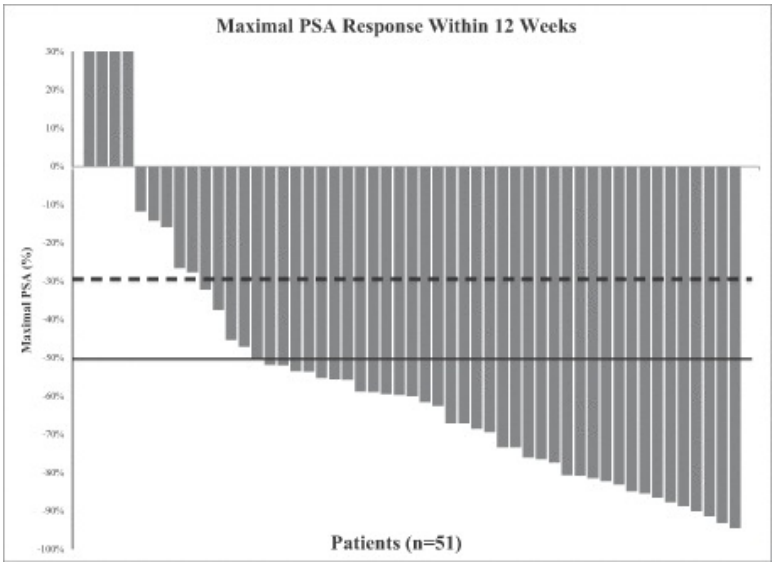
<table>
<tr><th>Patient Population</th><th>Number of Patients</th><th>Primary Endpoint</th></tr>
<tr><td>Non-metastatic CRPC treatment-naïve patients</td><td rowspan="2">Up to 48</td><td rowspan="2">Percentage of patients with a maximal reduction in PSA levels of at least 30% from baseline to the end of the primary treatment phase</td></tr>
<tr><td>Metastatic CRPC treatment-naïve patients</td></tr>
<tr><td>Zytiga-refractory patients</td><td>Up to 30</td><td rowspan="2">Percentage of change in PSA levels from baseline to the end of the primary treatment phase</td></tr>
<tr><td>Xtandi-refractory patients</td><td>Up to 30</td></tr>
</table>

Additional endpoints include incidence of adverse events, change from baseline in safety parameters, response rate and circulating tumor cell, or CTC, enumeration and characterization, including for the evaluation of C-terminal androgen receptor expression in relation to N-terminal androgen receptor expression to identify C-terminal loss and the lack of a functional ligand binding domain.

As of August 15, 2014, we had enrolled 93 patients in Part 2 of the trial. All patients in Part 2 of the trial receive treatment for an initial period of 12 weeks followed by optional continued dosing for those patients who tolerated treatment and did not show signs of disease progression. Treatment will be continued until disease progression or patient withdrawal due to adverse events or other reasons.

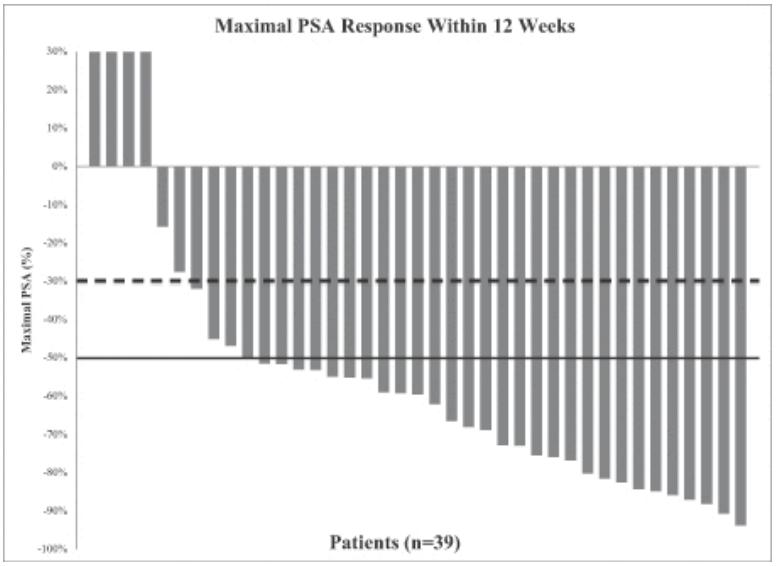
Clinical Data Presented at ASCO. In May 2014 at ASCO, we presented interim efficacy and safety data from our ARMOR2 trial for patients who received the 2550 mg/day dose of galeterone. In 51 evaluable CRPC treatment-naïve patients in Part 1 and Part 2 of the trial who received the 2550 mg/day dose of galeterone, during the first 12 weeks of dosing, 82% had a maximal reduction in PSA levels of at least 30%, and 75% had a maximal reduction in PSA levels of at least 50%, as described in Figure 3 below.

Figure 3: ARMOR2: Maximal PSA Response Waterfall Plot in All Pre-Chemotherapy CRPC Treatment-Naïve Patients (n=51) (2550 mg dose)



In 39 metastatic CRPC treatment-naïve patients who received the 2550 mg/day dose, during the first 12 weeks of dosing, 85% had a maximal reduction in PSA levels of at least 30%, and 77% had a maximal reduction in PSA levels of at least 50%, as described in Figure 4 below.

Figure 4: ARMOR2: Maximal PSA Response Waterfall Plot in Pre-Chemotherapy Metastatic CRPC Treatment-Naïve Patients Treated (n=39) (2550 mg dose)



We also reported 12-week data for 15 Zytiga-refractory patients, two of whom had a maximal reduction in PSA levels of at least 30%. Of the eight Zytiga-refractory patients evaluable by Response Evaluation Criteria in Solid Tumors, or RECIST, five patients had stable disease and three patients had progressive disease as measured by CT/MRI scans by modified RECIST criteria and as measured by bone scans by PCWG2 guidelines. As measured by RECIST criteria, stable disease is achieved when the tumor has not increased in size by 20% and has not decreased by 30%, a partial response occurs when the tumor has decreased in size by at least 30%, and progressive disease occurs when the tumor has increased in size by at least 20% or new tumor lesions are identified.

Our ARMOR2 trial included CTC enumeration and characterization. At ASCO, we presented data from a retrospective subset analysis in which four treatment-naïve CRPC patients in ARMOR2 were identified as having C-terminal loss as determined by the evaluation of C-terminal androgen receptor expression in relation to N-terminal androgen receptor expression. All four of these patients had maximal reductions in PSA levels of at least 50%. We believe that these data support our view that androgen receptor degradation may be active in patients without an intact ligand binding domain and are consistent with our preclinical studies of galeterone.

At ASCO, we also presented interim safety results from all 87 patients treated as of May 12, 2014 in ARMOR2. In these patients, galeterone was well tolerated. Approximately 90% of all treatment-emergent adverse events reported were grade 1 or 2 in severity and were generally manageable and reversible. The majority of these events were assessed as not related or unlikely related to galeterone. In addition, there were no reported cases of seizure or mineralocorticoid excess. The most common adverse events were nausea, decreased appetite, fatigue, diarrhea, pruritus and increased aminotransferase indicating elevated liver enzyme levels. Six of these patients (7%) experienced a grade 3 or grade 4 treatment-emergent increase in aminotransferase indicating elevated liver enzyme levels. These events were asymptomatic, transient and all six of these patients recovered following temporary drug withdrawal. Four of the six patients have been re-challenged at a reduced dose level with none showing a recurrence of a grade 3 or higher adverse event. There were three unexpected serious adverse events in the trial 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 acase of hypocalcemia and hyperparathyroidism in a patient with a history of hypocalcemia and hyperparathyroidism. Under the Common Terminology Criteria for Adverse Events established by the National Cancer Institutes, adverse events are reported by grade. Grades 1 or 2 indicate mild to moderate adverse events, grade 3 indicates a severe but not life threatening event with required hospitalization, grade 4 indicates that the event is life threatening and a grade 5 event is death.

Clinical Data to be Presented at ESMO 2014 Congress. We plan to announce additional interim data from our ARMOR2 trial in September 2014 at the ESMO 2014 Congress. We believe that these data are consistent with the efficacy and safety data that we reported at ASCO. Specifically, we expect to report, among other data, the following data collected as of August 15, 2014:

- *C-terminal Loss*. Seven treatment-naïve CRPC patients had been identified in the retrospective subset analysis as having truncated androgen receptors with C-terminal loss. Six of these patients had maximal reductions in PSA levels of at least 50%. The seventh patient, who did not show any PSA reduction, discontinued therapy due to an adverse event unrelated to galeterone after approximately six weeks in the trial and did not receive the full treatment regimen.
- *Non-metastatic and metastatic CRPC treatment-naïve patients at the selected Phase 2 dose of 2550 mg/day*. During the first 12 weeks of dosing, 83% of the 60 patients in this treatment group showed maximal reduction in PSA levels of at least 30%, and 70% of these patients showed maximal reduction in PSA levels of at least 50%.
- *Safety*. In 107 patients treated in our ARMOR2 trial, galeterone was well tolerated.

Reformulation of Galeterone

The ARMOR2 trial uses a proprietary spray dried dispersion formulation of galeterone in tablet form that we developed after we completed the ARMOR1 trial. Spray dried dispersion is a manufacturing technology used in the pharmaceutical industry to improve dissolution rates and enhance the bioavailability of poorly soluble compounds such as galeterone. During the spray dried dispersion manufacturing process, galeterone and an inert polymer are dissolved in organic solvents and spray dried to produce solid dispersion powder, which is then tableted. The final drug product is an oral tablet.

We developed the tableted spray dried dispersion formulation as a result of findings of exposure variability due to a pronounced food effect with the original drug product used in the ARMOR1 trial. The original formulation was micronized active pharmaceutical ingredient in capsule, which we refer to as the PIC formulation. The spray dried dispersion formulation minimizes the food effect, decreases the exposure variability and increases the exposure levels. We anticipate using our tableted spray dried dispersion formulation in all subsequent clinical trials for galeterone and, if approved for marketing, commercial sales of galeterone.

ARMOR1 Trial

In November 2009, we initiated our ARMOR1 trial, an open label, dose escalation Phase 1 clinical trial of galeterone. We conducted the ARMOR1 trial in 49 CRPC patients at eight sites in the United States using our prior PIC formulation of galeterone. The trial enrolled metastatic and non-metastatic CRPC treatment-naïve patients.

Patients were enrolled in the trial in eight cohorts based on dose level and dosing schedule. Escalating doses of galeterone were administered from 650 mg/day through 2600 mg/day as a single daily dose or a split dose twice daily. The monitoring committee for the trial reviewed all safety data prior to escalation. Galeterone was taken with a patient choice of meal or with a food supplement. Patients received treatment for an initial period of 12 weeks followed by optional continued dosing for those patients who tolerated treatment and did not show signs of disease progression. Treatment was continued until disease progression or patient withdrawal due to adverse events or other reasons.

The trial was designed as a dose finding trial. The primary endpoints for the trial were to assess incidence of adverse events and change from baseline in safety parameters. Secondary endpoints included the percentage of patients with a 50% or greater decrease in PSA during the period from baseline to the earlier of the end of the 12-week treatment period or PSA nadir and changes in disease status from baseline in CT/MRI scans and bone scans over the 12-week treatment period.

A total of 49 patients were enrolled in ARMOR1, of whom 37 patients completed the 12-week treatment period, and 22 patients entered the extension phase of the trial. Of the 12 patients who did not complete the 12-week treatment period, five discontinued treatment due to disease progression, five discontinued treatment due to adverse events and two voluntarily withdrew from the trial.

Safety. Galeterone was well tolerated in the trial. Patients in the trial, as a group, were dosed with galeterone, in the aggregate, for approximately 8,000 days, with individual patients receiving galeterone for up to 20 months. Approximately 90% of treatment-emergent adverse events reported for the first 12 weeks of treatment were grade 1 or grade 2 in severity and were generally manageable and reversible. The majority were assessed as not related or unlikely related to galeterone. The most common treatment-emergent adverse events reported for the first 12 weeks of treatment were fatigue, increased aminotransferase, nausea, diarrhea and pruritus. The incidence of treatment-emergent adverse events was comparable between cohorts and was not dose related. A total of eight patients (or 16%) experienced a grade 3 treatment-emergent increase in aminotransferase indicating elevated liver enzyme levels. These events were asymptomatic and transient. Of the eight patients, two patients voluntarily withdrew from the trial, and six patients restarted at the same dose level or one dose level below with no recurrence of a grade 3 or higher adverse event. A maximum tolerated dose was not reached in the

trial. In our ARMOR1 trial, there was only one unexpected serious adverse event assessed by the investigator as possibly related to treatment with galeterone: a case involving 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.

Efficacy. Patients in each of the doses tested experienced reductions in PSA. In the 12 patients who received the highest dose in the study, 2600 mg/day, maximal PSA decreases of at least 30% were observed in 75% of the patients, and maximal PSA decreases of at least 50% were observed in 42% of the patients. Of the 49 patients in the trial, 22% experienced maximal PSA decreases of at least 50%, and 49% experienced maximal PSA decreases of at least 30%. We believe that these results, while favorable, were adversely impacted by the exposure variability associated with the food effect of the PIC formulation. Radiographic evidence of tumor shrinkage and overall tumor stabilization was seen in multiple patients as assessed by CT/MRI scans and bone scans as measured by RECIST. Thirty-nine patients had measurable disease at baseline, including five patients receiving the 2600 mg/day dose. Of the five patients, two had partial responses, and a third patient had a near partial response with a reduction in maximal PSA levels of 28%. Of the 39 patients, 22 had stable disease at the end of the 12-week treatment period.

Phase 1 Trials in Healthy Volunteers in Connection with Galeterone Reformulation

During the course of the ARMOR1 trial, we conducted a retrospective analysis of data from the trial which suggested that the PIC formulation of galeterone used in the trial had a food effect, which may have introduced variability into the drug exposure levels. On the basis of this data, we conducted two Phase 1 trials (TOK-200-06 and TOK-200-07) in a total of 36 healthy volunteers to further evaluate the food effect of the PIC formulation of galeterone. In these trials, volunteers received a 975 mg/day dose of galeterone in a fed state with an FDA standardized high calorie/high fat meal or food supplement or in a fasted state in a cross-over design with a seven day washout between treatments. Treatment with galeterone was well tolerated by all volunteers in the trials. The pharmacokinetic results from the trials showed a substantial food effect with increased absorption of 10 to 12 fold in the fed versus the fasted volunteers. As a result, we pursued development of a new formulation to eliminate this food effect. In a Phase 1 clinical trial of galeterone (TOK-200-08), we explored the use of a coated tablet using the active ingredient of the PIC formulation but decided not to take this formulation forward.

We evaluated the proprietary spray dried dispersion formulation, in both capsule and tablet form, in a Phase 1 clinical trial (TOK-200-09) in 24 healthy volunteers. This trial was designed to assess single dose pharmacokinetics and relative bioavailability of the spray dried dispersion formulation under fed and fasted conditions as compared to the PIC formulation of galeterone under fed conditions. Treatment with galeterone was well tolerated by all volunteers in this trial. In addition, the new formulation eliminated the food effect observed with the PIC formulation, reduced drug exposure variability and increased drug exposure levels. We are using our proprietary spray dried dispersion formulation in tablet form in the ongoing ARMOR2 trial and plan to use it in all future trials and, if approved for marketing, commercial sales of galeterone.

Pivotal Phase 3 Clinical Trial

We are currently finalizing our plans for our pivotal Phase 3 clinical trial of galeterone, which we refer to as our ARMOR3-Splice Variant, or SV, trial. 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 up to 170 metastatic CRPC treatment-naïve patients whose prostate tumors express the AR-V7 splice variant. We expect that the trial will be conducted at approximately 60 to 80 sites worldwide.

Under the trial protocol, patients will be randomized to receive either galeterone or the control arm treatment, Xtandi. Patients in the galeterone arm will receive a dose of 2550 mg/day, and patients in the Xtandi arm will receive a dose of 60 mg/day. All patients will continue to receive treatment until they have radiographic

evidence of disease progression or patient withdrawal due to adverse events or other reasons. Other details of the trial design, such as the randomization ratio, stratification factors and plans for a futility analysis, are still under consideration and will be contained in the final protocol submitted to the FDA. We also plan to establish an independent data monitoring committee and, although the trial is planned to be an open label trial, to have the data collected and analyzed in a manner that leaves us blind to the data.

We expect that in the trial patients with the AR-V7 splice variant will be identified by a central laboratory using a CTC-based AR-V7 specific assay. Based on our discussions with the FDA, we will need to develop an analytically validated assay and submit an investigational device exemption application, or IDE, for the assay to the FDA before we screen patients in the trial. We are currently finalizing our strategy for developing this assay. Delays in developing this assay could delay the initiation of the trial. We expect that we may need to screen more than 1,000 patients to identify and enroll the target AR-V7 positive patients.

The primary endpoint of the trial will be radiographic progression-free survival measured from the time of patient randomization to the time of radiographic evidence of disease progression or time of death from any cause. The secondary endpoints of the trial will include reduction in PSA levels, overall survival and safety. In order to achieve the primary endpoint, results from the trial must demonstrate an 82% increase in median radiographic progression-free survival in the galeterone arm as compared to the Xtandi arm. Such a result would be statistically significant and would likely be considered a clinically relevant outcome.

We expect to commence the trial in the first half of 2015 and to have 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 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.

Prospective Identification of AR-V7

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. In addition, based on those discussions, we believe that the *in vitro* companion diagnostic test will need to be approved by the FDA through its Premarket Approval, or PMA, process.

Other Development Activities

We plan to explore galeterone's utility in 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, including with Xofigo, and novel targeted agents, and in other diseases in which the androgen receptor signaling pathway plays a role.

Galeterone Mechanisms of Action

The androgen receptor signaling pathway is the primary pathway that drives prostate cancer growth and has been implicated in other hormonally driven diseases. The pathway is ordinarily activated by the binding of androgens, such as testosterone and DHT, to the ligand binding domain of androgen receptors in prostate cancer cells. Galeterone disrupts the activation of the pathway through multiple mechanisms of action:

- inhibition of the enzyme CYP17, which blocks the synthesis of testosterone;
- androgen receptor antagonism, which blocks the binding of testosterone or DHT with the androgen receptor; and
- androgen receptor degradation, which reduces the amount of androgen receptor protein in the tumor cells.

In order to demonstrate galeterone's multiple mechanisms of action, we conducted preclinical studies with respect to each mechanism.

CYP17 Lyase Inhibition

Like Zytiga, galeterone is an inhibitor of CYP17, a protein with two enzymatic functions: hydroxylase and lyase. Because CYP17 plays a central role in synthesizing the androgens that drive tumor cell growth, CYP17 inhibitors have been developed to treat patients with CRPC. Selectively blocking CYP17 lyase reduces the production of key androgen precursors. However, inhibition of the CYP17 hydroxylase causes an accumulation of certain steroids, such as progesterone, deoxycorticosterone and corticosterone, and a reduction in cortisol, which can result in mineralocorticoid excess. An ideal CYP17 inhibitor will selectively block the lyase function of CYP17 relative to hydroxylase so that these steroids do not accumulate to the extent that they cause mineralocorticoid excess.

We conducted preclinical studies of galeterone and abiraterone to evaluate their relative selectivity with respect to the inhibition of the hydroxylase and lyase functions of CYP17. In these studies, galeterone was shown to selectively block the lyase function of CYP17 relative to the hydroxylase function. In contrast, abiraterone more selectively blocked the hydroxylase function relative to the lyase function, consistent with its published risk for mineralocorticoid excess.

Consistent with these findings, in further preclinical studies in cell cultures, we observed that galeterone inhibited testosterone synthesis comparable to abiraterone, but that abiraterone significantly lowered cortisol levels as compared to galeterone. We believe that this difference is due in part to galeterone's selective inhibition of the lyase function of CYP17.

Androgen Receptor Antagonism

Like Xtandi, galeterone blocks androgens from binding to the androgen receptor. This results in reduced translocation of the androgen receptor into the cell nucleus, which prevents the androgen receptor from acting as a transcription factor and decreases the expression of androgen-responsive genes that drive tumor growth. In *in vitro* studies, galeterone has shown potency of antagonism greater than or comparable to other androgen receptor antagonists, including enzalutamide.

Androgen Receptor Degradation

Galeterone decreases the amount of androgen receptor protein in prostate tumor cells by enhancing degradation of the androgen receptor. This reduces the number of androgen receptors in the tumor cells to which androgen can bind and decreases the sensitivity of androgen responsive cells to androgens. The effect of galeterone to reduce androgen receptor levels has been observed in tumor cell lines and a xenograft model in mice. We have observed this effect of galeterone in varying degrees in prostate cancer cell lines that express non-mutated full-length androgen receptors and multiple forms of androgen receptor alterations. These alterations

include splice variants, such as AR-V7, that are missing large portions of the protein sequence of the androgen receptor in the C-terminus and point mutations, which are single amino acid mutations in the protein sequence of the androgen receptor. In contrast to galeterone, which has been shown to lower androgen receptor levels, in independent preclinical studies and our preclinical studies, reductions in androgen receptor levels have not been observed using *in vitro* or *in vivo* models of prostate cancer treated with abiraterone, bicalutamide or enzalutamide. Abiraterone is the active ingredient in Zytiga, bicalutamide is the active ingredient in Casodex and enzalutamide is the active ingredient in Xtandi. To our knowledge, there are no approved drugs or drugs in clinical development, other than galeterone, with the mechanism of action of androgen receptor degradation.

Preclinical Development

We have conducted *in vitro* and *in vivo* preclinical studies to evaluate galeterone's effect on prostate cancer, including the efficacy of galeterone in hormone-sensitive tumor cell lines, in tumors expressing AR-V7 and other splice variants, in tumors expressing androgen receptor point mutations and in combination with novel targeted agents.

Activity in Hormone Treatment-Resistant Prostate Cancer

We believe that galeterone has the potential to treat tumors that are resistant to hormone treatments because of its differentiated mechanisms of action. In preclinical studies, others have reported key mechanisms of resistance in hormone treatment-resistant prostate cancer, which include:

- increased CYP17 enzyme levels;
- increased production of testosterone and DHT;
- increased wild type or mutant androgen receptor levels;
- alterations in the androgen receptor, such as splice variants and point mutations;
- mutations in the androgen receptor that result in activation by steroids, such as prednisone and progesterone; and
- androgen receptor mutations which convert androgen antagonists into agonists thus leading to activation of the receptor.

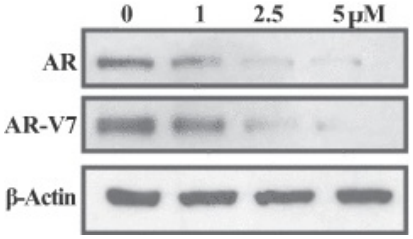
Activity in Tumors Expressing Splice Variants, including AR-V7

Androgen receptor splice variants are produced in tumor cells due to an aberrant RNA splicing event. As a result, a truncated androgen receptor protein is synthesized that lacks the C-terminal end of the protein, the region of the protein responsible for androgen binding. Tumor cells that express altered androgen receptors that lack the C-terminal end of the protein are not responsive to agents whose activity requires a functional ligand binding domain. In addition, the lack of the ligand binding domain causes the remaining splice variants to be constitutively active, or continuously signaling, meaning that activation of the androgen receptor pathway and tumor growth occurs even in the absence of androgens and androgen binding. This indicates the importance of androgen receptor degradation to the prevention of tumor growth.

As a follow-up to preclinical studies in which galeterone had caused degradation of full-length androgen receptors, preclinical studies were conducted in independent laboratories to determine whether galeterone also causes androgen receptor degradation in splice variant proteins.

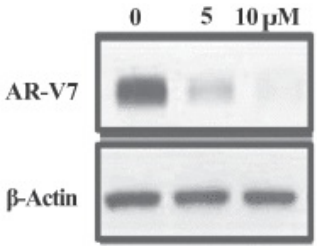
In preclinical studies, we measured androgen receptor degradation using cell lines that expressed full-length and splice variant androgen receptors. These cells model the expression patterns described in human tumor samples where full-length and splice variant androgen receptor proteins are co-expressed. As shown in Figure 5 below, levels of both full-length androgen receptor and AR-V7 were reduced in a dose dependent fashion following galeterone treatment. In the figures below, we use as a control beta-actin (ß-Actin), a protein commonly used as a control in these types of experiments.

Figure 5: Galeterone Causes Decreased Levels of Both Full-Length Androgen Receptor (AR) and AR-V7



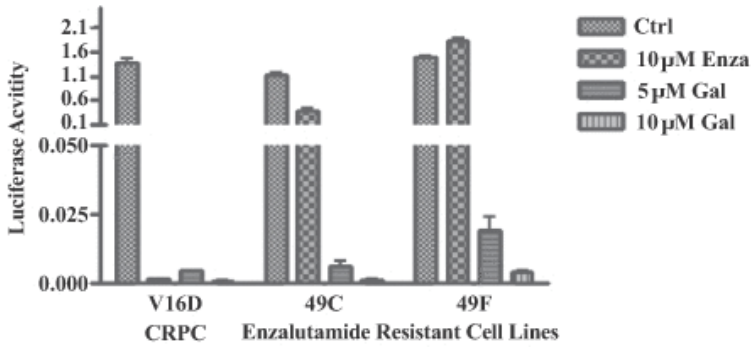
To demonstrate that galeterone would degrade the AR-V7 protein alone, in the absence of the full-length androgen receptor, we studied galeterone in a prostate cancer cell line that only expresses AR-V7, and not the full-length androgen receptor. As shown in Figure 6 below, in this study, AR-V7 protein levels were reduced in a dose dependent fashion in cells that only express AR-V7 and not the full-length androgen receptor, confirming that galeterone can act directly on the AR-V7.

Figure 6: Galeterone Causes Decreased Levels of Androgen Receptor in Cell Line Only Expressing AR-V7



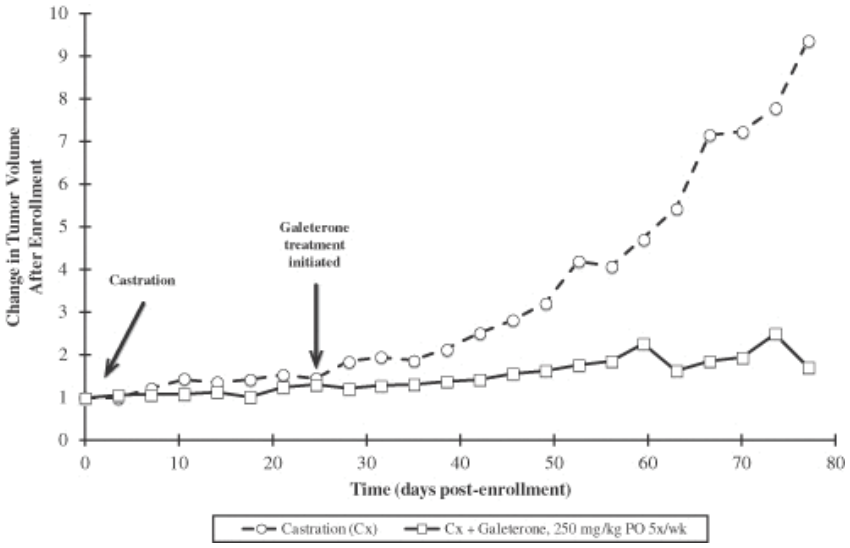
In addition, together with the Vancouver Prostate Centre, we examined whether degradation of androgen receptors translated into reduced androgen receptor signaling and reduced tumor growth in prostate cancer tumor cells which express AR-V7. The Vancouver Prostate Centre conducted a series of studies evaluating the anti-tumor activity of galeterone and enzalutamide in AR-V7 expressing cells. In these studies, galeterone reduced tumor cell proliferation, reduced androgen receptor levels, and decreased nuclear translocation of the androgen receptor, while enzalutamide was only weakly effective in these measures of anti-tumor activity. In these studies, the effect of galeterone or enzalutamide on androgen responsive gene expression was also evaluated by measuring the activity of luciferase, a fluorescent marker, inserted into tumor cells, with lower luciferase activity indicating greater inhibition of androgen signaling. As shown in Figure 7 below, in these studies, the tumor cell line that did not express AR-V7 (V16D) had reduced luciferase activity when treated with enzalutamide or galeterone. However, the enzalutamide-resistant tumor cell lines that did express AR-V7 (49C and 49F) only had reduced luciferase activity when treated with galeterone. When treated with enzalutamide, these tumor cells had increased luciferase activity or only a minimal reduction in luciferase activity, indicating a lower inhibition of androgen signaling relative to galeterone in enzalutamide-resistant tumor cells with AR-V7.

Figure 7: Comparison of Luciferase Activity of Galeterone and Enzalutamide in Enzalutamide-Resistant Tumor Cell Lines



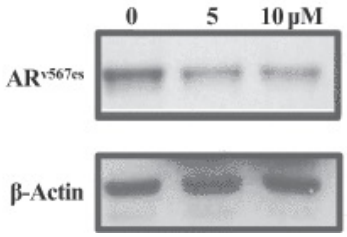
We also evaluated the *in vivo* activity of galeterone in a LuCaP136 xenograft model of human prostate cancer tumor cells grown in castrated mice. LuCaP136 is a prostate cancer cell line that expresses AR-V7. As shown in Figure 8 below, the tumors grew in control animals. However, castrated animals treated with galeterone showed a pronounced tumor growth inhibition.

Figure 8: Galeterone Shows Tumor Growth Inhibition in LuCaP136 (AR-V7 Positive) Castration-Resistant Xenograft Model



We have also evaluated galeterone against a second splice variant, AR^{v567es}. AR^{v567es}, like AR-V7, is a truncated androgen receptor with C-terminal loss. To demonstrate that galeterone would degrade the AR^{v567es} protein alone, in the absence of a full-length androgen receptor, we studied galeterone in a prostate cancer cell line that only expresses AR^{v567es}, and not the full-length androgen receptor. As shown in Figure 9 below, in this study, AR^{v567es} protein levels were reduced in a dose dependent fashion in cells that only express AR^{v567es} and not the full-length androgen receptor, confirming that galeterone can act directly on the AR^{v567es}.

Figure 9: Galeterone Causes Decreased Levels of Androgen Receptor in Cell Line Only Expressing AR^{v567es}



Activity in Androgen Receptor Point Mutations

Patients treated with Xtandi and Zytiga eventually develop resistance such that their tumors continue to grow despite continued treatment. In addition, some patients never respond to initial treatment with Zytiga or Xtandi. Preclinical studies have shown that this resistance may be caused by androgen receptor point mutations such as AR-F876L and AR-T878A. In preclinical studies, galeterone was active against prostate cancer cells that expressed these point mutations.

Galeterone in Combination with Other Therapeutic Drugs

The activation of the Akt/PI3K/mTOR pathway is one of the most frequent alterations observed in human tumor cells. There is growing evidence that the Akt/PI3K/mTOR pathway plays a significant role in prostate cancer tumor progression. Recent scientific publications have shown that there may be a linkage between the androgen receptor signaling pathway and the Akt/PI3K/mTOR pathway such that blocking androgen-dependent signaling may lead to a compensatory upregulation of the Akt/PI3K/mTOR pathway and thus enhanced tumor cell growth. As a result, combination therapies that target both the androgen receptor signaling pathway and the Akt/PI3K/mTOR pathway may have enhanced therapeutic benefit relative to monotherapy.

As part of our exploration of possible therapies to combine with galeterone, we have conducted *in vitro* studies to evaluate whether galeterone acts additively or synergistically with inhibitors of the Akt/PI3K/mTOR pathway, a signaling pathway associated with tumor cell survival, proliferation and invasiveness. In these preclinical studies, we observed that galeterone is synergistic with certain Akt, mTOR and PI3K inhibitors in suppressing prostate cancer cell proliferation. We plan to conduct *in vivo* studies to test drug combinations of galeterone with Akt, mTOR and PI3K inhibitors in xenograft models.

Androgen Receptor Degradation Compounds

We plan to identify and develop novel compounds that are designed to disrupt androgen receptor signaling through enhanced androgen receptor degradation under an exclusive license from UMB. We plan to develop these compounds for use as monotherapies or in combination with existing therapies. We plan to target these compounds for patients with androgen receptor signaling diseases, including prostate cancer patients with primary or acquired resistance to existing therapies.

Manufacturing

Galeterone is a small molecule drug candidate that is manufactured through a reproducible synthetic process from readily available raw materials. Galeterone is manufactured in a proprietary formulation based on spray dried dispersion technology that is designed to produce a product that can provide consistent drug exposure and can be administered with or without food.

We believe that we have sufficient supply of formulated drug to complete the ARMOR2 trial and have completed the production of formulated drug for use in our planned pivotal Phase 3 clinical trial using manufacturers operating under cGMP to manufacture pivotal clinical trial materials.

We do not have our own manufacturing facilities. We currently rely, and expect to continue to rely, on a limited number of third-party contract manufacturers for all of our required raw materials, drug substance and finished product for our preclinical research and clinical trials. We do not have long-term agreements with any of these third parties. We also do not have any current contractual relationships for the manufacture of commercial supplies of any of our product candidates after they are approved. If any of our products are approved by any regulatory agency, we intend to enter into agreements with third-party contract manufacturers and one or more backup manufacturers for the commercial production of those products. We believe that there are a number of qualified manufacturers with which we could enter into commercial supply arrangements. Further, we believe that the process to manufacture galeterone can be scaled up to commercial levels without any unusual equipment.

Commercialization Strategy

We have worldwide development and commercialization rights to galeterone. To maximize the value of these rights, we intend to build a urology- and oncology-focused specialty sales and marketing organization in the United States to support the commercialization of galeterone. We believe that a specialty sales force will be able to target the key prescribing physicians in urology and oncology that treat CRPC. We currently do not have any sales or marketing capabilities or experience. We plan to establish the required capabilities within an appropriate time frame ahead of any product approval and commercialization to support a product launch. To develop the appropriate internal commercial infrastructure in the United States, we will have to invest financial and management resources, some of which will have to be deployed prior to any confirmation that galeterone will be approved. We intend to commercialize galeterone outside the United States through collaborations with third parties.

Competition

The biopharmaceutical industry is characterized by intense competition and rapid innovation. Our potential competitors include large pharmaceutical and biotechnology companies, specialty pharmaceutical companies and generic drug companies. Many of our potential competitors have substantially greater financial, technical and human resources than we do, as well as greater experience in the discovery and development of product candidates, obtaining FDA and other regulatory approvals of products and the commercialization of those products. Accordingly, our potential competitors may be more successful than us in obtaining FDA approval for drugs and achieving widespread market acceptance. Our potential competitors' drugs may be more effective, or more effectively marketed and sold, than any product candidate we may commercialize and may render our product candidates obsolete or non-competitive before we can recover the expenses of their development and commercialization. We anticipate that we will face intense and increasing competition as new drugs enter the market and advanced technologies become available. Finally, the development of new treatment methods for the diseases we are targeting could render our product candidates non-competitive or obsolete.

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 and Johns Hopkins trials, we believe that Zytiga and Xtandi are 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. Zytiga is marketed in the United States by Johnson & Johnson, and Xtandi is marketed in the United States by Astellas Pharma Inc. and Medivation, Inc. ARN-509 is being developed by Johnson & Johnson and ODM-201 is being developed by Bayer Healthcare and Orion Corporation. In addition, depending on the indication for which galeterone is approved, galeterone may compete with chemotherapy and other compounds that are not secondary hormonal treatments, including Jevtana and Provenge, and compounds that are in clinical development, such as Exelixis, Inc.'s Cometriq and Bavarian Nordic A/S's Prostvac.

We believe the key competitive factors that will affect the development and commercial success of galeterone, if approved, will be efficacy, safety and tolerability profile, probability of drug resistance, convenience of the dosing regimen, price, the level of generic competition and the availability of reimbursement from government and other third-party payors.

Intellectual Property

We strive to protect the proprietary technologies that we believe are important to our business, including seeking and maintaining patent protection intended to cover the composition of matter of our product candidates, their methods of use, related technology and other inventions that are important to our business. In addition to patent protection, we also rely on trade secrets and careful monitoring of our proprietary information to protect aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection.

Our success will depend significantly on our ability to obtain and maintain patent and other proprietary protection for commercially important technology, inventions and know-how related to our business, defend and enforce our patents, maintain our licenses to use intellectual property owned by third parties, preserve the confidentiality of our trade secrets and operate without infringing the valid and enforceable patents and other proprietary rights of third parties.

A third party may hold intellectual property, including patent rights, that is important or necessary to commercialize our products. It may be necessary for us to use the patented or proprietary technology of third parties to commercialize our products, in which case we would be required to obtain a license from these third parties on commercially reasonable terms. We may not be able to obtain such licenses on commercially reasonable terms, or at all, in which case our business could be harmed, possibly materially.

We plan to continue to expand our intellectual property estate by filing patent applications directed to dosage forms, methods of treatment and additional compounds and their derivatives. Specifically, we seek patent protection in the United States and internationally for novel compositions of matter covering the compounds, the chemistries and processes for manufacturing these compounds and the use of these compounds in a variety of therapies.

The patent positions of biopharmaceutical companies like us are generally uncertain and involve complex legal, scientific and factual questions. In addition, the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted after issuance. Consequently, we do not know whether any of our product candidates will be protectable or remain protected by enforceable patents. We cannot predict whether the patent applications we are currently pursuing will issue as patents in any particular jurisdiction or whether the claims of any issued patents will provide sufficient proprietary protection from competitors. Any patents that we hold may be challenged, circumvented or invalidated by third parties.

Because patent applications in the United States and certain other jurisdictions are maintained in secrecy for 18 months, and since publication of discoveries in the scientific or patent literature often lags behind actual discoveries, we cannot be certain of the priority of inventions covered by pending patent applications. Moreover, we may have to participate in interference proceedings declared by the United States Patent and Trademark Office, or USPTO, to determine priority of invention or in post-grant challenge proceedings at the USPTO or at a

foreign patent office, such as inter partes review and post grant review proceedings at the USPTO and opposition proceedings at the European Patent Office, that challenge priority of invention or other features of patentability. Such proceedings could result in substantial cost, even if the eventual outcome is favorable to us.

We generally file a provisional patent application with the USPTO first and then subsequently file a corresponding non-provisional patent application, which enables us to establish an earlier effective filing date in the subsequently filed non-provisional patent application. In order to benefit from the earlier effective filing date, we must file a corresponding non-provisional patent application, such as a utility application in the United States or an international application under the Patent Cooperation Treaty, or PCT, within 12 months of the date of the provisional patent application filing. Based on the PCT filing, we may file national and regional patent applications in the United States, the European Union, China, Japan, Australia, Canada, Brazil, India, Indonesia, Israel, Mexico, New Zealand, South Korea, Singapore, South Africa or the Eurasian Patent Organization. To date, we have not filed for patent protection in all national and regional jurisdictions where such protection may be available, and we may decide to abandon national and regional patent applications before a patent is granted. In addition, the patent grant proceeding for each national or regional patent application that we file is an independent proceeding. As a result, it is possible for a patent application to be granted in one jurisdiction and denied in another jurisdiction, and depending on the jurisdiction, the scope of patent protection may vary.

Galeterone Patent Portfolio

As of July 31, 2014, we owned two issued U.S. patents, ten U.S. provisional and non-provisional patent applications, one issued foreign patent and 34 foreign applications in our galeterone patent portfolio. We also had rights under our license agreement with UMB to five issued U.S. patents and 42 issued foreign patents as well as three U.S. patent applications and 11 foreign applications. Our owned and licensed patent and patent applications, if issued, are expected to expire on various dates from 2017 through 2034, without taking into account any possible patent term extensions.

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.

Method of Use. We have licensed from UMB a U.S. patent covering a method of treating prostate cancer in a human subject by administering galeterone, which is expected to expire in 2027. The license also includes granted patents in the European Patent Convention and Japan covering the use of galeterone to treat prostate disease, including prostate cancer and prostatic hyperplasia. Similar patents have been granted or allowed in Australia, Canada, Hong Kong, South Korea, Mexico, New Zealand, Singapore, South Africa, and the Eurasian Patent Organization. These patents are expected to expire in 2026. In addition, we have pending applications in Brazil, China, the European Patent Convention, India, Israel, Indonesia and Japan.

We have also filed a PCT patent application covering the use of galeterone in treating prostate cancer mediated by androgen receptor variants, including splice variants such as AR-V7, as well as the use of biomarkers in identifying patients who are expected to respond to treatment with galeterone. This application is jointly owned with UMB and the University of Washington. The term of a patent derived from this PCT application, if issued, would be expected to expire in 2034.

Pharmaceutical Compositions. We have filed U.S. and international patent applications relating to a galeterone formulation and its use where the galeterone is present in a spray dried dispersion. We have pending

applications in the United States, the European Union, Australia, Brazil, Canada, China, India and Japan. The term of any patent in this family, if issued, would be expected to expire in 2032. In addition, we have licensed from UMB a U.S. patent application covering a pharmaceutical composition of galeterone. The term of any patent, if issued, claiming priority to this application would be expected to expire in 2026.

Combination Treatments. We have filed patent applications or licensed from UMB patent applications covering the use of galeterone in combination with other therapeutic drugs. For example, we have filed U.S. and foreign patent applications covering the use of galeterone in combination with inhibitors of the Akt/PI3K pathway. We have pending applications in the United States, the European Union, Australia, Canada and Japan. The term of any patent in this family, if issued, would be expected to expire in 2032.

Prodrugs, Metabolites and Analogs. We have filed patent applications or licensed from UMB patent applications directed to prodrugs, metabolites or analogs of galeterone. For example, we have licensed a U.S. patent application from UMB directed to certain prodrugs of galeterone. If issued, the term of the resulting patent, if issued, would be expected to expire in 2029. We have also filed patent applications in the United States and certain other countries including Australia, Brazil, Canada, China, the European Union, India and Japan directed to other prodrugs of galeterone. If issued, the term of the resulting patents would be expected to expire in 2030. Further, we have filed patent applications in the United States and certain other countries including Australia, Brazil, Canada, China, the European Union, India and Japan directed to compounds which have been identified as metabolites of galeterone and which may be biologically active. If issued, the term of the resulting patents would be expected to expire in 2030. We have also obtained a license to a UMB PCT patent application directed to analogs of galeterone that disrupt androgen receptor signaling by degrading the androgen receptor. The term of any patent, if issued, claiming priority to this PCT patent application would be expected to extend to 2034.

The term of individual patents depends upon the legal term of the patents in the countries in which they are obtained. In most countries in which we file, the patent term is 20 years from the earliest date of filing a non-provisional patent application. In the United States, the patent term of a patent that covers an FDA-approved drug may also be eligible for patent term extension, which permits patent term restoration as compensation for the patent term lost during the FDA regulatory review process. The Hatch-Waxman Act permits a patent term extension of up to five years beyond the expiration of the patent. The length of the patent term extension is related to the length of time the drug is under regulatory review. Patent extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only one patent applicable to an approved drug may be extended. Similar provisions are available in Europe and other non-United States jurisdictions to extend the term of a patent that covers an approved drug. In the future, if and when our pharmaceutical products receive FDA approval, we expect to apply for patent term extensions on patents covering those products. We intend to seek patent term extensions to any of our issued patents in any jurisdiction where these are available, however there is no guarantee that the applicable authorities, including the FDA in the United States, will agree with our assessment of whether such extensions should be granted, and even if granted, the length of such extensions.

We also rely on trade secret protection for our confidential and proprietary information. Although we take steps to protect our proprietary information and trade secrets, including through contractual means with our employees and consultants, third parties may independently develop substantially equivalent proprietary information and techniques or otherwise gain access to our trade secrets or disclose our technology. Thus, we may not be able to meaningfully protect our trade secrets. It is our policy to require our employees, consultants, outside scientific collaborators, sponsored researchers and other advisors to execute confidentiality agreements upon the commencement of employment or consulting relationships with us. These agreements provide that all confidential information concerning our business or financial affairs developed or made known to the individual during the course of the individual's relationship with us is to be kept confidential and not disclosed to third parties except in specific circumstances. In the case of employees, the agreements provide that all inventions conceived by the individual, and which are related to our current or planned business or research and development or made during normal working hours, on our premises or using our equipment or proprietary information, are our exclusive property.

License Agreement with University of Maryland, Baltimore

In May 2006, we entered into a master license agreement with UMB. Pursuant to the license agreement, UMB granted us 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, which we refer to as licensed products, and to otherwise practice the patent rights in any manner, for the prevention, diagnosis, treatment or control of any human or animal disease. In addition, UMB granted us a first option to receive an exclusive license to UMB's rights in certain improvements to the licensed products, which improvements we refer to as licensed improvements.

We have exercised our option and acquired exclusive rights to licensed improvements under three amendments to the license agreement. In March 2009, the license agreement was amended to grant us an exclusive license to oral prodrugs of the licensed products. In April 2012, the license agreement was amended to grant us an exclusive license to compositions and methods of inducing endoplasmic reticulum stress. In October 2013, the license agreement was amended to grant us an exclusive license to a patent application directed to analogs of galeterone that disrupt androgen receptor signaling by degrading the androgen receptor.

Under the terms of the license agreement, as amended, we are obligated to use commercially reasonable efforts to develop and commercialize one or more licensed products. We must also achieve specified milestone events by specified dates. Unless our license agreement with UMB is terminated earlier as provided below, our exclusive license from UMB expires on a country-by-country basis as of the later of the expiration date of the last to expire of the claims of the patent rights licensed to us under the agreement in such country or ten years after the first commercial sale of a licensed product in such country. UMB may terminate the agreement if we fail to achieve such milestone events and do not cure such failure within a specified termination notice period. UMB may also terminate the agreement upon our breach of our payment obligations or our other material breaches under the agreement if we do not cure such breach within a specified notice period or upon our bankruptcy or insolvency. We may terminate the agreement at any time, on a country-by-country basis, if we determine that a license under the licensed patent rights in an applicable country is not advantageous to our commercial success, provided that our payment obligations with respect to licensed products in such country would survive termination if we continued to develop and commercialize licensed products in such country following such a termination.

In consideration for the rights granted to us, we made an upfront payment to UMB of $20,000 following the execution of the license agreement and a payment of $10,000 following the execution of each of the March 2009, April 2012 and October 2013 amendments. We are obligated to pay UMB an annual maintenance fee of $10,000 each year until the first commercial sale. In addition, we paid UMB a $50,000 milestone payment upon the submission of our IND for galeterone and a $40,000 milestone payment upon the issuance of the first patent related to UMB's prodrug patent application. We are obligated to make an additional $50,000 milestone payment to UMB for each additional IND we file for a licensed product and a $100,000 milestone payment upon the approval of each NDA for a licensed product by the FDA. We must also pay UMB low-single digit percentage royalties on aggregate worldwide net sales of licensed products, including sales by our 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. Our royalty obligations are subject to specified reductions in the event that we are required to obtain additional licenses from third parties or in the event of specified competition from third-party products licensed by UMB. Our minimum annual royalty payment to UMB is $50,000 beginning in the year following the year in which the first commercial sale occurs. We must also pay UMB 10% of all non-royalty sublicense income received from sublicensees. Finally, as of April 10, 2012, we assumed responsibility for all patent expenses related to the prosecution and maintenance of the licensed patents.

Government Regulation and Product Approvals

Government authorities in the United States, at the federal, state and local level, and in other countries and jurisdictions, including the European Union, extensively regulate, among other things, the research, development, testing, manufacture, quality control, clearance, approval, packaging, storage, recordkeeping, labeling, advertising, promotion, distribution, marketing, post-approval monitoring and reporting, and import and export of pharmaceutical products and medical devices. The processes for obtaining regulatory clearances and approvals in the United States and in foreign countries and jurisdictions, along with subsequent compliance with applicable statutes and regulations and other regulatory authorities, require the expenditure of substantial time and financial resources.

Review and Approval of Drugs in the United States

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act, or FDCA, and implementing regulations. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval may subject an applicant and/or sponsor to a variety of administrative or judicial sanctions, including refusal by the FDA to approve pending applications, withdrawal of an approval, imposition of a clinical hold, issuance of warning letters and other types of letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement of profits, or civil or criminal investigations and penalties brought by the FDA and the Department of Justice, or DOJ, or other governmental entities.

An applicant seeking approval to market and distribute a new drug product in the United States must typically undertake the following:

- completion of preclinical laboratory tests, animal studies and formulation studies in compliance with the FDA's good laboratory practice, or GLP, regulations;
- submission to the FDA of an IND, which must take effect before human clinical trials may begin;
- approval by an independent institutional review board, or IRB, representing each clinical site before each clinical trial may be initiated;
- performance of adequate and well-controlled human clinical trials in accordance with good clinical practices, or GCP, to establish the safety and efficacy of the proposed drug product for each indication;
- preparation and submission to the FDA of a new drug application, or NDA;
- review of the product by an FDA advisory committee, where appropriate or if applicable;
- satisfactory completion of one or more FDA inspections of the manufacturing facility or facilities at which the product, or components thereof, are produced to assess compliance with current Good Manufacturing Practices, or cGMP, requirements and to assure that the facilities, methods and controls are adequate to preserve the product's identity, strength, quality and purity;
- satisfactory completion of FDA audits of clinical trial sites to assure compliance with GCPs and the integrity of the clinical data;
- payment of user fees and securing FDA approval of the NDA; and
- compliance with any post-approval requirements, including Risk Evaluation and Mitigation Strategies, or REMS, and post-approval studies required by the FDA.

Preclinical Studies

Preclinical studies include laboratory evaluation of the purity and stability of the manufactured drug substance or active pharmaceutical ingredient and the formulated drug or drug product, as well as *in vitro* and animal studies to assess the safety and activity of the drug for initial testing in humans and to establish a rationale

for therapeutic use. The conduct of preclinical studies is subject to federal regulations and requirements, including GLP regulations. The results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and plans for clinical trials, among other things, are submitted to the FDA as part of an IND. Some long-term preclinical testing, such as animal tests of reproductive adverse events and carcinogenicity, may continue after the IND is submitted.

Human Clinical Trials in Support of an NDA

Clinical trials involve the administration of the investigational product to human subjects under the supervision of qualified investigators in accordance with GCP requirements, which include, among other things, the requirement that all research subjects provide their informed consent in writing before their participation in any clinical trial. Clinical trials are conducted under written study protocols detailing, among other things, the inclusion and exclusion criteria, the objectives of the study, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. A protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND. An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions related to a proposed clinical trial and places the trial on clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. Accordingly, submission of an IND may or may not result in the FDA allowing clinical trials to commence.

In addition, an IRB representing each institution participating in the clinical trial must review and approve the plan for any clinical trial before it commences at that institution, and the IRB must conduct a continuing review and reapprove the study at least annually. The IRB must review and approve, among other things, the study protocol and informed consent information to be provided to study subjects. An IRB must operate in compliance with FDA regulations. Information about certain clinical trials must be submitted within specific timeframes to the National Institutes of Health for public dissemination on their ClinicalTrials.gov website.

A sponsor who wishes to conduct a clinical trial outside the United States may, but need not, obtain FDA authorization to conduct the clinical trial under an IND. If a foreign clinical trial is not conducted under an IND, the sponsor may submit data from the clinical trial to the FDA in support of an NDA or IND so long as the clinical trial is conducted in compliance with an international guideline for the ethical conduct of clinical research known as the Declaration of Helsinki and/or the laws and regulations of the country or countries in which the clinical trial is performed, whichever provides the greater protection to the participants in the clinical trial.

Human clinical trials are typically conducted in three sequential phases, which may overlap or be combined:

- ***Phase 1:*** The drug is initially introduced into a small number of healthy human subjects or, in certain indications such as cancer, patients with the target disease or condition and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an early indication of its effectiveness and to determine optimal dosage.
- ***Phase 2:*** The drug is administered to a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage.
- ***Phase 3:*** Phase 3 clinical trials are commonly referred to as “pivotal” trials, which typically denotes a trial which generates the principal data that the FDA or other relevant regulatory agency will use to determine whether or not to approve a drug. In Phase 3 trials, the drug is administered to an expanded patient population, generally at geographically dispersed clinical trial sites, in well-controlled clinical trials to generate enough data to statistically evaluate the efficacy and safety of the product for approval, to establish the overall risk-benefit profile of the product, and to provide adequate information for the labeling of the product.

Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and more frequently if serious adverse events occur. In addition, IND safety reports must be submitted to the FDA for any of the following: serious and unexpected suspected adverse reactions; findings from other studies or animal or *in vitro* testing that suggest a significant risk in humans exposed to the drug; and any clinically important increase in the case of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, or at all. Furthermore, the FDA or the sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research subjects are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution, or an institution it represents, if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug has been associated with unexpected serious harm to patients. The FDA will typically inspect one or more clinical sites to assure compliance with GCP and the integrity of the clinical data submitted.

Section 505(b)(2) NDAs

NDAs for most new drug products are based on two full clinical trials that must contain substantial evidence of the safety and efficacy of the proposed new product. These applications are submitted under Section 505(b)(1) of the FDCA. The FDA is, however, authorized to approve an alternative type of NDA under Section 505(b)(2) of the FDCA. This type of application allows the applicant to rely, in part, on the FDA's previous findings of safety and efficacy for a similar product, or published literature. Specifically, Section 505(b)(2) applies to NDAs for a drug for which the investigations made to show whether or not the drug is safe for use and effective in use and relied upon by the applicant for approval of the application "were not conducted by or for the applicant and for which the applicant has not obtained a right of reference or use from the person by or for whom the investigations were conducted."

Thus, Section 505(b)(2) authorizes the FDA to approve an NDA based on safety and effectiveness data that were not developed by the applicant. NDAs filed under Section 505(b)(2) may provide an alternate and potentially more expeditious pathway to FDA approval for new or improved formulations or new uses of previously approved products. If the 505(b)(2) applicant can establish that reliance on the FDA's previous approval is scientifically appropriate, the applicant may eliminate the need to conduct certain preclinical studies or clinical trials of the new product. The FDA may also require companies to perform additional studies, trials or measurements to support the change from the approved product. The FDA may then approve the new drug candidate for all or some of the label indications for which the referenced product has been approved, as well as for any new indication sought by the Section 505(b)(2) applicant.

Submission of an NDA to the FDA

Assuming successful completion of required clinical testing and other requirements, the results of the preclinical studies and clinical trials, together with detailed information relating to the product's chemistry, manufacture, controls and proposed labeling, among other things, are submitted to the FDA as part of an NDA requesting approval to market the drug product for one or more indications. Under federal law, the submission of most NDAs is additionally subject to an application user fee, currently exceeding $2.1 million, and the sponsor of an approved NDA is also subject to annual product and establishment user fees, currently exceeding $104,000 per product and $554,000 per establishment. These fees are typically increased annually.

The FDA conducts a preliminary review of an NDA within 60 days of its receipt and informs the sponsor by the 74th day after the FDA's receipt of the submission to determine whether the application is sufficiently complete to permit substantive review. The FDA may request additional information rather than accept an NDA for filing. In this event, the application must be resubmitted with the additional information. The resubmitted application is also subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review. The FDA has agreed to specified performance goals in the review process of NDAs. Most such applications are meant to be reviewed within ten months from the date of

filing, and most applications for "priority review" products are meant to be reviewed within six months of filing. The review process may be extended by the FDA for various reasons, including for three additional months to consider new information or clarification provided by the applicant to address an outstanding deficiency identified by the FDA following the original submission.

Before approving an NDA, the FDA typically will inspect the facility or facilities where the product is or will be manufactured. These pre-approval inspections cover all facilities associated with an NDA submission, including drug component manufacturing (such as Active Pharmaceutical Ingredients), finished drug product manufacturing, and control testing laboratories. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA will typically inspect one or more clinical sites to assure compliance with GCP.

The FDA may refer an application for a novel drug to an advisory committee or explain why such referral was not made. Typically, an advisory committee is a panel of independent experts, including clinicians and other scientific experts, that reviews, evaluates and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Fast Track, Breakthrough Therapy and Priority Review Designations

The FDA is authorized to designate certain products for expedited review if they are intended to address an unmet medical need in the treatment of a serious or life-threatening disease or condition. These programs are fast track designation, breakthrough therapy designation and priority review designation.

Specifically, the FDA may designate a product for fast track review if it is intended, whether alone or in combination with one or more other drugs, for the treatment of a serious or life-threatening disease or condition, and it demonstrates the potential to address unmet medical needs for such a disease or condition. For fast track products, sponsors may have greater interactions with the FDA and the FDA may initiate review of sections of a fast track product's NDA before the application is complete. This rolling review may be available if the FDA determines, after preliminary evaluation of clinical data submitted by the sponsor, that a fast track product may be effective. The sponsor must also provide, and the FDA must approve, a schedule for the submission of the remaining information and the sponsor must pay applicable user fees. However, the FDA's time period goal for reviewing a fast track application does not begin until the last section of the NDA is submitted. In addition, the fast track designation may be withdrawn by the FDA if the FDA believes that the designation is no longer supported by data emerging in the clinical trial process.

Second, in 2012, Congress enacted the Food and Drug Administration Safety and Innovation Act, or FDASIA. This law established a new regulatory scheme allowing for expedited review of products designated as "breakthrough therapies." A product may be designated as a breakthrough therapy if it is intended, either alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The FDA may take certain actions with respect to breakthrough therapies, including holding meetings with the sponsor throughout the development process; providing timely advice to the product sponsor regarding development and approval; involving more senior staff in the review process; assigning a cross-disciplinary project lead for the review team; and taking other steps to design the clinical trials in an efficient manner.

Third, the FDA may designate a product for priority review if it is a drug that treats a serious condition and, if approved, would provide a significant improvement in safety or effectiveness. The FDA determines, on a case- by-case basis, whether the proposed drug represents a significant improvement when compared with other available therapies. Significant improvement may be illustrated by evidence of increased effectiveness in the

treatment of a condition, elimination or substantial reduction of a treatment-limiting drug reaction, documented enhancement of patient compliance that may lead to improvement in serious outcomes, and evidence of safety and effectiveness in a new subpopulation. A priority designation is intended to direct overall attention and resources to the evaluation of such applications, and to shorten the FDA's goal for taking action on a marketing application from ten months to six months.

Accelerated Approval Pathway

The FDA may grant accelerated approval to a drug for a serious or life-threatening condition that provides meaningful therapeutic advantage to patients over existing treatments based upon a determination that the drug has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit. The FDA may also grant accelerated approval for such a condition when the product has an effect on an intermediate clinical endpoint that can be measured earlier than an effect on irreversible morbidity or mortality, or IMM, and that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. Drugs granted accelerated approval must meet the same statutory standards for safety and effectiveness as those granted traditional approval.

For the purposes of accelerated approval, a surrogate endpoint is a marker, such as a laboratory measurement, radiographic image, physical sign, or other measure that is thought to predict clinical benefit, but is not itself a measure of clinical benefit. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. An intermediate clinical endpoint is a measurement of a therapeutic effect that is considered reasonably likely to predict the clinical benefit of a drug, such as an effect on IMM. The FDA has limited experience with accelerated approvals based on intermediate clinical endpoints, but has indicated that such endpoints generally may support accelerated approval where the therapeutic effect measured by the endpoint is not itself a clinical benefit and basis for traditional approval, if there is a basis for concluding that the therapeutic effect is reasonably likely to predict the ultimate clinical benefit of a drug.

The accelerated approval pathway is most often used in settings in which the course of a disease is long and an extended period of time is required to measure the intended clinical benefit of a drug, even if the effect on the surrogate or intermediate clinical endpoint occurs rapidly. Thus, accelerated approval has been used extensively in the development and approval of drugs for treatment of a variety of cancers in which the goal of therapy is generally to improve survival or decrease morbidity and the duration of the typical disease course requires lengthy and sometimes large trials to demonstrate a clinical or survival benefit.

The accelerated approval pathway is usually contingent on a sponsor's agreement to conduct, in a diligent manner, additional post-approval confirmatory studies to verify and describe the drug's clinical benefit. As a result, a drug candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the effect on the clinical endpoint. Failure to conduct required post-approval studies, or confirm a clinical benefit during post-marketing studies, would allow the FDA to withdraw the drug from the market on an expedited basis. All promotional materials for drug candidates approved under accelerated regulations are subject to prior review by the FDA.

The FDA's Decision on an NDA

On the basis of the FDA's evaluation of the NDA and accompanying information, including the results of the inspection of the manufacturing facilities, the FDA may issue an approval letter or a complete response letter. An approval letter authorizes commercial marketing of the product with specific prescribing information for specific indications. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. If and when those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the NDA, the FDA will issue an approval letter. The FDA has committed to reviewing such resubmissions in two or six months

depending on the type of information included. Even with submission of this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

If the FDA approves a product, it may limit the approved indications for use for the product, require that contraindications, warnings or precautions be included in the product labeling, require that post-approval studies, including Phase 4 clinical trials, be conducted to further assess the drug's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution restrictions or other risk management mechanisms, including REMS, which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-market studies or surveillance programs. After approval, many types of changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to further testing requirements and FDA review and approval.

In addition, as a condition of approval, the FDA may require an applicant to develop a REMS. REMS use risk minimization strategies beyond the professional labeling to ensure that the benefits of the product outweigh the potential risks. To determine whether a REMS is needed, the FDA will consider the size of the population likely to use the product, seriousness of the disease, expected benefit of the product, expected duration of treatment, seriousness of known or potential adverse events, and whether the product is a new molecular entity. REMS can include medication guides, physician communication plans for healthcare professionals, and elements to assure safe use, or ETASU. ETASU may include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring, and the use of patient registries. The FDA may require a REMS before approval or post-approval if it becomes aware of a serious risk associated with use of the product. The requirement for a REMS can materially affect the potential market and profitability of a product.

Post-Approval Requirements

Drugs manufactured or distributed pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to recordkeeping, periodic reporting, product sampling and distribution, advertising and promotion and reporting of adverse experiences with the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims, are subject to prior FDA review and approval. There also are continuing, annual user fee requirements for any marketed products and the establishments at which such products are manufactured, as well as new application fees for supplemental applications with clinical data.

In addition, drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and state agencies, and are subject to periodic unannounced inspections by the FDA and these state agencies for compliance with cGMP requirements. Changes to the manufacturing process are strictly regulated and often require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP and impose reporting and documentation requirements upon the sponsor and any third-party manufacturers that the sponsor may decide to use. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance.

Once an approval is granted, the FDA may withdraw the approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;

- fines, warning letters or holds on post-approval clinical trials;
- refusal of the FDA to approve pending NDAs or supplements to approved NDAs, or suspension or revocation of product license approvals;
- product seizure or detention, or refusal to permit the import or export of products; or
- injunctions or the imposition of civil or criminal penalties.

The FDA strictly regulates marketing, labeling, advertising and promotion of products that are placed on the market. Drugs may be promoted only for the approved indications and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability.

In addition, the distribution of prescription pharmaceutical products is subject to the Prescription Drug Marketing Act, or PDMA, which regulates the distribution of drugs and drug samples at the federal level, and sets minimum standards for the registration and regulation of drug distributors by the states. Both the PDMA and state laws limit the distribution of prescription pharmaceutical product samples and impose requirements to ensure accountability in distribution.

Abbreviated New Drug Applications for Generic Drugs

In 1984, with passage of the Hatch-Waxman Amendments to the FDCA, Congress authorized the FDA to approve generic drugs that are the same as drugs previously approved by the FDA under the NDA provisions of the statute. To obtain approval of a generic drug, an applicant must submit an abbreviated new drug application, or ANDA, to the agency. In support of such applications, a generic manufacturer may rely on the preclinical and clinical testing previously conducted for a drug product previously approved under an NDA, known as the reference listed drug, or RLD.

Specifically, in order for an ANDA to be approved, the FDA must find that the generic version is identical to the RLD with respect to the active ingredients, the route of administration, the dosage form, and the strength of the drug. At the same time, the FDA must also determine that the generic drug is "bioequivalent" to the innovator drug. Under the statute, a generic drug is bioequivalent to a RLD if "the rate and extent of absorption of the drug do not show a significant difference from the rate and extent of absorption of the listed drug…"

Upon approval of an ANDA, the FDA indicates whether the generic product is "therapeutically equivalent" to the RLD in its publication "Approved Drug Products with Therapeutic Equivalence Evaluations," also referred to as the "Orange Book." Physicians and pharmacists consider a therapeutic equivalent generic drug to be fully substitutable for the RLD. In addition, by operation of certain state laws and numerous health insurance programs, the FDA's designation of therapeutic equivalence often results in substitution of the generic drug without the knowledge or consent of either the prescribing physician or patient.

Under the Hatch-Waxman Amendments, the FDA may not approve an ANDA until any applicable period of non-patent exclusivity for the RLD has expired. The FDCA provides a period of five years of non-patent data exclusivity for a new drug containing a new chemical entity. In cases where such exclusivity has been granted, an ANDA may not be filed with the FDA until the expiration of five years unless the submission is accompanied by a Paragraph IV certification, in which case the applicant may submit its application four years following the original product approval. The FDCA also provides for a period of three years of exclusivity if the NDA includes reports of one or more new clinical investigations, other than bioavailability or bioequivalence studies, that were conducted by or for the applicant and are essential to the approval of the application. This three-year exclusivity period often protects changes to a previously approved drug product, such as a new dosage form, route of administration, combination or indication.

Hatch-Waxman Patent Certification and the 30-Month Stay

Upon approval of an NDA or a supplement thereto, NDA sponsors are required to list with the FDA each patent with claims that cover the applicant's product or an approved method of using the product. Each of the patents listed by the NDA sponsor is published in the Orange Book. When an ANDA applicant files its application with the FDA, the applicant is required to certify to the FDA concerning any patents listed for the reference product in the Orange Book, except for patents covering methods of use for which the ANDA applicant is not seeking approval. To the extent that the Section 505(b)(2) applicant is relying on studies conducted for an already approved product, the applicant is required to certify to the FDA concerning any patents listed for the approved product in the Orange Book to the same extent that an ANDA applicant would.

Specifically, the applicant must certify with respect to each patent that:

- the required patent information has not been filed;
- the listed patent has expired;
- the listed patent has not expired, but will expire on a particular date and approval is sought after patent expiration; or
- the listed patent is invalid, unenforceable or will not be infringed by the new product.

A certification that the new product will not infringe the already approved product's listed patents or that such patents are invalid or unenforceable is called a Paragraph IV certification. If the applicant does not challenge the listed patents or indicates that it is not seeking approval of a patented method of use, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired (other than method of use patents involving indications for which the ANDA applicant is not seeking approval).

If the ANDA applicant or 505(b)(2) applicant has provided a Paragraph IV certification to the FDA, the applicant must also send notice of the Paragraph IV certification to the NDA and patent holders once the ANDA has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. The filing of a patent infringement lawsuit within 45 days after the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months after the receipt of the Paragraph IV notice, expiration of the patent, or a decision in the infringement case that is favorable to the ANDA applicant.

To the extent that the Section 505(b)(2) applicant is relying on studies conducted for an already approved product, the applicant is required to certify to the FDA concerning any patents listed for the approved product in the Orange Book to the same extent that an ANDA applicant would. As a result, approval of a 505(b)(2) NDA can be stalled until all the listed patents claiming the referenced product have expired, until any non-patent exclusivity, such as exclusivity for obtaining approval of a new chemical entity, listed in the Orange Book for the referenced product has expired, and, in the case of a Paragraph IV certification and subsequent patent infringement suit, until the earlier of 30 months, settlement of the lawsuit or a decision in the infringement case that is favorable to the Section 505(b)(2) applicant.

Pediatric Studies and Exclusivity

Under the Pediatric Research Equity Act of 2003, an NDA or supplement thereto must contain data that are adequate to assess the safety and effectiveness of the drug product for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. With enactment of the FDASIA in 2012, sponsors must also submit pediatric study plans prior to the assessment data. Those plans must contain an outline of the proposed pediatric study or studies the applicant plans to conduct, including study objectives and design, any deferral or waiver requests, and other information required by regulation. The applicant, the FDA, and the FDA's internal review committee must then review the information submitted, consult with each other, and agree upon a final plan. The FDA or the applicant may request an amendment to the plan at any time.

The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. Additional requirements and procedures relating to deferral requests and requests for extension of deferrals are contained in FDASIA. Unless otherwise required by regulation, the pediatric data requirements do not apply to products with orphan designation.

Pediatric exclusivity is another type of non-patent marketing exclusivity in the United States and, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity, including the non-patent and orphan exclusivity. This six-month exclusivity may be granted if an NDA sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The data do not need to show the product to be effective in the pediatric population studied; rather, if the clinical trial is deemed to fairly respond to the FDA's request, the additional protection is granted. If reports of requested pediatric studies are submitted to and accepted by the FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity or patent protection cover the product are extended by six months. This is not a patent term extension, but it effectively extends the regulatory period during which the FDA cannot approve another application.

Orphan Designation and Exclusivity

Under the Orphan Drug Act, the FDA may designate a drug product as an "orphan drug" if it is intended to treat a rare disease or condition (generally meaning that it affects fewer than 200,000 individuals in the United States, or more in cases in which there is no reasonable expectation that the cost of developing and making a drug product available in the United States for treatment of the disease or condition will be recovered from sales of the product). A company must request orphan product designation before submitting an NDA. If the request is granted, the FDA will disclose the identity of the therapeutic agent and its potential use. Orphan product designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a product with orphan status receives the first FDA approval for the disease or condition for which it has such designation or for a select indication or use within the rare disease or condition for which it was designated, the product generally will be receiving orphan product exclusivity. Orphan product exclusivity means that the FDA may not approve any other applications for the same product for the same indication for seven years, except in certain limited circumstances. Competitors may receive approval of different products for the indication for which the orphan product has exclusivity and may obtain approval for the same product but for a different indication. If a drug or drug product designated as an orphan product ultimately receives marketing approval for an indication broader than what was designated in its orphan product application, it may not be entitled to exclusivity.

Patent Term Restoration and Extension

A patent claiming a new drug product or medical device may be eligible for a limited patent term extension under the FDCA, which permits a patent restoration of up to five years for patent term lost during product development and the FDA regulatory review. The restoration period granted on a patent covering a new drug product or medical device is typically one-half the time between the date a clinical investigation on human beings is begun and the submission date of an application for approval of the product, plus the time between the submission date of an application for approval of the product and the ultimate approval date. Patent term restoration cannot be used to extend the remaining term of a patent past a total of 14 years from the product's approval date. Only one patent applicable to an approved drug product or medical device is eligible for the extension, and the application for the extension must be submitted prior to the expiration of the patent in question. A patent that covers multiple drugs or medical devices for which approval is sought can only be extended in connection with one of the approvals. The USPTO reviews and approves the application for any patent term extension or restoration in consultation with the FDA.

Review and Approval of Companion Diagnostics in the United States

We expect that we will rely upon an *in vitro* companion diagnostic test for use in selecting patients with AR-V7. In July 2014, the FDA issued final guidance stating that if an *in vitro* diagnostic is essential to the safe and effective use of a therapeutic product, then the FDA generally will require approval or clearance of the diagnostic at the same time that the FDA approves the therapeutic product. *In vitro* diagnostics marketed in the United States are regulated as medical devices. As a result, unless an exemption applies, a new medical device may not be marketed in the United States unless and until it has been cleared through filing of a 510(k) premarket notification, or 510(k), or approved by the FDA pursuant to the PMA process. Based on our discussions with the FDA, we believe that the companion diagnostic for galeterone will need to be approved through the PMA process.

510(k) Premarket Notification

To obtain 510(k) clearance, a manufacturer must submit a premarket notification demonstrating that the proposed device is "substantially equivalent" to a predicate device, which is a previously cleared 510(k) device or a pre-amendment device that was in commercial distribution before May 28, 1976, that is a class I or II device, or a class III device for which the FDA has not yet called for the submission of a PMA application. The FDA's 510(k) clearance pathway usually takes from three to 12 months from the date the notification is submitted and filed with the FDA, but it can take significantly longer and clearance is never assured. The FDA has issued guidance documents meant to expedite review of a 510(k) and facilitate interactions between manufacturers and the agency. To demonstrate substantial equivalence, a manufacturer must show that the device has the same intended use as a predicate device and the same technological characteristics, or the same intended use and different technological characteristics and does not raise new questions of safety and effectiveness than the predicate device. Most 510(k)s do not require clinical data for clearance, but the FDA may request such data. If the FDA concludes that the device is not substantially equivalent to a predicate device, the manufacturer will need to submit a PMA to market the device. Alternatively, a manufacturer may request a *de novo* classification if the device is of low to moderate risk and there is no predicate device upon which to base a substantial equivalence determination.

Premarket Approval

The PMA process for approval to market a medical device is more complex, costly, and time consuming than the 510(k) clearance procedure. A PMA must be supported by extensive data, including technical information regarding device design and development, preclinical studies, clinical studies, manufacturing and controls information and labeling information, that demonstrates the safety and effectiveness of the device for its intended use. The FDA may refer a PMA to an advisory committee for its recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions. If the FDA's evaluations of both the PMA and the manufacturing facility for the device are favorable, the FDA will either issue an approval letter authorizing commercial marketing or an approvable letter that usually contains a number of conditions that must be met in order to secure final approval. If the FDA's evaluations are not favorable, the FDA will deny approval of the PMA or issue a not approvable letter. The PMA process, including the gathering of clinical and preclinical data and the submission to and review by the FDA, can take several years, and the process can be expensive and uncertain. Moreover, even if the FDA approves a PMA, the FDA may approve the device with an indication that is narrower or more limited than originally sought, and the agency may impose post-approval conditions that it believes necessary to ensure the safety and effectiveness of the device.

Investigational Device Exemption

A clinical trial is typically required for a PMA and, in a small percentage of cases, the FDA may require a clinical trial in support of a 510(k) submission. A manufacturer that wishes to conduct a clinical trial involving the device is subject to the FDA's IDE regulation. The IDE regulation distinguishes between significant and nonsignificant risk device trials and the procedures for obtaining approval to begin the trial differ accordingly.

Also, some types of trials are exempt from the IDE regulations. A significant risk device presents a potential for serious risk to the health, safety or welfare of a subject. Significant risk devices are devices that are substantially important in diagnosing, curing, mitigating or treating disease or in preventing impairment to human health. Trials of devices that pose a significant risk require both FDA and an IRB approval prior to initiation of a clinical trial. Nonsignificant risk devices are devices that do not pose a significant risk to the human subjects. A nonsignificant risk device trial requires only IRB approval prior to initiation of a clinical trial.

An IDE application must be supported by appropriate data, such as animal and laboratory testing results, showing that it is safe to test the device in humans and that the testing protocol is scientifically sound. An IDE application is considered approved 30 days after it has been received by the FDA, unless the FDA otherwise informs the sponsor, prior to 30 calendar days from the date of receipt, that the IDE is approved, approved with conditions or disapproved. The FDA typically grants IDE approval for a specified number of subjects to be enrolled at specified trial centers. The clinical trial must be conducted in accordance with applicable regulations, including but not limited to the FDA's IDE regulations. The investigators must obtain subject informed consent, rigorously follow the investigational plan and trial protocol, control the disposition of investigational devices, and comply with all reporting and record keeping requirements. A clinical trial may be suspended or terminated by the FDA, the IRB or the sponsor at any time for various reasons, including a belief that the risks to the trial participants outweigh the benefits of participation in the trial. Approval of an IDE does not bind the FDA to accept the results of the trial as sufficient to prove the product's safety and efficacy, even if the trial meets its intended success criteria.

Review and Approval of Drug Products in the European Union

In order to market any product outside of the United States, a company must also comply with numerous and varying regulatory requirements of other countries and jurisdictions regarding quality, safety and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of drug products. Whether or not it obtains FDA approval for a product, the company would need to obtain the necessary approvals by the comparable foreign regulatory authorities before it can commence clinical trials or marketing of the product in those countries or jurisdictions. The approval process ultimately varies between countries and jurisdictions and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries and jurisdictions might differ from and be longer than that required to obtain FDA approval. Regulatory approval in one country or jurisdiction does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country or jurisdiction may negatively impact the regulatory process in others.

Pursuant to the European Clinical Trials Directive, a system for the approval of clinical trials in the European Union has been implemented through national legislation of the member states. Under this system, an applicant must obtain approval from the competent national authority of a European Union member state in which the clinical trial is to be conducted. Furthermore, the applicant may only start a clinical trial after a competent ethics committee has issued a favorable opinion. Clinical trial application must be accompanied by an investigational medicinal product dossier with supporting information prescribed by the European Clinical Trials Directive and corresponding national laws of the member states and further detailed in applicable guidance documents.

To obtain marketing approval of a drug under European Union regulatory systems, an applicant must submit a marketing authorization application, or MAA, either under a centralized or decentralized procedure.

The centralized procedure provides for the grant of a single marketing authorization by the European Commission that is valid for all European Union member states. The centralized procedure is compulsory for specific products, including for medicines produced by certain biotechnological processes, products designated as orphan medicinal products, advanced therapy products and products with a new active substance indicated for the treatment of certain diseases. For products with a new active substance indicated for the treatment of other diseases and products that are highly innovative or for which a centralized process is in the interest of patients, the centralized procedure may be optional.

Under the centralized procedure, the Committee for Medicinal Products for Human Use, or the CHMP, established at the European Medicines Agency, or EMA, is responsible for conducting the initial assessment of a drug. The CHMP is also responsible for several post-authorization and maintenance activities, such as the assessment of modifications or extensions to an existing marketing authorization. Under the centralized procedure in the European Union, the maximum timeframe for the evaluation of an MAA is 210 days, excluding clock stops, when additional information or written or oral explanation is to be provided by the applicant in response to questions of the CHMP. Accelerated evaluation might be granted by the CHMP in exceptional cases, when a medicinal product is of major interest from the point of view of public health and in particular from the viewpoint of therapeutic innovation. In this circumstance, the EMA ensures that the opinion of the CHMP is given within 150 days.

The decentralized procedure is available to applicants who wish to market a product in various European Union member states where such product has not received marketing approval in any European Union member states before. The decentralized procedure provides for approval by one or more other, or concerned, member states of an assessment of an application performed by one member state designated by the applicant, known as the reference member state. Under this procedure, an applicant submits an application based on identical dossiers and related materials, including a draft summary of product characteristics, and draft labeling and package leaflet, to the reference member state and concerned member states. The reference member state prepares a draft assessment report and drafts of the related materials within 210 days after receipt of a valid application. Within 90 days of receiving the reference member state's assessment report and related materials, each concerned member state must decide whether to approve the assessment report and related materials.

If a member state cannot approve the assessment report and related materials on the grounds of potential serious risk to public health, the disputed points are subject to a dispute resolution mechanism and may eventually be referred to the European Commission, whose decision is binding on all member states.

Review and Approval of In Vitro Diagnostics in the European Union

In the European Economic Area, or EEA, *in vitro* diagnostic medical devices are regulated as medical devices and are required to conform with the essential requirements of the E.U. Directive on *in vitro* diagnostic medical devices (Directive No 98/79/EC, as amended). As medical devices, *in vitro* diagnostic medical devices must comply with the Essential Requirements in Annex I to the EU Medical Devices Directive (Council Directive 93/42/EEC), or the Essential Requirements. Specifically, a manufacturer must undergo a conformity assessment procedure, which varies according to the type of medical device and its classification. Except for low risk medical devices, where the manufacturer can issue a CE Declaration of Conformity based on a self-assessment of the conformity of its products with the Essential Requirements, a conformity assessment procedure requires the intervention of a third-party organization designated by competent authorities of a European Union country to conduct conformity assessments, or a Notified Body. Notified Bodies are independent testing houses, laboratories, or product certifiers typically based within the European Union and authorized by the European member states to perform the required conformity assessment tasks, such as quality system audits and device compliance testing. The Notified Body would typically audit and examine the product's Technical File and the quality system for the manufacture, design and final inspection of the product before issuing a CE Certificate of Conformity demonstrating compliance with the relevant Essential Requirements.

Pharmaceutical Coverage, Pricing and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of products approved by the FDA and other government authorities. Sales of products will depend, in part, on the extent to which the costs of the products will be covered by third-party payors, including government health programs in the United States such as Medicare and Medicaid, commercial health insurers and managed care organizations. The process for determining whether a payor will provide coverage for a product may be separate from the process for setting the

price or reimbursement rate that the payor will pay for the product once coverage is approved. Third-party payors may limit coverage to specific products on an approved list, or formulary, which might not include all of the approved products for a particular indication.

Additionally, the containment of healthcare costs has become a priority of federal and state governments, and the prices of drugs have been a focus in this effort. The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost-containment programs, including price controls, restrictions on reimbursement and requirements for substitution of generic products. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit net revenue and results.

In order to secure coverage and reimbursement for any product that might be approved for sale, a company may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of the product, in addition to the costs required to obtain FDA or other comparable regulatory approvals. A payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Third-party reimbursement may not be sufficient to maintain price levels high enough to realize an appropriate return on investment in product development.

In the European Union, pricing and reimbursement schemes vary widely from country to country. Some countries provide that drug products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost-effectiveness of a particular product candidate to currently available therapies. For example, the European Union provides options for its member states to restrict the range of drug products and devices for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. European Union member states may approve a specific price for a drug product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the drug product on the market. Other member states allow companies to fix their own prices for drug products, but monitor and control company profits. The downward pressure on health care costs in general, particularly prescription drugs, has become intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross-border imports from low-priced markets exert competitive pressure that may reduce pricing within a country. Any country that has price controls or reimbursement limitations for drug products may not allow favorable reimbursement and pricing arrangements.

Healthcare Law and Regulation

Healthcare providers, physicians and third-party payors play a primary role in the recommendation and prescription of drug products that are granted marketing approval. Arrangements with third-party payors and customers are subject to broadly applicable fraud and abuse and other healthcare laws and regulations. Such restrictions under applicable federal and state healthcare laws and regulations, include the following:

- the federal 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, in whole or in part, under a federal healthcare program such as Medicare and Medicaid;
- the federal False Claims Act imposes civil penalties, and provides for 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, or HIPAA, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, 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 payments and other transfers of value to physicians and teaching hospitals and physician ownership and investment interests; and
- analogous state and foreign 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.

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. 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 HIPAA, thus complicating compliance efforts.

Legal Proceedings

We are not currently a party to any material legal proceedings.

Facilities

Our headquarters are located in Cambridge, Massachusetts, where we occupy approximately 4,150 square feet of office space. The term of the lease expires on a month-to-month basis.

Employees

As of September 2, 2014, we had 17 full-time employees, nine of whom were primarily engaged in research and development activities.

Management

The following table sets forth the name, age and position of each of our executive officers and directors as of September 2, 2014.

Name	Age	Position(s)
Executive Officers		
Jodie P. Morrison	39	President and Chief Executive Officer, Director
John S. McBride	62	Chief Operating Officer
Karen J. Ferrante, M.D.	56	Head of Research and Development and Chief Medical Officer
Lee H. Kalowski	33	Chief Financial Officer
Non-Employee Directors		
Seth L. Harrison, M.D.(1)(2)(3)	54	Chairman of the Board of Directors
Reinhard J. Ambros, Ph.D.*	58	Director
Timothy J. Barberich(1)(2)(3)	66	Director
David A. Kessler, M.D.(2)(3)	63	Director
Campbell Murray, M.D.*	38	Director
Joseph A. Yanchik, III(1)	50	Director

(1) Member of audit committee.

(2) Member of compensation committee.

(3) Member of nominating and corporate governance committee.

* Drs. Ambros and Murray have notified us that they will resign from our board of directors contingent upon and effective immediately prior to the effectiveness of the registration statement of which this prospectus forms a part.

** Committee memberships will be effective upon the effectiveness of the registration statement of which this prospectus forms a part.

Executive Officers

Jodie P. Morrison has served as our President and Chief Executive Officer and as a member of our board of directors since March 2013. From December 2006 until March 2013, Ms. Morrison held other senior positions with us, including Chief Operating Officer, Head of Clinical Affairs and Program Operations and Vice President of Clinical Affairs and Program Operations. Prior to joining our company, Ms. Morrison served as Director of Clinical Operations and Medical Affairs at Dyax Corporation, or Dyax. Prior to joining Dyax, Ms. Morrison held clinical management positions at both Curis, Inc. and at Diacrin, Inc. Ms. Morrison received a B.A. in neuroscience from Mount Holyoke College, her clinical research certification from the Boston University School of Medicine and her business training through the Greater Boston Executive Program at the MIT Sloan School of Management. We believe Ms. Morrison is qualified to serve on our board of directors due to her service as our President and Chief Executive Officer, her years of service as our Chief Operating Officer and her extensive knowledge of our company and industry.

John S. McBride has served as our Chief Operating Officer since February 2014 and served as our Chief Financial Officer from April 2014 until September 2014. Prior to joining our company, Mr. McBride founded and served as President of Alliance Life Science Advisors, Inc., a consulting firm focused on assisting life science companies with strategic planning, business development and financing projects from March 2012 until February 2014. Prior to founding Alliance Life Science Advisors, Inc., Mr. McBride was an independent consultant from January 2009 until March 2012. In addition, Mr. McBride previously served as Executive Vice President and Chief Operating Officer of Gloucester Pharmaceuticals, Inc., Global Head of Oncology Licensing at Pharmacia Corporation, Executive Vice President, Business Operations and Chief Financial Officer at CytoTherapeutics, Inc., Vice President, Business Development and Treasurer at Phytera, Inc., Vice President, Commercial Development at Sparta Pharmaceuticals, Inc. and Vice President, Business Development at U.S.

Bioscience, Inc. Currently, Mr. McBride serves as a member of the board of directors of Intezyne, Inc. From August 2008 until June 2013, Mr. McBride served as a member of the board of directors of Niiki Pharma Inc. Mr. McBride received a B.S. in biochemistry and an M.S. in chemical engineering from the University of Wisconsin and an M.B.A. from the Wharton School, University of Pennsylvania.

Karen J. Ferrante, M.D. has served as our Head of Research and Development and Chief Medical Officer since April 2014. Prior to joining our company, Dr. Ferrante served as oncology therapeutic area head and Takeda Cambridge, USA site head for Takeda Pharmaceuticals from May 2013 until July 2013 and held senior positions at Millennium Pharmaceuticals, which was acquired by Takeda Pharmaceuticals in May 2008, including Chief Medical Officer, a Head of Research and Development and Senior Vice President, Clinical Development from September 2007 until May 2013. In addition, Dr. Ferrante previously held positions of increasing responsibility at Pfizer Global Research & Development, including Vice President and Therapeutic Area Clinical Leader in Oncology Development, and served as Associate Director of Clinical Oncology at Bristol-Myers Squibb Company, or BMS. Prior to joining BMS, Dr. Ferrante served as a staff physician at the Beth Israel Deaconess Hospital. She also served as instructor, clinical instructor and clinical fellow in medicine at the Harvard Medical School while completing her internship and residency in internal medicine followed by her fellowship in hematology and oncology at Beth Israel Deaconess Hospital. Currently, Dr. Ferrante serves as a member of the board of directors of Progenics Pharmaceuticals, Inc. Dr. Ferrante received a B.S. in chemistry and biology from Providence College and an M.D. from Georgetown University.

Lee H. Kalowski has served as our Chief Financial Officer since September 2014. Prior to joining our company, Mr. Kalowski served in global biotechnology equity research at Credit Suisse where he covered companies in the biopharmaceutical industry, including companies developing prostate cancer therapies, as a vice president from January 2012 until September 2014, as a senior analyst from May 2011 until September 2014 and as an associate from June 2010 until May 2011. Prior to joining Credit Suisse, Mr. Kalowski worked in mergers and acquisitions for the pharmaceutical division of Johnson & Johnson from May 2009 until August 2009 while attending the Wharton School, University of Pennsylvania, from July 2008 until May 2010. Prior to that, Mr. Kalowski held global pharmaceutical equity research positions at Sanford C. Bernstein & Co. LLC and Prudential Equity Group, LLC. Mr. Kalowski received a B.A. in biology and economics from Union College and an M.B.A. in finance and health care management from the Wharton School, University of Pennsylvania.

Non-Employee Directors

Seth L. Harrison, M.D. is one of our founders and has served as a member of our board of directors since April 2005 and as Chairman of our board of directors since August 2005. In September 1999, Dr. Harrison founded Apple Tree Partners, or Apple Tree, a life sciences investment firm, and since that time has served as Apple Tree's Managing Partner. In addition, Dr. Harrison previously served as our Chief Executive Officer from August 2008 until September 2011. Currently, Dr. Harrison serves as a member of the boards of directors of Heartware International, Inc., or Heartware, and Aileron Therapeutics, Inc., or Aileron, and as Chairman of the board of directors of Braeburn Pharmaceuticals. From 2002 until 2010, Dr. Harrison served as a member of the board of directors of the International Partnership for Microbicides, a Rockefeller Foundation/Gates Foundation sponsored public-private partnership engaged in the development of anti-HIV microbicides. Dr. Harrison received an A.B. from Princeton University, an M.D. and M.B.A., both from Columbia University, and completed a surgery internship at the Presbyterian Hospital in the City of New York. We believe Dr. Harrison is qualified to serve on our board of directors due to his strong medical and venture capital background, his extensive experience with development-stage companies such as ours and his service on the boards of directors of a range of public and private companies.

Reinhard J. Ambros, Ph.D. has served as a member of our board of directors since May 2009. Dr. Ambros has served as Global Head of Novartis Venture Funds since August 2005. Previously, he served as Head of Group Strategic Planning for Novartis Corporation from 2001 until 2005. Prior to that, he served as global head of business development and licensing for cardiovascular and metabolic diseases at Novartis Pharma

AG. Currently, Dr. Ambros serves as a member of the boards of directors of Aileron, FORMA Therapeutics, Inc., Genedata AG and Symetis SA. Dr. Ambros received an M.S. from the University of Regensburg, Germany, and a Ph.D. in medicinal chemistry and pharmacology from the University of Regensburg, Germany. We believe Dr. Ambros is qualified to serve on our board of directors due to his management experience in the biotechnology sector and his service on other boards of directors.

Timothy J. Barberich has served as a member of our board of directors since February 2010. Mr. Barberich founded Sepracor, Inc., or Sepracor, in 1984 and served as Chief Executive Officer and Chairman of the board of directors of Sepracor until November 2009 when Sepracor was acquired by Dainippon Sumitomo. Prior to founding Sepracor, Mr. Barberich served as a senior executive at Millipore Corporation. Mr. Barberich currently serves on the boards of directors of Heartware, GI Dynamics, Inc., Verastem Pharmaceuticals, Inc., Neurovance and BioNevia, Inc. He previously served on the Board of Trustees of the Boston Medical Center and the board of the Pharmaceutical Research and Manufacturers' Association. Mr. Barberich received a B.S. in chemistry from Kings College. We believe Mr. Barberich is qualified to serve on our board of directors due to his significant experience in the development and commercialization of pharmaceutical products, his leadership experience at other pharmaceutical companies and his service on other boards of directors.

David A. Kessler, M.D. has served as a member of our board of directors since March 2009. Dr. Kessler has served as Professor of Pediatrics and Epidemiology and Biostatistics at the University of California, San Francisco, or UCSF, School of Medicine since 2003. Dr. Kessler served as the Dean of the School of Medicine and the Vice Chancellor for Medical Affairs at UCSF from 2003 until 2007 and Dean of the Yale University School of Medicine from 1997 until 2003. Dr. Kessler served as Commissioner of the FDA from November 1990 until March 1997. He also currently serves as a senior advisor to TPG Capital. Dr. Kessler was elected a member of the Institute of Medicine in 1993. Currently, Dr. Kessler serves on the board of directors of Immucor, Inc. He previously served on the board of directors of Aptalis. Dr. Kessler received a B.A. from Amherst College, a J.D. from The University of Chicago Law School and an M.D. from Harvard Medical School. In addition, Dr. Kessler received an Advanced Professional Certificate from the New York University Graduate School of Business Administration. We believe Dr. Kessler is qualified to serve on our board of directors due to his extensive healthcare and regulatory experience.

Campbell Murray, M.D. has served as a member of our board of directors since May 2009. Dr. Murray has served as a Managing Director of Novartis Venture Funds since August 2005. Previously, Dr. Murray served as the Director of Special Projects at the Novartis Institutes for BioMedical Research from July 2004 until July 2005. Currently, Dr. Murray serves as a member of the boards of directors of Aerpio Therapeutics, Alios BioPharm, Euthymics Bioscience, Inc., Galera Therapeutics, ImaginAb and Neurovance. He previously served on the boards of directors of Akebia Therapeutics, Aileron and ProCertus BioPharm. Dr. Murray received a bachelor of human biology from the University of Auckland Medical School, an M.B.A. from Harvard Business School, an M.P.P. from the John F. Kennedy School of Government, and an MBChB (M.D.) from the University of Auckland Medical School. We believe that Dr. Murray is qualified to serve on our board of directors due to his extensive investment experience in the biotechnology sector.

Joseph A. Yanchik, III is one of our founders and has served as a member of our board of directors since August 2005. Mr. Yanchik served as our Chief Executive Officer from August 2005 until August 2008. Mr. Yanchik has served as the President and Chief Executive Officer of Aileron since July 2005. Mr. Yanchik previously served as Venture Partner at Apple Tree from June 2005 until September 2009, Vice President of Corporate Development at Mendel Biotechnology and founder and Chief Business Officer of Poetic Genetics, Inc. Prior to that, Mr. Yanchik specialized in corporate and securities law at Cahill Gordon & Reindel and Venture Law Group. Mr. Yanchik received a B.B.A. from Loyola College and a J.D. from the Villanova University School of Law. We believe Mr. Yanchik is qualified to serve on our board of directors due to his extensive business, legal and investment experience and experience as an executive.

Family Relationships

There are no family relationships among any of our directors or executive officers.

Board Composition

Our board of directors currently consists of seven members, all of whom were elected as directors pursuant to a voting agreement that we have entered into with the holders of our redeemable convertible preferred stock and certain of our founders. The voting agreement will terminate upon the closing of this offering, and there will be no further contractual obligations regarding the election of our directors. Our directors hold office until their successors have been elected and qualified or until the earlier of their resignation or removal.

Our restated certificate of incorporation and our amended and restated bylaws that will become effective upon the closing of this offering provide that the authorized number of directors may be changed only by resolution of the board of directors. Our restated certificate of incorporation and our amended and restated bylaws that will become effective upon the closing of this offering also provide that our directors may be removed only for cause by the affirmative vote of the holders of at least 75% of the votes that all our stockholders would be entitled to cast in an annual election of directors, and that any vacancy on our board of directors, including a vacancy resulting from an enlargement of our board of directors, may be filled only by vote of a majority of our directors then in office.

In accordance with the terms of our certificate of incorporation and bylaws that will become effective upon the closing of this offering, our board of directors will be divided into three classes, class I, class II and class III, with members of each class serving staggered three-year terms. Upon the closing of this offering, the members of the classes will be divided as follows:

- the class I directors will be Jodie P. Morrison and Joseph A. Yanchik, III, and their term will expire at the annual meeting of stockholders to be held in 2015;
- the class II directors will be Timothy J. Barberich and David A. Kessler, and their term will expire at the annual meeting of stockholders to be held in 2016; and
- the class III directors will be Seth L. Harrison, and his term will expire at the annual meeting of stockholders to be held in 2017.

Upon the expiration of the term of a class of directors, directors in that class will be eligible to be elected for a new three-year term at the annual meeting of stockholders in the year in which their term expires.

We have no formal policy regarding board diversity. Our priority in selection of a board member is identification of a member who will further the interests of our stockholders through his or her established record of professional accomplishment, the ability to contribute positively to the collaborative culture among board members, knowledge of our business and understanding of the competitive landscape.

Director Independence

Rule 5605 of the NASDAQ Listing Rules requires a majority of a listed company's board of directors to be comprised of independent directors within one year of listing. In addition, the NASDAQ Listing Rules require that, subject to specified exceptions, each member of a listed company's audit, compensation and nominating and corporate governance committees be independent under the Securities Exchange Act of 1934, as amended, or the Exchange Act. Audit committee members must also satisfy the independence criteria set forth in Rule 10A-3 under the Exchange Act, and compensation committee members must also satisfy the independence criteria set forth in Rule 10C-1 under the Exchange Act. Under Rule 5605(a)(2) of the NASDAQ Listing Rules, a director will only qualify as an "independent director" if, in the opinion of our board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director. In order to be considered independent for purposes of Rule 10A-3, a member of an audit committee of a listed company may not, other than in his or her capacity as a member of the audit committee, the board of directors, or any other board committee, accept, directly or indirectly, any consulting, advisory, or other compensatory fee from the listed company or any of its subsidiaries or otherwise be an affiliated person of the listed company or any of its subsidiaries. In order to be considered independent for purposes of Rule 10C-1, the board must consider, for

each member of a compensation committee of a listed company, all factors specifically relevant to determining whether a director has a relationship to such company which is material to that director's ability to be independent from management in connection with the duties of a compensation committee member, including, but not limited to: (1) the source of compensation of the director, including any consulting advisory or other compensatory fee paid by such company to the director; and (2) whether the director is affiliated with the company or any of its subsidiaries or affiliates.

In August 2014, our board of directors undertook a review of the composition of our board of directors and its committees and the independence of each director. Based upon information requested from and provided by each director concerning his or her background, employment and affiliations, including family relationships, our board of directors has determined that each of our directors, with the exception of Jodie P. Morrison and Seth L. Harrison, is an "independent director" as defined under Rule 5605(a)(2) of the NASDAQ Listing Rules. Ms. Morrison is not an independent director under Rule 5605(a)(2) because she is our President and Chief Executive Officer. Dr. Harrison is not an independent director under Rule 5605(a)(2) because he served as our Chief Executive Officer from August 2008 until September 2011. We expect Dr. Harrison will become an independent director as of September 23, 2014. Our board of directors also determined that Messrs. Yanchik and Barberich, who will be members of our audit committee upon the effectiveness of the registration statement of which this prospectus forms a part, and Messrs. Kessler and Barberich, and Dr. Harrison as of September 23, 2014, who will comprise our compensation committee upon the effectiveness of the registration statement of which this prospectus forms a part, satisfy the independence standards for such committees established by the SEC and the NASDAQ Listing Rules, as applicable. Dr. Harrison is not an independent director for the purpose of membership on our audit committee under Rule 10A-3 because of his affiliation with entities affiliated with Apple Tree Partners II, L.P., which beneficially own approximately 49% of our outstanding common stock prior to this offering.

Under applicable NASDAQ rules, we are permitted to phase-in our compliance with the independence requirements for our audit, compensation and nominating and corporate governance committees. The phase-in periods with respect to director independence allow us to have only one independent member on each of the audit committee, compensation committee and nominating and corporate governance committee upon the listing date of our common stock, a majority of independent members on each of these committees and our audit committee within 90 days of the listing date and fully independent committees within one year of the listing date. We expect that by the first anniversary of our listing on The NASDAQ Global Market, each of these committees will comply with the applicable independence requirements.

Board Committees

Our board of directors has established an audit committee and a compensation committee and, effective upon the effectiveness of the registration statement of which this prospectus forms a part, will establish a nominating and corporate governance committee. Each of these committees will operate under a charter that will be approved by our board of directors.

Audit Committee

Effective upon the effectiveness of the registration statement of which this prospectus forms a part, the members of our audit committee will be Joseph A. Yanchik, III, Timothy J. Barberich and Seth L. Harrison. Mr. Yanchik will be the chair of the audit committee. None of the members of our audit committee qualifies as an audit committee financial expert within the meaning of SEC regulations and the NASDAQ Listing Rules. Following this offering, we plan to seek to identify a director to serve on the audit committee who would qualify as an audit committee financial expert. Our audit committee assists our board of directors in its oversight of our accounting and financial reporting process and the audits of our financial statements. Our audit committee's responsibilities will include:

- appointing, approving the compensation of, and assessing the independence of our registered public accounting firm;

- overseeing the work of our independent registered public accounting firm, including through the receipt and consideration of reports from such firm;
- reviewing and discussing with management and our independent registered public accounting firm our annual and quarterly financial statements and related disclosures;
- monitoring our internal control over financial reporting, disclosure controls and procedures and code of business conduct and ethics;
- overseeing our internal audit function, if any;
- discussing our risk management policies;
- establishing policies regarding hiring employees from our independent registered public accounting firm and procedures for the receipt and retention of accounting related complaints and concerns;
- meeting independently with our internal auditing staff, our independent registered public accounting firm and management;
- reviewing and approving or ratifying any related person transactions; and
- preparing the audit committee report required by SEC rules. All audit services to be provided to us and all non-audit services, other than de minimis non-audit services, to be provided to us by our registered public accounting firm must be approved in advance by our audit committee.

Compensation Committee

Effective upon the effectiveness of the registration statement of which this prospectus forms a part, the members of our compensation committee will be Timothy J. Barberich, Seth L. Harrison and David A. Kessler. Mr. Barberich will be the chair of the compensation committee. Our compensation committee assists our board of directors in the discharge of its responsibilities relating to the compensation of our executive officers. Our compensation committee's responsibilities will include:

- reviewing and approving, or making recommendations to our board with respect to, the compensation of our Chief Executive Officer and other executive officers;
- overseeing the evaluation of our senior executives;
- reviewing and making recommendations to our board of directors with respect to our incentive-compensation and equity-based compensation plans;
- overseeing and administering our equity-based plans;
- reviewing and making recommendations to our board with respect to director compensation;
- reviewing and discussing with management our "Compensation Discussion and Analysis" disclosure to the extent such disclosure is required by SEC rules; and
- preparing the compensation committee report required by SEC rules.

Nominating and Corporate Governance Committee

Effective upon the effectiveness of the registration statement of which this prospectus forms a part, the members of our nominating and corporate governance committee will be Seth L. Harrison, Timothy J. Barberich and David A. Kessler. Dr. Harrison will be the chair of the nominating and corporate governance committee. Upon the closing of this offering, the nominating and corporate governance committee's responsibilities will include:

- identifying individuals qualified to become members of our board;
- recommending to our board the persons to be nominated for election as directors and to each of our board's committees;

- developing and recommending to our board corporate governance principles; and
- overseeing an annual evaluation of our board.

Compensation Committee Interlocks and Insider Participation

None of our executive officers serves, or in the past has served, as a member of the board of directors or compensation committee, or other committee serving an equivalent function, of any entity that has one or more executive officers who serve as members of our board of directors or our compensation committee. None of the members of our compensation committee is an officer or employee of our company, and other than Dr. Harrison, who served as our Chief Executive Officer from August 2008 until September 2011, none of the members of our compensation committee has ever been an officer or employee of our company.

Code of Business Conduct and Ethics

We have adopted a written code of business conduct and ethics that will be effective upon the closing of this offering and apply to our directors, officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. Following this offering, a copy of the code will be posted on the Corporate Governance section of our website, which is located at www.tokaipharma.com. If we make any substantive amendments to, or grant any waivers from, the code of business conduct and ethics for any officer or director, we will disclose the nature of such amendment or waiver on our website or in a current report on Form 8-K.

Director Compensation

Prior to this offering, we did not have a formal non-employee director compensation policy. In 2013, we paid $2,000 and $6,000 in cash to Dr. Kessler and Mr. Barberich, respectively, as compensation for board of directors meetings attended in person. In addition, on June 26, 2013, we granted options to purchase 10,374 shares and 7,795 shares of common stock to Dr. Kessler and Mr. Barberich, respectively, with an exercise price of $1.58 per share. With respect to 5,845 of the shares of common stock underlying the option granted to Dr. Kessler, those shares vested as to 2.083% of the shares on July 1, 2013 and as to an additional 2.083% of the shares at the beginning of each successive month thereafter until June 1, 2017, and with respect to 4,529 of the shares of common stock underlying the option granted to Dr. Kessler, those shares vested as to 8.333% of the shares on October 24, 2013 and as to an additional 2.083% of the shares at the beginning of each successive month thereafter until June 1, 2017. With respect to 4,392 of the shares of common stock underlying the option granted to Mr. Barberich, those shares vested as to 2.083% of the shares on July 1, 2013 and as to an additional 2.083% of the shares at the beginning of each successive month thereafter until June 1, 2017, and with respect to 3,403 of the shares of common stock underlying the option granted to Mr. Barberich, those shares vested as to 8.333% of the shares on October 24, 2013 and as to an additional 2.083% of the shares at the beginning of each successive month thereafter until June 1, 2017.

None of our other non-employee directors received any compensation in 2013. We do not pay any compensation to our President and Chief Executive Officer in connection with her service on our board of directors. The compensation that we pay to our President and Chief Executive Officer is discussed in the "Executive Compensation" section of this prospectus.

Our board of directors has approved a compensation policy for our non-employee directors that will become effective upon the closing of this offering. This policy is designed to provide a total compensation package that enables us to attract and retain qualified and experienced individuals to serve as directors and to align our directors' interests with those of our stockholders. Following this offering, our non-employee directors will be compensated for their services on our board of directors as follows:

- each new non-employee director will receive an initial grant of an option under our 2014 Stock Incentive Plan, or the 2014 Plan, to purchase 25,000 shares of common stock upon his or her initial election to our board of directors;

- each non-employee director who has served on the board for at least three months will receive an annual grant of an option under our 2014 Plan to purchase 12,000 shares of common stock on the date of the first meeting of our board of directors held after each annual meeting of our stockholders;
- each non-employee director will receive an annual cash fee of $35,000 ($60,000 for the chairman of the board of directors);
- each non-employee director who is a member of the audit committee will receive an additional annual cash fee of $7,500 ($15,000 for the audit committee chairman);
- each non-employee director who is a member of the compensation committee will receive an additional annual cash fee of $5,000 ($10,000 for the compensation committee chairman); and
- each non-employee director who is a member of the nominating and corporate governance committee will receive an additional annual cash fee of $3,750 ($7,500 for the nominating and corporate governance committee chairman).

The stock options granted to our non-employee directors will have an exercise price equal to the fair market value of our common stock on the date of grant and will expire ten years after the date of grant. The initial stock options granted to our future newly elected non-employee directors will, subject to the director's continued service on our board, vest with respect to one-third of the shares on the first anniversary of the grant date and quarterly thereafter until the third anniversary of the date of grant. The annual stock options granted to our non-employee directors will, subject to the director's continued service on our board, vest with respect to 100% of the shares on the first anniversary of the grant date. The initial and annual stock options granted to our non-employee directors will vest in full with respect to the shares then underlying such options upon a change of control.

The annual cash fee will be payable in arrears on the last day of each quarter. The amount of each payment will be prorated for any portion of a quarter that a director is not serving on our board. In addition, no fee will be payable in respect of any period prior to the effective date of the registration statement of which this prospectus is a part, and the first payment after the effective date will be prorated therefor.

Each non-employee director will also be entitled to reimbursement for reasonable travel and other expenses incurred in connection with attending meetings of the board of directors and any committee on which he or she serves.

Executive Compensation

This section discusses the material elements of our executive compensation policies for our "named executive officers" and the most important factors relevant to an analysis of these policies. It provides qualitative information regarding the manner and context in which compensation is awarded to and earned by our executive officers named in the "Summary Compensation Table" below, or our "named executive officers," and is intended to place in perspective the data presented in the following tables and the corresponding narrative.

In preparing to become a public company, we have begun a thorough review of all elements of our executive compensation program, including the function and design of our equity incentive programs. We have begun, and we expect to continue in the coming months, to evaluate the need for revisions to our executive compensation program to ensure our program is competitive with the companies with which we compete for executive talent and is appropriate for a public company.

Summary Compensation Table

The following table sets forth information regarding compensation earned by our President and Chief Executive Officer and our next two highest paid executive officers during the year ended December 31, 2013. We refer to these individuals as our named executive officers.

Name	Year	Salary ($)	Bonus ($)	Option Awards ($)(1)	All Other Compensation ($)	Total ($)
Jodie P. Morrison(2) *President and Chief Executive Officer*	2013	330,103	91,875	547,134	565(3)	969,677
Martin D. Williams *Former President and Chief Executive Officer*	2013	87,500	—	—	268,215(4)	355,715
Adrian Senderowicz, M.D. *Former Chief Medical Officer*	2013	68,750	—	—	118,227(5)	186,977

(1) Amounts listed represent the aggregate fair value amount computed as of the grant date of the option awards granted during 2013 in accordance with FASB ASC Topic 718. Assumptions used in the calculation of these amounts are included in Note 9, Stock-Based Awards, to our consolidated financial statements included elsewhere in this prospectus.
(2) Ms. Morrison also serves as a member of our board of directors but does not receive any additional compensation for her service as a director.
(3) Represents the dollar value of a group life insurance premium paid during the fiscal year with respect to life insurance for Ms. Morrison.
(4) Consists of (i) $262,500 paid as severance to Mr. Williams following his departure from our company effective March 27, 2013, (ii) $5,385 in accrued vacation and (iii) $330, which represents the dollar value of a group life insurance premium paid during the fiscal year with respect to life insurance for Mr. Williams.
(5) Consists of (i) $114,594 paid as severance to Dr. Senderowicz following his departure from our company effective March 27, 2013, (ii) $3,173 in accrued vacation and (iii) $460, which represents the dollar value of a group life insurance premium paid during the fiscal year with respect to life insurance for Dr. Senderowicz.

Narrative Disclosure to Summary Compensation Table

Base salary. In 2013, we paid $330,103 in base salary to Ms. Morrison and, before their departure from our company effective March 27, 2013, $87,500 in base salary to Mr. Williams and $68,750 in base salary to Dr. Senderowicz. We use base salaries to recognize the experience, skills, knowledge and responsibilities required of all our employees, including our named executive officers. None of our named executive officers is currently party to an employment agreement or other agreement or arrangement that provides for automatic or scheduled increases in base salary. In connection with the departures from our company of Mr. Williams and

Dr. Senderowicz effective March 27, 2013, we agreed to make severance payments to Mr. Williams and Dr. Senderowicz pursuant to a separation agreement entered into with each of them. See "—Employment Agreements, Severance and Change in Control Agreements" for additional information.

Annual bonus. Our board of directors may, in its discretion, award bonuses to our named executive officers from time to time. We typically establish annual bonus targets based around a set of specified corporate goals for our named executive officers and conduct an annual performance review to determine the attainment of such goals. Our management may propose bonus awards to the compensation committee of the board or the board primarily based on such review process. Our board of directors makes the final determination of the eligibility requirements for and the amount of such bonus awards. With respect to 2013, we awarded a bonus of $91,875 to Ms. Morrison based on her individual performance and our performance as a company that year. We did not award a bonus to any other named executive officer in 2013.

Equity incentives. Although we do not have a formal policy with respect to the grant of equity incentive awards to our executive officers or any formal equity ownership guidelines applicable to them, we believe that equity grants provide our executives with a strong link to our long-term performance, create an ownership culture and help to align the interests of our executives and our stockholders. In addition, we believe that equity grants with a time-based vesting feature promote executive retention because this feature incentivizes our executive officers to remain in our employment during the vesting period. Accordingly our compensation committee and board of directors periodically review the equity incentive compensation of our named executive officers and from time to time may grant equity incentive awards to them in the form of stock options. In 2013, we granted options to purchase 506,551 shares of our common stock to Ms. Morrison in connection with her elevation to President and Chief Executive Officer, of which options to purchase 341,300 shares are subject to time-based vesting and options to purchase 165,251 shares are subject to performance-based vesting. See "—Outstanding Equity Awards at Year End." We did not grant equity awards to any of our other named executive officers in 2013.

Outstanding Equity Awards at Year End

The following table sets forth information regarding outstanding stock options held by our named executive officers as of December 31, 2013.

	Option Awards			
Name	Number of Securities Underlying Unexercised Options (#) exercisable	Number of Securities Underlying Unexercised Options (#) unexercisable	Option Exercise Price ($)	Option Expiration Date
Jodie P. Morrison	2,574	—	2.31	5/27/2018
President and Chief Executive Officer	22,441	—	0.63	5/6/2019
	38,049	22,829(1)	1.37	6/28/2021
	2,887	2,244(2)	1.37	9/7/2021
	3,608	2,806(3)	1.37	9/7/2021
	11,833	9,204(4)	1.37	9/7/2021
	14,864	11,561(5)	1.37	9/7/2021
	32,353	226,472(6)	1.58	6/26/2023
	10,310	72,165(7)	1.58	6/26/2023
	—	165,251(8)	1.58	6/26/2023
Martin D. Williams	—	—	—	—
Former President and Chief Executive Officer				
Adrian Senderowicz, M.D.	—	—	—	—
Former Chief Medical Officer				

(1) This option vested as to 2.083% of the shares underlying the option on July 1, 2011 and vests as to an additional 2.083% of the shares underlying the option monthly thereafter through June 1, 2015.

(2) This option vested as to 2.083% of the shares underlying the option on October 1, 2011 and vests as to an additional 2.083% of the shares underlying the option monthly thereafter through September 1, 2015.
(3) This option vested as to 2.083% of the shares underlying the option on October 7, 2011 and vests as to an additional 2.083% of the shares underlying the option monthly thereafter through September 1, 2015.
(4) This option vested as to 8.333% of the shares underlying the option on January 27, 2012 and vests as to an additional 2.083% of the shares underlying the option monthly thereafter through September 1, 2015.
(5) This option vested as to 20.833% of the shares underlying the option on July 12, 2012 and vests as to an additional 2.083% of the shares underlying the option monthly thereafter through September 1, 2015.
(6) This option vested as to 2.083% of the shares underlying the option on July 1, 2013 and vests as to an additional 2.083% of the shares underlying the option monthly thereafter through June 1, 2017.
(7) This option vested as to 8.333% of the shares underlying the option on October 24, 2013 and vests as to an additional 2.083% of the shares underlying the option monthly thereafter through June 1, 2017. This option is also subject to acceleration by 12 months upon the consummation of this offering.
(8) This option is a performance-based option that vests as to 100% of the shares underlying the option upon the consummation of this offering.

Employment Agreements, Severance and Change in Control Agreements

Jodie P. Morrison

In June 2013, in connection with our appointment of Ms. Morrison as our President and Chief Executive Officer, we entered into an employment agreement with Ms. Morrison. The employment agreement establishes Ms. Morrison's title, her base salary, her eligibility for an annual bonus of up to 25% of her base salary, and her eligibility for benefits made available to employees generally and also provides for certain benefits upon termination of her employment under specified conditions. Ms. Morrison's employment is at will. We granted Ms. Morrison two stock options pursuant to the employment agreement: an option for the purchase of 341,300 shares that is subject to time-based vesting and an option for the purchase of 165,251 shares that is subject to performance-based vesting.

Under the terms of the employment agreement, if Ms. Morrison's employment is terminated by us without cause or by Ms. Morrison for good reason, each as defined in her employment agreement, and subject to Ms. Morrison's execution of a general release of potential claims against us, we have agreed to continue to pay her then-current base salary for a period of 12 months. In addition, if Ms. Morrison's employment is terminated by us without cause or by Ms. Morrison for good reason within one year following a change of control, as defined in her stock option agreement, and subject to Ms. Morrison's execution of a general release of potential claims against us, the time-vested option granted to Ms. Morrison upon her appointment as Chief Executive Officer will accelerate in full.

The time-based option is also subject to acceleration by 12 months upon the consummation of this offering. The performance-based option granted to Ms. Morrison upon her appointment as Chief Executive Officer will vest as to 100% of the number of shares of common stock underlying the option upon the consummation of this offering.

In addition, under each stock option agreement that we have entered into with Ms. Morrison, other than the stock option agreement for the performance-based option described above, we have agreed that if Ms. Morrison is terminated without cause or resigns for good reason in connection with or within one year after a change in control of our company (as defined in the applicable stock option agreement), then that stock option will vest in full.

Martin D. Williams

In connection with Mr. Williams' departure from our company effective March 27, 2013, we entered into a separation agreement with Mr. Williams under which we agreed to make severance payments to Mr. Williams in the amount of his then-current base salary for 12 months following his departure.

Adrian Senderowicz, M.D.

In connection with Dr. Senderowicz's departure from our company effective March 27, 2013, we entered into a separation agreement with Dr. Senderowicz under which we agreed to make severance payments to Dr. Senderowicz in the amount of his then-current base salary for six months following his departure.

In 2014, we entered into employment agreements with Mr. McBride, Dr. Ferrante and Mr. Kalowski in connection with their commencing employment with us.

John S. McBride

Mr. McBride's employment agreement establishes his title, his base salary, his eligibility for an annual bonus of up to 20% of his base salary, and his eligibility for benefits made available to employees generally and also provides for certain benefits upon termination of his employment under specified conditions. Mr. McBride's employment is at will. Pursuant to Mr. McBride's employment agreement, we granted Mr. McBride a stock option for the purchase of 209,178 shares that is subject to time-based vesting.

Under the terms of the employment agreement, if Mr. McBride's employment is terminated by us without cause, as defined in his employment agreement, and subject to Mr. McBride's execution of a general release of potential claims against us, we have agreed to continue to pay his then-current base salary for a period of six months.

In addition, under the terms of Mr. McBride's stock option agreement, if Mr. McBride's employment is terminated by us without cause or by Mr. McBride for good reason, each as defined in his stock option agreement, within one year following a change of control event, the option will become exercisable in full with respect to the shares then underlying the option.

Karen J. Ferrante, M.D.

The employment agreement establishes Dr. Ferrante's title, her base salary, her eligibility for an annual bonus of up to 20% of her base salary, and her eligibility for benefits made available to employees generally and also provides for certain benefits upon termination of her employment under specified conditions. Dr. Ferrante's employment is at will. Pursuant to Dr. Ferrante's employment agreement, we granted Dr. Ferrante a stock option for the purchase of 258,036 shares that is subject to time-based vesting.

Under the terms of the employment agreement, if Dr. Ferrante's employment is terminated by us without cause, as defined in her employment agreement, and subject to Dr. Ferrante's execution of a general release of potential claims against us, we have agreed to continue to pay her then-current base salary:

- for a period of six months if Dr. Ferrante's termination occurs within six months of Dr. Ferrante's commencement of employment with us;
- for a period equal to the number of full months worked if Dr. Ferrante's termination occurs more than six months but less than 12 months after Dr. Ferrante's commencement of employment with us; and
- for a period of 12 months if Dr. Ferrante's termination occurs on or after the one year anniversary of Dr. Ferrante's commencement of employment with us.

In addition, under the terms of Dr. Ferrante's stock option agreement, if Dr. Ferrante's employment is terminated by us without cause or by Dr. Ferrante for good reason, each as defined in her stock option agreement, within one year following a change of control event, the option will become exercisable in full with respect to the shares then underlying the option.

Lee H. Kalowski

Mr. Kalowski's employment agreement establishes his title, his base salary, his eligibility for an annual bonus of up to 30% of his base salary and his eligibility for benefits made available to employees generally and also provides for certain benefits upon termination of his employment under specified conditions. Mr. Kalowski's employment agreement also provided for a $45,000 cash signing bonus. Mr. Kalowski's employment is at will. Pursuant to Mr. Kalowski's employment agreement, Mr. Kalowski was granted (1) an option to purchase 218,417 shares of our common stock at an exercise price equal to the price per share of the shares sold to the public in this offering and (2) a restricted stock unit award for 54,604 shares of our common stock. Each of these awards will be effective upon the day following the effectiveness of the registration statement of which this prospectus forms a part (and prior to the commencement of trading of our common stock on The NASDAQ Global Market). These awards will be subject to time-based vesting.

Under the terms of the employment agreement, if Mr. Kalowski's employment is terminated by us without cause, as defined in his employment agreement, and subject to Mr. Kalowski's execution of a general release of potential claims against us, we have agreed to continue to pay his then-current base salary for a period of six months or until Mr. Kalowski commences employment with or begins providing services to another person, employer or entity.

In addition, under the terms of the employment agreement, if Mr. Kalowski's employment is terminated by us without cause or by Mr. Kalowski for good reason, each as defined in his employment agreement, within one year following a change of control event, the option will become exercisable in full with respect to the shares then underlying the option, and the restricted stock unit will vest in full with respect to the shares then underlying the restricted stock unit.

Other Agreements

We have also entered into employee confidentiality, inventions, non-solicitation and non-competition agreements with each of our named executive officers. Under the employee confidentiality, inventions, non-solicitation and non-competition agreements, each named executive officer has agreed (1) not to compete with us during his or her employment and for a period of one year after the termination of his or her employment, (2) not to solicit our employees during his or her employment and for a period of one year after the termination of his or her employment, (3) to protect our confidential and proprietary information and (4) to assign to us related intellectual property developed during the course of his or her employment.

Stock Option and Other Compensation Plans

2007 Stock Incentive Plan

Our 2007 Stock Incentive Plan, as amended, or the 2007 Plan, was first adopted by our board of directors and first approved by our stockholders in May 2007. Our 2007 Plan was amended in June 2008, March 2009, May 2009, September 2011, May 2013, February 2014 and April 2014. Our employees, officers, directors, consultants and advisors are eligible to receive awards under the 2007 Plan; however, incentive stock options may only be granted to employees. In accordance with the terms of the 2007 Plan, our board of directors, or a committee or executive officer appointed by our board, administers the 2007 Plan and, subject to any limitations in the 2007 Plan, selects the recipients of awards and determines:

- the number of shares of common stock covered by options and the dates upon which those options become exercisable;
- the type of options to be granted;
- the exercise prices of options;
- the duration of options; and

- the number of shares of common stock subject to any restricted stock or other stock-based awards and the terms and conditions of those awards, including the issue price, conditions for repurchase or forfeiture and repurchase price.

If our board of directors delegates authority to an executive officer to grant awards under the 2007 Plan, the executive officer has the power to make awards to employees, directors, consultants and advisors, except officers or executive officers. Our board of directors will fix the terms of the awards to be granted by such executive officer, including the exercise price of such awards, and the maximum number of shares subject to awards that such executive officer may make.

In the event of a reorganization event, as defined in the 2007 Plan, our board shall take any one or more of the following actions as to all or any outstanding awards on such terms as the board determines:

- provide that all outstanding awards shall be assumed, or substantially equivalent awards shall be substituted, by the acquiring or succeeding corporation (or an affiliate thereof);
- upon written notice to a participant, provide that all of the participant's unexercised awards shall become exercisable in full and will terminate immediately prior to the consummation of such reorganization event, unless exercised by the participant within a specified period following the date of such notice;
- provide that all outstanding awards shall become realizable or deliverable, or restrictions applicable to an award shall lapse, in whole or in part, prior to or upon such reorganization event;
- in the event of a reorganization event pursuant to which holders of shares of our common stock will receive a cash payment for each share surrendered in the reorganization event, which we refer to as the acquisition price, make or provide for a cash payment to the participants with respect to each award held by a participant equal to (a) the acquisition price times the number of shares of our common stock subject to the participant's awards (to the extent the exercise price of such awards does not exceed the acquisition price) minus (b) the aggregate exercise price of all such outstanding awards, in exchange for the termination of such options or other awards;
- in connection with a liquidation or dissolution, provide that awards shall convert into the right to receive liquidation proceeds (if applicable, net of the exercise price thereof); and
- provide for any combination of the foregoing.

As of July 31, 2014, there were options to purchase an aggregate of 1,634,275 shares of common stock outstanding under the 2007 Plan at a weighted average exercise price of $2.86 per share, and an aggregate of 171,994 shares of common stock had been issued upon the exercise of options granted under the 2007 Plan. As of July 31, 2014, there were 43,945 shares of common stock reserved for future issuance under the 2007 Plan. On and after the effective date of the 2014 Plan described below, we will grant no further stock options or other awards under the 2007 Plan.

2014 Stock Incentive Plan

In August 2014, our board of directors adopted and our stockholders approved the 2014 Plan, which will become effective upon effectiveness of the registration statement of which this prospectus forms a part. The 2014 Plan provides for the grant of incentive stock options, nonstatutory stock options, restricted stock, restricted stock units, stock appreciation rights and other stock-based awards. Upon effectiveness of the 2014 Plan, the number of shares of our common stock that will be reserved for issuance under the 2014 Plan will be the sum of (1) 1,700,000 shares, plus (2) the number of shares (up to 1,678,220 shares) equal to the sum of the number of shares reserved for issuance under the 2007 Plan that remain available for grant under the 2007 Plan immediately prior to the effectiveness of the 2014 Plan and the number of shares of our common stock subject to outstanding awards under our 2007 Plan that expire, terminate or are otherwise surrendered, cancelled, forfeited or repurchased by us 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 our common stock, 4% of the number of shares of our common stock outstanding on the first day of the applicable fiscal year and an amount determined by our board of directors.

Our employees, officers, directors, consultants and advisors will be eligible to receive awards under the 2014 Plan; however, incentive stock options may only be granted to our employees.

Subject to any limitation in the 2014 Plan, our board of directors, or any committee or officer to which our board of directors has delegated authority, will select the recipients of awards and determine:

- the number of shares of common stock covered by options and the dates upon which those options become exercisable;
- the exercise price of options;
- the duration of options;
- the methods of payment of the exercise price of options; and
- the number of shares of common stock subject to any restricted stock or other stock-based awards and the terms and conditions of such awards, including the issue price, conditions for repurchase, repurchase price and performance conditions, if any.

Upon a merger or other reorganization event, our board of directors, may, in its sole discretion, take any one or more of the following actions pursuant to the 2014 Plan, as to some or all outstanding awards, other than restricted stock awards:

- provide that all outstanding awards will be assumed or substituted by the successor corporation;
- upon written notice to a participant, provide that the participant's unexercised options or awards will terminate immediately prior to the consummation of such transaction unless exercised by the participant;
- provide that outstanding awards will become exercisable, realizable or deliverable, or restrictions applicable to an award will lapse, in whole or in part, prior to or upon the reorganization event;
- in the event of a reorganization event pursuant to which holders of our common stock will receive a cash payment for each share surrendered in the reorganization event, make or provide for a cash payment to the participants equal to the excess, if any, of the acquisition price times the number of shares of our common stock subject to such outstanding awards (to the extent then exercisable at prices not in excess of the acquisition price), over the aggregate exercise price of all such outstanding awards and any applicable tax withholdings, in exchange for the termination of such awards; and
- provide that, in connection with a liquidation or dissolution, awards convert into the right to receive liquidation proceeds.

Upon the occurrence of a reorganization event other than a liquidation or dissolution, the repurchase and other rights under each outstanding restricted stock award will continue for the benefit of the successor company and will, unless our board of directors may otherwise determine, apply to the cash, securities or other property into which our common stock is converted pursuant to the reorganization event. Upon the occurrence of a reorganization event involving a liquidation or dissolution, all restrictions and conditions on each outstanding restricted stock award will automatically be deemed terminated or satisfied, unless otherwise provided in the agreement evidencing the restricted stock award.

No award may be granted under the 2014 Plan after the tenth anniversary of the effective date of the 2014 Plan. Our board of directors may amend, suspend or terminate the 2014 Plan at any time, except that stockholder approval will be required to comply with applicable law or stock market requirements.

2014 Employee Stock Purchase Plan

In August 2014, our board of directors adopted and our stockholders approved the 2014 ESPP. Our 2014 ESPP will become effective upon effectiveness of the registration statement of which this prospectus forms a part. The 2014 ESPP will initially provide participating employees with the opportunity to purchase an aggregate of 225,000 shares of our common stock. The number of shares of our common stock reserved for issuance under the 2014 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 our common stock, (2) 1% of the total number of shares of our common stock outstanding on the first day of the applicable fiscal year and (3) an amount determined by our board of directors.

All employees and all employees of a designated subsidiary, as defined in the 2014 ESPP, are eligible to participate in the 2014 ESPP, subject to limited exceptions set forth in the 2014 ESPP.

However, no employee is eligible to receive an option to purchase shares of our common stock under the 2014 ESPP that would result in the employee owning 5% or more of the total combined voting power or value of our common stock immediately after the grant of an option under the 2014 ESPP. Additionally, no employee may purchase shares of our common stock with an aggregate value of more than $25,000 per calendar year in which the option is outstanding under the 2014 ESPP, as determined by the value of such shares as of the date the option is granted.

We may make one or more offerings to our employees to purchase stock under the 2014 ESPP at such time or times as determined by our board of directors with each offering continuing for a six-month period, which we refer to as a plan period. However, our board of directors or a committee appointed by our board of directors may, in its discretion, choose a different plan period of twelve months or less for any offerings made under the 2014 ESPP. Our board of directors has not yet determined when the first plan period under the 2014 ESPP will commence. Payroll deductions made during each plan period will be held in payroll deductions accounts for all participating employees for the purchase of our common stock at the end of each plan period.

On the commencement date of each plan period, we will grant to each eligible employee who is then a participant in the 2014 ESPP an option to purchase shares of our common stock. The employee may authorize up to a maximum of 15% of his or her base pay to be deducted by us during the plan period. Each employee who continues to be a participant in the 2014 ESPP on the last business day of the plan period will be deemed to have exercised the option to the extent of the employee's accumulated payroll deductions, subject to the maximum share ownership limits for the 2014 ESPP. Under the terms of the 2014 ESPP, the option exercise price will be determined by our board of directors or a committee appointed by our board of directors for each plan period. Our board of directors or a committee appointed by our board of directors may set whether the option exercise price will be based on the closing price of our common stock on (1) the first business day of the plan period or (2) the last business day of the plan period, or the lower of such closing prices, provided that the option exercise price will be at least 85% of the applicable closing price. In no event may an employee purchase in any one plan period a number of shares that exceeds the number of shares determined by dividing (1) the product of $2,083 and the number of full months in the plan period by (2) the closing price of a share of our common stock on the commencement date of the plan period.

An employee who is not a participant in the 2014 ESPP on the last day of the plan period is not entitled to exercise any option, and any balance held in the employee's accumulated payroll deduction account will be refunded. An employee's rights under the 2014 ESPP terminate upon voluntary withdrawal from the purchase plan at any time prior to the last business day of the applicable plan period or when the employee ceases employment for any reason, as defined in the 2014 ESPP, before the last business day of the applicable plan period.

In the event of any stock splits, reverse stock splits, stock dividends, recapitalizations, combination of shares, reclassification of shares, spin-offs or other similar events or changes in capitalization or any dividend or

distribution to holders of our common stock other than an ordinary cash dividend, we will be required to make equitable adjustments in connection with the 2014 ESPP to the extent determined by our board of directors or a committee appointed by our board of directors.

Upon a merger or other reorganization event, our board of directors or a committee appointed by our board of directors may take any one or more of the following actions pursuant to the 2014 ESPP as to some or all outstanding options:

- provide that options will be assumed, or substantially equivalent options shall be substituted, by the acquiring or succeeding corporation (or an affiliate thereof);
- upon written notice to employees, provide that all outstanding options will terminate immediately prior to the consummation of such reorganization event and that all such outstanding options will become exercisable to the extent of accumulated payroll deductions as of a date specified by our board of directors or by a committee appointed by our board of directors;
- upon written notice to employees, provide that all outstanding options shall be cancelled as of a date prior to the effective date of such reorganization event and that all accumulated payroll deductions will be returned to participating employees on such date;
- in the event of a reorganization event pursuant to which holders of shares of our common stock will receive a cash payment for each share surrendered in the reorganization event, change the last day of the plan period to be the date of the consummation of the reorganization event and make or provide for a cash payment equal to (1) the acquisition price multiplied by the number of shares of our common stock subject to the participant's option that could be purchased based on the employee's accumulated payroll deductions at such time, minus (2) the aggregate option price of such option; or
- provide that, in connection with a liquidation or dissolution, options shall convert into the right to receive liquidation proceeds (net of the option price).

Our board of directors may at any time amend or terminate the 2014 ESPP, except that we must obtain stockholder approval for any amendment that requires stockholder approval under Section 423 of the Internal Revenue Code, and our board of directors may not make any amendment that would cause the 2014 ESPP to fail to comply with Section 423 of the Internal Revenue Code. Upon termination of the 2014 ESPP, we will refund any balance held in the payroll deduction accounts of participating employees.

401(k) Retirement Plan

We maintain a 401(k) retirement plan that is intended to be a tax-qualified defined contribution plan under Section 401(k) of the Internal Revenue Code. In general, all of our employees are eligible to participate, beginning on the first day of the month following commencement of their employment. The 401(k) plan includes a salary deferral arrangement pursuant to which participants may elect to reduce their current compensation by up to the statutorily prescribed limit, equal to $17,500 in 2014, and have the amount of the reduction contributed to the 401(k) plan. Currently, we do not match employee contributions.

Limitation of Liability and Indemnification

As permitted by Delaware law, we expect our board of directors and stockholders to adopt provisions in our restated certificate of incorporation, which will be effective as of the closing date of this offering, that limit or eliminate the personal liability of our directors. Our certificate of incorporation limits the liability of directors to the maximum extent permitted by Delaware law. Delaware law provides that directors of a corporation will not be personally liable for monetary damages for breaches of their fiduciary duties as directors, except liability for:

- any breach of the director's duty of loyalty to us or our stockholders;
- any act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;

- any unlawful payments related to dividends or unlawful stock repurchases, redemptions or other distributions; or
- any transaction from which the director derived an improper personal benefit.

These limitations do not apply to liabilities arising under federal securities laws and do not affect the availability of equitable remedies, including injunctive relief or rescission. If Delaware law is amended to authorize the further elimination or limiting of a director, then the liability of our directors will be eliminated or limited to the fullest extent permitted by Delaware law as so amended.

As permitted by Delaware law, our certificate of incorporation that will be effective as of the closing date of this offering will also provide that:

- we will indemnify our directors and officers to the fullest extent permitted by law;
- we may indemnify our other employees and other agents to the same extent that we indemnify our officers and directors, unless otherwise determined by our board of directors; and
- we will advance expenses to our directors and officers in connection with legal proceedings in connection with a legal proceeding to the fullest extent permitted by law.

The indemnification provisions contained in our certificate of incorporation that will be effective as of the closing date of this offering are not exclusive. In addition, we plan to enter into indemnification agreements with each of our directors and executive officers. We expect that each of these indemnification agreements will provide, among other things, that we will indemnify such director or executive officer to the fullest extent permitted by law for claims arising in his or her capacity as a director or officer, as applicable, provided that he or she acted in good faith and in a manner that he or she reasonably believed to be in, or not opposed to, our best interests and, with respect to any criminal proceeding, had no reasonable cause to believe that his or her conduct was unlawful. We expect that each of these indemnification agreements will provide that in the event that we do not assume the defense of a claim against a director or officer, as applicable, we will be required to advance his or her expenses in connection with his or her defense, provided that he or she undertakes to repay all amounts advanced if it is ultimately determined that he or she is not entitled to be indemnified by us.

We believe that these provisions and agreements are necessary to attract and retain qualified persons as directors and officers. Insofar as indemnification for liabilities arising under the Securities Act of 1933, which we refer to as the Securities Act, may be permitted to directors, officers or persons controlling our company pursuant to the foregoing provisions, we understand that in the opinion of the SEC such indemnification is against public policy as expressed in the Securities Act and is therefore unenforceable.

In addition, we maintain standard policies of insurance under which coverage is provided to our directors and officers against losses arising from claims made by reason of breach of duty or other wrongful act, and to us with respect to payments which may be made by us to such directors and officers pursuant to the above indemnification provisions or otherwise as a matter of law.

Related Person Transactions

The following is a description of transactions since January 1, 2011 to which we have been a party, and in which any of our directors, executive officers or beneficial owners of more than 5% of our voting securities, or affiliates or immediate family members of any of our directors, executive officers or beneficial owners of more than 5% of our voting securities, had or will have a direct or indirect material interest. We believe the terms obtained or consideration that we paid or received, as applicable, in connection with the transactions described below were comparable to terms available or the amounts that would be paid or received, as applicable, from unrelated third parties.

Series D-3 Redeemable Convertible Preferred Stock Financing

During September 2011, January 2012 and July 2012, we issued and sold an aggregate of 42,935,192 shares of our Series D-3 redeemable convertible preferred stock at a purchase price per share of $0.54617142 for an aggregate purchase price of $23.4 million.

The following table sets forth the number of shares of Series D-3 redeemable convertible preferred stock that were issued to our directors, executive officers and holders of more than 5% of our voting securities, and affiliates or immediate family members of our directors, executive officers and holders of more than 5% of our voting securities in connection with the Series D-3 redeemable convertible preferred stock financing and the aggregate cash purchase price paid by such persons and entities.

Purchaser	Shares of Series D-3 Redeemable Convertible Preferred Stock	Purchase Price
Entities affiliated with Apple Tree Partners II, L.P.(1)	24,046,035	$13,133,257
Novartis BioVentures Ltd.(2)	13,222,826	$ 7,221,930
Trusts and other entities affiliated with Muneer A. Satter(3)	3,931,085	$ 2,147,046

(1) Consists of 13,370,422 shares of Series D-3 redeemable convertible preferred stock and 10,675,613 shares of Series D-3 redeemable convertible preferred stock purchased by Apple Tree Partners II, L.P. and Apple Tree Partners II – Annex, L.P., respectively. Dr. Seth L. Harrison, a member of our board of directors, is a principal of the general partner of each of Apple Tree Partners II, L.P. and Apple Tree Partners II – Annex, L.P. and is affiliated with these entities. See "Principal Stockholders."

(2) Dr. Reinhard J. Ambros and Dr. Campbell Murray, two members of our board of directors, are employees of a corporation that is affiliated with Novartis BioVentures Ltd. See "Principal Stockholders."

(3) Consists of shares of Series D-3 redeemable convertible preferred stock purchased by the Muneer A. Satter Revocable Trust and various other trusts and other entities for which Mr. Satter serves as trustee, investment advisor or manager and, in such capacity, has sole voting and dispositive control over all such shares.

Series E Redeemable Convertible Preferred Stock Financing

During May 2013 and October 2013, we issued and sold an aggregate of 56,892,391 shares of our Series E redeemable convertible preferred stock at a purchase price per share of $0.62398475 for an aggregate purchase price of $35.5 million.

The following table sets forth the number of shares of Series E redeemable convertible preferred stock that were issued to our directors, executive officers and holders of more than 5% of our voting securities, and affiliates or immediate family members of our directors, executive officers and holders of more than 5% of our voting securities in connection with the Series E redeemable convertible preferred stock financing and the aggregate cash purchase price paid by such persons and entities.

Purchaser	Shares of Series E Redeemable Convertible Preferred Stock	Purchase Price
Apple Tree Partners II – Annex, L.P.(1)	24,199,308	$ 15,099,997
Novartis BioVentures Ltd.(2)	15,064,469	$ 9,399,999
Trusts and other entities affiliated with Muneer A. Satter(3)	8,013,003	$ 4,999,992

(1) Dr. Seth L. Harrison, a member of our board of directors, is a principal of the general partner of Apple Tree Partners II – Annex, L.P. and is affiliated with this entity. See "Principal Stockholders."

(2) Dr. Reinhard J. Ambros and Dr. Campbell Murray, two members of our board of directors, are employees of a corporation that is affiliated with Novartis BioVentures Ltd. See "Principal Stockholders."

(3) Consists of shares of Series E redeemable convertible preferred stock purchased by the Muneer A. Satter Revocable Trust and various other trusts and other entities for which Mr. Satter serves as trustee, investment advisor or manager and, in such capacity, has sole voting and dispositive control over all such shares.

Agreements with Our Stockholders

We have entered into a fifth amended and restated investor rights agreement with the purchasers of our redeemable convertible preferred stock, including some of our 5% stockholders and their affiliates and entities affiliated with our directors. The investor rights agreement provides those holders with the right to demand that we file a registration statement, subject to certain limitations, and to request that their shares be covered by a registration statement that we are otherwise filing. See "Description of Capital Stock—Registration Rights" for additional information.

We have also entered into a stockholders' agreement with certain purchasers of our common stock and redeemable convertible preferred stock. The stockholders' agreement provides for rights of first refusal and co-sale rights in respect of sales of securities by certain holders of our capital stock. The stockholders' agreement also provides holders of our redeemable convertible preferred stock with a participation right to purchase their pro rata share of new securities that we may propose to sell and issue, subject to specified exceptions. The stockholders' agreement also contains provisions with respect to the election of our board of directors and its composition. The rights of first refusal, co-sale rights and participation rights under this agreement do not apply to this offering, and the stockholders' agreement will terminate upon the closing of this offering.

Severance and Change in Control Agreements

See the "Management—Employment Agreements, Severance and Change in Control Agreements" section of this prospectus for a further discussion of these arrangements.

Participation in this Offering

Certain of our existing stockholders and their affiliates, including our existing principal stockholders, have agreed to purchase an aggregate of 1,105,000 shares of our common stock in this offering at the initial public offering price for an aggregate purchase price of $16,575,000.

Indemnification of Officers and Directors

Our certificate of incorporation that will be effective as of the closing date of this offering provides that we will indemnify our directors and officers to the fullest extent permitted by Delaware law. In addition, we expect to enter into indemnification agreements with each of our officers and directors that may be broader in scope than the specific indemnification provisions contained in the Delaware General Corporation Law. See the "Executive Compensation—Limitation of Liability and Indemnification" section of this prospectus for a further discussion of these arrangements.

Policies and Procedures for Related Person Transactions

Our board of directors has adopted a written related person transaction policy, which will become effective upon effectiveness of the registration statement of which this prospectus forms a part, to set forth policies and procedures for the review and approval or ratification of related person transactions. Effective upon the closing of this offering, this policy will cover any transaction, arrangement or relationship, or any series of similar transactions, arrangements or relationships, in which we were or are to be a participant, the amount involved exceeds $120,000, and a related person had or will have a direct or indirect material interest, including, without limitation, purchases of goods or services by or from the related person or entities in which the related person has a material interest, indebtedness, guarantees of indebtedness and employment by us of a related person.

Our related person transaction policy contains exceptions for any transaction or interest that is not considered a related person transaction under SEC rules as in effect from time to time. In addition, the policy provides that an interest arising solely from a related person's position as an executive officer of another entity that is a participant in a transaction with us will not be subject to the policy if each of the following conditions is met:

- the related person and all other related persons own in the aggregate less than a 10% equity interest in such entity;
- the related person and his or her immediate family members are not involved in the negotiation of the terms of the transaction with us and do not receive any special benefits as a result of the transaction; and
- the amount involved in the transaction is less than the greater of $200,000 and 5% of the annual gross revenue of the company receiving payment under the transaction.

The policy provides that any related person transaction proposed to be entered into by us must be reported to our Chief Executive Officer or Chief Financial Officer and will be reviewed and approved by our audit committee in accordance with the terms of the policy, prior to effectiveness or consummation of the transaction whenever practicable. The policy provides that if our Chief Executive Officer or Chief Financial Officer determines that advance approval of a related person transaction is not practicable under the circumstances, our audit committee will review and, in its discretion, may ratify the related person transaction at the next meeting of the audit committee. The policy also provides that alternatively, our Chief Executive Officer or Chief Financial Officer may present a related person transaction arising in the time period between meetings of the audit committee to the chair of the audit committee, who will review and may approve the related person transaction, subject to ratification by the audit committee at the next meeting of the audit committee.

In addition, the policy provides that any related person transaction previously approved by the audit committee or otherwise already existing that is ongoing in nature will be reviewed by the audit committee annually to ensure that such related person transaction has been conducted in accordance with the previous approval granted by the audit committee, if any, and that all required disclosures regarding the related person transaction are made.

The policy provides that transactions involving compensation of executive officers will be reviewed and approved by our compensation committee in the manner to be specified in the charter of the compensation committee.

A related person transaction reviewed under this policy will be considered approved or ratified if it is authorized by the audit committee in accordance with the standards set forth in the policy after full disclosure of the related person's interests in the transaction. As appropriate for the circumstances, the policy provides that the audit committee will review and consider:

- the related person's interest in the related person transaction;
- the approximate dollar value of the amount involved in the related person transaction;
- the approximate dollar value of the amount of the related person's interest in the transaction without regard to the amount of any profit or loss;
- whether the transaction was undertaken in the ordinary course of business of our company;
- whether the transaction with the related person is proposed to be, or was, entered into on terms no less favorable to us than the terms that could have been reached with an unrelated third party;
- the purpose of, and the potential benefits to us of, the transaction; and
- any other information regarding the related person transaction or the related person in the context of the proposed transaction that would be material to investors in light of the circumstances of the particular transaction.

The policy provides that the audit committee will review all relevant information available to it about the related person transaction. The policy provides that the audit committee may approve or ratify the related person transaction only if the audit committee determines that, under all of the circumstances, the transaction is in, or is not inconsistent with, our best interests. The policy provides that the audit committee may, in its sole discretion, impose such conditions as it deems appropriate on us or the related person in connection with approval of the related person transaction.

Principal Stockholders

The following table sets forth information regarding the beneficial ownership of our common stock as of July 31, 2014 by:

- each person, or group of affiliated persons, who is known by us to beneficially own more than 5% of our common stock;
- each of our named executive officers;
- each of our directors; and
- all of our directors and executive officers as a group.

Beneficial ownership is determined in accordance with the rules and regulations of the SEC. These rules generally attribute beneficial ownership of securities to persons who possess sole or shared voting power or investment power with respect to those securities and include shares of common stock issuable upon the exercise of stock options that are immediately exercisable or exercisable within 60 days after July 31, 2014. Except as otherwise indicated, all of the shares reflected in the table are shares of common stock and all persons listed below have sole voting and investment power with respect to the shares beneficially owned by them, subject to community property laws, where applicable. The information is not necessarily indicative of beneficial ownership for any other purpose.

The number of shares beneficially owned in the following table assumes the automatic conversion of all outstanding shares of our redeemable convertible preferred stock into shares of common stock upon the closing of this offering. The percentage ownership calculations for beneficial ownership prior to this offering are based on 15,361,742 shares outstanding as of July 31, 2014, assuming the automatic conversion of all outstanding shares of our redeemable convertible preferred stock into shares of common stock upon the closing of this offering. Percentage ownership calculations for beneficial ownership after this offering also include the shares we are offering hereby. Except as otherwise indicated in the table below, addresses of named beneficial owners are in care of Tokai Pharmaceuticals, Inc., One Broadway, 14th Floor, Cambridge, Massachusetts 02142.

In computing the number of shares of common stock beneficially owned by a person and the percentage ownership of that person, we deemed outstanding shares of common stock subject to options held by that person that are currently exercisable or exercisable within 60 days after July 31, 2014. We did not deem these shares outstanding, however, for the purpose of computing the percentage ownership of any other person.

Certain of our existing stockholders and their affiliates, including our existing principal stockholders, have agreed to purchase an aggregate of 1,105,000 shares of our common stock in this offering at the initial public offering price. In addition, Novo A/S has agreed to purchase 1,000,000 shares of our common stock in this offering at the initial public offering price. The following table does not reflect any purchases by these parties or their affiliates.

Name and Address of Beneficial Owner	Number of Shares Beneficially Owned	Percentage of Shares Beneficially Owned	
		Before Offering	After Offering
5% Stockholders:			
Entities affiliated with Apple Tree Partners[1] 47 Hulfish Street, Suite 441 Princeton, NJ 08542	7,549,579	49.15%	34.56%
Novartis BioVentures Ltd.[2] PO Box HM 2899 Hamilton HM LX Bermuda	4,319,328	28.12%	19.78%
Trusts and other entities affiliated with Muneer A. Satter[3] Satter Investment Management, LLC 676 North Michigan Ave., Suite 4000 Chicago, IL 60611	1,621,688	10.56%	7.42%
Executive Officers and Directors:			
Jodie P. Morrison[4]	234,939	1.51%	1.06%
Martin D. Williams	168,850	1.10%	*
Adrian Senderowicz, M.D.	14,782	*	*
Seth L. Harrison, M.D.[5]	7,762,273	50.53%	35.54%
Reinhard J. Ambros, Ph.D.[2]	4,319,328	28.12%	19.78%
Timothy J. Barberich[6]	136,511	*	*
David A. Kessler, M.D.[7]	20,540	*	*
Campbell Murray, M.D.[2]	4,319,328	28.12%	19.78%
Joseph A. Yanchik, III[8]	47,204	*	*
All executive officers and directors as a group (11 persons)[9]	12,795,368	81.31%	57.60%

* Represents beneficial ownership of less than one percent of our outstanding stock.

(1) Consists of (i) 9 shares of common stock and 4,218,632 shares of common stock underlying shares of redeemable convertible preferred stock held by Apple Tree Partners II, L.P. and (ii) 3,330,938 shares of common stock underlying shares of redeemable convertible preferred stock held by Apple Tree Partners II – Annex, L.P. Dr. Seth L. Harrison, a member of our board of directors, is a principal of the general partner of each of Apple Tree Partners II, L.P. and Apple Tree Partners II –Annex, L.P., and Dr. Harrison disclaims beneficial ownership of the shares held by each of Apple Tree Partners II, L.P. and Apple Tree Partners II – Annex, L.P., except to the extent of his pecuniary interest therein. Dr. Harrison has sole voting and investment control and power over the shares held by Apple Tree Partners II, L.P. and Apple Tree Partners II – Annex, L.P.

(2) Consists of 4,319,328 shares of common stock underlying shares of redeemable convertible preferred stock held by Novartis BioVentures Ltd., a Bermuda corporation. The board of directors of Novartis BioVentures Ltd. has sole voting and investment control and power over such shares. None of the members of its board of directors has individual voting or investment power with respect to such shares and each disclaims beneficial ownership of such shares. Dr. Reinhard J. Ambros and Dr. Campbell Murray, two members of our board of directors, are also employees of a corporation that is affiliated with Novartis BioVentures Ltd. Each of Drs. Murray and Ambros disclaims beneficial ownership of the shares held by Novartis BioVentures Ltd., except to the extent of their pecuniary interest arising as a result of their employment by such affiliate of Novartis BioVentures Ltd. Novartis BioVentures Ltd. is an indirectly owned subsidiary of Novartis AG.

(3) Consists of 1,621,688 shares of common stock underlying shares of redeemable convertible preferred stock held by the Muneer A. Satter Revocable Trust and various other trusts and other entities for which Mr. Satter serves as trustee, investment advisor or manager and, in such capacity, has sole voting and dispositive control over all such shares.

(4) Consists of (i) 9,551 shares of common stock and (ii) 225,388 shares of common stock underlying options that are exercisable as of July 31, 2014 or will become exercisable within 60 days after such date.

(5) Consists of (i) 212,694 shares of common stock held by Dr. Harrison, (ii) 9 shares of common stock and 4,218,641 shares of common stock underlying shares of redeemable convertible preferred stock held by Apple Tree Partners II, L.P. and (iii) 3,330,938 shares of common stock underlying shares of redeemable convertible preferred stock held by Apple Tree Partners II – Annex, L.P. Dr. Harrison is a principal of the general partner of each of Apple Tree Partners II, L.P. and Apple Tree Partners II – Annex, L.P., and Dr. Harrison disclaims beneficial ownership of the shares held by each of Apple Tree Partners II, L.P. and Apple Tree Partners II – Annex, L.P., except to the extent of his pecuniary interest therein. Dr. Harrison has sole voting and investment control and power over the shares held by Apple Tree Partners II, L.P. and Apple Tree Partners II – Annex, L.P.

(6) Consists of (i) 121,077 shares of common stock underlying shares of redeemable convertible preferred stock and (ii) 15,434 shares of common stock underlying options that are exercisable as of July 31, 2014 or will become exercisable within 60 days after such date.

(7) Consists of 20,540 shares of common stock underlying options that are exercisable as of July 31, 2014 or will become exercisable within 60 days after such date.

(8) Consists of (i) 25,546 shares of common stock and (ii) 21,658 shares of common stock underlying options that are exercisable as of July 31, 2014 or will become exercisable within 60 days after such date.

(9) Includes 373,961 shares of common stock underlying options that are exercisable as of July 31, 2014 or will become exercisable within 60 days after such date. See footnote 2 with respect to Drs. Ambros and Murray and footnotes 1 and 5 with respect to Dr. Harrison.

Description of Capital Stock

General

Following the closing of this offering, our authorized capital stock will consist of 200,000,000 shares of common stock, par value $0.001 per share, and 5,000,000 shares of preferred stock, par value $0.001 per share, all of which preferred stock will be undesignated. The following description of our capital stock and provisions of our restated certificate of incorporation and amended and restated bylaws are summaries and are qualified by reference to the certificate of incorporation and the bylaws that will be in effect upon the closing of this offering. We have filed copies of these documents with the SEC as exhibits to our registration statement of which this prospectus forms a part. The descriptions of the common stock and preferred stock reflect changes to our capital structure that will occur upon the closing of this offering.

Common Stock

As of July 31, 2014, we had outstanding 15,361,742 shares of common stock, held of record by 41 stockholders, assuming the automatic conversion of all outstanding shares of our preferred stock into common stock upon the closing of this offering.

Holders of our common stock are entitled to one vote for each share held on all matters submitted to a vote of stockholders and do not have cumulative voting rights. An election of directors by our stockholders shall be determined by a plurality of the votes cast by the stockholders entitled to vote on the election. Other matters shall be decided by the affirmative vote of our stockholders having a majority in voting power of the votes cast by the stockholders present or represented and voting on such matter, except as otherwise disclosed below. Holders of common stock are entitled to receive proportionately any dividends as may be declared by our board of directors, subject to any preferential dividend rights of outstanding preferred stock.

In the event of our liquidation or dissolution, the holders of common stock are entitled to receive proportionately all assets available for distribution to stockholders after the payment of all debts and other liabilities and subject to the prior rights of any outstanding preferred stock. Holders of common stock have no preemptive, subscription, redemption or conversion rights. The rights, preferences and privileges of holders of common stock are subject to and may be adversely affected by the rights of the holders of shares of any series of preferred stock that we may designate and issue in the future.

Preferred Stock

Under the terms of our certificate of incorporation that will become effective upon the closing of this offering, our board of directors is authorized to issue shares of preferred stock in one or more series without stockholder approval. Our board of directors has the discretion to determine the rights, preferences, privileges and restrictions, including voting rights, dividend rights, conversion rights, redemption privileges and liquidation preferences, of each series of preferred stock.

The purpose of authorizing our board of directors to issue preferred stock and determine its rights and preferences is to eliminate delays associated with a stockholder vote on specific issuances. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions, future financings and other corporate purposes, could have the effect of making it more difficult for a third party to acquire, or could discourage a third party from seeking to acquire, a majority of our outstanding voting stock. Upon the closing of this offering, there will be no shares of preferred stock outstanding, and we have no present plans to issue any shares of preferred stock.

Stock Options

As of July 31, 2014, options to purchase 1,634,275 shares of our common stock at a weighted average exercise price of $2.86 per share were outstanding, of which options to purchase 525,068 shares of our common stock were exercisable, at a weighted average exercise price of $1.89 per share.

Registration Rights

We have entered into a fifth amended and restated investor rights agreement, dated as of May 13, 2013, which we refer to as the Investor Rights Agreement, with certain of our stockholders. Upon the closing of this offering, holders of a total of 14,764,034 shares of our common stock, including for this purpose 14,764,025 shares of our common stock issuable upon conversion of our preferred stock upon the closing of this offering, will have the right to require us to register these shares under the Securities Act under specified circumstances as described below and will have incidental registration rights as described below. After registration pursuant to these rights, these shares will become freely tradable without restriction under the Securities Act.

Demand Registration Rights

Beginning on the 180th day after the effective date of the registration statement of which this prospectus forms a part, subject to specified limitations set forth in the Investor Rights Agreement, at any time the holders of a majority of the then outstanding shares of our common stock issuable upon conversion of our Series C, Series D and Series E preferred stock upon the closing of this offering, acting together, may demand in writing that we register registrable securities, as defined under the Investor Rights Agreement, under the Securities Act so long as the total amount of registrable shares requested to be registered represents at least 20% of the then-outstanding registrable shares or has an aggregate expected price to the public of at least $10.0 million. We are not obligated to file a registration statement pursuant to this demand provision on more than two occasions during the term of the Investor Rights Agreement, subject to specified exceptions.

In addition, at any time after we become eligible to file a registration statement on Form S-3 under the Securities Act, subject to specified limitations, a holder or holders of a majority of the then outstanding shares of our common stock issuable upon conversion of our Series C, Series D and Series E preferred stock upon the closing of this offering may demand in writing that we register on Form S-3 registrable shares held by them so long as the total amount of registrable shares requested to be registered has an aggregate expected price to the public of at least $7.5 million unless such request is for all remaining registrable securities. We are not obligated to file a registration statement pursuant to this demand provision on more than two occasions during any 12-month period, subject to specified exceptions.

Incidental Registration Rights

If, at any time after the closing of this offering, we propose to file a registration statement to register any of our securities under the Securities Act, either for our own account or for the account of any of our stockholders, other than pursuant to the demand registration rights described above and other than pursuant to a Form S-4 or Form S-8, the holders of our registrable securities are entitled to notice of registration and, subject to specified exceptions, we will be required to use commercially reasonable efforts to register the registrable securities then held by them that they request that we register.

Expenses

Pursuant to the Investor Rights Agreement, we are required to pay all registration expenses, including registration fees, printing expenses, fees and disbursements of our counsel and accountants and reasonable fees and disbursements of one counsel representing any selling stockholders, other than any underwriting discounts and commissions, related to any demand or incidental registration. The Investor Rights Agreement contains customary cross-indemnification provisions, pursuant to which we are obligated to indemnify any selling

stockholders in the event of material misstatements or omissions in the registration statement attributable to us, and they are obligated to indemnify us for material misstatements or omissions in the registration statement attributable to them.

Anti-Takeover Effects of Delaware Law and our Charter and Bylaws

Delaware law contains, and upon the closing of this offering our certificate of incorporation and our bylaws will contain, provisions that could have the effect of delaying, deferring or discouraging another party from acquiring control of us. These provisions, which are summarized below, are expected to discourage coercive takeover practices and inadequate takeover bids. These provisions are also designed to encourage persons seeking to acquire control of us to first negotiate with our board of directors.

Staggered Board; Removal of Directors

Upon the closing of this offering, our certificate of incorporation and bylaws will divide our board of directors into three classes with staggered three-year terms. In addition, a director will only be able to be removed for cause and only by the affirmative vote of the holders of at least 75% of the votes that all of our stockholders would be entitled to cast in an annual election of directors. Any vacancy on our board of directors, including a vacancy resulting from an enlargement of our board of directors, will only be able to be filled by vote of a majority of our directors then in office. The classification of our board of directors and the limitations on the removal of directors and filling of vacancies could make it more difficult for a third party to acquire, or discourage a third party from seeking to acquire, control of our company.

Stockholder Action by Written Consent; Special Meetings

Upon the closing of this offering, our certificate of incorporation will provide that any action required or permitted to be taken by our stockholders must be effected at a duly called annual or special meeting of such holders and may not be effected by any consent in writing by such holders. Upon the closing of this offering, our certificate of incorporation and bylaws will also provide that, except as otherwise required by law, special meetings of our stockholders can only be called by our chairman of the board, our chief executive officer or our board of directors.

Advance Notice Requirements for Stockholder Proposals

Upon the closing of this offering, our bylaws will establish an advance notice procedure for stockholder proposals to be brought before an annual meeting of stockholders, including proposed nominations of persons for election to our board of directors. Stockholders at an annual meeting will only be able to consider proposals or nominations specified in the notice of meeting or brought before the meeting by or at the direction of our board of directors or by a stockholder of record on the record date for the meeting who is entitled to vote at the meeting and who has delivered timely written notice in proper form to our secretary of the stockholder's intention to bring such business before the meeting. These provisions could have the effect of delaying until the next stockholder meeting stockholder actions that are favored by the holders of a majority of our outstanding voting securities.

Delaware Business Combination Statute

Upon the closing of this offering, we will be subject to Section 203 of the Delaware General Corporation Law. Subject to certain exceptions, Section 203 prevents a publicly-held Delaware corporation from engaging in a "business combination" with any "interested stockholder" for three years following the date that the person became an interested stockholder, unless the interested stockholder attained such status with the approval of our board of directors or unless the business combination is approved in a prescribed manner. A "business combination" includes, among other things, a merger or consolidation involving us and the "interested stockholder" and the sale of more than 10% of our assets. In general, an "interested stockholder" is any entity or person beneficially owning 15% or more of our outstanding voting stock and any entity or person affiliated with or controlling or controlled by such entity or person.

Amendment of Certificate of Incorporation and Bylaws

The Delaware General Corporation Law provides generally that the affirmative vote of a majority of the shares entitled to vote on any matter is required to amend a corporation's certificate of incorporation or bylaws, unless a corporation's certificate of incorporation or bylaws, as the case may be, requires a greater percentage. Effective upon the closing of this offering, our bylaws may be amended or repealed by a majority vote of our board of directors or by the affirmative vote of the holders of at least 75% of the votes that all of our stockholders would be entitled to cast in any annual election of directors. In addition, the affirmative vote of the holders of at least 75% of the votes that all of our stockholders would be entitled to cast in any annual election of directors is required to amend or repeal or to adopt any provisions inconsistent with any of the provisions of our certificate of incorporation described above under "—Staggered Board; Removal of Directors" and "—Stockholder Action by Written Consent; Special Meetings."

Listing on The NASDAQ Global Market

Our common stock has been approved for listing on The NASDAQ Global Market under the symbol "TKAI."

Authorized but Unissued Shares

The authorized but unissued shares of common stock and preferred stock are available for future issuance without stockholder approval, subject to any limitations imposed by the NASDAQ Listing Rules. These additional shares may be used for a variety of corporate finance transactions, acquisitions and employee benefit plans. The existence of authorized but unissued and unreserved common stock and preferred stock could make it more difficult or discourage an attempt to obtain control of us by means of a proxy contest, tender offer, merger or otherwise.

Transfer Agent and Registrar

The transfer agent and registrar for our common stock will be Continental Stock Transfer & Trust Company.

Shares Eligible for Future Sale

Prior to this offering, there has been no public market for our common stock, and a liquid public trading market for our common stock may not develop or be sustained after this offering. Future sales of substantial amounts of our common stock in the public market, including shares issued upon exercise of outstanding options or in the public market after this offering, or the anticipation of those sales, could adversely affect market prices prevailing from time to time and could impair our ability to raise capital through sales of our equity securities. Our common stock has been approved for listing on The NASDAQ Global Market under the symbol "TKAI."

Upon the closing of this offering, we will have outstanding 21,841,742 shares of common stock, after giving effect to the issuance of 6,480,000 shares of common stock in this offering and the conversion of all outstanding shares of our preferred stock into 14,860,173 shares of common stock upon the closing of this offering, and assuming no exercise of outstanding options after July 31, 2014. Of the shares to be outstanding immediately after the closing of this offering, 5,375,000 shares sold by us in this offering, assuming that the underwriters do not exercise their over-allotment option, will be freely tradable without restriction or further registration under the Securities Act, except for any shares purchased by our "affiliates," as that term is defined in Rule 144 under the Securities Act, whose sales would be subject to the Rule 144 resale restrictions described below, other than the holding period requirement.

The remaining 16,466,742 shares of common stock, including 1,105,000 shares which our existing stockholders and their affiliates have agreed to purchase in this offering, will be "restricted securities," as that term is defined in Rule 144 under the Securities Act and will further be subject to either restrictions on transfer under the lock-up agreements described below or restrictions on transfer for a period of 180 days from the effectiveness of the registration statement of which this prospectus forms a part under stock option agreements entered into between us and the holders of those shares. Following the expiration of these restrictions, these shares will become eligible for public sale if they are registered under the Securities Act or if they qualify for an exemption from registration under Rules 144 or 701 under the Securities Act, which are summarized below.

Subject to the lock-up agreements described below and any additional contractual lock-up period and the provisions of Rules 144 and 701 under the Securities Act, these restricted securities will be available for sale in the public market as follows:

Date Available for Sale	Number of Shares Eligible for Sale	Comment
On the date of this prospectus	0	Shares sold in this offering and shares saleable under Rule 144 that are not subject to a lock-up
90 days after the date of this prospectus	0	Shares saleable under Rules 144 and 701 that are not subject to a lock-up
180 days after the date of this prospectus	16,466,742	Lock-up released; shares saleable under Rules 144 and 701

Rule 144

Affiliate Resales of Restricted Securities

In general, beginning 90 days after the effective date of the registration statement of which this prospectus forms a part, a person who is an affiliate of ours, or who was an affiliate at any time during the 90 days before a

sale, who has beneficially owned shares of our common stock for at least six months would be entitled to sell in "broker's transactions" or certain "riskless principal transactions" or to market makers, a number of shares within any three-month period that does not exceed the greater of:

- 1% of the number of shares of our common stock then outstanding, which will equal approximately 218,417 shares immediately after this offering; or
- the average weekly trading volume in our common stock on The NASDAQ Global Market during the four calendar weeks preceding the filing of a notice on Form 144 with respect to such sale.

Affiliate resales under Rule 144 are also subject to the availability of current public information about us. In addition, if the number of shares being sold under Rule 144 by an affiliate during any three-month period exceeds 5,000 shares or has an aggregate sale price in excess of $50,000, the seller must file a notice on Form 144 with the SEC and The NASDAQ Stock Market concurrently with either the placing of a sale order with the broker or the execution directly with a market maker.

Non-Affiliate Resales of Restricted Securities

In general, beginning 90 days after the effective date of the registration statement of which this prospectus forms a part, a person who is not an affiliate of ours at the time of sale, and has not been an affiliate at any time during the three months preceding a sale, and who has beneficially owned shares of our common stock for at least six months but less than a year, is entitled to sell such shares subject only to the availability of current public information about us. If such person has held our shares for at least one year, such person can resell under Rule 144(b)(1) without regard to any Rule 144 restrictions, including the 90-day public company requirement and the current public information requirement.

Non-affiliate resales are not subject to the manner of sale, volume limitation or notice filing provisions of Rule 144.

Lock-Up Agreements

We and each of our directors and executive officers and holders of our outstanding common stock, who collectively own 100% of our common stock, based on shares outstanding as of July 31, 2014, have agreed that, without the prior written consent of BMO Capital Markets Corp. and Stifel, Nicolaus & Company, Incorporated on behalf of the underwriters, we and they will not, subject to limited exceptions, during the period ending 180 days after the date of this prospectus, either directly or indirectly:

- offer, sell, pledge, contract to sell, purchase any option to sell, grant any option for the purchase of, lend, or otherwise dispose of, or require us to file with the SEC a registration statement under the Securities Act to register, any shares of our common stock or any securities convertible into, exercisable for or exchangeable for our common stock, whether any such transaction is to be settled by delivery of shares of our common stock or other securities, in cash or otherwise, or publicly disclose the intention to enter into any such transaction; or
- enter into any swap or other derivative transaction that transfers to another, in whole or in part, any of the economic benefits or risks of ownership of such shares of our common stock, whether any such transaction is to be settled by delivery of shares of our common stock or other securities, in cash or otherwise, or publicly disclose the intention to enter into any such transaction.

These agreements are subject to certain exceptions, as described in the section of this prospectus entitled "Underwriting." Upon the expiration of the applicable lock-up periods and any additional contractual lock-up period, substantially all of the shares subject to such lock-up restrictions will become eligible for sale, subject to the limitations discussed above.

These agreements apply to the shares purchased in this offering by certain of our existing stockholders and their affiliates, including our existing principal stockholders, who have agreed to purchase an aggregate of 1,105,000 shares of our common stock in this offering at the initial public offering price. Novo A/S, which has agreed to purchase 1,000,000 shares of our common stock in this offering at the initial public offering price, is not a party to a lock-up agreement.

Registration Rights

Subject to the lock-up agreements described above, upon the closing of this offering, the holders of an aggregate of 14,764,034 shares of our common stock, including for this purpose 14,764,025 shares of our common stock issuable upon conversion of our preferred stock upon the closing of this offering, will have the right to require us to register these shares under the Securities Act under specified circumstances. After registration pursuant to these rights and expiration of the lock-up agreement, these shares will become freely tradable without restriction under the Securities Act. See "Description of Capital Stock—Registration Rights" for additional information regarding these registration rights.

Stock Options

As of July 31, 2014, options to purchase 1,634,275 shares of our common stock at a weighted average exercise price of $2.86 per share were outstanding, of which options to purchase 525,068 shares of our common stock were exercisable, at a weighted average exercise price of $1.89 per share. Following this offering, we intend to file registration statements on Form S-8 under the Securities Act to register all of the shares of common stock subject to outstanding options and options and other awards issuable pursuant to our equity compensation plans.

Material U.S. Federal Tax Considerations for Non-U.S. Holders of Common Stock

The following is a general discussion of the material U.S. federal income and estate tax considerations applicable to non-U.S. holders with respect to their ownership and disposition of shares of our common stock issued pursuant to this offering. For purposes of this discussion, a non-U.S. holder means a beneficial owner of our common stock that is not for U.S. federal income tax purposes:

- an individual who is a citizen or resident of the United States;
- a corporation or any other organization taxable as a corporation for U.S. federal income tax purposes, created or organized in the United States or under the laws of the United States or of any state thereof or the District of Columbia;
- an estate, the income of which is subject to U.S. federal income tax regardless of its source; or
- a trust if (1) a U.S. court is able to exercise primary supervision over the trust's administration and one or more U.S. persons have the authority to control all of the trust's substantial decisions or (2) the trust has a valid election in effect under applicable U.S. Treasury Regulations to be treated as a U.S. person.

This discussion does not address the tax treatment of partnerships or other entities that are pass-through entities for U.S. federal income tax purposes or persons that hold their common stock through partnerships or other pass-through entities. A partner in a partnership or other pass-through entity that will hold our common stock should consult his, her or its own tax advisor regarding the tax consequences of acquiring, holding and disposing of our common stock through a partnership or other pass-through entity, as applicable.

This discussion is based on current provisions of the U.S. Internal Revenue Code of 1986, as amended, which we refer to as the Code, existing and proposed U.S. Treasury Regulations promulgated thereunder, current administrative rulings and judicial decisions, all as in effect as of the date of this prospectus, all of which are subject to change or to differing interpretation, possibly with retroactive effect. Any change could alter the tax consequences to non-U.S. holders described in this prospectus. There can be no assurance that the Internal Revenue Service, which we refer to as the IRS, will not challenge one or more of the tax consequences described herein. We assume in this discussion that a non-U.S. holder holds shares of our common stock as a capital asset, generally property held for investment.

This discussion does not address all aspects of U.S. federal income and estate taxation that may be relevant to a particular non-U.S. holder in light of that non-U.S. holder's individual circumstances nor does it address any aspects of U.S. state, local or non-U.S. taxes, the alternative minimum tax, or the Medicare tax on net investment income. This discussion also does not consider any specific facts or circumstances that may apply to a non-U.S. holder and does not address the special tax rules applicable to particular non-U.S. holders, such as:

- insurance companies;
- tax-exempt organizations;
- financial institutions;
- brokers or dealers in securities;
- regulated investment companies;
- pension plans;
- controlled foreign corporations;
- passive foreign investment companies;
- owners deemed to sell our common stock under the constructive sale provisions of the Code;
- persons that have a functional currency other than the U.S. dollar;

- owners that hold our common stock as part of a straddle, hedge, conversion transaction, synthetic security or other integrated investment; and
- certain U.S. expatriates.

This discussion is for general information only and is not tax advice. Accordingly, all prospective non-U.S. holders of our common stock should consult their own tax advisors with respect to the U.S. federal, state, local and non-U.S. tax consequences of the purchase, ownership and disposition of our common stock.

Distributions on Our Common Stock

Distributions, if any, on our common stock generally will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. If a distribution exceeds our current and accumulated earnings and profits, the excess will be treated as a tax-free return of the non-U.S. holder's investment, up to such holder's tax basis in the common stock. Any remaining excess will be treated as capital gain, subject to the tax treatment described below in "Gain on sale, exchange or other disposition of our common stock." Any such distributions will also be subject to the discussion below under the section titled "Withholding and information reporting requirements—FATCA."

Dividends paid to a non-U.S. holder generally will be subject to withholding of U.S. federal income tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence.

Dividends that are treated as effectively connected with a trade or business conducted by a non-U.S. holder within the United States and, if an applicable income tax treaty so provides, that are attributable to a permanent establishment or a fixed base maintained by the non-U.S. holder within the United States, are generally exempt from the 30% withholding tax if the non-U.S. holder satisfies applicable certification and disclosure requirements. However, such U.S. effectively connected income, net of specified deductions and credits, is taxed at the same graduated U.S. federal income tax rates applicable to United States persons (as defined in the Code). Any U.S. effectively connected income received by a non-U.S. holder that is a corporation may also, under certain circumstances, be subject to an additional "branch profits tax" at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence.

A non-U.S. holder of our common stock who claims the benefit of an applicable income tax treaty between the United States and such holder's country of residence generally will be required to provide a properly executed IRS Form W-8BEN or W-8BEN-E (or successor form) and satisfy applicable certification and other requirements. Non-U.S. holders are urged to consult their tax advisors regarding their entitlement to benefits under a relevant income tax treaty.

A non-U.S. holder that is eligible for a reduced rate of U.S. withholding tax under an income tax treaty may obtain a refund or credit of any excess amounts withheld by timely filing a U.S. tax return with the IRS.

Gain on Sale, Exchange or Other Disposition of Our Common Stock

In general, a non-U.S. holder will not be subject to any U.S. federal income tax on any gain realized upon such holder's sale, exchange or other disposition of shares of our common stock unless:

- the gain is effectively connected with the non-U.S. holder's conduct of a U.S. trade or business and, if an applicable income tax treaty so provides, is attributable to a permanent establishment or a fixed-base maintained by such non-U.S. holder in the United States, in which case the non-U.S. holder generally will be taxed on a net income basis at the graduated U.S. federal income tax rates applicable to United States persons (as defined in the Code) and, if the non-U.S. holder is a foreign corporation, the branch profits tax described above in "Distributions on our common stock" also may apply;

- the non-U.S. holder is a nonresident alien individual who is present in the United States for 183 days or more in the taxable year of the disposition and certain other conditions are met, in which case the non-U.S. holder will be subject to a 30% tax (or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence) on the net gain derived from the disposition, which may be offset by certain U.S. source capital losses of the non-U.S. holder, if any (even though the individual is not considered a resident of the United States), provided that the non-U.S. holder has timely filed U.S. federal income tax returns with respect to such losses; or
- we are, or have been, at any time during the five-year period preceding such disposition (or the non-U.S. holder's holding period, if shorter) a "U.S. real property holding corporation," unless our common stock is regularly traded on an established securities market and the non-U.S. holder holds no more than 5% of our outstanding common stock, directly or indirectly, actually or constructively, during the shorter of the 5-year period ending on the date of the disposition or the period that the non-U.S. holder held our common stock. If we are determined to be a U.S. real property holding corporation and the foregoing exception does not apply, then a purchaser may withhold 10% of the proceeds payable to a non-U.S. holder from a sale of our common stock and the non-U.S. holder generally will be taxed on its net gain derived from the disposition at the graduated U.S. federal income tax rates applicable to United States persons (as defined in the Code). Generally, a corporation is a U.S. real property holding corporation only if the fair market value of its U.S. real property interests equals or exceeds 50% of the sum of the fair market value of its worldwide real property interests plus its other assets used or held for use in a trade or business. Although there can be no assurance, we do not believe that we are, or have been, a U.S. real property holding corporation, or that we are likely to become one in the future. No assurance can be provided that our common stock will be regularly traded on an established securities market for purposes of the rules described above.

U.S. Federal Estate Tax

Shares of our common stock that are owned or treated as owned at the time of death by an individual who is not a citizen or resident of the United States, as specifically defined for U.S. federal estate tax purposes, are considered U.S. situs assets and will be included in the individual's gross estate for U.S. federal estate tax purposes. Such shares, therefore, may be subject to U.S. federal estate tax, unless an applicable estate tax or other treaty provides otherwise.

Backup Withholding and Information Reporting

We must report annually to the IRS and to each non-U.S. holder the gross amount of the distributions on our common stock paid to such holder and the tax withheld, if any, with respect to such distributions. Non-U.S. holders may have to comply with specific certification procedures to establish that the holder is not a United States person (as defined in the Code) in order to avoid backup withholding at the applicable rate with respect to dividends on our common stock. Dividends paid to non-U.S. holders subject to withholding of U.S. federal income tax, as described above in "Distributions on our common stock," generally will be exempt from U.S. backup withholding.

Information reporting and backup withholding will generally apply to the proceeds of a disposition of our common stock by a non-U.S. holder effected by or through the U.S. office of any broker, U.S. or foreign, unless the holder certifies its status as a non-U.S. holder and satisfies certain other requirements, or otherwise establishes an exemption. Generally, information reporting and backup withholding will not apply to a payment of disposition proceeds to a non-U.S. holder where the transaction is effected outside the United States through a non-U.S. office of a broker. However, for information reporting purposes, dispositions effected through a non-U.S. office of a broker with substantial U.S. ownership or operations generally will be treated in a manner similar to dispositions effected through a U.S. office of a broker. Non-U.S. holders should consult their own tax advisors regarding the application of the information reporting and backup withholding rules to them.

Copies of information returns may be made available to the tax authorities of the country in which the non-U.S. holder resides or is incorporated under the provisions of a specific treaty or agreement.

Backup withholding is not an additional tax. Any amounts withheld under the backup withholding rules from a payment to a non-U.S. holder can be refunded or credited against the non-U.S. holder's U.S. federal income tax liability, if any, provided that an appropriate claim is timely filed with the IRS.

Withholding and Information Reporting Requirements—FATCA

The Foreign Account Tax Compliance Act, or FATCA, generally imposes a U.S. federal withholding tax at a rate of 30% on payments of dividends on, or gross proceeds from the sale or other disposition of, our common stock paid to a foreign entity unless (i) if the foreign entity is a "foreign financial institution," such foreign entity undertakes certain due diligence, reporting, withholding, and certification obligations, (ii) if the foreign entity is not a "foreign financial institution," such foreign entity identifies certain of its U.S. investors, if any, or (iii) the foreign entity is otherwise exempt under FATCA. Under applicable U.S. Treasury regulations, withholding under FATCA will only apply (1) to payments of dividends on our common stock made after June 30, 2014, and (2) to payments of gross proceeds from a sale or other disposition of our common stock made after December 31, 2016. Under certain circumstances, a non-U.S. holder may be eligible for refunds or credits of the tax. An intergovernmental agreement between the United States and an applicable foreign country may modify the requirements described in this paragraph. Non-U.S. holders should consult their own tax advisors regarding the possible implications of this legislation on their investment in our common stock and the entities through which they hold our common stock, including, without limitation, the process and deadlines for meeting the applicable requirements to prevent the imposition of the 30% withholding tax under FATCA.

Underwriting

We and the underwriters named below have entered into an underwriting agreement with respect to the shares being offered. Subject to certain conditions, each underwriter has severally agreed to purchase the number of shares indicated in the following table. BMO Capital Markets Corp., Stifel, Nicolaus & Company, Incorporated and William Blair & Company, L.L.C. are the representatives of the underwriters.

Underwriters	Number of Shares
BMO Capital Markets Corp.	2,430,000
Stifel, Nicolaus & Company, Incorporated	2,430,000
William Blair & Company, L.L.C.	1,166,400
Janney Montgomery Scott LLC	453,600
Total	6,480,000

The underwriters are committed to take and pay for all of the shares being offered, if any are taken, other than the shares covered by the option described below unless and until that option is exercised. If an underwriter fails or refuses to purchase any of its committed shares, the purchase commitments of the non-defaulting underwriters may be increased or the offering may be terminated.

The underwriters have an option to buy up to an additional 972,000 shares from us to cover sales by the underwriters of a greater number of shares than the total number set forth in the table above. They may exercise this option for 30 days. If any shares are purchased pursuant to this option, the underwriters will severally purchase shares in approximately the same proportion as set forth in the table above, and the underwriters will offer the additional shares on the same terms as those on which the shares are being offered.

The underwriters propose to offer the shares of our common stock directly to the public at the initial public offering price set forth on the cover of this prospectus and to certain dealers at such offering price less a concession not in excess of $0.63 per share. After the initial public offering of the shares, the offering price and the selling concession may be changed by the underwriters.

Certain of our existing stockholders and their affiliates, including our existing principal stockholders, have agreed to purchase an aggregate of 1,105,000 shares of our common stock in this offering at the initial public offering price. In addition, Novo A/S has agreed to purchase 1,000,000 shares of our common stock in this offering at the initial public offering price.

The following table shows the per share and total underwriting discounts and commissions to be paid by us to the underwriters assuming both no exercise and full exercise of the underwriters' option to purchase additional shares.

	No Exercise	Full Exercise
Per Share	$ 1.05	$ 1.05
Total	$6,804,000	$7,824,600

We estimate that the total expenses of the offering, including registration, filing and listing fees, printing fees and legal and accounting expenses, but excluding underwriting discounts and commissions, will be approximately $2.7 million, all of which will be paid by us. We have agreed to reimburse the underwriters for certain of their expenses, in an amount up to $25,000, incurred in connection with the clearance of this offering with the Financial Industry Regulatory Authority, Inc.

In addition, we have previously agreed to pay a fee upon the closing of this offering to a financial advisor equal to the greater of $0.5 million and 1% of the gross proceeds of this offering in connection with strategic and financial advisory services unrelated to this offering.

We and our officers and directors and the holders of substantially all of our capital stock and options have agreed with the underwriters that, for a period of 180 days after the date of this prospectus, subject to certain exceptions, we and they will not (1) offer, sell, pledge, contract to sell, purchase any option to sell, grant any option for the purchase of, lend, or otherwise dispose of, or require us to file with the SEC a registration statement under the Securities Act to register, any shares of common stock or any securities convertible into, exercisable for or exchangeable for common stock of which the undersigned is now, or may in the future become, the beneficial owner (within the meaning of Rule 13d-3 under the Exchange Act), or (2) enter into any swap or other derivative transaction that transfers to another, in whole or in part, any of the economic benefits or risks of ownership of common stock, whether any such transaction described in clause (1) or (2) above is to be settled by delivery of common stock or other securities, in cash or otherwise, or publicly disclose the intention to enter into any transaction described in clause (1) or (2) above, except with the prior written consent of BMO Capital Markets Corp. and Stifel, Nicolaus & Company, Incorporated; provided that BMO Capital Markets Corp. and Stifel, Nicolaus & Company, Incorporated, on behalf of the underwriters, have agreed to notify us at least three business days before the effective date of any release or waiver granted to one of our officers or directors, and we have agreed to announce the impending release or waiver by issuing a press release through a major news service at least two business days before the effective date of the release or waiver.

The restrictions described in this paragraph do not apply to the following, subject to certain limitations set forth in the lock-up agreements:

- transfers of securities as a bona fide gift;
- the surrender or forfeiture of securities to us to satisfy tax withholding obligations upon exercise or vesting of stock options or equity awards;
- transfers of securities to any immediate family member or any trust for the direct or indirect benefit of the lock-up signatory or an immediate family member of the lock-up signatory or to any corporation, partnership, limited liability company or other entity all of the beneficial ownership interests of which are held by the lock-up signatory and/or one or more immediate family members of the lock-up signatory in a transaction not involving a disposition for value;
- transfers of securities upon death of the lock-up signatory by will or intestate succession;
- if the lock-up signatory is a corporation, partnership, limited liability company, trust or other business entity, transfers of securities to one or more affiliates of the lock-up signatory or transfers or distributions of securities to the partners, members or stockholders or other equityholders of the lock-up signatory or, in the case of a corporation, transfers of securities to a wholly-owned subsidiary of the lock-up signatory; and
- the entry into any trading plan established pursuant to Rule 10b5-1 under the Exchange Act for the transfer of shares of securities, provided that such plan does not provide for any sales or other dispositions of securities during the lock-up period and no public announcement or filing under the Exchange Act is made by us or on our behalf regarding the establishment of such plan.

The lock-up agreements apply to any shares purchased in this offering by certain of our existing stockholders and their affiliates, including our existing principal stockholders, who have agreed to purchase an aggregate of 1,105,000 shares of our common stock in this offering at the initial public offering price. Novo A/S, which has agreed to purchase 1,000,000 shares of our common stock in this offering at the initial public offering price, is not a party to a lock-up agreement.

See “Shares Eligible for Future Sale” for a discussion of certain transfer restrictions.

Prior to the offering, there has been no public market for our common stock. The initial public offering price was negotiated among us and the representatives. Among the factors considered in determining the initial public offering price of the shares, in addition to prevailing market conditions, were our historical performance, estimates of our business potential and earnings prospects, an assessment of our management and the consideration of the above factors in relation to market valuation of companies in related businesses.

Our common stock has been approved for listing on The NASDAQ Global Market under the symbol “TKAI.”

In connection with the offering, the underwriters may purchase and sell shares of our common stock in the open market. These transactions may include short sales, stabilizing transactions and purchases to cover positions created by short sales. Short sales involve the sale by the underwriters of a greater number of shares than they are required to purchase in the offering, and a short position represents the amount of such sales that have not been covered by subsequent purchases. A "covered short position" is a short position that is not greater than the amount of additional shares for which the underwriters' option described above may be exercised. The underwriters may cover any covered short position by either exercising their option to purchase additional shares or purchasing shares in the open market. In determining the source of shares to cover the covered short position, the underwriters will consider, among other things, the price of shares available for purchase in the open market as compared to the price at which they may purchase additional shares pursuant to the option described above. "Naked" short sales are any short sales that create a short position greater than the amount of additional shares for which the option described above may be exercised. The underwriters must cover any such naked short position by purchasing shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market after pricing that could adversely affect investors who purchase in the offering. Stabilizing transactions consist of various bids for or purchases of common stock made by the underwriters in the open market prior to the completion of the offering.

The underwriters may also impose a penalty bid. This occurs when a particular underwriter repays to the underwriters a portion of the underwriting discount received by it because the representatives have repurchased shares sold by or for the account of such underwriter in stabilizing or short covering transactions.

Purchases to cover a short position and stabilizing transactions, as well as other purchases by the underwriters for their own accounts, may have the effect of preventing or retarding a decline in the market price of our stock, and together with the imposition of the penalty bid, may stabilize, maintain or otherwise affect the market price of the common stock. As a result, the price of our common stock may be higher than the price that otherwise might exist in the open market. The underwriters are not required to engage in these activities and may end any of these activities at any time. These transactions may be effected on The NASDAQ Global Market, in the over-the-counter market or otherwise.

In connection with this offering, the underwriters may engage in passive market making transactions in the common stock on The NASDAQ Global Market in accordance with Rule 103 of Regulation M under the Exchange Act during a period before the commencement of offers or sales of common stock and extending through the completion of distribution. A passive market maker must display its bid at a price not in excess of the highest independent bid of that security. However, if all independent bids are lowered below the passive market maker's bid, that bid must then be lowered when specified purchase limits are exceeded. Passive market making may cause the price of our common stock to be higher than the price that otherwise would exist in the open market in the absence of those transactions. The underwriters are not required to engage in passive market making and may end passive market making activities at any time.

The underwriters do not expect sales to discretionary accounts to exceed five percent of the total number of shares offered.

We have agreed to indemnify the several underwriters against certain liabilities, including liabilities under the Securities Act and to contribute to payments that the underwriters may be required to make for these liabilities.

A prospectus in electronic format may be made available on websites maintained by one or more underwriters, or selling group members, if any, participating in this offering. The representatives may agree to allocate a number of shares of our common stock to underwriters for sale to their online brokerage account holders. Internet distributions will be allocated by the representatives to underwriters that may make Internet distributions on the same basis as other allocations.

The underwriters and their respective affiliates are full service financial institutions engaged in various activities, which may include sales and trading, commercial and investment banking, advisory, investment

management, investment research, principal investment, hedging, market making, brokerage and other financial and non-financial activities and services. Certain of the underwriters and their respective affiliates have provided, and may in the future provide, a variety of these services to the issuer and to persons and entities with relationships with the issuer, for which they received or will receive customary fees and expenses.

In the ordinary course of their various business activities, the underwriters and their respective affiliates, officers, directors and employees may purchase, sell or hold a broad array of investments and actively trade securities, derivatives, loans, commodities, currencies, credit default swaps and other financial instruments for their own account and for the accounts of their customers, and such investment and trading activities may involve or relate to assets, securities and/or instruments of the issuer (directly, as collateral securing other obligations or otherwise) and/or persons and entities with relationships with the issuer. The underwriters and their respective affiliates may also communicate independent investment recommendations, market color or trading ideas and/or publish or express independent research views in respect of such assets, securities or instruments and may at any time hold, or recommend to clients that they should acquire, long and/or short positions in such assets, securities and instruments.

Selling Restrictions

European Economic Area

In relation to each Member State of the European Economic Area which has implemented the Prospectus Directive (each, a "Relevant Member State") an offer to the public of any shares of our common stock may not be made in that Relevant Member State, except that an offer to the public in that Relevant Member State of any shares of our common stock may be made at any time under the following exemptions under the Prospectus Directive, if they have been implemented in that Relevant Member State:

(a) to any legal entity which is a qualified investor as defined in the Prospectus Directive;

(b) to fewer than 100 or, if the Relevant Member State has implemented the relevant provision of the 2010 PD Amending Directive, 150, natural or legal persons (other than qualified investors as defined in the Prospectus Directive), as permitted under the Prospectus Directive, subject to obtaining the prior consent of the representatives for any such offer; or

(c) in any other circumstances falling within Article 3(2) of the Prospectus Directive, provided that no such offer of shares of our common stock shall result in a requirement for the publication by us or any underwriter of a prospectus pursuant to Article 3 of the Prospectus Directive.

For the purposes of this provision, the expression an "offer to the public" in relation to any shares of our common stock in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and any shares of our common stock to be offered so as to enable an investor to decide to purchase any shares of our common stock, as the same may be varied in that Member State by any measure implementing the Prospectus Directive in that Member State, the expression "Prospectus Directive" means Directive 2003/71/EC (and amendments thereto, including the 2010 PD Amending Directive, to the extent implemented in the Relevant Member State), and includes any relevant implementing measure in the Relevant Member State, and the expression "2010 PD Amending Directive" means Directive 2010/73/EU.

United Kingdom

Each underwriter has represented and agreed that:

(a) it has only communicated or caused to be communicated and will only communicate or cause to be communicated an invitation or inducement to engage in investment activity (within the meaning of Section 21 of the Financial Services and Markets Act of 2000, or the FSMA) received by it in connection with the issue or sale of the shares of our common stock in circumstances in which Section 21(1) of the FSMA does not apply to us; and

(b) it has complied and will comply with all applicable provisions of the FSMA with respect to anything done by it in relation to the shares of our common stock in, from or otherwise involving the United Kingdom.

Legal Matters

The validity of the shares of common stock being offered will be passed upon for us by Wilmer Cutler Pickering Hale and Dorr LLP, Boston, Massachusetts. The underwriters are represented by Goodwin Procter LLP, Boston, Massachusetts, in connection with certain legal matters related to this offering.

Experts

The financial statements as of December 31, 2012 and 2013 and for each of the two years in the period ended December 31, 2013 and, cumulatively, for the period from March 26, 2004 (date of inception) to December 31, 2013 included in this prospectus have been so included in reliance on the report of PricewaterhouseCoopers LLP, an independent registered public accounting firm, given on the authority of said firm as experts in auditing and accounting.

Where You Can Find More Information

We have filed with the SEC a registration statement on Form S-1 under the Securities Act with respect to the shares of common stock to be sold in this offering. This prospectus, which constitutes part of the registration statement, does not include all of the information contained in the registration statement and the exhibits, schedules and amendments to the registration statement. Some items are omitted in accordance with the rules and regulations of the SEC. For further information with respect to us and our common stock, we refer you to the registration statement and to the exhibits and schedules to the registration statement. Statements contained in this prospectus about the contents of any contract or any other document filed as an exhibit are not necessarily complete, and in each instance, we refer you to the copy of the contract or other documents filed as an exhibit to the registration statement. Each of these statements is qualified in all respects by this reference.

You may read and copy the registration statement of which this prospectus is a part at the SEC's public reference room, which is located at 100 F Street, N.E., Room 1580, Washington, D.C. 20549. You can request copies of the registration statement by writing to the SEC and paying a fee for the copying cost. Please call the SEC at 1-800-SEC-0330 for more information about the operation of the SEC's public reference room. In addition, the SEC maintains an Internet website, which is located at www.sec.gov, that contains reports, proxy and information statements and other information regarding issuers that file electronically with the SEC. You may access the registration statement of which this prospectus is a part at the SEC's Internet website.

Upon the closing of this offering, we will be subject to the informational and periodic reporting requirements of the Exchange Act. We will fulfill our obligations with respect to such requirements by filing periodic reports and other information with the SEC. We intend to furnish our stockholders with annual reports containing consolidated financial statements certified by an independent registered public accounting firm. We also maintain a website at www.tokaipharma.com. Our website is not a part of this prospectus.

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Report of Independent Registered Public Accounting Firm

To the Board of Directors and Stockholders of
Tokai Pharmaceuticals, Inc.

In our opinion, the accompanying consolidated balance sheets and the related consolidated statements of operations and comprehensive loss, of redeemable convertible preferred stock and stockholders' deficit and of cash flows present fairly, in all material respects, the financial position of Tokai Pharmaceuticals, Inc. and its subsidiary (a development stage company) at December 31, 2013 and 2012, and the results of their operations and their cash flows for the years then ended and, cumulatively, for the period from March 26, 2004 (date of inception) to December 31, 2013 in conformity with accounting principles generally accepted in the United States of America. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits. We conducted our audits of these statements in accordance with the standards of the Public Company Oversight Board (United States), which require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

/s/ PricewaterhouseCoopers LLP

Boston, Massachusetts
May 2, 2014, except for the last paragraph of Note 16, as to which the date is August 29, 2014

Tokai Pharmaceuticals, Inc.
(A Development Stage Company)

CONSOLIDATED BALANCE SHEETS
(In thousands, except share and per share data)

	December 31,		June 30,	Pro Forma June 30,
	2012	2013	2014	2014
			(unaudited)	
Assets				
Current assets:				
Cash and cash equivalents	$ 11,691	$ 31,753	$ 21,150	$ 21,150
Prepaid expenses and other current assets	235	425	589	589
Total current assets	11,926	32,178	21,739	21,739
Property and equipment, net	16	29	36	36
Deferred offering costs	—	30	1,524	1,524
Restricted cash	20	50	50	50
Other Assets	—	—	71	71
Total assets	$ 11,962	$ 32,287	$ 23,420	$ 23,420
Liabilities, Redeemable Convertible Preferred Stock and Stockholders' Equity (Deficit)				
Current liabilities:				
Accounts payable	$ 764	$ 5	$ 1,216	$ 1,216
Accrued expenses	1,254	2,204	2,472	2,472
Total current liabilities	2,018	2,209	3,688	3,688
Total liabilities	2,018	2,209	3,688	3,688
Commitments and contingencies (Note 11)				
Redeemable convertible preferred stock (Series A, B-1, B-2, C, D-1, D-2, D-3 and E), $0.001 par value; 98,693,763 and 155,586,141 shares authorized at December 31, 2012 and 2013, respectively, and 155,586,141 shares authorized at June 30, 2014 (unaudited); 98,693,750 and 155,586,141 shares issued and outstanding at December 31, 2012 and 2013, respectively, and 155,586,141 shares issued and outstanding at June 30, 2014 (unaudited); aggregate liquidation preference of $83,528 at December 31, 2013 and June 30, 2014 (unaudited); no shares issued or outstanding pro forma at June 30, 2014 (unaudited)	49,845	85,345	85,345	—
Stockholders' equity (deficit):				
Common stock, $0.001 par value; 112,182,244 and 173,018,331 shares authorized at December 31, 2012 and 2013, respectively, and 178,408,438 shares authorized at June 30, 2014 (unaudited); 464,633 and 493,292 shares issued and outstanding at December 31, 2012 and 2013, respectively, and 501,569 shares issued and outstanding at June 30, 2014 (unaudited); 15,361,742 shares issued and outstanding pro forma at June 30, 2014 (unaudited)	—	—	1	15
Additional paid-in capital	7,429	7,788	8,139	93,470
Deficit accumulated during the development stage	(47,330)	(63,055)	(73,753)	(73,753)
Total stockholders' equity (deficit)	(39,901)	(55,267)	(65,613)	19,732
Total liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)	$ 11,962	$ 32,287	$ 23,420	$ 23,420

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

Tokai Pharmaceuticals, Inc.
(A Development Stage Company)

CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(In thousands, except share and per share data)

	Year Ended December 31,		Six Months Ended June 30,		Cumulative Period From Inception (March 26, 2004) to December 31, 2013	Cumulative Period From Inception (March 26, 2004) to June 30, 2014
	2012	2013	2013	2014		
			(unaudited)			(unaudited)
Revenue	$ —	$ —	$ —	$ —	$ —	$ —
Operating expenses:						
Research and development	7,370	12,201	5,148	7,948	49,366	57,314
General and administrative	2,279	3,548	1,687	2,829	13,457	16,286
Total operating expenses	9,649	15,749	6,835	10,777	62,823	73,600
Loss from operations	(9,649)	(15,749)	(6,835)	(10,777)	(62,823)	(73,600)
Other income (expense):						
Interest income	—	—	—	—	216	216
Interest expense	—	—	—	—	(302)	(302)
Other income (expense), net	—	24	—	79	263	342
Total other income, net	—	24	—	79	177	256
Net loss and comprehensive loss	(9,649)	(15,725)	(6,835)	(10,698)	(62,646)	(73,344)
Accretion of redeemable convertible preferred stock to redemption value	(34)	(94)	(79)	—	(3,359)	(3,359)
Accrual of dividend on redeemable convertible preferred stock	—	—	—	—	(347)	(347)
Modifications of redeemable convertible preferred stock	—	—	—	—	9,925	9,925
Net loss attributable to common stockholders	$ (9,683)	$ (15,819)	$ (6,914)	$ (10,698)	$ (56,427)	$ (67,125)
Net loss per share attributable to common stockholders, basic and diluted	$ (31.09)	$ (38.02)	$ (20.49)	$ (21.48)		
Weighted average common shares outstanding, basic and diluted	311,474	416,037	337,495	498,107		
Pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)		$ (1.29)		$ (0.70)		
Pro forma weighted average common shares outstanding, basic and diluted (unaudited)		12,230,206		15,358,280		

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

Tokai Pharmaceuticals, Inc.
(A Development Stage Company)

CONSOLIDATED STATEMENTS OF REDEEMABLE CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' DEFICIT

(In thousands, except share data)

	Series A, B-1, B-2, C, D-1, D-2, D-3 and E Redeemable Convertible Preferred Stock		Common Stock		Additional Paid-in Capital	Stockholder Receivable	Deficit Accumulated During the Development Stage	Total Stockholders' Deficit
	Shares	Amount	Shares	Par Value				
Balances at Inception (March 26, 2004)	—	$ —	—	$ —	$ —	$ —	$ —	$ —
Issuance of common stock	—	—	403,483	—	58	—	—	58
Issuance of common stock upon exercise of stock options	—	—	3,070	—	1	—	—	1
Repurchase and forfeiture of unvested restricted stock	—	—	(99,736)	—	(33)	—	—	(33)
Issuance of Series A, Series B-2 and Series D-1, D-2 and D-3 redeemable convertible preferred stock, net of issuance costs of $693	47,119,526	30,272	—	—	—	—	—	—
Conversion of promissory notes and accrued interest into Series B-1 and B-2 and Series C redeemable convertible preferred stock	16,878,182	6,970	—	—	—	—	—	—
Modification of Series A redeemable convertible preferred stock in 2007	—	45	—	—	—	—	(45)	(45)
Modification of Series A and Series B-1 and B-2 redeemable convertible preferred stock in 2009	—	(9,970)	—	—	9,970	—	—	9,970
Accrual of Series A preferred stock cumulative dividend	—	347	—	—	—	—	(347)	(347)
Accretion of Series A, Series B-1 and B-2 and Series D-1 and D-3 redeemable convertible preferred stock to redemption value	—	3,231	—	—	(3,214)	—	(17)	(3,231)
Cancellation of warrants	—	—	—	—	125	—	—	125
Forgiveness of accrued interest on convertible promissory note in 2009	—	—	—	—	94	—	—	94
Loans to stockholders	—	—	—	—	—	(350)	—	(350)
Collection of loans to stockholders	—	—	—	—	—	130	—	130
Reserve for loan to stockholder	—	—	—	—	—	220	—	220
Stock-based compensation expense	—	—	—	—	248	—	—	248
Net loss	—	—	—	—	—	—	(37,272)	(37,272)
Balances at December 31, 2011	63,997,708	30,895	306,817	—	7,249	—	(37,681)	(30,432)
Issuance of Series D-3 redeemable convertible preferred stock, net of issuance costs of $34	34,696,042	18,916	—	—	—	—	—	—
Issuance of common stock upon exercise of stock options	—	—	2,843	—	4	—	—	4
Issuance of common stock	—	—	154,973	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	210	—	—	210
Accretion of Series D-3 redeemable convertible preferred stock to redemption value	—	34	—	—	(34)	—	—	(34)
Net loss	—	—	—	—	—	—	(9,649)	(9,649)
Balances at December 31, 2012	98,693,750	49,845	464,633	—	7,429	—	(47,330)	(39,901)
Issuance of Series E redeemable convertible preferred stock, net of issuance costs of $94	56,892,391	35,406	—	—	—	—	—	—
Issuance of common stock upon exercise of stock options	—	—	157,804	—	215	—	—	215
Repurchase and forfeiture of unvested restricted stock	—	—	(129,145)	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	238	—	—	238
Accretion of Series E redeemable convertible preferred stock to redemption value	—	94	—	—	(94)	—	—	(94)
Net loss	—	—	—	—	—	—	(15,725)	(15,725)
Balances at December 31, 2013	155,586,141	85,345	493,292	—	7,788	—	(63,055)	(55,267)
Issuance of common stock upon exercise of stock options	—	—	8,277	1	11	—	—	12
Stock-based compensation expense	—	—	—	—	340	—	—	340
Net loss	—	—	—	—	—	—	(10,698)	(10,698)
Balances at June 30, 2014 (unaudited)	155,586,141	$ 85,345	501,569	$ 1	$ 8,139	$ —	$ (73,753)	$ (65,613)

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

Tokai Pharmaceuticals, Inc.
(A Development Stage Company)

CONSOLIDATED STATEMENTS OF CASH FLOWS
(In thousands)

	Year Ended December 31,		Six Months Ended June 30,		Cumulative Period From Inception (March 26, 2004) to December 31, 2013	Cumulative Period From Inception (March 26, 2004) to June 30, 2014
	2012	2013	2013	2014		
			(unaudited)			(unaudited)
Cash flows from operating activities:						
Net loss	$ (9,649)	$ (15,725)	$ (6,835)	$ (10,698)	$ (62,646)	$ (73,344)
Adjustments to reconcile net loss to net cash used in operating activities:						
Stock-based compensation expense	210	238	90	340	696	1,036
Non-cash interest expense	—	—	—	—	299	299
Depreciation expense	9	10	3	11	65	76
Reserve for (release of reserve for) loan to stockholder	—	—	—	(79)	220	141
Loss on disposal of property and equipment	—	—	—	—	5	5
Changes in operating assets and liabilities:						
Prepaid expenses and other current assets	139	(190)	37	(164)	(425)	(589)
Accounts payable	(119)	(759)	621	507	5	512
Accrued expenses	77	950	410	(43)	2,204	2,161
Other assets	—	—	—	(71)	—	(71)
Net cash used in operating activities	(9,333)	(15,476)	(5,674)	(10,197)	(59,577)	(69,774)
Cash flows from investing activities:						
Purchases of property and equipment	(8)	(23)	(3)	(18)	(99)	(117)
Change in restricted cash	—	(30)	(30)	—	(50)	(50)
Net cash used in investing activities	(8)	(53)	(33)	(18)	(149)	(167)
Cash flows from financing activities:						
Proceeds from issuance of redeemable convertible preferred stock, net of issuance costs	18,775	35,406	19,921	—	84,594	84,594
Proceeds from issuance of convertible promissory notes, net of issuance costs	—	—	—	—	6,890	6,890
Proceeds from issuance of common stock	—	—	—	—	58	58
Proceeds from exercise of common stock options	4	215	215	12	220	232
Loans made to stockholders	—	—	—	—	(350)	(350)
Collection of loans made to stockholders	—	—	—	79	130	209
Repurchase of common stock at cost	—	—	—	—	(33)	(33)
Payments of initial public offering costs	—	(30)	—	(479)	(30)	(509)
Net cash provided by (used in) financing activities	18,779	35,591	20,136	(388)	91,479	91,091
Net increase (decrease) in cash and cash equivalents	9,438	20,062	14,429	(10,603)	31,753	21,150
Cash and cash equivalents at beginning of period	2,253	11,691	11,691	31,753	—	—
Cash and cash equivalents at end of period	$ 11,691	$ 31,753	$ 26,120	$ 21,150	$ 31,753	$ 21,150
Supplemental disclosure cash flow information:						
Cash paid for interest	$ —	$ —	$ —	$ —	$ 3	$ 3
Supplemental disclosure of non-cash investing and financing activities:						
Accretion of redeemable convertible preferred stock to redemption value	$ 34	$ 94	$ 79	$ —	$ 3,359	$ 3,359
Accrual of dividend on redeemable convertible preferred stock	$ —	$ —	$ —	$ —	$ 347	$ 347
Modifications of redeemable convertible preferred stock	$ —	$ —	$ —	$ —	$ 9,925	$ 9,925
Issuance of warrant in connection with convertible notes	$ —	$ —	$ —	$ —	$ 125	$ 125
Forgiveness of interest	$ —	$ —	$ —	$ —	$ 94	$ 94
Warrant cancellation	$ —	$ —	$ —	$ —	$ 125	$ 125
Conversion of convertible promissory notes and accrued interest and advance from stockholder to shares of redeemable convertible preferred stock	$ 141	$ —	$ —	$ —	$ 6,970	$ 6,970
Deferred offering costs included in accounts payable and accrued expenses	$ —	$ —	$ —	$ 1,015	$ —	$ 1,015

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

Tokai Pharmaceuticals, Inc.
(A Development Stage Company)

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Amounts in thousands, except share and per share data)

1. Nature of the Business and Basis of Presentation

Tokai Pharmaceuticals, Inc. (the "Company") (a development stage 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. Accordingly, the Company is considered to be in the development stage.

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.

Galeterone is in the development stage. 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 Company's consolidated financial statements have been prepared on the basis of continuity of operations, realization of assets and the satisfaction of liabilities in the ordinary course of business. The Company has experienced negative cash flows and has a deficit accumulated during the development stage of $63,055 as of December 31, 2013. The Company expects that its existing cash and cash equivalents as of December 31, 2013 will enable the Company to fund its operating expenses and capital expenditure requirements through at least December 31, 2014. In addition, the Company expects that its cash and cash equivalents as of June 30, 2014 (unaudited) will be sufficient to fund its operating expenses and capital expenditure requirements through at least June 30, 2015 (unaudited). The future viability of the Company is largely dependent on its ability to generate cash from operating activities or to raise additional capital to finance its operations. The Company's failure to raise capital as and when needed could have a negative impact on its financial condition and ability to pursue its business strategies.

The Company is seeking to complete an initial public offering of its common stock. If the gross proceeds from the initial public offering are at least $40,000, subject to a minimum per share price for the shares of common stock sold in the initial public offering, the Company's outstanding redeemable convertible preferred stock will automatically convert into shares of common stock upon the completion of the Company's initial public offering.

In the event the Company does not complete an initial public offering, the Company expects to seek additional funding through a combination of equity offerings, debt financings, marketing and distribution

Tokai Pharmaceuticals, Inc.
(A Development Stage Company)

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Amounts in thousands, except share and per share data)

arrangements and other collaborations, strategic alliances and licensing arrangements. Additional funding may not be available to the Company on acceptable terms or at all. The terms of any financing may adversely affect the holdings or the rights of the Company's stockholders. If the Company is unable to obtain sufficient funding, it may have to curtail the development of galeterone, reduce or delay its development program or one or more of its other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase its expenditures and undertake development or commercialization activities at its own expense, which could adversely affect its business prospects.

The accompanying consolidated financial statements and footnotes include Diotima Pharmaceuticals, Inc. ("Diotima"), a variable interest entity in which the Company has a variable financial interest and is the primary beneficiary but has no ownership interest. In September 2010, the Company formed and incorporated Diotima, which has since operated as a stand-alone company with limited activity. In November 2010, the Company contributed certain assets to Diotima in exchange for all of the issued and outstanding shares of common and preferred stock of Diotima (see Note 8). All significant intercompany balances and transactions between the Company and Diotima have been eliminated in consolidation.

These consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America ("GAAP").

At December 31, 2013 and June 30, 2014, the Company is considered a development stage enterprise. Until planned principal operations have commenced and significant revenue is generated, financial statements prepared in accordance with GAAP are required to report cumulative statements of operations and comprehensive loss, stockholders' equity (deficit) and cash flows from the date of inception.

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.

Unaudited Interim Financial Information

The accompanying consolidated balance sheet as of June 30, 2014, the consolidated statements of operations and comprehensive loss and consolidated statements of cash flows for the six months ended June 30, 2013 and 2014 and for the cumulative period from inception (March 26, 2004) to June 30, 2014, and the consolidated statement of redeemable convertible preferred stock and stockholders' deficit for the six months ended June 30, 2014 are unaudited. The unaudited interim consolidated financial statements have been prepared on the same basis as the audited annual consolidated financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary for the fair statement of the Company's financial position as of June 30, 2014 and the results of its operations and its cash flows for the six

Tokai Pharmaceuticals, Inc.
(A Development Stage Company)

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Amounts in thousands, except share and per share data)

months ended June 30, 2013 and 2014 and for the cumulative period from inception (March 26, 2004) to June 30, 2014. The financial data and other information disclosed in these notes related to the six months ended June 30, 2013 and 2014 and the cumulative period from inception (March 26, 2004) to June 30, 2014 are unaudited. The results for the six months ended June 30, 2014 are not necessarily indicative of results to be expected for the year ending December 31, 2014, any other interim periods, or any future year or period.

Unaudited Pro Forma Information

The accompanying unaudited pro forma consolidated balance sheet as of June 30, 2014 has been prepared to give effect to the automatic conversion of all outstanding shares of redeemable convertible preferred stock into 14,860,173 shares of common stock as if the Company's proposed initial public offering (see Note 1) had occurred on June 30, 2014. In the accompanying consolidated statements of operations, unaudited pro forma basic and diluted net loss per share attributable to common stockholders for the year ended December 31, 2013 and the six months ended June 30, 2014 have been prepared to give effect to the automatic conversion of all outstanding shares of redeemable convertible preferred stock as if the Company's proposed initial public offering (see Note 1) had occurred on the later of January 1, 2013 or the issuance date of the redeemable convertible preferred stock.

Cash Equivalents

The Company considers all short-term, highly liquid investments with original maturities of ninety days or less at acquisition date 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.

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.

Tokai Pharmaceuticals, Inc.
(A Development Stage Company)

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Amounts in thousands, except share and per share data)

- 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 $1,311 as of December 31, 2012 and 2013 and June 30, 2014 (unaudited) 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 and June 30, 2014 (unaudited), the Company had recorded $30 and $1,524, respectively, of deferred offering costs in the accompanying consolidated balance sheet in contemplation of a probable 2014 equity financing. Should the equity financing no longer be considered probable of being consummated, the deferred offering costs would be expensed immediately as a charge to operating expenses in the consolidated statement of operations. The Company did not record any deferred offering costs as of December 31, 2012.

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.

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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.

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.

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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.

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. There was no difference between net loss and comprehensive loss for the years ended December 31, 2012 and 2013, the six months ended June 30, 2013 and 2014 (unaudited), the cumulative period from inception (March 26, 2004) to December 31, 2013 and the cumulative period from inception (March 26, 2004) to June 30, 2014 (unaudited).

Carrying Value of Redeemable Convertible Preferred Stock

The Company recognizes changes in the redemption values of its outstanding redeemable convertible preferred stock immediately as they occur and adjusts the carrying value of the redeemable convertible preferred stock to equal the redemption value at the end of each reporting period as if the end of each reporting period were the redemption date.

Reductions in the carrying value of each series of redeemable convertible preferred stock are only recorded to the extent that the Company has previously recorded increases in the carrying value of the security.

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Net Income (Loss) Per Share

The Company follows the two-class method when computing net income (loss) per share, as the Company has issued shares that meet 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.

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 shares of common stock outstanding for the period. Diluted net income (loss) attributable to common stockholders is computed by adjusting income (loss) attributable to common stockholders to reallocate undistributed earnings based on the potential impact of dilutive securities, including outstanding stock options and unvested restricted common stock. 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 outstanding for the period, including potential dilutive common shares assuming the dilutive effect of outstanding stock options and unvested restricted common stock.

The Company's redeemable convertible preferred stock contractually entitles the holders of such shares to participate in dividends but does not contractually require the holders of such shares to participate in losses of the Company. Similarly, restricted stock awards granted by the Company entitle the holder of such awards to dividends declared or paid by the board of directors, regardless of whether such awards are unvested, as if such shares were outstanding common shares at the time of the dividend. However, the unvested restricted stock awards are not entitled to share in the residual net assets (deficit) of the Company. Accordingly, in periods in which the Company reports a net loss attributable to common stockholders, diluted net loss per share attributable to common stockholders is the same as basic net loss per 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 years ended December 31, 2012 and 2013 and the six months ended June 30, 2013 and 2014 (unaudited).

Recently Issued and Adopted Accounting Pronouncements

In June 2014, the Financial Accounting Standards Board, (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. These presentation and disclosure requirements will no longer be required for the first annual period beginning after December 15, 2014 for public companies. Early application is permitted for interim and annual periods for which financial statements have not yet been issued or made available for issuance. Effective upon the Company's adoption of this guidance, the Company will no longer disclose inception-to-date information currently included in its consolidated statements of operations and comprehensive loss, of cash flows, and of redeemable convertible preferred stock and stockholders' deficit and the related notes thereto.

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Other accounting standards that have been issued or proposed by the FASB or other standards-setting bodies that do not require adoption until a future date are not expected to have a material impact on the Company's consolidated financial statements upon adoption.

3. Property and Equipment, net

Property and equipment, net consisted of the following as of December 31, 2012 and 2013 and June 30, 2014 (unaudited):

	December 31,		June 30,
	2012	2013	2014
			(unaudited)
Computer equipment	$ 67	$ 72	$ 86
	67	72	86
Less: Accumulated depreciation	(51)	(43)	(50)
	$ 16	$ 29	$ 36

Depreciation expense was $9 and $10 for the years ended December 31, 2012 and 2013, respectively, $3 and $11 for the six months ended June 30, 2013 and 2014 (unaudited), respectively, $65 for the cumulative period from inception (March 26, 2004) through December 31, 2013 and $76 for the cumulative period from inception (March 26, 2004) through June 30, 2014 (unaudited).

4. Accrued Expenses

Accrued expenses consisted of the following as of December 31, 2012 and 2013 and June 30, 2014 (unaudited):

	December 31,		June 30,
	2012	2013	2014
			(unaudited)
Accrued research and development expenses	$ 766	$1,370	$ 1,595
Accrued payroll and related expenses	291	436	320
Accrued professional fees	146	338	511
Accrued other	51	60	46
	$1,254	$2,204	$ 2,472

5. Convertible Promissory Notes

In March 2007, the Company issued a convertible promissory note in the aggregate principal amount of $2,935 (the "Series B Note") to one of its existing stockholders. The Series B Note accrued interest at an annual rate of 6% payable at maturity. In May 2007, the total outstanding principal and accrued interest on the Series B note of $3,050 converted into 798,067 and 80,117 shares of the Company's Series B-1 and B-2 redeemable convertible preferred stock, respectively (see Note 6).

In October 2008 and February 2009, the Company issued $2,000 and $2,000 in aggregate principal amount, respectively, of convertible promissory notes (collectively, the "Series C Notes") to certain existing holders of

the Company's Series B-2 redeemable convertible preferred stock. The Series C Notes accrued interest at an annual rate of 6% and were due at any time on or after April 14, 2010 upon the written demand of the holders of at least 60% of the aggregate principal amount under all Series C Notes then outstanding. The Company incurred financing costs of $45 related to the issuance of the Series C Notes. In connection with the issuance of the Series C Notes, the Company also issued warrants to the holders of the Series C Notes for the purchase of an aggregate of 572,683 shares of Series C redeemable convertible preferred stock at a purchase price of $0.25 per warrant share (the "Series C Warrants"). At the date of issuance, the Series C Warrants were valued using the Black-Scholes option-pricing model, which resulted in a total fair value of $125 at the date of issuance. The Series C Warrants were remeasured at December 31, 2008 using the Black-Scholes option-pricing model and again upon cancellation of the Series C Warrants in May 2009. There was no significant change in the value of the Series C Warrants from October 2008 through May 2009. Issuance costs and the value of the Series C Warrants were recorded initially as deferred financing costs included in other assets on the consolidated balance sheet and amortized to interest expense. The Company recorded $90 of interest expense during 2008 and 2009 related to the amortization of these deferred financing costs.

In May 2009, the aggregate outstanding principal of $4,000 on the Series C Notes were converted into an aggregate of 15,999,998 shares of Series C redeemable convertible preferred stock at a conversion price of $0.25 per share, the outstanding Series C Warrants were cancelled and the accrued interest on the Series C Notes payable was forgiven. As the holders of the Series C Notes were also the Company's majority stockholders, the Company considered the cancellation of the Series C Warrants and forgiveness of interest to represent contributed capital and, accordingly, recorded $125 and $94 for the cancellation of the Series C Warrants and forgiveness of interest, respectively, as additional paid-in capital.

6. Redeemable Convertible Preferred Stock

As of December 31, 2013 and June 30, 2014 (unaudited), the Company's certificate of incorporation, as amended and restated (the "Certificate of Incorporation"), authorizes the Company to issue 155,586,141 shares of preferred stock, $0.001 par value per share.

The Company has issued Series A, Series B-1, Series B-2, Series C, Series D-1, Series D-2 and Series D-3, and Series E redeemable convertible preferred stock (collectively, the "Redeemable Preferred Stock"). The Series C, D and E redeemable convertible preferred stock are collectively referred to as the "Senior Preferred Stock." The Redeemable Preferred Stock is classified outside of stockholders' equity (deficit) because the shares contain redemption features that are not solely within the control of the Company.

During 2004, 2005 and 2007, the Company issued a total of 4,500,000 shares of Series A redeemable convertible preferred stock at an issuance price equal to $0.50 per share and received aggregate gross proceeds of $2,250. In connection with these preferred stock financings, the Company paid total issuance costs of $20. Holders of the Series A redeemable convertible preferred stock were initially entitled to cumulative dividends of $0.04 per share.

During 2007, the outstanding principal and accrued interest of $3,050 on the Series B Note was converted into 798,067 shares of Series B-1 redeemable convertible preferred stock at $3.383176 per share and 80,117 shares of Series B-2 redeemable convertible preferred stock at $4.365388 per share, respectively (see Note 5). The Company issued an additional 1,423,702 shares of Series B-2 redeemable convertible preferred stock in 2007 for aggregate gross proceeds of $6,215. The Company incurred issuance costs of $240 relating to the sale and issuance of these shares of Series B-2 redeemable convertible preferred stock.

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In May 2007, in connection with the authorization and issuance of the Series B-1 and Series B-2 redeemable convertible preferred stock, the rights and preferences of the Series A redeemable convertible preferred stock were modified such that the holders of Series A were no longer entitled to cumulative dividends but instead became entitled to non-cumulative dividends when and if declared by the Company's board of directors. In addition, the maximum participation amount of the Series A redeemable convertible preferred stock upon liquidation was increased from 200% to 300% of the Series A liquidation preference. The modification of these rights and preferences resulted in a transfer of value between common and preferred stockholders and was treated as a deemed dividend to the Company's preferred stockholders. Accordingly, the Company recorded the deemed dividend of $45, representing the decrease in fair value of the Company's common stock as a result of the modification, by increasing the carrying value of the Series A redeemable convertible preferred stock by $45 and increasing accumulated deficit, as the Company had no additional paid-in capital.

As the Company immediately accretes the carrying value of its redeemable convertible preferred stock to redemption value at the end of each reporting period as if the end of the reporting period were the redemption date, as of December 31, 2007, the Company reduced the carrying value of the Series A redeemable convertible preferred stock by $347, which represented the amount of cumulative dividends recorded through the modification date to which holders of Series A redeemable convertible preferred stock were no longer entitled upon liquidation or redemption as a result of the modification.

In May 2009, the aggregate outstanding principal of $4,000 on the Series C Notes was converted into an aggregate of 15,999,998 shares of Series C redeemable convertible preferred stock at a conversion price of $0.25 per share, and the accrued interest on the Series C Notes payable was forgiven (see Note 5). In 2009, the Company issued 29,294,828 shares of Series D redeemable convertible preferred stock at $0.54617142 per share to new and existing investors for gross proceeds of $16,000. The Company incurred issuance costs of $209 in connection with the sale and issuance of these shares of Series D redeemable convertible preferred stock.

In connection with the issuance of the Series C and Series D redeemable convertible preferred stock in 2009, the rights and preferences of the Series A, Series B-1 and Series B-2 redeemable convertible preferred stock outstanding were modified such that the liquidation and redemption price of the Series A was increased from $0.50 per share to $0.70 per share, the liquidation and redemption price of the Series B-1 was decreased from $3.38 to $0.70 per share, and the liquidation and redemption price of the Series B-2 was decreased from $4.365388 to $0.70 per share. In addition, certain voting rights were modified and the maximum participation amount for the holders of the Series A, Series B-1 and Series B-2 redeemable convertible preferred stock was eliminated. The holders of the Series A, Series B-1 and Series B-2 redeemable convertible preferred stock also waived adjustment to the conversion prices of the Series A and Series B redeemable convertible preferred stock that should have occurred as a result of anti-dilution provisions due to the issuance price of the Series C redeemable convertible preferred stock.

Due to the significant change in the fair value of the Series A, Series B-1 and Series B-2 redeemable convertible preferred stock as a result of the modification, such changes were, for accounting purposes only, treated by the Company as extinguishments and reissuances of these securities. Accordingly, the Company recorded an adjustment to remove the carrying value of the Series A, Series B-1 and Series B-2 redeemable convertible preferred stock with a corresponding adjustment to additional paid-in capital. The Company recorded the adjustment to additional paid-in capital because the holders of the Series A, Series B-1 and Series B-2 redeemable convertible preferred stock were also the majority shareholders of the Series C and Series D redeemable convertible preferred stock. Subsequently, the Company recorded the reissuance of the modified

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Series A, Series B-1 and Series B-2 redeemable convertible preferred stock at their respective fair values with a corresponding entry to additional paid-in capital. The extinguishment and reissuance of these securities resulted in a net decrease to the carrying values of the Series A, Series B-1 and Series B-2 redeemable convertible preferred stock of $1,395, $2,461 and $6,114, respectively, as well as an aggregate net capital contribution of $9,970 recorded as additional paid-in capital.

As the Company immediately accretes the carrying value of its redeemable convertible preferred stock to redemption value at the end of each reporting period as if the end of the reporting period were the redemption date, the Company recorded an entry of $3,380 as of December 31, 2009 to increase the carrying values of each series of outstanding redeemable convertible preferred stock to their respective redemption values

In May 2010, the Company issued 3,661,846 shares of Series D redeemable convertible preferred stock to certain existing investors at $0.54617142 per share for gross proceeds of $2,000. The Company incurred issuance costs of $30 in connection with the sale and issuance of these shares of Series D redeemable convertible preferred stock. In November 2010, all outstanding shares of Series D redeemable convertible preferred stock were exchanged for 29,294,828 shares of Series D-1 redeemable convertible preferred stock and 3,661,846 shares of Series D-2 redeemable convertible preferred stock. The Company treated this exchange as an extinguishment of the Series D redeemable convertible preferred stock and the issuance of Series D-1 and D-2 preferred stock at their respective fair values. As the rights and preferences of the shares exchanged were identical, the Company determined that the fair value of the Series D-1 and Series D-2 redeemable convertible preferred stock was the same as the carrying value of the Series D redeemable convertible preferred stock at the time of the exchange. As a result, there was no change in the aggregate carrying values of these securities.

In November 2010, in connection with the distribution of all outstanding shares of convertible preferred stock of Diotima, which was a wholly owned subsidiary of the Company, to holders of the Company's Series A, Series B-1, Series B-2, Series C and Series D-1 redeemable convertible preferred stock on a pro rata basis (see Note 8), the liquidation and redemption amounts of each of the Company's outstanding shares of Series A, Series B-1, Series B-2, Series C and Series D-1 redeemable convertible preferred stock were decreased by $0.0419 per share. Accordingly, the Company recorded an adjustment to decrease the aggregate carrying value of the Series A, Series B-1 and Series B-2 redeemable convertible preferred stock by $285, or $0.0419 per share, to reflect their adjusted redemption values. Although the liquidation preferences of the Series C and D-1 redeemable convertible preferred stock were decreased as a result of the distribution, the downward adjustments to their carrying values was limited to $80, representing the amount of accretion previously recorded by the Company related to the Series C and D-1 redeemable convertible preferred stock.

During 2011, the Company issued 8,239,150 shares of Series D-3 redeemable convertible preferred stock to certain existing investors at $0.54617142 per share for gross proceeds of $4,500. The Company incurred issuance costs of $194 in connection with the sale and issuance of these shares of Series D-3 redeemable convertible preferred stock.

During 2012, the Company issued 34,696,042 shares of Series D-3 redeemable convertible preferred stock to certain existing investors at $0.54617142 per share for gross proceeds of $18,950. The Company incurred issuance costs of $34 in connection with the sale and issuance of these shares of Series D-3 redeemable convertible preferred stock.

In May and October 2013, the Company issued an aggregate of 56,892,391 shares of Series E redeemable convertible preferred stock to existing and new investors at $0.62398475 per share for gross proceeds of $35,500.

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The Company incurred issuance costs of $94 in connection with the sale and issuance of these shares of Series E redeemable convertible preferred stock.

Redeemable Preferred Stock consisted of the following as of December 31, 2012:

	Preferred Shares Authorized	Preferred Shares Issued and Outstanding	Liquidation Preference	Carrying Value	Common Stock Issuable Upon Conversion
Series A redeemable convertible preferred stock	4,500,000	4,500,000	$ 2,961	$ 2,961	429,799
Series B-1 redeemable convertible preferred stock	798,067	798,067	525	525	76,224
Series B-2 redeemable convertible preferred stock	1,503,819	1,503,819	989	989	143,631
Series C redeemable convertible preferred stock	15,999,998	15,999,998	3,330	3,920	1,528,176
Series D-1 redeemable convertible preferred stock	29,294,828	29,294,828	14,773	16,000	2,797,978
Series D-2 redeemable convertible preferred stock	3,661,846	3,661,846	2,000	2,000	349,747
Series D-3 redeemable convertible preferred stock	42,935,205	42,935,192	23,450	23,450	4,100,782
	98,693,763	98,693,750	$ 48,028	$49,845	9,426,337

Redeemable Preferred Stock consisted of the following as of December 31, 2013 and as of June 30, 2014 (unaudited):

	Preferred Shares Authorized	Preferred Shares Issued and Outstanding	Liquidation Preference	Carrying Value	Common Stock Issuable Upon Conversion
Series A redeemable convertible preferred stock	4,500,000	4,500,000	$ 2,961	$ 2,961	429,799
Series B-1 redeemable convertible preferred stock	798,067	798,067	525	525	76,224
Series B-2 redeemable convertible preferred stock	1,503,819	1,503,819	989	989	143,631
Series C redeemable convertible preferred stock	15,999,998	15,999,998	3,330	3,920	1,528,176
Series D-1 redeemable convertible preferred stock	29,294,828	29,294,828	14,773	16,000	2,797,978
Series D-2 redeemable convertible preferred stock	3,661,846	3,661,846	2,000	2,000	349,747
Series D-3 redeemable convertible preferred stock	42,935,192	42,935,192	23,450	23,450	4,100,782
Series E redeemable convertible preferred stock	56,892,391	56,892,391	35,500	35,500	5,433,836
	155,586,141	155,586,141	$ 83,528	$85,345	14,860,173

The holders of the Redeemable Preferred Stock have the following rights and preferences:

Voting Rights

The holders of Redeemable Preferred Stock are entitled to vote, together with the holders of common stock, on all matters submitted to stockholders for a vote. Holders of all Redeemable Preferred Stock, with the exception of holders of Series C redeemable convertible preferred stock, have the right to vote the number of shares equal to the number of shares of common stock into which such Redeemable Preferred Stock could convert on the record date for determination of stockholders entitled to vote. Holders of Series C redeemable convertible preferred stock are entitled to cast 0.45773175 of a vote for each share of common stock into which one share of Series C redeemable convertible preferred stock is convertible.

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Dividends

The holders of all Redeemable Preferred Stock are entitled to receive dividends at an annual rate of 8% of the Original Issue Price of the applicable series when and if declared by the Company's board of directors, provided that the holders of the Series A redeemable convertible preferred stock are entitled to receive the greater of 8% of the original issuance price of the Series A redeemable convertible preferred stock or $0.04 per share when and if declared by the Company's board of directors. Dividends are non-cumulative, and holders of Redeemable Preferred Stock holders are not entitled to any accruing dividends. In addition, any dividends declared by the Company's board of directors are required to be paid: first, to the holders of the Senior Preferred Stock; second, to the holders of Series B-1 and B-2 redeemable convertible preferred stock; and last, to the holders of Series A redeemable convertible preferred stock. The Original Issue Price ("OIP") for the Series A, Series B-1, Series B-2, Series C, Series D-1, Series D-2, Series D-3 and Series E redeemable convertible preferred stock is equal to $0.50, $3.383176, $4.365388, $0.25, $0.54617142, $0.54617142, $0.54617142 and $0.62398475 per share, respectively, subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Redeemable Preferred Stock. As of December 31, 2013 and June 30, 2014 (unaudited), no dividends had been declared by the Company's board of directors.

Liquidation Preference

In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Company (each, a "Liquidation Event"), the holders of Series C, Series D and Series E redeemable convertible preferred stock (collectively, the "Senior Preferred Stockholders") are entitled to be paid out of the assets of the Company prior to any payments made to the holders of Series A, Series B-1 or Series B-2 redeemable convertible preferred stock, and the holders of Series B-1 and B-2 redeemable convertible preferred stock are entitled to be paid out of any remaining assets prior to the holders of the Series A redeemable convertible preferred stock.

Based on the liquidation preferences under the Certificate of Incorporation and assuming sufficient assets available for distribution to the Company's stockholders, upon a Liquidation Event, holders of Redeemable Preferred Stock would be entitled to receive $0.6581 per share for Series A redeemable convertible preferred stock, $0.6581 per share for Series B-1 and B-2 redeemable convertible preferred stock, $0.2081 per share for Series C redeemable convertible preferred stock, $0.5043 per share for Series D-1 redeemable convertible preferred stock, $0.54617142 per share for Series D-2 and D-3 redeemable convertible preferred stock and $0.62398475 per share for Series E redeemable convertible preferred stock (in each case, subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization).

In the event that the assets available for distribution to the Company's stockholders are not sufficient to permit payment in full to the holders of Redeemable Preferred Stock, the holders of the Senior Preferred Stock are entitled to receive such amount prior to and in preference of the holders of the Series B-1 and B-2 redeemable convertible preferred stock and Series A redeemable convertible preferred stock, and the holders of Series B-1 and B-2 redeemable convertible preferred stock are entitled to receive such amount prior to and in preference of the holders of Series A redeemable convertible preferred stock.

In the event that the assets available for distribution to the Company's stockholders are not sufficient to permit payment to any class of holders in order of preference and in the full amount to which they are entitled, the assets available for distribution are distributed on a pro rata basis. After the payment of all preferential amounts to the holders of the Senior Preferred Stock, the Series B redeemable convertible preferred stock and the Series A

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redeemable convertible preferred stock, any remaining assets available for distribution will be distributed among the holders of the Redeemable Preferred Stock and the holders of the Company's common stock on a pro rata basis based on the number of shares held by each holder on an as converted to common stock basis.

Conversion

Each share of Redeemable Preferred Stock is convertible at the option of the stockholder at any time without the payment of additional consideration, or will automatically be converted into shares of common stock at the applicable conversion ratio then in effect, upon the closing of a firm commitment underwritten public offering with gross proceeds of at least $40,000 and a minimum price per share to the public or upon the vote or written consent of the holders of at least 75% of the outstanding shares of the Senior Preferred Stockholders voting together as a single class. The conversion ratio of the Redeemable Preferred Stock is determined by dividing the OIP of the applicable series by the Conversion Price (as defined in the Certificate of Incorporation) of the applicable series with the exception of Series B redeemable convertible preferred stock, which is calculated by dividing $0.54617142 by the Conversion Price. The Conversion Price is subject to adjustment as set forth in the Certificate of Incorporation. As of December 31, 2013 and June 30, 2014 (unaudited), the Conversion Price for the Series A, Series B-1, Series B-2, Series C, Series D-1, Series D-2, Series D-3 and Series E redeemable convertible preferred stock is equal to $5.235, $5.71841477, $5.71841477, $2.6175, $5.71841477, $5.71841477, $5.71841477 and $6.53312033 per share, respectively. As of December 31, 2013 and June 30, 2014 (unaudited), all shares of Redeemable Preferred Stock are convertible into shares of the Company's common stock on a 10.47-for-1 basis.

Redemption Rights

At any time on or after May 10, 2018, shares of each of the Series A, Series B-1 and Series B-2 redeemable convertible preferred stock are subject to mandatory redemption by the Company in three equal annual installments beginning 60 days after receipt of a notice of redemption from the holders of at least 60% of the combined voting power of the holders of the outstanding Series A, Series B-1 and Series B-2 redeemable convertible preferred stock, voting as a single class. As of December 31, 2013 and June 30, 2014 (unaudited), the redemption price for the Series A, Series B-1 and Series B-2 redeemable convertible preferred stock is equal to $0.6581 per share, subject to appropriate adjustment for any stock splits, stock dividends, combinations or any other similar recapitalization affecting such shares, plus any dividends declared but unpaid thereon.

At any time on or after May 10, 2018, shares of the Senior Preferred Stock are subject to mandatory redemption by the Company in three equal annual installments beginning 60 days after receipt of a notice of redemption from the holders of at least 75% of the combined voting power of holders of the outstanding Senior Preferred Stock. As of December 31, 2013 and June 30, 2014 (unaudited), the redemption price for the Series C, Series D-1, Series D-2, Series D-3 and Series E convertible preferred stock is equal to $0.2081, $0.5043, $0.54617142, $0.54617142 and $0.62398475 per share, respectively, subject to appropriate adjustment for any stock splits, stock dividends, combinations or any other similar recapitalization affecting such shares, plus any dividends declared but unpaid thereon.

Reissuance

Shares of any Redeemable Preferred Stock that are redeemed or converted will be retired or canceled and may not be reissued by the Company.

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7. Common Stock

As of December 31, 2013 and June 30, 2014 (unaudited), the Certificate of Incorporation authorizes the Company to issue 173,018,331 and 178,408,438 shares, respectively, of common stock, $0.001 par value per share.

Each share of common stock entitles the holder to one vote on all matters submitted to a vote of the Company's stockholders. Common stockholders are not entitled to receive dividends, unless declared by the board of directors.

As of December 31, 2013 and June 30, 2014 (unaudited), the Company had reserved 16,031,807 and 16,538,393 shares of common stock, respectively, for the conversion of the outstanding shares of Series A, Series B-1, Series B-2, Series C, Series D-1, Series D-2, Series D-3 and Series E redeemable convertible preferred stock (see Note 6) and the exercise of outstanding stock options and the number of shares of common stock remaining available for grant under the Company's Amended and Restated 2007 Stock Option Plan (see Note 9).

8. Diotima Distribution

In September 2010, the Company formed and incorporated Diotima, a wholly owned subsidiary of the Company (see Note 1). In November 2010, the Company entered into a contribution agreement with Diotima (the "Contribution Agreement"), pursuant to which the Company assigned rights to develop and commercialize certain compounds that were unrelated to the Company's core operations to Diotima in exchange for all outstanding shares of common and preferred stock of Diotima. The book value of the assets contributed to Diotima was $0. Effective in November 2010, the Company distributed to stockholders of the Company who were record holders as of May 21, 2010, on a pro rata basis, all of the issued and outstanding shares of common and preferred stock of Diotima (the "Diotima Spin-off").

In connection with the Diotima Spin-off, the Company entered into various agreements with Diotima. Under the terms of these agreements, the Company has funded the payment of license and license maintenance fees related to intellectual property licenses held by Diotima. As a result of this funding activity, the Company has determined that Diotima is a variable interest entity, in which the Company has a variable financial interest and is the primary beneficiary but has no ownership interest. Accordingly, the Company has continued to consolidate Diotima subsequent to the Diotima Spin-off. Diotima has had limited activity. Expenses incurred by Diotima for the years ended December 31, 2012 and 2013 and for the cumulative period from the incorporation of Diotima in 2010 to December 31, 2013 were $85, $60 and $233, respectively. Expenses incurred by Diotima for the six months ended June 30, 2013 and 2014 (unaudited) and for the cumulative period from the incorporation of Diotima in 2010 to June 30, 2014 (unaudited) were $58, $8 and $241, respectively. In 2014, the license agreements relating to these compounds were terminated. Additionally, in April 2014, the board of directors and stockholders of Diotima approved the dissolution of Diotima, and Diotima was dissolved.

9. Stock-Based Awards

2007 Stock Incentive Plan

The Company's 2007 Stock Incentive Plan, as amended (the "2007 Plan") provides for the Company to sell or issue restricted common stock or to grant stock options for the purchase of common stock to employees, members of the board of directors and consultants of the Company. The 2007 Plan is administered by the board of directors,

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or at the discretion of the board of directors, by a committee of the board. The exercise prices, vesting and other restrictions are determined at the discretion of the board of directors, or their committee if so delegated, except that the exercise price per share of stock options may not be less than 100% of the fair market value of the share of common stock on the date of grant and the term of stock option may not be greater than ten years.

Stock options granted under the 2007 Plan with service-based vesting conditions generally vest over four years and expire after ten years, although options have been granted with vesting terms of less than four years.

The total number of shares of common stock that may be issued under the 2007 Plan was 1,590,580 shares as of December 31, 2013, of which 47,535 shares remained available for future issuance as of December 31, 2013. As of June 30, 2014 (unaudited), the total number of shares of common stock that may be issued under the 2007 Plan was 2,105,395 shares, of which 43,945 shares remained available for issuance as of June 30, 2014 (unaudited).

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

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. The Company historically has been a private company and lacks company-specific historical and implied volatility information. Therefore, it estimates its expected stock volatility based on the historical volatility of a publicly traded group of peer companies and 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.

The following table sets forth the assumptions that the Company used to determine the fair value of the stock options granted, presented on a weighted average basis:

	Year Ended December 31,		Six Months Ended June 30,	
	2012	2013	2013	2014
			(unaudited)	
Risk-free interest rate	0.79%	1.72%	1.71%	1.87%
Expected term (in years)	6.07	5.98	5.99	5.89
Expected volatility	65.5%	79.7%	79.6%	79.2%
Expected dividend yield	0%	0%	0%	0%

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The following table summarizes the Company's stock option activity from January 1, 2012 through June 30, 2014:

	Shares Issuable Under Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (In years)	Aggregate Intrinsic Value
Outstanding as of December 31, 2011	769,600	$ 1.26	9.3	$ 74
Granted	99,130	1.37		
Exercised	(2,843)	1.37		
Forfeited	(58,230)	1.16		
Outstanding as of December 31, 2012	807,657	$ 1.26	8.5	$ 52
Granted	786,532	1.74		
Exercised	(157,804)	1.37		
Forfeited	(312,286)	1.37		
Outstanding as of December 31, 2013	1,124,099	$ 1.58	8.8	$ 2,346
Granted (unaudited)	524,133	5.57		
Exercised (unaudited)	(8,277)	1.47		
Forfeited (unaudited)	(5,680)	1.58		
Outstanding as of June 30, 2014 (unaudited)	1,634,275	$ 2.86	8.7	$ 5,941
Options vested and expected to vest as of December 31, 2013	946,438	$ 1.58	8.6	$ 1,992
Options exercisable as of December 31, 2013	329,738	$ 1.26	7.5	$ 790
Options vested and expected to vest as of June 30, 2014 (unaudited)	1,450,528	$ 2.94	8.7	$ 5,098
Options exercisable as of June 30, 2014 (unaudited)	470,751	$ 1.58	7.6	$ 2,300

As of December 31, 2013 and June 30, 2014 (unaudited), outstanding options for the purchase of 165,251 shares of common stock at an exercise price of $1.58 per share have performance-based vesting conditions that have been deemed to be not probable of vesting.

The aggregate intrinsic value of stock options is calculated as the difference between the exercise price of the stock options and the fair value of the Company's common stock for those stock options that had exercise prices lower than the fair value of the Company's common stock. The aggregate intrinsic value of stock options exercised was $1 and $33 for the years ended December 31, 2012 and 2013, respectively, and $33 and $34 for the six months ended June 30, 2013 and 2014, respectively (unaudited).

The Company received cash proceeds from the exercise of stock options of $4 and $215 during the years ended December 31, 2012 and 2013, respectively, and $215 and $12 during the six months ended June 30, 2013 and 2014, respectively (unaudited).

The weighted average grant date fair value of stock options granted during the years ended December 31, 2012 and 2013 was $0.84 and $1.17 per share, respectively, and $1.05 and $3.77 per share for the six months ended June 30, 2013 and 2014, respectively (unaudited).

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Restricted Common Stock

The 2007 Plan provides for the award of restricted common stock. The Company has granted restricted common stock with time-based vesting conditions. Unvested shares of restricted common stock may not be sold or transferred by the holder. These restrictions lapse according to the time-based vesting conditions of each award.

The table below summarizes the Company's restricted stock activity since January 1, 2012:

	Shares	Weighted Average Grant Date Fair Value
Unvested restricted common stock as of December 31, 2011	2,739	$ —
Issued	154,973	1.37
Vested	(18,155)	1.26
Forfeited	—	—
Unvested restricted common stock as of December 31, 2012	139,557	$ 1.37
Issued	—	—
Vested	(10,412)	1.26
Forfeited	(129,145)	1.37
Unvested restricted common stock as of December 31, 2013	—	$ —

The aggregate intrinsic value of restricted stock awards is calculated as the difference between the price paid for the restricted stock awards and the fair value of the Company's common stock for those restricted stock awards that had a purchase price lower than the fair value of the Company's common stock. The aggregate intrinsic value of restricted stock awards that vested during the years ended December 31, 2012 and 2013 was $23 and $14, respectively, and during the six months ended June 30, 2013 (unaudited) was $14. As of December 31, 2013 and June 30, 2014 (unaudited), there were no unvested restricted stock awards subject to repurchase.

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:

	Year Ended December 31,		Six Months Ended June 30,		Cumulative Period From Inception (March 26, 2004) to December 31, 2013	Cumulative Period From Inception (March 26, 2004) to June 30, 2014
	2012	2013	2013	2014		
			(unaudited)			(unaudited)
Research and development	$ 87	$ 91	$ 41	$ 136	$ 263	$ 399
General and administrative	123	147	49	204	433	637
	$ 210	$ 238	$ 90	$ 340	$ 696	$ 1,036

As of December 31, 2013, the Company had an aggregate of $645 of unrecognized stock-based compensation cost, which is expected to be recognized over a weighted average period of 2.95 years. As of June 30, 2014 (unaudited), the Company had an aggregate of $2,238 of unrecognized stock-based compensation cost, which is expected to be recognized over a weighted average period of 3.20 years.

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10. Net Loss Per Share and Unaudited Pro Forma Net Loss Per Share

Net Loss Per Share

Basic and diluted net loss per share attributable to common stockholders was calculated as follows for the years ended December 31, 2012 and 2013 and the six months ended June 30, 2013 and 2014 (unaudited):

	Year Ended December 31,		Six Months Ended June 30,	
	2012	2013	2013	2014
			(unaudited)	
Numerator:				
Net loss	$ (9,649)	$ (15,725)	$ (6,835)	$ (10,698)
Accretion of redeemable convertible preferred stock to redemption value	(34)	(94)	(79)	—
Net loss attributable to common stockholders	$ (9,683)	$ (15,819)	$ (6,914)	$ (10,698)
Denominator:				
Weighted average common shares outstanding, basic and diluted	311,474	416,037	337,495	498,107
Net loss per share attributable to common stockholders, basic and diluted	$ (31.09)	$ (38.02)	$ (20.49)	$ (21.48)

The Company excluded the following common stock equivalents, outstanding as of December 31, 2012 and 2013 and as of June 30, 2013 and 2014 (unaudited), from the computation of diluted net loss per share attributable to common stockholders for the years ended December 31, 2012 and 2013 and the six months ended June 30, 2013 and 2014 (unaudited) because they had an anti-dilutive impact due to the net loss attributable to common stockholders incurred for the periods:

	December 31,		June 30,	
	2012	2013	2013	2014
			(unaudited)	
Stock options to purchase common stock	807,657	1,124,099	1,062,445	1,634,275
Unvested restricted common stock	139,557	—	—	—
Redeemable convertible preferred stock (as converted to common stock)	9,426,337	14,860,173	12,487,660	14,860,173
	10,373,551	15,984,272	13,550,105	16,494,448

Unaudited Pro Forma Net Loss Per Share

The unaudited pro forma basic and diluted net loss per share attributable to common stockholders for the year ended December 31, 2013 and the six months ended June 30, 2014 gives effect to adjustments arising upon the closing of the proposed initial public offering. The unaudited pro forma net loss attributable to common stockholders used in the calculation of unaudited basic and diluted pro forma net loss per share attributable to common stockholders does not include the effects of the adjustments to the carrying value of Redeemable Preferred Stock to equal redemption value because it assumes that the conversion of Redeemable Preferred Stock into common stock had occurred on the later of January 1, 2013 or the issuance date of the Redeemable Preferred Stock.

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The unaudited pro forma basic and diluted weighted average common shares outstanding used in the calculation of unaudited pro forma basic and diluted net loss per share attributable to common stockholders for the year ended December 31, 2013 and the six months ended June 30, 2014 gives effect to the automatic conversion upon the closing of the proposed initial public offering of all outstanding shares of Redeemable Preferred Stock as of December 31, 2013 and June 30, 2014 into 14,860,173 shares of common stock as if the conversion had occurred on the later of January 1, 2013 or the issuance date of the Redeemable Preferred Stock.

The computation of unaudited pro forma basic and diluted net loss per share attributable to common stockholders is as follows:

	Year Ended December 31, 2013	Six Months Ended June 30, 2014
		(unaudited)
Numerator:		
Net loss	$ (15,725)	$ (10,698)
Pro forma net loss attributable to common stockholders	$ (15,725)	$ (10,698)
Denominator:		
Weighted average common shares outstanding, basic and diluted	416,037	498,107
Pro forma adjustment for assumed automatic conversion of all outstanding shares of redeemable convertible preferred stock upon the closing of the proposed initial public offering	11,814,169	14,860,173
Pro forma weighted average common shares outstanding, basic and diluted	12,230,206	15,358,280
Pro forma net loss per share attributable to common stockholders, basic and diluted	$ (1.29)	$ (0.70)

11. 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 years ended December 31, 2012 and 2013, the Company recognized $341 and $366, respectively, of rental expense related to office space. For the cumulative period from inception (March 26, 2004) to December 31, 2013, the Company recognized $1,557 of rental expense related to office space. For the six months ended June 30, 2013 and 2014 (unaudited), the Company recognized $173 and $209, respectively, of rental expense related to office space. For the cumulative period from inception (March 26, 2004) to June 30, 2014 (unaudited), the Company recognized $1,766 of rental expense related to office space.

Intellectual Property Licenses

In May 2006, the Company entered into a master license agreement with the University of Maryland, Baltimore ("UMB"). 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

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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.

In consideration for the rights granted, the Company made an upfront payment to UMB of $20 following the execution of the license agreement and a payment of $10 following the execution of each of the amendments in 2009, 2012 and 2013. In addition, the Company paid UMB a $50 milestone payment in 2009 upon the submission of an investigational new drug application ("IND") for galeterone and a $40 milestone payment in 2013 upon the issuance of the first patent related to UMB's prodrug patent application.

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 IND filed for a licensed product and a $100 milestone payment upon the approval of each NDA 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 June 30, 2014 (unaudited), 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 June 30, 2014 (unaudited), 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 license, milestone and maintenance fees of $60 and $20 for the years ended December 31, 2012 and 2013, respectively, and $210 for the cumulative period from inception (March 26, 2004) to December 31, 2013. In connection with this license agreement, the Company incurred license, milestone and maintenance fees of $10 in each of the six months ended June 30, 2013 and 2014 (unaudited) and $220 for the cumulative period from inception (March 26, 2004) to June 30, 2014 (unaudited).

The Company also had two license agreements for compounds and indications unrelated to its core strategy that were assigned to Diotima in November 2010 (see Note 8). Under the terms of the Contribution Agreement with Diotima, the Company funded the payment of annual license maintenance fees for the years ended December 31, 2011, 2012 and 2013. In early 2014, the Company, on behalf of Diotima, notified the licensors that they were terminating the two license agreements. In connection with these license agreements, the Company incurred license and maintenance fees of $50 for each of the years ended December 31, 2012 and 2013 and $1,710 for the cumulative period from inception (March 26, 2004) to December 31, 2013. The Company incurred license and maintenance fees of $50 for the six months ended June 30, 2013 (unaudited). There were no license or maintenance fees associated with these licenses incurred by the Company during the six months ended June 30, 2014 (unaudited).

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The Company also entered into a license agreement in 2006 for certain technologies, under which the Company paid a total of $100 prior to the license agreement being terminated effective December 31, 2008.

Advisor Agreement

The Company is obligated to pay a fee to a financial advisor equal to the greater of $500 and 1% of the gross proceeds of an initial public offering of the Company's common stock, upon the closing of such event, in connection with strategic and financial advisory services unrelated to the offering.

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. 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 such indemnifications. 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 consolidated financial statements as of December 31, 2013 or June 30, 2014 (unaudited).

12. Income Taxes

During the years ended December 31, 2012 and 2013 and the six months ended June 30, 2013 and 2014 (unaudited), the Company recorded no income tax benefits for the net operating losses incurred in each year, due to its uncertainty of realizing a benefit from those items.

A reconciliation of the U.S. federal statutory income tax rate to the Company's effective income tax rate is as follows:

	Year Ended December 31,	
	2012	2013
Federal statutory income tax rate	(34.0)%	(34.0)%
Federal and state research and development tax credit	(1.2)	(0.7)
State taxes, net of federal benefit	(5.4)	(5.6)
Stock-based compensation expense	0.6	0.4
Other	—	0.1
Change in deferred tax asset valuation allowance	40.0	39.8
Effective income tax rate	0.0%	0.0%

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Net deferred tax assets as of December 31, 2012 and 2013 consisted of the following:

	December 31,	
	2012	2013
Current deferred tax assets:		
Accrued expenses	$ 43	$ 201
Total current deferred tax assets	43	201
Noncurrent deferred tax assets:		
Capitalized research and development expenses	14,455	19,250
Net operating loss carryforwards	2,862	3,989
Research and development tax credit carryforwards	718	889
Other	60	73
Total noncurrent deferred tax assets	18,095	24,201
Total gross deferred tax assets	18,138	24,402
Valuation allowance	(18,138)	(24,402)
Net deferred tax assets	$ —	$ —

Changes in the valuation allowance for deferred tax assets during the years ended December 31, 2012 and 2013 related primarily to the increase in net operating loss carryforwards, capitalized research and development expenses and research and development tax credit carryforwards and were as follows:

	Year Ended December 31,	
	2012	2013
Valuation allowance as of beginning of year	$ 14,129	$ 18,138
Decreases recorded as benefit to income tax provision	—	—
Increases recorded to income tax provision	4,009	6,264
Valuation allowance as of end of year	$ 18,138	$ 24,402

As of December 31, 2013, the Company had net operating loss carryforwards for federal and state income tax purposes of $10,471 and $8,116, respectively, which begin to expire in 2024 and 2014, respectively. As of December 31, 2013, the Company also had available research and development tax credit carryforwards for federal and state income tax purposes of $610 and $422, respectively, which begin to expire in 2025 and 2023, respectively. Utilization of the net operating loss carryforwards and research and development tax credit carryforwards may be subject to a substantial annual limitation under Section 382 of the Internal Revenue Code of 1986, as amended (the "Code") due to ownership changes that have occurred previously or that could occur in the future. These ownership changes may limit the amount of carryforwards that can be utilized annually to offset future taxable income. In general, an ownership change, as defined by Section 382 of the Code, results from transactions increasing the ownership of certain shareholders or public groups in the stock of a corporation by more than 50% over a three-year period. The Company has not conducted a study to assess whether a change of control has occurred or whether there have been multiple changes of control since inception due to the significant complexity and cost associated with such a study. If the Company has experienced a change of control, as defined by Section 382 of the Code, at any time since inception, utilization of the net operating loss carryforwards or research and development tax credit carryforwards would be subject to an annual limitation under Section 382 of the Code, which is determined by first multiplying the value of the Company's stock at the

time of the ownership change by the applicable long-term tax-exempt rate, and then could be subject to additional adjustments, as required. Any limitation may result in expiration of a portion of the net operating loss carryforwards or research and development tax credit carryforwards before utilization. Further, until a study is completed and any limitation is known, no amounts are being presented as an uncertain tax position.

As of December 31, 2012 and 2013, the Company's gross deferred tax asset balance of $18,138 and $24,402, respectively, was comprised principally of capitalized research and development expenses, net operating loss carryforwards, and research and development tax credit carryforwards. During the years ended December 31, 2012 and 2013, gross deferred tax assets increased due to additional net operating loss carryforwards, research and development tax credits generated and additional research and development expenses capitalized for tax purposes. During the six months ended June 30, 2014 (unaudited), the Company's gross deferred tax assets increased by approximately $4,300 due to the operating losses incurred by the Company during that period.

The Company has evaluated the positive and negative evidence bearing upon its ability to realize the deferred tax assets. Management has considered the Company's history of cumulative net losses incurred since inception and its lack of commercialization of any products or generation of any revenue from product sales since inception and has concluded that it is more likely than not that the Company will not realize the benefits of the deferred tax assets. Accordingly, a full valuation allowance has been established against the deferred tax assets as of December 31, 2012 and 2013 and June 30, 2014 (unaudited). Management reevaluates the positive and negative evidence at each reporting period.

The Company has not recorded any amounts for unrecognized tax benefits as of December 31, 2012 or 2013 or June 30, 2014 (unaudited).

The Company files tax returns as prescribed by the tax laws of the jurisdictions in which it operates. In the normal course of business, the Company is subject to examination by federal and state jurisdictions, where applicable. There are currently no pending income tax examinations. The Company's tax years are still open under statute from 2010 to the present. Earlier years may be examined to the extent that tax credit or net operating loss carryforwards are used in future periods. The Company's policy is to record interest and penalties related to income taxes as part of its income tax provision.

13. 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.

14. Qualifying Therapeutic Discovery Project Program

In 2010, the Company received $244 for a research project under the Qualifying Therapeutic Discovery Project Credit program under the Patient Protection and Affordable Care Act, covering 50% of qualifying expenses incurred. The Company recorded the proceeds received as other income in its consolidated statements of operations for the cumulative period from inception (March 26, 2004) to December 31, 2013 and for the cumulative period from inception (March 26, 2004) to June 30, 2014 (unaudited).

Tokai Pharmaceuticals, Inc.
(A Development Stage Company)

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
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15. Related Party Transactions

In 2005, the Company loaned $250 to and entered into a promissory note with an advisor and stockholder of the Company that accrued interest at 2.92% per annum and was due in 2007. In 2007, unpaid principal and interest in the amount of $220 was deemed uncollectable by the Company, and as a result, was 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 $79 for the six months ended June 30, 2014 (unaudited), representing cash collected during that period.

In May 2009, the Company loaned $100 to and entered into a promissory note with an officer of the Company. The note and accrued interest was fully paid in 2011.

16. Subsequent Events

For its consolidated financial statements as of December 31, 2013 and for the year then ended, the Company evaluated subsequent events through May 2, 2014, the date on which those financial statements were issued, and, with respect to the reverse stock split described below, through August 29, 2014.

Increases in Authorized Common Shares under the Certificate of Incorporation and in Shares Reserved for Issuance under the 2007 Plan

In February 2014, the Company effected an increase in the number of authorized shares of its common stock under its Certificate of Incorporation to 177,408,438 shares and an increase in the number of shares of common stock available for issuance under the 2007 Plan to 2,009,884 shares.

In April 2014, the Company effected an increase in the number of authorized shares of its common stock under its Certificate of Incorporation to 178,408,438 shares and an increase in the number of shares of common stock available for issuance under the 2007 Plan to 2,105,395 shares.

Reverse Stock Split

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 Preferred Stock (see Note 6). 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 preferred stock conversion ratios.

17. Subsequent Events (unaudited)

For its interim consolidated financial statements as of June 30, 2014 and for the six months then ended, the Company evaluated subsequent events through August 11, 2014, the date on which those financial statements were issued.

Tokai Pharmaceuticals, Inc.
(A Development Stage Company)

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(Amounts in thousands, except share and per share data)

2014 Stock Incentive Plan

On August 29, 2014, the Company's stockholders approved the 2014 Stock Incentive Plan (the "2014 Plan"), which will become effective upon the effectiveness of the registration statement for the Company's initial public offering of shares of common stock. The 2014 Plan provides for the grant of incentive stock options, nonstatutory stock options, restricted stock, restricted stock units, stock appreciation rights and other stock-based awards. The number of shares initially reserved for issuance under the 2014 Plan is the sum of 1,700,000 shares of common stock, plus the number of shares (up to 1,678,220 shares) equal to the sum of (i) the number of shares reserved for issuance under the 2007 Plan that remain 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. In addition, the number of shares of common stock that may be issued under the 2014 Plan is subject to increase 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.

2014 Employee Stock Purchase Plan

On August 29, 2014, the Company's stockholders approved the 2014 Employee Stock Purchase Plan. A total of 225,000 shares of common stock were reserved for issuance under this plan. The 2014 Employee Stock Purchase Plan will become effective upon the effectiveness of the registration statement for the Company's initial public offering of shares of common stock. In addition, the number of shares of common stock that may be issued under the plan 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 450,000 shares of the Company's common stock, 1% 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.

6,480,000 Shares

Tokai Pharmaceuticals, Inc.

Common Stock

Prospectus

BMO Capital Markets

Stifel

William Blair

Janney Montgomery Scott

September 16, 2014

Until October 11, 2014, 25 days after the date of this prospectus, all dealers that buy, sell or trade our common stock, whether or not participating in this offering, may be required to deliver a prospectus. This is in addition to the dealer's obligation to deliver a prospectus when acting as an underwriter and with respect to unsold allotments or subscriptions.