

Viking Therapeutics, Inc.  
Form 10-K  
March 08, 2016

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

(Mark One)

ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934  
For the fiscal year ended December 31, 2015

OR

TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT  
OF 1934 FOR THE TRANSITION PERIOD FROM \_\_\_\_\_ TO \_\_\_\_\_  
Commission File Number 001-37355

Viking Therapeutics, Inc.

(Exact name of Registrant as specified in its Charter)

|  |                     |
|--|---------------------|
| Delaware                                 | 46-1073877          |
| ( State or other jurisdiction of         |                     |
| incorporation or organization)           | (I.R.S. Employer    |
| 12340 El Camino Real, Suite 250          | Identification No.) |
| San Diego, California                    | 92130               |
| (Address of principal executive offices) | (Zip Code)          |

Registrant's telephone number, including area code: (858) 704-4660

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

| Title of Each Class                         | Name of Each Exchange on Which Registered |
|---|---|
| Common Stock, par value \$0.00001 per share | The Nasdaq Capital Market                 |

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

None

Indicate by check mark if the Registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act.

Yes  No

Indicate by check mark if the Registrant is not required to file reports pursuant to Section 13 or 15(d) of the Act.

Yes  No

Indicate by check mark whether the Registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the Registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes  No

Indicate by check mark whether the Registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the Registrant was required to submit and post such files). Yes  No

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K (§229.405) is not contained herein, and will not be contained, to the best of Registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K.

Indicate by check mark whether the Registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See the definition of "large accelerated filer", "accelerated filer", and "smaller reporting company" in Rule 12b-2 of the Exchange Act. (Check one):

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

Indicate by check mark whether the Registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act).

Yes  No

The aggregate market value of the voting and non-voting common equity held by non-affiliates of the Registrant, based on the closing price of the shares of common stock on the Nasdaq Capital Market on June 30, 2015 (the last

trading day of the registrant's second fiscal quarter of 2015), was \$18,289,869. Shares of voting stock held by directors, officers and stockholders or stockholder groups whose beneficial ownership exceeds 5% of the registrant's common stock outstanding have been excluded in that such persons may be deemed to be affiliates. The number of shares owned by stockholders whose beneficial ownership exceeds 5% was determined based upon information supplied by such persons and upon Schedules 13D and

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13G, if any, filed with the Securities and Exchange Commission. This assumption regarding affiliate status is not necessarily a conclusive determination for other purposes.

The number of shares of the Registrant's Common Stock outstanding as of February 29, 2016 was 9,683,741.

#### DOCUMENTS INCORPORATED BY REFERENCE

Portions of the Registrant's Definitive Proxy Statement for its 2016 Annual Meeting of Stockholders or an amendment to this Annual Report on Form 10-K to be filed with the Securities and Exchange Commission within 120 days of the registrant's fiscal year ended December 31, 2015 are incorporated by reference into Part III of this Annual Report on Form 10-K.

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This Annual Report on Form 10-K contains “forward-looking statements” as defined in Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, in connection with the Private Securities Litigation Reform Act of 1995 that involve risks and uncertainties, as well as assumptions that, if they never materialize or prove incorrect, could cause our results to differ materially and adversely from those expressed or implied by such forward-looking statements. Such forward-looking statements include estimates of our expenses, future revenue, capital requirements and our needs for additional financing; statements regarding our ability to develop, acquire and advance drug candidates into, and successfully complete, clinical trials and preclinical studies; statements concerning new product candidates; risks and uncertainties associated with our research and development activities, including our clinical trials and preclinical studies; our expectations regarding the potential market size and the size of the patient populations for our drug candidates, if approved for commercial use, and our ability to serve such markets; statements regarding our ability to maintain and establish collaborations or obtain additional funding; statements regarding developments and projections relating to our competitors and our industry and other matters that do not relate strictly to historical facts or statements of assumptions underlying any of the foregoing. These statements are often identified by the use of words such as “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “will,” the negative versions of these terms and similar expressions or variations. These statements are based on the beliefs and assumptions of our management based on information currently available to management. Such forward-looking statements are subject to risks, uncertainties and other factors that could cause actual results and the timing of certain events to differ materially and adversely from future results expressed or implied by such forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those identified below, and those discussed in the section titled “Risk Factors” included elsewhere in this Annual Report on Form 10-K and in our other Securities and Exchange Commission filings. Furthermore, such forward-looking statements speak only as of the date of this report. We undertake no obligation to update any forward-looking statements to reflect events or circumstances occurring after the date of such statement.

Throughout this Annual Report on Form 10-K, unless the context otherwise requires, the terms “Viking,” “we,” “us” and “our” in this Annual Report on Form 10-K refer to Viking Therapeutics, Inc.

## PART I

### Item 1. Business.

#### Overview

We are a clinical-stage biopharmaceutical company focused on the development of novel, first-in-class or best-in-class therapies for metabolic and endocrine disorders. We have exclusive worldwide rights to a portfolio of five drug candidates in clinical trials or preclinical studies, which are based on small molecules licensed from Ligand Pharmaceuticals Incorporated, or Ligand. Details regarding our license agreement with Ligand are discussed under the heading “Agreements with Ligand” under Part I, “Item 1. Business” of this Annual Report on Form 10-K.

Our lead clinical program is VK5211, an orally available drug candidate, currently in a Phase 2 clinical trial for acute rehabilitation following non-elective hip fracture surgery. Hip fracture is a common injury among persons aged 60 and older. The acute recovery period post-injury is characterized by significant and rapid declines in bone mineral density, or BMD, and lean body mass, or LBM, which contributes to substantial morbidity and mortality in these patients. VK5211 is a non-steroidal selective androgen receptor modulator, or SARM. A SARM is designed to selectively interact with a subset of receptors that have a normal physiologic role of interacting with naturally-occurring hormones called androgens. Broad activation of androgen receptors with drugs, such as exogenous testosterone, can stimulate muscle growth and improve BMD, but often results in unwanted side effects such as prostate growth, hair growth and acne. VK5211 is expected to selectively produce the therapeutic benefits of testosterone in muscle and bone tissue, potentially accelerating rehabilitation and improving patient outcomes.

We expect VK5211 to produce the therapeutic benefits of testosterone, including increased LBM and BMD, with improved safety, tolerability and patient acceptance due to a tissue-selective mechanism of action and an oral route of administration. Tissue selectivity is particularly important in treating patients recovering from non-elective hip fracture surgery, as these patients experience abnormally elevated losses of muscle tissue and BMD. This results in a loss of muscle strength, an increased risk of additional fractures and increased mortality. We believe the selective stimulation of androgen receptors in muscle and bone provides an attractive therapeutic approach for patients recovering from hip fractures. In Phase 1 clinical trials, VK5211 demonstrated statistically significant increases in LBM among treated subjects following 21 days of treatment. Statistically significant refers to a low probability, generally regarded as less than or equal to 5%, of obtaining the observed result under a hypothesis that assumes no difference between treatment groups. We also observed positive dose-dependent trends in functional exercise and strength measures consistent with anabolic activity. In addition, no drug-related serious adverse events were reported. In an established animal model of osteoporosis, treatment with VK5211 resulted in significant increases in BMD and bone strength. In October 2015, we commenced enrollment for a Phase 2 proof-of-concept clinical trial in patients recovering from non-elective hip fracture surgery, and we expect to enroll a total of 120 patients

and complete enrollment in this clinical trial in the second half of 2016. We also plan to discuss with the U.S. Food and Drug Administration, or the FDA, potential clinical development of VK5211 in other acute use settings, such as cancer cachexia.

Hip fractures occur in over 300,000 persons in the U.S. annually. Most hip fractures occur in the elderly, often resulting from minimal trauma, such as a fall from standing height. Unfortunately, elderly individuals are at higher risk of substantial morbidity and mortality due to these fractures as a result of higher rates of frailty and undernourishment. Furthermore, the rate of hip fracture is known to increase with age, doubling every 5-6 years after age 60. Hip fractures can lead to devastating consequences. Disability frequently results from persistent pain and limited physical mobility. Hip fractures are associated with substantial morbidity and mortality, with approximately 15%-20% of patients dying within one year of fracture. There are currently no approved therapies in the U.S. for restoration or preservation of LBM, BMD or physical function in patients who have suffered a hip fracture. Pharmacological interventions, including with steroids, have demonstrated limited clinical benefit or expose patients to the risk of undesirable side-effects, such as virilization in women and prostate growth in men. We believe the potential size of the worldwide hip fracture treatment market for a SARM exceeds \$1.0 billion annually.

Our second program is focused on the development of orally available small molecule thyroid hormone receptor beta, or TR $\beta$ , agonists. Our two lead molecules are VK2809 and VK0214. We believe selective thyroid receptor agonists have the potential to treat a variety of lipid disorders. Thyroid hormone receptors are found in several tissues throughout the body. The TR $\beta$  isoform is the major receptor subtype expressed in the liver and the TR $\alpha$  isoform is the major subtype expressed in the heart. Selective activation of the TR $\beta$  receptor in liver tissue is believed to favorably affect cholesterol and lipoprotein levels via multiple mechanisms, including increasing the expression of low-density lipoprotein receptors, or LDL, receptors and increasing mitochondrial fatty acid oxidation. These characteristics in turn lead to reductions of LDL cholesterol, or LDL-C, plasma and liver triglycerides. We are developing VK2809 for the potential treatment of hypercholesterolemia and fatty liver disease. We are developing VK0214 for the potential treatment of X-linked adrenoleukodystrophy, or X-ALD. We believe our selective TR $\beta$  agonists are capable of achieving this unique lipid lowering profile without eliciting unwanted effects on the heart and thyroid hormone axis.

VK2809 is an orally available, tissue and receptor-subtype selective agonist of the thyroid beta receptor that is entering Phase 2 development for the treatment of patients with hypercholesterolemia and fatty liver disease. VK2809 belongs to a family of novel prodrugs which are cleaved in vivo to release potent thyromimetics.

In a Phase 1 multiple ascending dose clinical trial, patients with mild hypercholesterolemia who were treated with VK2809 at doses of 5 mg and above experienced significant placebo-adjusted LDL-C reductions from baseline, ranging from approximately 15%-41%. In addition, placebo-adjusted triglyceride levels were reduced by more than 30% at doses of 2.5 mg and above. There were no serious adverse events observed in this trial, and no differences in heart rate, heart rhythm or blood pressure were observed between VK2809 and placebo-treated patients. In addition, VK2809 has demonstrated significant reductions in liver fat content in multiple animal models of fatty liver disease, suggesting potential efficacy in the setting of nonalcoholic steatohepatitis, or NASH. We plan to commence a Phase 2 clinical trial of VK2809 in approximately 100 patients with hypercholesterolemia and fatty liver disease in the first half of 2016 and to complete this clinical trial in the fourth quarter of 2016 or the first quarter of 2017.

In the U.S., the number of patients with dyslipidemia was estimated to be greater than 100 million in 2013. In the U.S., 33.5% of adults, or 71.0 million people, have high LDL-C. NASH is a growing epidemic in the U.S., and is quickly becoming a leading cause of cirrhosis and liver failure. It is estimated that NASH affects 2% to 5% of Americans, or 6.0 to 15.0 million people. As a result, we believe the global market opportunity for VK2809 in hypercholesterolemia or NASH exceeds \$1.0 billion.



We are also developing VK0214 for X-ALD, a rare X-linked, inherited neurological disorder characterized by a breakdown in the protective barriers surrounding brain and nerve cells. The disease is caused by mutations in a peroxisomal transporter of very long chain fatty acids, or VLCFA, known as the adenosine triphosphate binding cassette transporter D1, or ABCD1. As a result, transporter function is impaired and patients are unable to efficiently metabolize VLCFA. The TR $\beta$  receptor is known to regulate expression of an alternative VLCFA transporter, known as ABCD2. Various preclinical models have demonstrated that increased expression of ABCD2 can lead to normalization of VLCFA metabolism. Preliminary in vitro data suggest that VK0214 stimulates ABCD2 expression. We are conducting studies of VK0214 in an in vivo model of disease. Pending completion of this work, we expect in 2016 to commence work directed toward filing an Investigational New Drug Application, or IND.

X-ALD is a rare, often fatal condition believed to occur with an incidence of approximately one in 17,000 births. X-ALD is caused by mutations in the gene encoding for ABCD1, which is located on the X chromosome. Men have one X chromosome, while women have two copies. Because of this, an inherited mutation in the ABCD1 gene is more likely to manifest in males relative to females. The ABCD1 protein plays a critical role in the transport of VLCFA into a cellular organelle called the peroxisome, where VLCFA metabolism and disposal occur. Without functional ABCD1, VLCFA accumulate in cells, including neural cells, where they can lead to membrane disruption and damage to the myelin sheath, a protective and insulating membrane that surrounds nerve cells in the brain. This damage can result in decreased motor coordination and function, visual and hearing disturbances, the loss of cognitive

function, dementia, seizures, adrenal dysfunction and other complications, including death. There are currently no approved therapies for X-ALD and pharmacologic interventions have demonstrated limited clinical benefit. As a result, we believe the worldwide X-ALD market exceeds \$1.0 billion.

We have a pipeline with three additional programs targeting metabolic diseases and anemia. Our most advanced pipeline program is VK0612, a first-in-class, orally available Phase 2b-ready drug candidate for type 2 diabetes. Preliminary clinical data suggest VK0612 has the potential to provide substantial glucose-lowering effects, with an attractive safety and convenience profile compared with existing type 2 diabetes therapies. Our preclinical programs are focused on identifying orally available erythropoietin receptor, or EPOR, agonists, for the potential treatment of anemia, and on the development of tissue-selective inhibitors of diacylglycerol acyltransferase-1, or DGAT-1, for the potential treatment of obesity and dyslipidemia.

### Our Product Pipeline

The following table highlights our product pipeline:

Key: SARM, selective androgen receptor modulator; TR $\beta$ , thyroid receptor beta; NASH, nonalcoholic steatohepatitis.

### Our Strategy

We intend to become a leading biopharmaceutical company focused on the development of novel, first-in-class or best-in-class therapies for metabolic and endocrine disorders. The key elements of our strategy include:

- Advance the development of VK5211 for hip fracture and other muscle wasting disorders. We have commenced enrollment for a Phase 2 proof-of-concept clinical trial in patients recovering from non-elective hip fracture surgery, and we expect to enroll a total of 120 patients and complete enrollment in this clinical trial in the second half of 2016 and complete the trial in the first quarter of 2017. Pending positive data from this clinical trial, we plan to advance VK5211 in further clinical trials.
- Advance the development of VK2809 for hypercholesterolemia and fatty liver disease. We plan to commence a Phase 2 clinical trial in approximately 100 patients with hypercholesterolemia and fatty liver disease in the first half of 2016 and expect to complete this clinical trial in the fourth quarter of 2016 or the first quarter of 2017.
  - Advance the development of VK0214 for X-ALD. We are evaluating VK0214 in an animal model of X-ALD and expect to complete the study in 2016.
- Advance the development of VK0612 for type 2 diabetes. Pending receipt of sufficient funding, we intend to commence clinical development of VK0612 to evaluate once-daily doses of VK0612 in patients with poorly-controlled type 2 diabetes.
- Advance the development of our preclinical programs. We currently have two additional preclinical programs in development. Pending receipt of sufficient funding, we also plan to further advance our EPOR agonist and DGAT-1 inhibitor programs.
- Evaluate strategic partnership and collaboration opportunities. We plan to selectively evaluate partnership and collaboration opportunities throughout the duration of our development programs. In addition, we may opportunistically pursue in-licensing opportunities.

## VK5211: A Selective Androgen Receptor Modulator (SARM) for Hip Fracture

### Product Summary

Our lead clinical program, VK5211, is an orally available, non-steroidal SARM in development for the treatment of patients recovering from non-elective hip fracture surgery. VK5211 is designed to selectively produce the therapeutic benefits of testosterone in muscle and bone tissue with improved safety and tolerability. Tissue selectivity is critical in treating patients recovering from hip fracture. These patients experience elevated rates of metabolic breakdown of muscle tissue and loss of BMD. This results in a loss of muscle strength, an increased risk of additional fractures and increased mortality. Androgens, such as testosterone, are hormones that stimulate a variety of physiologic processes, including muscle, bone, hair and prostate growth. However, testosterone's lack of selectivity can produce undesirable side effects such as prostate growth in men, and hair growth and masculinization in women.

VK5211 has been evaluated in three Phase 1 clinical trials. Based on these clinical and additional preclinical data, we believe VK5211 has the following important characteristics that may suggest therapeutic benefits in patients recovering from hip fracture surgery:

- Improvement in lean body mass: Preliminary Phase 1 data suggest VK5211 rapidly stimulates the formation of LBM, an important property for the hip fracture recovery setting, where patients can lose up to 6% of lean body mass in the two months following injury.
- Improvement in bone growth and density: VK5211 has demonstrated encouraging efficacy in a standard animal model of osteoporosis, demonstrating improved bone mineral content, density and strength. This may benefit patients following hip surgery, where loss of bone mineral density can exceed 12 times the background rate for patients with osteoporosis.
- Encouraging tolerability: VK5211 has been well-tolerated at and above doses that we are currently administering in our Phase 2 clinical trial.
- Novel mechanism of action: Based on the anabolic characteristics imparted by selective activation of the androgen receptor, we believe VK5211 may stimulate bone and muscle growth, without demonstrating adverse bone remodeling properties that are a potential concern for osteoporosis drugs such as bisphosphonates. We expect VK5211's novel mechanism of action to provide critical bone and muscle growth promoting advantages.
- Once-daily, oral convenience: Clinical data suggest that VK5211 has the potential to provide therapeutic benefits via once-daily oral dosing. This may represent an important advantage among elderly patients, relative to injectable protein or bisphosphonate therapies.

The initial IND filing for VK5211 was submitted in December 2008 by Ligand. The subject of the IND was an application to begin clinical investigations of the drug substance in healthy volunteers. In a Phase 1 clinical trial, VK5211 was shown to be safe and well-tolerated following daily oral administration for 21 days. In this clinical trial, statistically significant increases in lean muscle mass were observed in drug-treated subjects compared to subjects treated with placebo ( $p=0.047$ ), and positive dose-dependent trends in functional exercise and strength measures were consistent with anabolic activity. Statistically significant refers to a low probability, generally regarded as less than or equal to 5%, of obtaining the observed result under a hypothesis that assumes no difference between treatment groups. No clinically significant drug-related adverse events were reported. In animal models, VK5211 has demonstrated anabolic activity in muscles, anti-resorptive and anabolic activity in bones, and robust selectivity for muscle and bone versus prostate and sebaceous glands.

In October 2015, we commenced enrollment for a Phase 2 proof-of-concept clinical trial in patients recovering from non-elective hip fracture surgery, and we expect to enroll a total of 120 patients and complete enrollment in this clinical trial in the second half of 2016. Pending positive data from this clinical trial, we plan to advance VK5211 in further clinical trials. We also plan to discuss with the FDA potential clinical development of VK5211 in other settings, such as cancer cachexia.

## Androgens and Androgen Receptors

Androgens are important for the proper regulation of the reproductive system, and play critical roles in the homeostasis of the muscular, skeletal, cardiovascular, metabolic and central nervous systems. The most predominant androgen hormone is testosterone. Testosterone is predominately produced in the testes in men and in the adrenal glands and ovaries in women, albeit at lower levels than in men. Testosterone stimulates the growth of muscle and bone, also known as anabolic effects, as well as the growth of the prostate and sebaceous gland, also known as androgenic effects and, as such, testosterone is considered a non-tissue-selective androgen.

While testosterone preparations are widely used for the treatment of male hypogonadism, the androgenic activity of testosterone limits its use in women and in elderly men who have a higher risk of developing benign prostatic hyperplasia, or BPH, a benign increase in prostate size, and prostate cancer. In men, the lack of selectivity of anabolic steroids may result in side effects such as acne, hair loss and progression of BPH and/or prostate cancer. In women, exposure to exogenous testosterone can be associated with hair growth, acne and masculinization. Furthermore, testosterone must be administered by intramuscular injections, transdermal patches or gels. These routes of administration can be inconvenient or associated with potential safety issues. We believe VK5211's selectivity, limited off-target effects and convenient route of administration may make it superior to off-label testosterone for treating hip fracture and other muscle wasting disorders.

SARMs are a class of small molecules designed to elicit the benefits of androgens on tissues such as muscle and bone, without the undesirable effects on prostate and sebaceous glands, by selectively activating androgen receptors in certain tissues. We believe that, based on their robust activity on muscle and bone, SARMs can be used for the potential treatment of a number of diseases or disorders, including hip fracture, muscle wasting, osteoporosis, frailty and hormone deficiency in both men and women in cases where testosterone supplements or anabolic steroid treatments are ineffective or where the side effect profile is inappropriate.

#### Hip Fracture and Other Muscle Wasting Market Opportunities

We are currently conducting a Phase 2 clinical trial for acute rehabilitation following non-elective hip fracture surgery. More than 300,000 patients in the U.S. experience hip fractures each year, and approximately 50% lose the ability to live independently following the injury. The number of hip fractures is expected to grow in the U.S. as the population ages. Due to required limitations in mobility following hip fracture, patients experience muscle atrophy, or deterioration from lack of use, which impacts the time required for rehabilitation to restore physical function. We believe VK5211's potential stimulatory effect on lean body mass could result in benefits to patients recovering from hip fracture or other conditions requiring orthopedic intervention, such as hip or knee replacement surgery. Currently, there are no approved therapies to assist in the maintenance or restoration of LBM, BMD or restoration of functional performance for these patients.

Hip fracture in the elderly is a serious and debilitating condition with a high mortality rate. One year mortality in this group is estimated to range from 20% to 30% and an estimated 50% of patients lose the ability to walk independently. As a result of the loss of mobility, and additional morbidities caused by the hip fracture, 20% of patients will require stays at long-term care facilities. Studies show that following hip fracture, patients experience a severe and rapid decline in LBM and BMD. These reported rates of decline are 12 to 75 times the rates observed in persons of similar age and demographics who have not sustained a hip fracture. Loss of LBM is believed to contribute to morbidity, disability and risk of re-fracture in hip fracture patients. Loss of BMD is associated with an increased risk of mortality and re-fracture. The following graphs illustrate the representative losses in LBM and BMD in the 60 days following hip fracture, as measured in the total hip and at the femoral neck.

No therapies are currently approved to treat patients experiencing loss of LBM and BMD in the acute setting following non-elective hip fracture surgery. In addition, there are no approved therapies to facilitate improved functional performance following surgery.

VK5211: A Potent, Non-Steroidal SARM

VK5211 is an orally available, non-steroidal SARM. VK5211 is a third generation SARM with greatly improved tissue-selectivity and other characteristics relative to earlier-generation SARM-targeting drug candidates. VK5211 selectively activates androgen receptors

in muscle and bone, stimulating muscle and bone growth, while avoiding undesirable side effects, such as unwanted hair growth, acne or stimulation of sebaceous glands and prostate growth. We believe VK5211 is a potential best-in-class compound due to its selectivity, potency and ability to show positive effects within a short treatment duration.

#### Clinical Data for VK5211

In three Phase 1 clinical trials, VK5211 was shown to be safe and well-tolerated at all doses following daily oral administration for up to 21 days. There were no reported serious adverse events determined to be related to treatment, and no clinically significant changes in liver function tests, prostate-specific antigen, hematocrit or electrocardiogram readings were observed. Moreover, subjects treated with VK5211 for 21 days experienced statistically significant increases in lean muscle mass, and positive dose-dependent trends in functional exercise and strength measures were consistent with anabolic activity.

The first Phase 1 clinical trial was a randomized, double-blind, placebo-controlled trial in 48 healthy male volunteers conducted in 2009. In this clinical trial, six cohorts received an escalating single dose of VK5211 ranging from 0.1 mg to 22 mg. The primary objective of this clinical trial was to evaluate the safety and tolerability of escalating single doses of VK5211 in healthy male subjects. Secondary objectives of the first Phase 1 clinical trial included a determination of the pharmacokinetics, or PK, and pharmacodynamics, or PD, of single escalating doses of VK5211 in healthy male subjects. The results showed that single doses at the levels administered were well-tolerated and no serious or severe adverse events were observed among subjects receiving VK5211. The PD results showed dose-related decreases in total testosterone and sex-hormone binding protein, consistent with the mechanism of action of selective androgen receptor modulation. A dose-related decrease in fasting serum HDL was also observed. VK5211 was well-tolerated and demonstrated predictable dose-proportional increases in systemic exposure.

In a subsequent Phase 1 multiple ascending dose clinical trial, which commenced in 2010 and was completed in 2011, 76 healthy men in three cohorts were dosed daily with placebo, 0.1 mg, 0.3 mg or 1 mg of VK5211 for 21 days. The primary objective of the second Phase 1 clinical trial for VK5211 was to assess the safety and tolerability of escalating doses of VK5211 following repeated once-daily oral administration for 21 days in healthy men. Secondary objectives included a determination of the PK and PD of VK5211 following repeated once-daily oral administration for 21 days. Exploratory objectives included a determination of the effects of 21 days of treatment with VK5211 on lean body mass measured by dual energy X-ray absorptiometry scan, maximal voluntary strength measured by the one repetition maximum method, and stair climbing power. The average body mass index in all cohorts ranged from 24.6 kg/m<sup>2</sup> to 27.0 kg/m<sup>2</sup>. In this clinical trial, subjects receiving 1 mg doses of VK5211 demonstrated a statistically significant 1.21 kilogram average increase in lean body mass. Positive, dose-dependent trends in strength and performance measurements were also observed. There were no significant changes or trends in fat mass across cohorts. VK5211 was shown to be safe and well tolerated, with a similar frequency of adverse events between the treated and placebo groups. There were no drug-related serious adverse events. In addition, there were no significant changes in hemoglobin, prostate-specific antigen, liver function tests or QT interval at any dose. VK5211 also displayed a favorable pharmacokinetic profile, without any changes in prostate-specific antigen.

In September 2015, we completed a Phase 1 clinical trial of VK5211 in 24 healthy male and female subjects aged 65 and over. Subjects received once-daily oral doses of VK5211 for seven days. The results of this study showed VK5211 to be safe and well-tolerated, with predictable pharmacokinetic properties.





## Preclinical Data

VK5211 has also demonstrated anabolic activity in muscles, anti-resorptive and anabolic activity in bone, and robust selectivity for muscle and bone versus prostate and sebaceous glands in animal models. The effects of VK5211 on bone strength, bone mineral content and BMD were evaluated in ovariectomized female rats, which are rats that have undergone surgical removal of the ovaries. The ovariectomized rat model is a standard animal model for evaluating the effect of pharmaceutical agents in osteoporosis, because removal of ovaries stimulates high bone turnover and subsequent bone loss, creating a simulated post-menopausal state that models the metabolic changes in post-menopausal osteoporosis patients. As shown below, in ovariectomized rats, at the two highest doses, VK5211 produced significant increases in femur bone mineral content and bone strength relative to ovariectomized rats treated with vehicle. In addition, VK5211 demonstrated anabolic effects in bone formation rates, bone density, bone volume and trabecular thickness.

### Effects of VK5211 on Bone in Ovariectomized Rats

The tissue-selectivity of VK5211 was examined in a castrated rat model. The castrated rat model is a standard animal model for examining tissue selectivity for SARMs due to the rapid nature of muscle atrophy in castrated animals and the high sensitivity to muscle growth upon androgen-based treatment. Muscle mass can be restored with a potent androgen-receptor agonist, such as testosterone. Initially, rats are castrated or receive sham surgery. Sham rats are rats that receive surgical procedures that do not remove the ovaries or have other physiologic purposes. Upon recovering from the surgery, castrated and sham rats are administered either an active therapy such as VK5211 or testosterone. The effects of therapy in this model are assessed by measuring muscle and prostate tissue mass. Muscle mass in castrated animals treated with vehicle is assigned 0% relative efficacy, while muscle mass in non-castrated animals that underwent sham surgery is assigned 100% relative efficacy. For example, a castrated rat treated with a drug that demonstrates 100% relative efficacy would have equivalent tissue mass to a non-castrated rat.

In this model, VK5211 demonstrated greater than 500-fold selectivity for maintaining muscle weight at non-castrate levels relative to the effects on prostate weight. By comparison, testosterone shows similar effects on both muscle and prostate tissue. These data suggest that VK5211 is highly tissue-selective for muscle, potentially leading to an improved therapeutic profile relative to testosterone.

## Effects of VK5211 in Muscle and Prostate Tissue in Castrated Rats

In studies of VK5211 in non-human primates, treatment periods of 14 days and 13 weeks resulted in significant increases in body mass relative to baseline. For example, in a 13 week good laboratory practice-safety study, treated animals experienced progressive body weight gain of 20% to 47% from baseline. Most of this increase in body weight was retained after a four-week recovery period.

## Development Plans

We expect to develop VK5211 for potential treatment of a wide range of diseases and disorders in both men and women. In October 2015, we commenced enrollment for a randomized, double-blind, placebo-controlled, multicenter Phase 2 proof-of-concept trial in patients who recently underwent non-elective hip fracture surgery. We expect to enroll approximately 120 patients in this clinical trial. We will evaluate three doses of VK5211 and plan to assess changes in LBM, BMD, functional status and quality of life among treated versus untreated patients after 12 weeks of therapy. We expect enrollment in this clinical trial to be completed in the second half of 2016, and the trial to be completed in the first quarter of 2017. Pending positive data from this study, we plan to advance VK5211 in further clinical trials. We also plan to discuss with the FDA potential clinical development of VK5211 in other settings, such as cancer cachexia.

## Future Opportunity in Cancer Cachexia

Cachexia is a complex disease characterized by an uncontrolled decline in muscle mass. Patients suffering from cachexia experience increased rates of metabolic breakdown of muscle tissue, resulting in a loss of muscle strength and reduced body weight. The condition is often found secondary to an underlying disease, such as cancer, chronic obstructive pulmonary disease, heart failure and HIV/AIDS. It is estimated that a combined total of approximately 9.0 million people suffer from cachexia in the U.S., Europe and Japan. A combination of factors tied to the underlying disease, including reduced growth factor production and overproduction of inflammatory and apoptosis, or cell-death, mediators, create an imbalance in muscle formation and degradation. The resulting dysregulation and associated weight loss leads to increased mortality rates in affected patients. Common clinical symptoms include decline in physical function and impaired immune function, which contribute to increased disability, fatigue, diminished quality of life and reduced rate of survival.

Although muscle wasting associated with cancer can be partially attributed to nutritional deficiencies, the use of appetite stimulants and nutritional interventions are generally ineffective. This is likely due to the failure of these approaches to address the underlying catabolic processes contributing to muscle wasting. Additionally, cancer patients with severe weight loss, poor performance status and metastatic disease who no longer respond to therapy may be less likely to respond to single therapies designed to increase muscle mass and improve physical function. Because muscle wasting, which often leads to refractory cachexia, has significant negative

impacts on patients and their families, early intervention with therapeutic agents aimed at stimulating muscle mass is critically important.

Approximately 2.0 million cancer patients in North America and Europe suffer from cachexia, and it is estimated that up to 20% of all cancer deaths are a direct result of cachexia. It is particularly common among patients with lung, gastric, colorectal or pancreatic cancers, with up to 80% of patients with gastric or pancreatic cancers, and approximately 50% of patients with lung or colorectal cancers, suffering from the syndrome. There are currently no approved therapies in the U.S. for cancer cachexia, and pharmacological interventions have demonstrated limited clinical benefit or expose patients to the risk of undesirable side-effects such as virilization in women and prostate growth in men. As a result, we believe the potential size of the worldwide cancer cachexia market exceeds \$1.0 billion.

Pending approval of VK5211 in the acute hip fracture setting, we may seek to pursue label expansion for VK5211 in non-small cell lung cancer, or NSCLC, patients with cachexia. According to the American Cancer Society, an estimated 224,000 patients in the U.S. are projected to be diagnosed with lung cancer in 2014, of which approximately 85% of these cases are expected to be NSCLC. At diagnosis, approximately half of NSCLC patients present with some form of muscle wasting syndrome. Muscle wasting in this population is associated with reduced strength, increased fatigue and a decrease in overall quality of life. In addition, data indicate that lean body mass may correlate with overall survival, suggesting a potential link between improvement in lean body mass and survival. We believe VK5211 may benefit a large segment of the NSCLC patient population, due to the drug's potential therapeutic benefits on muscle mass and associated functional gains.

In the U.S., the number of patients with dyslipidemia was estimated to be greater than 100 million in 2013. In the U.S., 33.5% of adults, or 71.0 million people, have high LDL-C. NASH is a growing epidemic in the U.S., and is quickly becoming a leading cause of cirrhosis and liver failure. It is estimated that NASH affects 2% to 5% of Americans, or 6.0 to 15.0 million people. As a result, we believe the global market opportunity for VK2809 in hypercholesterolemia or NASH exceeds \$1.0 billion.

VK2809 and VK0214: Novel Selective Thyroid Hormone Receptor- $\beta$ , or TR $\beta$ , Agonists for Lipid Disorders and Adrenoleukodystrophy

#### Product Summary

VK2809 and VK0214 are novel, orally available, selective TR $\beta$  agonists in development for lipid disorders and X-ALD. Thyroid hormone receptors are found in various tissues throughout the body. TR $\beta$  is the major receptor isoform expressed in the liver and TR $\alpha$  is the major isoform expressed in the heart. The unique properties of our TR $\beta$  agonists are designed to reduce or eliminate the deleterious effects of extra-hepatic thyroid receptor activation. In particular, high tissue and TR $\beta$  selectivity may lead to reduced activity at the TR $\alpha$  receptor, which can be associated with increased respiration and cardiac tissue hypertrophy. Selective activation of the TR $\beta$  receptor in liver tissue is believed to favorably affect cholesterol and lipoprotein levels via multiple mechanisms, including increasing the expression of low-density lipoprotein receptors and increasing mitochondrial fatty acid oxidation. These characteristics in turn lead to reductions of LDL-C, plasma and liver triglycerides. In addition, our chemical structures are not substrates for certain transporters involved in the uptake of thyroid hormone. Various animal models have shown that our molecules, as a result of their unique profiles, may have reduced cardiovascular effects versus thyroid hormone and other thyromimetics.

As a result of these characteristics, we believe our selective TR $\beta$  agonists are capable of eliciting a unique lipid lowering profile without eliciting unwanted effects on the heart and thyroid hormone axis. In Phase 1 clinical trials, subjects treated with VK2809 at doses of 5 mg and above experienced significant placebo-adjusted LDL-C reductions

from baseline, ranging from approximately 15-41%. In addition, placebo-adjusted triglyceride levels were reduced by more than 30% at doses of 2.5 mg and above. There were no serious adverse events observed in this trial, and no differences in heart rate, heart rhythm or blood pressure were observed between VK2809 and placebo-treated patients. In addition, VK2809 has demonstrated significant reductions in liver fat content in multiple animal models of fatty liver disease, suggesting potential efficacy in the NASH setting.

We are developing VK2809 for the potential treatment of cholesterolemia and fatty liver disease. Because prior studies have shown excellent data in both the lipid-lowering setting and in models of fatty liver disease, we plan to conduct a Phase 2 clinical trial to evaluate both potential indications. We will target patients who have elevated cholesterol, fatty liver disease, and at least three risk factors for metabolic syndrome. Metabolic syndrome is considered a major driver for the onset of NASH. The primary endpoint of this trial will assess changes in LDL-C, with exploratory endpoints evaluating changes in liver fat content, inflammatory markers, and histological changes. We expect to commence the study in the first half of 2016 and complete the study in the fourth quarter of 2016 or the first quarter of 2017. Upon conclusion, we expect to be in a position to move forward in either hypercholesterolemia or NASH.

## VK2809 Summary

VK2809 has been evaluated in two Phase 1 clinical trials. Based on these clinical and additional preclinical data, we believe VK2809 has the following important characteristics that may benefit patients with metabolic or lipid disorders:

- Broader efficacy: Preliminary Phase 1 data suggest VK2809 could reduce plasma LDL-C, triglyceride and atherogenic protein levels by greater amounts than existing oral therapies. Such broad and potent lipid lowering-activity may be particularly desirable for poorly-controlled patients with hypercholesterolemia or mixed dyslipidemia, or among patients with risk factors such as chronic kidney disease.
  - Encouraging safety profile: VK2809 has demonstrated encouraging safety to date in over 110 subjects. No drug related serious adverse events were observed. In addition, no cardiovascular abnormalities were reported, in-line with the expected high tissue and receptor selectivity for VK2809.
- Encouraging tolerability: VK2809 has been well-tolerated at and above doses that we plan to administer in future clinical trials.
- Novel mechanism of action: Based on its selective thyroid receptor targeting mechanism of action, we believe VK2809 has the potential to lower plasma and liver lipid levels in a manner complementary to existing agents such as statins. In particular, we expect the unique liver-targeting properties of VK2809 will impart a robust lipid lowering effect within hepatic tissue, with potential therapeutic applications in fatty liver diseases such as NASH.
- Combinability: VK2809's novel mechanism of action is expected to allow combinability with many existing therapies, leading to enhanced efficacy and potentially delaying transition to subsequent therapies.
- Once-daily convenience: Clinical data suggest that VK2809 has the potential to lower plasma lipid levels in hypercholesterolemia patients as a once-daily oral therapy.

## Clinical Data for VK2809

VK2809 has been evaluated in two Phase 1 clinical trials. The initial Phase 1 safety, tolerability and pharmacokinetic study of VK2809 was conducted in 2006. This was followed by a 14-day Phase 1b clinical trial in 56 patients with mild hypercholesterolemia, defined as baseline plasma LDL-C of at least 100 mg/dL. This study was initiated in 2007 and completed in 2008. VK2809 was shown to be safe and well-tolerated across doses ranging from 0.25 mg to 40 mg per day. There were no serious adverse events, and the frequency of adverse events in VK2809-treated subjects was similar to placebo-treated subjects. No differences in heart rate, heart rhythm or blood pressure were observed between VK2809 and placebo-treated patients. Mild increases in liver enzymes were observed at the higher doses of VK2809 along with dose-related mean shifts in thyroid hormone levels. The clinical trial results also showed dose-related reductions in fasting LDL-C and fasting triglyceride, or TG, levels at day 14. Significant placebo-adjusted LDL-C reductions from baseline were observed at doses of 5 mg and above and ranged from approximately 15%-41%, while placebo-adjusted TG levels were reduced by more than 30% at doses of 2.5 mg and above. In addition, statistically significant reductions of lipoprotein a (Lp(a)) and apolipoprotein (Apo(B)), which are believed to be positively associated with a patient's risk of developing cardiovascular disease, were observed in certain cohorts. We believe these preliminary results compare favorably with the lipid lowering activities of existing oral agents for hyperlipidemia. A comparison of the Phase 1b efficacy results of VK2809 and data from existing hyperlipidemia agents is shown in the table below.

## Preclinical Data

VK2809, which is our most advanced TR $\beta$  agonist, is a potent small molecule that is selective for the TR $\beta$  receptor compared with the alpha receptor. VK2809 has an equilibrium dissociation constant  $K_i$ , which refers to the concentration of drug required to occupy 50% of available TR $\beta$  receptors, of approximately 2 nanomoles per liter, and has approximately 16:1 selectivity for the beta receptor over the alpha receptor. VK2809 has demonstrated cholesterol-lowering activity in five animal species. In addition, VK2809 has demonstrated additive cholesterol lowering activity when combined with atorvastatin, an approved and widely prescribed medication for lowering cholesterol. Treatment of rodents with VK2809 also led to a beneficial reduction in liver fat content. For example, histologic evaluation of liver tissue, as well as quantitative data showing liver triglyceride content was reduced by more than 40% in some rodent models of hepatic steatosis, demonstrated encouraging preliminary signs of efficacy in the reduction of liver fat. We believe the reduction of liver fat content results suggest a potential benefit in diseases characterized by excessive accumulation of lipids in liver tissue, such as NASH. We believe the totality of results from our TR $\beta$  agonist program suggest that VK2809 possesses an attractive profile for potential future development in a variety of lipid disorders, including dyslipidemia, hypercholesterolemia and NASH.

## VK2809 Development Plans

We are developing VK2809 for potential treatment of cholesterolemia and fatty liver disease. Because prior studies have shown excellent data in both the lipid-lowering setting and in models of fatty liver disease, we plan to conduct a Phase 2 clinical trial to evaluate both potential indications. We will target patients who have elevated cholesterol, fatty liver disease, and at least three of the five criteria developed by the National Cholesterol Education Program Adult Treatment Panel (NCEP ATP, 2005 revision) that are used to diagnose patients with metabolic syndrome. Metabolic syndrome is considered a major driver for the onset of NASH. The primary endpoint of this trial will assess changes in LDL-C, with exploratory endpoints evaluating changes in liver fat content, inflammatory markers and histological changes. We expect to commence the clinical trial in the first half of 2016 and complete the clinical trial in the fourth quarter of 2016 or the first quarter of 2017. Upon conclusion, we expect to be in a position to move forward in either hypercholesterolemia or NASH.

## Opportunity in X-ALD

We are developing VK0214 for X-ALD, a rare X-linked, inherited neurological disorder characterized by a breakdown in the protective barriers surrounding brain and nerve cells. The disease, for which there is no approved treatment, is caused by mutations in a peroxisomal transporter of very long chain fatty acids, or VLCFA, known as ABCD1. As a result, transporter function is impaired and patients are unable to efficiently metabolize VLCFA. VK0214 is a novel selective TR $\beta$  agonist. TR $\beta$  is known to regulate expression of an alternative VLCFA transporter, known as ABCD2. Various preclinical models have demonstrated that increased expression of ABCD2 can lead to normalization of VLCFA metabolism. Preliminary in vitro data suggest that VK0214 stimulates

ABCD2 expression. We are conducting studies of VK0214 in an in vivo model of disease. Pending completion of this work, we expect in 2016 to commence work directed toward filing an IND.

X-ALD is a rare, often fatal condition believed to occur with an incidence of approximately one in 17,000 births. X-ALD is caused by mutations in the gene encoding for ABCD1, which is located on the X chromosome. Men have one X chromosome, while women have two copies. Because of this, an inherited mutation in the ABCD1 gene is more likely to manifest in males relative to females. The ABCD1 protein plays a critical role in the transport of VLCFA into a cellular organelle called the peroxisome, where VLCFA metabolism and disposal occur. Without functional ABCD1, VLCFA accumulate in cells, including neural cells, where they can lead to membrane disruption and damage to the myelin sheath, a protective and insulating membrane that surrounds nerve cells in the brain. This damage can result in decreased motor coordination and function, visual and hearing disturbances, the loss of cognitive function, dementia, seizures, adrenal dysfunction and other complications, including death. X-ALD is divided into various sub-segments, which are broadly characterized by the presence or absence of brain inflammation:

- Cerebral adrenoleukodystrophy, or CALD: The most severe form of X-ALD is CALD. CALD is characterized by a progressive inflammatory destruction of myelin, leading to severe loss of neurological function and eventual death. Approximately 35% to 40% of male X-ALD patients present with cerebral involvement at a younger age, between the ages of 5 and 12 years. However, up to 20% of male X-ALD patients develop cerebral involvement later in life, between the ages of 20 and 35 years. In male children affected by CALD, learning and behavioral problems are often the first clinical manifestations of disease. In the absence of intervention, patients affected by CALD typically experience rapid degeneration into vegetative state within 3 to 5 years, often resulting in death within 10 years of diagnosis.

- Adrenomyeloneuropathy, or AMN: AMN is the more common form of X-ALD and is considered the default form of the disease in patients surviving beyond childhood. AMN is expected to affect all adult males with ABCD1 mutations, and approximately 65% of females. In males, the diagnosis is usually made between the ages of 20 and 50 and in females after the age of 65. AMN accounts for approximately half of all patients diagnosed with X-ALD. Patients with AMN generally present with slowly progressive symptoms resulting from (non-inflammatory) disruption of the axons, which are a fundamental component of the central nervous system (which allows nerve signals to be transmitted), in the spinal cord. Patients experience a variety of symptoms, including weakness in the legs, impaired vibration sense, incontinence and impotence. Severe motor disability, requiring the use of a wheelchair or cane, develops over a 3 to 15 year period. Many patients experience lower limb paralysis. While AMN is generally considered to develop more gradually relative to CALD, approximately 35% of AMN patients experience a rapid progression of myelopathy over a three to five year period. In addition, approximately 40% of AMN patients have or will develop CALD, with varying degrees of associated inflammation.

#### No Treatment Options for Majority of X-ALD Manifestations

There is a clear unmet medical need for patients suffering from X-ALD. CALD has been more commonly targeted for treatment due to its devastating effects, which are often manifested at a young age. For these patients, the only currently effective treatment option is allogeneic hematopoietic stem cell, or HSC, transplant. In this procedure, the patient is treated with HSCs containing the properly functioning copy of the ABCD1 gene, contributed by a donor other than the patient. Additionally, a method of ex vivo insertion of a functional copy of the ABCD1 gene via an HIV-1 based lentiviral vector into the patient's own HSCs to correct the aberrant expression of ABCD1 in patients with CALD is also in development. Over time with either method, as the transplanted cells grow and repopulate, a partial restoration of ABCD1 function can be achieved, leading many patients to resolution of progression in the cerebral form of the disease. While these forms of genetic correction have also shown potential clinical benefits, there is currently no approved therapy for X-ALD. In addition, recent data suggest that, even among successfully transplanted patients, AMN can develop. We believe our thyroid receptor agonists, which have the potential to

normalize metabolism of VLCFAs peripherally, and potentially centrally, may positively impact all forms of X-ALD, including the currently untreatable AMN form.

#### VK0214 Development Plans

We also plan to complete ongoing preclinical experiments with VK0214 in cell and animal models of X-ALD. We plan to complete an in vivo study in an established model of disease and report preliminary data in 2016. Pending completion of this work, we expect in 2016 to commence work directed toward filing an IND.

#### Three Pipeline Programs Target Metabolic Disease with Large Unmet Medical Need

We have a pipeline with three additional programs targeting metabolic diseases and anemia. Our pipeline programs include VK0612, a first-in-class, orally available Phase 2b-ready drug candidate for type 2 diabetes. Preliminary clinical data suggest VK0612 has the potential to provide substantial glucose-lowering effects, with an attractive safety and convenience profile compared with existing type 2 diabetes therapies. Our preclinical programs are focused on identifying orally available erythropoietin receptor, or EPOR,



agonists, for the potential treatment of anemia, and on the development of tissue-selective inhibitors of diacylglycerol acyltransferase-1, or DGAT-1, for the potential treatment of obesity and dyslipidemia.

#### Fructose-1,6-bisphosphatase (FBPase) Inhibitor Program

VK0612 is a first-in-class, orally available drug candidate for type 2 diabetes, one of the largest global healthcare challenges today. Preliminary clinical data suggest VK0612 has the potential to provide substantial glucose-lowering effects, with an attractive safety and convenience profile compared with existing type 2 diabetes therapies.

VK0612 is a potent, selective inhibitor of fructose-1,6-bisphosphatase, or FBPase, an enzyme that plays an important role in endogenous glucose production, or the synthesis of glucose by the body. We believe the inhibition of FBPase provides an attractive approach to controlling blood glucose levels in patients with diabetes. VK0612 has demonstrated potent glucose lowering effects in diabetic animal models. Clinical trials have shown that VK0612 is safe, well-tolerated and leads to significant glucose-lowering effects in patients with type 2 diabetes. Pending receipt of sufficient funding, we intend to commence additional clinical trials of VK0612 in patients with type 2 diabetes.

VK0612 has been evaluated in seven clinical trials, including one Phase 2a and six Phase 1 clinical trials. The first five Phase 1 clinical trials were conducted between 2006 and 2007. These were followed by the Phase 2a clinical trial, which was initiated in 2007 and completed in 2008, and the Phase 1b clinical trial, which was conducted in 2008. Based on these clinical and additional preclinical data, we believe VK0612 has the following important advantages over many existing type 2 diabetes therapies:

- Greater efficacy: Preliminary Phase 1 and 2 data suggest VK0612 could reduce plasma glycated hemoglobin A1c, or HbA1c, an important measure of long-term blood glucose levels, by 1% or more, potentially exceeding the typical anti-glycemic effects of newer drug classes.
- Encouraging safety profile: VK0612 has demonstrated encouraging safety to date in over 250 subjects. No cases of hypoglycemia, or low blood glucose levels, lacticemia, or sustained lactic acid in the blood, or other drug-related safety issues were observed in these subjects.
- Improved tolerability: VK0612 has been well-tolerated at and above doses that we plan to administer in our Phase 2b clinical trial, which we expect to be at or below 300 mg, with specific doses to be chosen based on the outcome of planned pharmacokinetic and pharmacodynamic calculations.
- Novel mechanism of action: Based on its insulin-independent mechanism of action, we believe VK0612 lowers blood glucose levels independently of pancreatic function. We expect VK0612's novel mechanism of action to provide critical durability and combinability advantages.
- Durability: Diabetes is characterized by deteriorating pancreatic beta cell function. Given VK0612's insulin-independent mechanism of action, the drug could provide a more durable therapeutic effect than many currently available type 2 diabetes therapies.
- Combinability: VK0612's novel mechanism of action is expected to allow combinability with many existing type 2 diabetes therapies, leading to enhanced efficacy and potentially delaying transition to subsequent therapies.
- Weight and lipid neutral profile: Clinical and preclinical data suggest VK0612 has the potential to provide robust anti-glycemic effects while maintaining a weight and lipid neutral profile.
- Once-daily convenience: Clinical data suggest that VK0612 has the potential to lower blood glucose levels in type 2 diabetes patients as a once-daily oral therapy.

We plan to commence a Phase 2b clinical trial with VK0612 in type 2 diabetes patients at a future date. Pending clinical data from this clinical trial, we may request an end-of-Phase 2 meeting with the FDA or may seek to commence Phase 3 clinical trials in type 2 diabetes patients either on our own or with a third party. The purpose of an end-of-Phase 2 meeting with the FDA would be to review our data with the FDA, discuss appropriate potential Phase 3 clinical trial designs and obtain agreement between us and the FDA on Phase 3 efficacy and safety objectives.

EPO Receptor (EPOR) Agonist Program

We are developing small molecule agonists of the erythropoietin, or EPO, receptor, or EPOR, for the potential treatment of anemia. Anemia results from a decrease in red blood cells and is typically experienced by patients with renal complications, cancer patients and HIV/AIDS patients. These patients currently receive recombinant human EPO and other erythropoiesis-stimulating agents, or ESAs. Total worldwide sales of these agents exceeded \$6.0 billion in 2014. However, these agents have a number of limitations, including cost of drug manufacturing, cost of treatment, a non-oral route of administration, and potential for immunogenicity, or possibility of inducing an immune response. Furthermore, ESA treatment is associated with an increased risk of adverse

cardiovascular complications in patients with kidney disease when used to increase hemoglobin levels above 13.0 g/dL, and may be related to an increase in mortality in cancer patients. We believe that our drug candidates have the potential to treat anemia with improved safety, tolerability and route of administration. We plan to conduct further preclinical studies and file an IND with the FDA at a future date.

#### Diacylglycerol Acyltransferase-1 (DGAT-1) Inhibitor Program

We are developing small molecule inhibitors of the enzyme DGAT-1 for the potential treatment of lipid disorders such as obesity and dyslipidemia. According to the CDC, approximately 36% of the adult U.S. population is obese, with the prevalence expected to exceed 40% by 2018. The World Health Organization estimates at least 500.0 million people are currently obese worldwide. DGAT-1 is a potential therapeutic target for reduction of triglyceride levels in the circulation and fat accumulation in adipose tissues. DGAT-1 null mice exhibit both reduced post-meal plasma triglyceride levels and increased energy expenditure, but have normal levels of circulating free fatty acids. Conversely, transgenic mice that overexpress DGAT-1 in adipose tissue are predisposed to obesity when fed a high-fat diet and have elevated levels of circulating free fatty acids. We have developed a series of novel compounds with tissue-targeting properties intended to mitigate potential side effects by selectively targeting the enterocyte, or intestinal absorptive cells, in the intestine, to inhibit dietary triglyceride uptake, or the liver, to inhibit de novo triglyceride synthesis. We plan to conduct further preclinical studies and file an IND with the FDA at a future date.

#### Competition

The biopharmaceutical industry is characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. While we believe that our technology, knowledge, experience and scientific resources provide us with competitive advantages, we face potential competition from many different sources, including commercial biopharmaceutical enterprises, academic institutions, government agencies and private and public research institutions. Any drug candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future.

Many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical studies, clinical trials, regulatory approvals and marketing approved products than we do. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. Our competitors may succeed in developing technologies and therapies that are more effective, better tolerated or less costly than any which we are developing, or that would render our drug candidates obsolete and noncompetitive. Even if we obtain regulatory approval of any of our drug candidates, our competitors may succeed in obtaining regulatory approvals for their products earlier than we do. We will also face competition from these third parties in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, and in acquiring and in-licensing technologies and products complementary to our programs or advantageous to our business.

The key competitive factors affecting the success of each of our drug candidates, if approved, are likely to be its efficacy, safety, tolerability, frequency and route of administration, convenience and price, the level of branded and generic competition and the availability of coverage and reimbursement from government and other third-party payors.

#### VK5211

In the U.S., there are currently no marketed therapies for the maintenance or improvement of LBM, BMD or physical function in patients recovering from non-elective hip fracture surgery. However, VK5211, if approved, will face competition from several experimental therapies that are in various stages of development for acute rehabilitation

following hip fracture surgery, including programs in development at Novartis AG and Morphosys AG. There are also several experimental therapies that are in various stages of clinical development for conditions characterized by muscle wasting by companies including GTx, Inc., Helsinn Group and Morphosys AG. In addition, nutritional and growth hormone-based therapies are sometimes used in patients experiencing muscle wasting.

#### VK2809

There are many therapies currently available and numerous others being developed for the treatment of hypercholesterolemia and dyslipidemia. If approved, VK2809 will face competition from therapies that are currently available and from therapies that may become available in the future. Generic statin therapies such as atorvastatin are widely prescribed for the initial treatment of hypercholesterolemia. Cholesterol absorption inhibitors such as Merck & Co., Inc.'s Zetia (ezetimibe), generic bile acid sequestrants such as colestevam and generic fibrates such as fenofibrate are also prescribed for the treatment of hypercholesterolemia. Various combinations of these therapies are often prescribed for patients suffering from dyslipidemia. In addition, recently-approved antibody

therapies targeting the proprotein convertase subtilisin/kexin type 9 (PCSK9) gene are expected to be prescribed for patients whose LDL remains elevated despite treatment with existing cholesterol-lowering agents. While no therapies are currently approved for the treatment of NASH, we are aware of several development-stage programs targeting this disease, including obeticholic acid from Intercept Pharmaceuticals, Inc., GFT505 from Genfit SA, aramchol from Galmed Pharmaceuticals Ltd., simtuzumab from Gilead Sciences, Inc., and emricisan from Conatus Pharmaceuticals Inc.

#### VK0214

In the U.S., there are currently no marketed therapies for the treatment of X-ALD. Hematopoietic stem cell therapy has been used to treat the most severe form of X-ALD, CALD. More recently, gene therapy has been shown to be effective in CALD as well. However, both treatments are invasive, requiring surgical intervention, and these do not appear to have an effect on the most pervasive form of X-ALD, AMN. High-dose biotin is under investigation for treatment of AMN. There are several experimental therapies that are in various stages of clinical development for X-ALD by companies, including MedDay Pharmaceuticals SAS and bluebird bio, Inc., which may be competitive with VK0214, if approved.

#### VK0612

In the U.S., VK0612, if approved, will face competition from a variety of currently marketed oral type 2 diabetes therapies, including metformin (generic), pioglitazone (generic), glimepiride (generic), sitagliptin (Merck & Co., Inc.) and canagliflozin (Johnson & Johnson). These therapies are well-established and are widely accepted by physicians, patients, caregivers and third-party payors as the standard of care for the treatment of type 2 diabetes. Physicians, patients and third-party payors may not accept the addition of VK0612 to their current treatment regimens for a variety of potential reasons, including:

- if they do not wish to incur any potential additional costs related to VK0612; or
- if they perceive the use of VK0612 to be of limited additional benefit to patients.

In addition to the currently approved and marketed type 2 diabetes therapies, there are a number of experimental drugs that are in various stages of clinical development by companies such as Eli Lilly and Company, Takeda Pharmaceutical Company Limited and TransTech Pharma, Inc.

#### Preclinical Programs Focused on EPOR Agonists and DGAT-1 Inhibitors

If any of our preclinical programs are ultimately determined safe and effective and approved for marketing, they may compete for market share with established therapies from a number of competitors, including large biopharmaceutical companies. Many therapies are currently available and numerous others are being developed for the treatment of anemia and obesity. Any products that we may develop from our preclinical programs may not be able to compete effectively with existing or future therapies.

#### Manufacturing and Supply

We do not have any manufacturing facilities and do not intend to develop any manufacturing capabilities. We believe that we currently possess sufficient VK5211 and VK2809 drug substance to allow for completion of our planned VK5211 and VK2809 Phase 2 clinical trials. Bulk active pharmaceutical ingredient, or API, and certain dosage forms are currently in storage in compliance with cGMP requirements. We believe that a majority of the existing API will be suitable for formulation into clinical trial material. We also have identified multiple contract manufacturers to provide commercial supplies of the formulated drug candidates if they are approved for marketing. We intend to secure contract manufacturers with established track records of quality product supply and significant experience with the

regulatory requirements of the FDA and EMA.

#### Our History

We were incorporated under the laws of the State of Delaware on September 24, 2012. Since our incorporation, we have devoted substantially all of our efforts to raising capital, building infrastructure, obtaining the worldwide rights to certain technology from Ligand, including VK5211, VK2809 and VK0612, and more recently following the IPO to planning, preparing for and commencing certain preclinical studies and clinical trials of our drug candidates. Each of our programs is based on small molecules licensed from Ligand pursuant to our Master License Agreement with Ligand, which we entered into on May 21, 2014.

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## Agreements with Ligand

### Master License Agreement

On May 21, 2014, we entered into a Master License Agreement, as amended on each of September 6, 2014 and April 8, 2015, or the Master License Agreement, with Ligand pursuant to which, among other things, Ligand granted to us and our affiliates an exclusive, perpetual, irrevocable, worldwide, royalty-bearing right and license under (1) patents related to (a) our VK5211 program and any other compounds comprised by specified SARM patents and derivatives of such compounds, or SARM Compounds, (b) our VK2809 and VK0214 programs and any other compounds comprised by specified TR $\beta$  patents and any derivatives of such compounds, or TR $\beta$  Compounds, (c) our VK0612 program and any other compounds comprised by specified FBPase patents and derivatives of such compounds, or FBPase Compounds, (d) our EPOR program and any other compounds comprised by specified EPOR patents and derivatives of such compounds, or EPOR Compounds, and (e) our DGAT-1 program and any other compounds comprised by specified DGAT-1 patents and derivatives of such compounds, or DGAT-1 Compounds; (2) related know-how controlled by Ligand; and (3) physical quantities of SARM, TR $\beta$ , FBPase, EPOR and DGAT-1 Compounds, or, collectively, the Licensed Technology, to research, develop, manufacture, have manufactured, use and commercialize the Licensed Technology in and for all therapeutic and diagnostic uses in humans or animals. We have the right to sublicense these rights in certain circumstances. Pursuant to the terms of the Master License Agreement, we have the exclusive right and sole responsibility and decision-making authority for researching and developing any pharmaceutical products that contain or comprise one or any combination of a SARM Compound, TR $\beta$  Compound, FBPase Compound, EPOR Compound or DGAT-1 Compound, or, collectively, the Licensed Products. We also have the exclusive right and sole responsibility and decision-making authority to conduct all clinical trials and preclinical studies that we believe are appropriate to obtain the regulatory approvals necessary for commercialization of the Licensed Products, and we will own and maintain all regulatory filings and all regulatory approvals for the Licensed Products. Additionally, pursuant to the terms of the Master License Agreement, we have the sole decision-making authority and responsibility and the exclusive right to commercialize any of the Licensed Products, either by ourselves or, in certain circumstances, through sublicensees selected by us. We also have the exclusive right to manufacture or have manufactured any Licensed Product ourselves or, in certain circumstances, through sublicensees or third parties selected by us. We will own any intellectual property that we develop in connection with the license granted under the Master License Agreement.

As partial consideration for the grant of the rights and licenses to us under the Master License Agreement, we issued to Ligand at the closing of the IPO 3,655,964 shares of our common stock having an estimated aggregate value of \$29.2 million. Furthermore, as partial consideration for the grant of the rights and licenses to us under the Master License Agreement, we entered into the Loan and Security Agreement with Ligand (as discussed below).

As further partial consideration for the grant of the rights and licenses to us by Ligand under the Master License Agreement, we have agreed to pay to Ligand certain one-time, non-refundable milestone payments in connection with licensed products containing (1) VK5211 or any other SARM Compound, in an aggregate amount of up to \$85.0 million per indication (for up to a total of two indications) upon the achievement of certain development and regulatory milestones and up to \$100.0 million upon the achievement of certain sales milestones; (2) VK2809, VK0214 or any other TR $\beta$  Compound, in an aggregate amount of up to \$75.0 million per indication (for up to a total of three indications) upon the achievement of certain development and regulatory milestones and up to \$150.0 million upon the achievement of certain sales milestones; (3) VK0612 or any other FBPase Compound, in an aggregate amount of up to \$60.0 million per indication (for up to a total of four indications) upon the achievement of certain development and regulatory milestones and up to \$150.0 million upon the achievement of certain sales milestones; (4) any EPOR Compound, in an aggregate amount of up to \$48.0 million per indication (for up to a total of three indications) upon the achievement of certain development and regulatory milestones and up to \$50.0 million upon the achievement of certain sales milestones; and (5) any DGAT-1 Compound, in an aggregate amount of up to

\$78.0 million per indication (for up to a total of two indications) upon the achievement of certain development and regulatory milestones and up to \$150.0 million upon the achievement of certain sales milestones. Additionally, we will pay to Ligand a one-time, non-refundable milestone payment of \$2.5 million upon the occurrence of the first commercial sale of VK0612 or any other FBPase Compound by one of our sublicensees. We will also pay to Ligand royalties on aggregate annual worldwide net sales of Licensed Products by us, our affiliates and our sublicensees at tiered percentage rates in the following ranges based upon net sales: (a) upper single digit royalties upon sales of VK5211 or any other SARM Compound, (b) low-to-middle single digit royalties upon sales of VK2809, VK0214 or any other TR $\beta$  Compound, (c) upper single digit royalties upon sales of VK0612 or any other FBPase Compound, (d) middle-to-upper single digit royalties upon sales of any EPOR Compound, and (e) low-to-middle single digit royalties upon sales of any DGAT-1 Compound; in each case subject to reduction in certain circumstances.

The term of the Master License Agreement will continue unless the agreement is terminated by us or Ligand. Ligand has the right to terminate the Master License Agreement under certain circumstances, including, but not limited to: (1) in the event of our insolvency or bankruptcy; (2) if we do not pay an undisputed amount owing under the Master License Agreement when due and fail to cure such default within a specified period of time; or (3) if we default on certain of our material and substantial obligations and fail to cure the default within a specified period of time. We have the right to terminate the Master License Agreement under certain circumstances,



including, but not limited to: (i) if Ligand does not pay an undisputed amount owing under the Master License Agreement when due and fails to cure such default within a specified period of time, or (ii) if Ligand defaults on certain of its material and substantial obligations and fails to cure the default within a specified period of time. In addition, provisions of the Master License Agreement can be terminated on a licensed program-by-program basis under certain circumstances. In the event that the Master License Agreement is terminated in its entirety or with respect to a specific licensed program for any reason: (A) all licenses granted to us under the Master License Agreement (or with respect to the specific licensed program) will terminate and we will, upon Ligand's request (subject to Ligand assuming legal responsibility for any clinical trials of the Licensed Products then ongoing), assign and transfer to Ligand (or to such transferee as Ligand may direct), at no cost to Ligand, all regulatory documentation and all regulatory approvals prepared or obtained by us or on our behalf related to the Licensed Products (or those related to the specific licensed program), or, if Ligand does not make such a request, we will wind down any ongoing clinical trials with respect to the Licensed Products (or those related to the specific licensed program) at no cost to Ligand; (B) we will, upon Ligand's request, sell and transfer to Ligand (or to such transferee as Ligand may direct), at a price equal to 125% of our costs of goods, any and all chemical, biological or physical materials relating to or comprising the Licensed Products (or those related to the specific licensed program); (C) we will have, for a period of six months following termination, the right to sell on the normal business terms in existence before such termination any finished commercial inventory of Licensed Products (or those related to the specific licensed program) which remains on hand, so long as we pay to Ligand the applicable royalties and sales milestones; (D) Ligand has the right to require us to assign to Ligand the trademarks owned by us relating to the Licensed Products (or those related to the specific licensed program); and (E) we will grant to Ligand a non-exclusive, worldwide, royalty-bearing sublicensable license under any patent rights and know-how controlled by us to the extent necessary to make, have made, import, use, offer to sell and sell the Licensed Products (or those related to the specific licensed program) anywhere in the world at a royalty rate in the low single digits.

Under the Master License Agreement, we have agreed to indemnify Ligand for claims relating to the performance of our obligations under the Master License Agreement, any breach of the representations and warranties made by us under the Master License Agreement, clinical trials conducted by us and the research, development and commercialization of the Licensed Products by us and our affiliates, sublicensees, distributors and agents. In addition, Ligand has agreed to indemnify us for claims relating to the performance of its obligations under the Master License Agreement, its breach of representations and warranties under the agreement and its research and development of the licensed compounds before the effective date of the Master License Agreement. Each party's indemnification obligations will not apply to the extent the claims result from the negligence or willful misconduct of the indemnified party or any of its employees, agents, officers or directors or from the indemnified party's breach of its representations or warranties set forth in the Master License Agreement.

#### Loan and Security Agreement

In connection with entering into the Master License Agreement, we entered into a Loan and Security Agreement with Ligand, dated May 21, 2014, as amended on April 8, 2015 and January 22, 2016, or the Loan and Security Agreement, pursuant to which, among other things, Ligand agreed to provide us with loans in the aggregate amount of up to \$2.5 million. Pursuant to the Loan and Security Agreement, Ligand loaned us \$2.5 million through December 31, 2014. From May 21, 2014 to January 21, 2016, the principal amount outstanding under the loans accrued interest at a fixed per annum rate equal to the lesser of 5.0% and the maximum interest rate permitted by law. Effective as of January 22, 2016, the principal amount outstanding under the loans accrue interest at a fixed per annum rate equal to the lesser of 2.5% and the maximum interest rate permitted by law. In the event we default under the loans, the loans will accrue interest at a fixed per annum rate equal to the lesser of 8% and the maximum interest rate permitted by law.

Each of the loans is evidenced by a Secured Convertible Promissory Note, or the Ligand Note. Pursuant to the terms of the Loan and Security Agreement and the Ligand Note, the loans will become due and payable upon the written demand of Ligand at any time after the earlier to occur of an event of default under the Loan and Security Agreement or the Note, or May 21, 2017, or the Maturity Date, unless the loans are repaid in cash or converted into equity prior to such time. Upon the consummation of our first bona fide capital financing transaction occurring after January 22, 2016, but prior to the Maturity Date, with aggregate net proceeds to us of at least \$2,000,000, or the Next Financing, we will be required to repay \$1,500,000 of the Ligand Note obligation to Ligand, with at least \$300,000 of such payment to be paid in cash (with any greater cash amount determined by us in our sole and absolute discretion), and the balance of the \$1,500,000 payment, or the Balance, to be paid in the form of such number of shares of our equity securities that are issued in the Next Financing equal to the quotient obtained by dividing the Balance by the lesser of (1) the lowest price per share paid by investors in the Next Financing, or the Financing Price, and (2) \$8.00 (subject to adjustment for stock dividends, splits, combinations or similar transactions). Notwithstanding the foregoing, the number of shares that we may issue to Ligand will be reduced to the extent that the issuance of such shares would increase Ligand's beneficial ownership of our common stock to greater than 49.9%, and any remaining amount of the Balance would have to be paid in cash. Each \$1.00 of the \$1,500,000 payment will reduce the amount of accrued and unpaid interest and then unpaid principal amount of the loans under the Ligand Note by \$0.50.

In addition, following the consummation of the Next Financing, we may repay any portion of the outstanding principal amount of the loans under the Ligand Note, plus accrued and previously unpaid interest thereon, by delivering a notice to Ligand, or the Additional Repayment Notice, specifying the amount that we wish to repay, or the Additional Payment Amount. Ligand will have five days to elect to receive the Additional Payment Amount in cash, shares of our common stock or a combination of cash and shares of our common stock. If Ligand does not make an election within such five-day period, the form of the Additional Payment Amount will be at our sole election and discretion, subject to the number of shares of common stock being reduced to the extent that the issuance of shares would increase Ligand's beneficial ownership of our common stock to greater than 49.9%. To the extent that any portion of an Additional Payment Amount will be paid in the form of shares of our common stock, the number of shares issuable will be equal to the quotient obtained by dividing the portion of the Additional Payment Amount that will be paid in shares by the lesser of (1) (a) if we deliver the Additional Repayment Notice within 180 days of the closing of the Next Financing, the Financing Price, or (b) if we deliver the Additional Repayment Notice 180 days or more after the closing of the Next Financing, the volume weighted average closing price of our common stock for the 30 days prior to the date we deliver the Additional Repayment Notice, and (2) \$8.00 (subject to adjustment for stock dividends, splits, combinations or similar transactions). Each \$1.00 of any Additional Repayment Amount will reduce the amount of accrued and unpaid interest and then unpaid principal amount under the Ligand Note by \$0.50. Additionally, pursuant to the Loan and Security Agreement, Ligand has agreed that it will not, until January 23, 2017, sell or otherwise transfer or dispose of any of our securities, including shares issuable upon conversion of the Ligand Note.

Following the Maturity Date, Ligand may demand repayment of the loans under the Ligand Note in full. If (1) the Ligand Note is not repaid in full prior to the Maturity Date and Ligand demands repayment, or (2) we wish to repay the full amount owed under the Ligand Note prior to the Maturity Date, we will be obligated to repay to Ligand an amount equal to 200% of the aggregate of the outstanding principal amount of the loans under the Ligand Note and of all accrued and unpaid interest thereon, or the Remaining Balance.

In either case, we may, at our sole election and discretion, elect to pay the Remaining Balance solely in cash. If we do not elect to repay the Remaining Balance in cash, the form of payment and mix of cash and shares of our common stock will be at Ligand's sole election and discretion. To the extent that any portion of the Remaining Balance will be paid in the form of shares of our common stock, the number of shares issuable will be equal to the quotient obtained by dividing the portion of the Remaining Balance that will be paid in shares by the lesser of (1) the volume weighted average closing price of our common stock for the 30 days prior to the date of Ligand's demand for repayment or the date of our repayment of the Remaining Balance in full, and (2) \$8.00 (subject to adjustment for stock dividends, splits, combinations or similar transactions).

We also granted Ligand a continuing security interest in all of our right, title and interest in and to our assets as collateral for the full, prompt, complete and final payment and performance when due of all obligations under the Loan and Security Agreement and the Ligand Note.

Under the Loan and Security Agreement and the Ligand Note, we are subject to affirmative and negative covenants. We agreed to, among other things, deliver financial statements, forecasts and budget information to Ligand. In addition, we agreed to use the proceeds from the loans solely as working capital and to fund our general business requirements in accordance with our forecast and budget. Under the Loan and Security Agreement and the Ligand Note, we may not take certain actions without Ligand's consent, such as declare or pay dividends, incur or repay certain indebtedness or engage in certain related party transactions. Ligand has the right to transfer the Ligand Note at any time without our permission.

An event of default under the Loan and Security Agreement will be deemed to occur or exist upon the termination of the Master License Agreement; in the event we fail to make principal or interest payments under the Ligand Note

when due; if we become insolvent or breach and fail to cure within a specified period of time any representation, warranty, covenant or agreement in the Loan and Security Agreement, the Master License Agreement, the Option Agreement, dated September 27, 2012, by and between us and Ligand, as amended, the Voting Agreement (as defined below) or the Management Rights Letter (as defined below); or upon the occurrence of certain other events. Additionally, pursuant to the Loan and Security Agreement, Ligand has agreed that it will not, until January 23, 2017, sell or otherwise transfer or dispose of any of our securities, including shares issuable upon conversion of the Ligand Note.

#### Management Rights Letter

As a condition to entering into the Master License Agreement, the Loan and Security Agreement and the Ligand Note, we entered into a Management Rights Letter with Ligand, dated as of May 21, 2014, or the Management Rights Letter. Pursuant to the Management Rights Letter, we agreed to: (1) expand the size of our board of directors so as to create one new directorship on our board of directors, and (2) appoint an individual named by Ligand, or the Ligand Director, to fill the newly-created directorship. Pursuant to the terms of the Management Rights Letter, the Ligand Director is entitled to receive the same compensation, including cash payments and equity incentive grants, as is provided to our other directors; however, the Ligand Director is not entitled to receive the compensation

provided to our directors in their capacity as members of a committee of our board of directors. Furthermore, we agreed to provide Ligand with advance written notice of the date of the annual meeting of our stockholders for each year in which the Ligand Director is up for election so as to permit Ligand to designate the Ligand Director for election at such annual meeting, and to nominate the Ligand Director to our board of directors at each such annual meeting of our stockholders. In addition, under the Management Rights Letter, we granted Ligand certain contractual management rights in the event Ligand is not represented on our board of directors, including the right to consult with us and offer advice to our management on significant business issues and the right to receive copies of all notices, minutes, consents and other material that we provide to our directors, subject to certain exceptions. We also agreed that, upon the consummation of the IPO, we would appoint a Chairperson of our board of directors who is “independent” under applicable SEC rules and the rules and listings standards of The Nasdaq Stock Market LLC. In accordance with the terms of the Management Rights Letter, Matthew W. Foehr was appointed to our board of directors as the Ligand Director and Lawson Macartney, DVM, Ph.D. was appointed Chairperson of our board of directors. The Management Rights Letter will terminate upon the earliest to occur of: (a) the liquidation, dissolution or indefinite cessation of our business operations; (b) the execution by us of a general assignment for the benefit of creditors or the appointment of a receiver or trustee to take possession of our property and assets; (c) an acquisition of us by means of any transaction or series of related transactions (including, without limitation, any reorganization, merger or consolidation) if our stockholders of record as constituted immediately prior to such transaction hold less than 50% of the voting power of the surviving or acquiring entity; (d) the date that Ligand and its affiliates collectively cease to beneficially own at least 7.5% of our outstanding voting stock; or (e) May 21, 2024.

#### Voting Agreement

In connection with the terms of the Management Rights Letter, we, Ligand, Brian Lian, Ph.D., and Michael Dinerman, M.D., our former Chief Operating Officer, entered into a Voting Agreement dated as of May 21, 2014, or the Voting Agreement, pursuant to which each of Ligand, Dr. Lian and Dr. Dinerman agreed to vote all of his or its shares of our voting securities so as to elect the Ligand Director as a member of our board of directors, and, if requested by Ligand, to vote in favor of any removal of the Ligand Director or selection of a new Ligand Director. The Voting Agreement will terminate under the same circumstances in which the Management Rights Letter will terminate.

#### Registration Rights Agreement

As a condition to the parties entering into the Master License Agreement and the Loan and Security Agreement, we entered into a Registration Rights Agreement, dated May 21, 2014, as amended on January 22, 2016, with Ligand, or the Registration Rights Agreement, pursuant to which we granted certain registration rights to Ligand with respect to (1) the securities issued by us to Ligand pursuant to the Master License Agreement and the securities issuable by us to Ligand pursuant to the Ligand Note, or, collectively, the Viking Securities, (2) the shares of our common stock issued or issuable upon conversion of the Viking Securities, if applicable, and (3) the shares of our common stock issued as a dividend or other distribution with respect to, in exchange for or in replacement of the Viking Securities, or, collectively, the Registrable Securities.

#### Mandatory Resale Registration Rights

Pursuant to the Registration Rights Agreement, we have agreed that we will file with the Securities and Exchange Commission, or the SEC, by no later than January 23, 2017, a Registration Statement on Form S-1 under the Securities Act of 1933, as amended, or the Securities Act, that covers the resale of the full amount of the Registrable Securities. We are obligated to use commercially reasonable efforts to have the Registration Statement declared effective by the SEC as soon as practicable after it is filed with the SEC, but in no event later than (1) in the event the SEC Staff does not review the Registration Statement, March 24, 2017 or (2) in the event the SEC Staff reviews the Registration Statement, May 23, 2017. If we do not file a Registration Statement for the resale of the Registrable

Securities within the requisite time period, if such Registration Statement is not declared effective by the SEC Staff by a certain date, or if, on any day after the Registration Statement is declared effective by the SEC Staff, sales of all of the Registrable Securities required to be included in the Registration Statement cannot be made pursuant to the Registration Statement, then we will, subject to certain exceptions, be obligated to pay to Ligand an amount in cash equal to 1% of the aggregate value of the Registrable Securities, measured as of the date of their issuance, on the day of such failure or ineffectiveness of, or inability to use, the Registration Statement and on every thirtieth day thereafter (pro-rated for partial periods) until such failure or ineffectiveness of, or inability to use, the Registration Statement is cured; up to a maximum of 5% of the aggregate value of the Registrable Securities, measured as of the date of their issuance.

Pursuant to the Registration Rights Agreement, in the event the SEC Staff takes the position that the registration of some or all of the Registrable Securities is not eligible to be made on a delayed or continuous basis under the provisions of Rule 415 under the Securities Act, or would require Ligand to be named as an “underwriter” in the Registration Statement, we have agreed to use our commercially reasonable efforts to persuade the SEC Staff that the offering contemplated by the Registration Statement is a valid secondary offering, is not made “by or on behalf of the issuer” (as defined in Rule 415 under the Securities Act) and that Ligand is not an

“underwriter” for purposes of the registration. If the SEC Staff does not agree with our proposal, we will remove from the Registration Statement the portion of the Registrable Securities, and/or we and Ligand will agree to certain restrictions and limitations on the registration and resale of the Registrable Securities, as the SEC Staff may require to ensure the registration complies with Rule 415 under the Securities Act. In the event the SEC Staff imposes restrictions or limitations on the registration and resale of the Registrable Securities, then any amounts that we would be obligated to pay to Ligand as a result of the failure or ineffectiveness of, or inability to use, the Registration Statement, will not accrue until a certain period of time after the date that we determine we are able to effect the registration of such Registrable Securities in accordance with SEC rules and regulations.

Pursuant to the terms of the Registration Rights Agreement, we also agreed to use our commercially reasonable efforts to keep each Registration Statement filed pursuant to the agreement effective with respect to all Registrable Securities until the earlier of (1) the date on which all shares of Registrable Securities may immediately be sold under Rule 144, as promulgated by the SEC under the Securities Act, or Rule 144, during any 90-day period, or (2) the date on which all of the Registrable Securities covered by the Registration Statement that are held by Ligand are sold.

Additionally, we have the right during certain periods after the effective date of the Registration Statement covering the resale of the Registrable Securities, to delay the disclosure of material, non-public information if, in the good faith opinion of our board of directors, it is not in our best interests to disclose the information. In addition, we have the ability to prohibit sales under the Registration Statement during certain periods, subject to certain limitations.

#### Form S-3 Registration Rights

The Registration Rights Agreement also provides that after the IPO, we will use our commercially reasonable efforts to qualify for the use of Form S-3 for purposes of registering the issuance and/or resale of the Registrable Securities. Once we have qualified for the use of Form S-3, we have agreed to convert the Registration Statement on Form S-1 that is initially to be filed to register the resale of the Registrable Securities into a Registration Statement on Form S-3.

#### Limitation on Registration Rights

Pursuant to the terms of the Registration Rights Agreement, we have agreed that we will not, except with Ligand’s prior written consent, from and after the date of the Registration Rights Agreement and prior to the date the Registration Statement covering the resale of the full amount of the Registrable Securities is declared effective by the SEC, enter into an agreement with another holder or prospective holder of our securities which provides demand registration rights that are more favorable than the registration rights provided to Ligand under the Registration Rights Agreement.

#### Termination of Registration Rights

Ligand’s registration rights terminate upon the earlier of (1) the date on which all shares of Registrable Securities may immediately be sold under Rule 144 during any 90-day period, or (2) the date on which all of the Registrable Securities covered by the Registration Statement that are held by Ligand are sold.

#### Expenses

We will bear all registration expenses in connection with the mandatory resale registration rights granted pursuant to the Registration Rights Agreement, including but not limited to all registration, qualification and filing fees, except that we will not be required to pay selling expenses, fees and disbursements of counsel for the holders of our capital stock other than the fees and disbursements of one special counsel in an amount of up to \$20,000.

Representative's Warrant Registration Rights

Upon the closing of the IPO, on May 4, 2015, we issued to the representative of the underwriters in the IPO as additional compensation a warrant to purchase an aggregate of 82,500 shares of our common stock, or the Representative's Warrant.

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### Demand Registration Rights

Pursuant to the terms of the Representative's Warrant, we are obligated, upon the written demand of the holders of at least 51% of the shares issuable upon exercise of the Representative's Warrant, or the Registrable Warrant Shares, to register all or a portion of the Registrable Warrant Shares, on one occasion. Upon our receipt of a written demand notice, we must file a registration statement with the SEC covering the Registrable Warrant Shares within 60 days and use our commercially reasonable efforts to have the registration statement declared effective promptly thereafter. The holder of the Representative's Warrant may exercise this demand registration right at any time from April 28, 2016 until April 28, 2020. However, we will not be required to register any Registrable Warrant Shares that are the subject of a then-effective registration statement. Additionally, we will not be obligated to file a registration statement in connection with a demand notice if the holder of the Registrable Warrant Shares is entitled to certain piggyback registration rights.

To the extent we file a registration statement in connection with the demand registration rights granted under the Representative's Warrant, we have agreed to keep the registration statement effective until the earlier of (1) the one year anniversary of the effective date of the registration statement or (2) the date when all Registrable Warrant Shares covered by the registration statement have been sold.

### Piggyback Registration Rights

Pursuant to the terms of the Representative's Warrant, we have also agreed to provide the holder of the Registrable Warrant Shares with certain piggyback registration rights. Until April 28, 2022, the holder of the Registrable Warrant Shares has a right to include all or any portion of the Registrable Warrant Shares in a registration statement filed by us, subject to certain exceptions. However, we will not be required to register any Registrable Warrant Shares that are the subject of a then-effective registration statement.

### Expenses

We will pay all fees and expenses incurred in registering the Registrable Warrant Shares, but the holder of the Registrable Warrant Shares will pay any and all underwriting commissions and the expenses of any legal counsel selected by the holder of the Registrable Warrant Shares to represent it in connection with the sale of the Registrable Warrant Shares.

### Research Services Agreement with the Academic Medical Center at the University of Amsterdam

Effective January 27, 2015, we entered into a Research Services Agreement with the Academic Medical Center at the University of Amsterdam, or the Academic Medical Center, pursuant to which the Academic Medical Center agreed to perform from time to time certain research projects for us on a series of one or more compounds. The agreement provided that, following completion of its research under the agreement, the Academic Medical Center would provide us with a report of the results of each study performed, and that we would solely own all right, title and interest in the results of the research services. The Research Services Agreement further provided that, as compensation for its services, and after our acceptance of the applicable services, we would be obligated to pay to the Academic Medical Center the amount set forth in the Statement of Work relating to the specific research project.

The Research Services Agreement also provided that it would remain in effect until the end date provided for in the last Statement of Work to be executed under the agreement. We entered into one Statement of Work under the Research Services Agreement, pursuant to which the Academic Medical Center evaluated the ability of our proprietary TR $\beta$  agonists, including VK2809 and VK0214, to induce ABCD2 expression in both control cell lines and in cells from X-ALD patients. This Statement of Work was completed in July 2015, at which time the Research

Services Agreement terminated in accordance with its terms.

## Government Regulation

### FDA Regulation and Marketing Approval

In the U.S., the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act of 1938, as amended, or FDCA, and related regulations. Drugs are also subject to other federal, state and local statutes and regulations. Failure to comply with the applicable U.S. regulatory requirements at any time during the drug development process, approval process or after approval may subject an applicant to administrative or judicial sanctions and non-approval of drug candidates. These sanctions could include the imposition by the FDA or an Institutional Review Board, or IRB, of a clinical hold on clinical trials, the FDA's refusal to approve pending applications or related supplements, withdrawal of an approval, untitled or warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, restitution, disgorgement, civil penalties or criminal prosecution. Such actions by government agencies could also require us to expend a large amount of resources to respond to the actions. Any agency or judicial enforcement action could have a material adverse effect on us.

The FDA and comparable regulatory agencies in state and local jurisdictions and in foreign countries impose substantial requirements upon the clinical development, manufacture and marketing of pharmaceutical products.

These agencies and other federal, state and local entities regulate research and development activities and the testing, manufacture, quality control, safety, effectiveness, labeling, packaging, storage, distribution, record-keeping, approval, post-approval monitoring, advertising, promotion, sampling and import and export of our products. Our drugs must be approved by the FDA through the new drug application, or NDA, process before they may be legally marketed in the U.S. See “The NDA Approval Process” under Part I, “Item 1. Business” of this Annual Report on Form 10-K.

The process required by the FDA before drugs may be marketed in the U.S. generally involves the following:

- completion of non-clinical laboratory tests, animal studies and formulation studies conducted according to good laboratory practice or other applicable regulations;
- submission of an IND, which allows clinical trials to begin unless the FDA objects within 30 days;
- adequate and well-controlled human clinical trials to establish the safety and efficacy of the proposed drug for its intended use or uses conducted in accordance with FDA regulations, good clinical practices, or GCP, which are international ethical and scientific quality standards meant to assure that the rights, safety and well-being of trial participants are protected, and to define the roles of clinical trial sponsors, administrators and monitors and to assure clinical trial data integrity;
- pre-approval inspection of manufacturing facilities and clinical trial sites; and
- FDA approval of an NDA, which must occur before a drug can be marketed or sold.

#### IND and Clinical Trials

Prior to commencing the first clinical trial, an IND, which contains the results of preclinical studies along with other information, such as information about product chemistry, manufacturing and controls and a proposed protocol, must be submitted to the FDA. The IND automatically becomes effective 30 days after receipt by the FDA unless the FDA within the 30-day time period raises concerns or questions about the conduct of the clinical trial. In such a case, the IND sponsor must resolve any outstanding concerns with the FDA before the clinical trial may begin. A separate submission to the existing IND must be made for each successive clinical trial to be conducted during drug development. Further, an independent IRB for each site proposing to conduct the clinical trial must review and approve the investigational plan for any clinical trial before it commences at that site. Informed written consent must also be obtained from each trial subject. Regulatory authorities, including the FDA, an IRB, a data safety monitoring board or the sponsor, may suspend or terminate a clinical trial at any time on various grounds, including a finding that the participants are being exposed to an unacceptable health risk or that the clinical trial is not being conducted in accordance with FDA requirements.

For purposes of NDA approval, human clinical trials are typically conducted in sequential phases that may overlap:

- Phase 1 – the drug is initially given to healthy human subjects or patients in order to determine metabolism and pharmacologic actions of the drug in humans, side effects and, if possible, to gain early evidence on effectiveness. During Phase 1 clinical trials, sufficient information about the investigational drug’s pharmacokinetics and pharmacologic effects may be obtained to permit the design of well-controlled and scientifically valid Phase 2 clinical trials.
- Phase 2 – clinical trials are conducted to evaluate the effectiveness of the drug for a particular indication or in a limited number of patients in the target population to identify possible adverse effects and safety risks, to determine the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage. Multiple Phase 2 clinical trials may be conducted by the sponsor to obtain information prior to beginning larger and more expensive Phase 3 clinical trials. Throughout this Annual Report on Form 10-K, we refer to our initial Phase 2

clinical trials as “Phase 2a clinical trials” and our subsequent Phase 2 clinical trials as “Phase 2b clinical trials.”

·Phase 3 – when Phase 2 clinical trials demonstrate that a dosage range of the product appears effective and has an acceptable safety profile, and provide sufficient information for the design of Phase 3 clinical trials, Phase 3 clinical trials in an expanded patient population at multiple clinical sites may be undertaken. They are performed after preliminary evidence suggesting effectiveness of the drug has been obtained, and are intended to further evaluate dosage, effectiveness and safety, to establish the overall benefit-risk relationship of the investigational drug and to provide an adequate basis for product labeling and approval by the FDA. In most cases, the FDA requires two adequate and well-controlled Phase 3 clinical trials to demonstrate the efficacy of the drug in an expanded patient population at multiple clinical trial sites.

All clinical trials must be conducted in accordance with FDA regulations, GCP requirements and their protocols in order for the data to be considered reliable for regulatory purposes.

An investigational drug product that is a combination of two different drugs in the same dosage form must comply with an additional rule that requires that each component make a contribution to the claimed effects of the drug product. This typically requires larger studies that test the drug against each of its components. In addition, typically, if a drug product is intended to treat a chronic disease, as is the case with our products, safety and efficacy data must be gathered over an extended period of time, which can range from six months to three years or more. Government regulation may delay or prevent marketing of drug candidates or new drugs for a considerable period of time and impose costly procedures upon our activities.

#### Disclosure of Clinical Trial Information

Sponsors of clinical trials of FDA-regulated products, including drugs, are required to register and disclose certain clinical trial information. Information related to the product, patient population, phase of investigation, study sites and investigators, and other aspects of the clinical trial, is then made public as part of the registration. Sponsors are also obligated to discuss the results of their clinical trials after completion. Disclosure of the results of these trials can be delayed until the new product or new indication being studied has been approved. Competitors may use this publicly available information to gain knowledge regarding the progress of development programs.

#### The NDA Approval Process

In order to obtain approval to market a drug in the U.S., a marketing application must be submitted to the FDA that provides data establishing to the FDA's satisfaction the safety and effectiveness of the investigational drug for the proposed indication. Each NDA submission requires a substantial user fee payment (currently exceeding \$2.3 million for fiscal year 2016) unless a waiver or exemption applies. The application includes all relevant data available from pertinent non-clinical studies, or preclinical studies and clinical trials, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product's chemistry, manufacturing, controls and proposed labeling, among other things. Data can come from company-sponsored clinical trials intended to test the safety and effectiveness of a use of a product, or from a number of alternative sources, including studies initiated by investigators that meet GCP requirements.

During the development of a new drug, sponsors are given opportunities to meet with the FDA at certain points. These points may be prior to submission of an IND, at the end of Phase 2 clinical trials, and before an NDA is submitted. Meetings at other times may be requested. These meetings can provide an opportunity for the sponsor to share information about the data gathered to date, for the FDA to provide advice and for the sponsor and the FDA to reach agreement on the next phase of development. Sponsors typically use the end-of-Phase 2 clinical trials meetings to discuss their Phase 2 clinical trials results and present their plans for the pivotal Phase 3 registration trial that they believe will support approval of the new drug.

Concurrent with clinical trials, companies usually complete additional preclinical safety studies and must also develop additional information about the chemistry and physical characteristics of the drug and finalize a process for the NDA sponsor's manufacturing the product in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the drug candidate and the manufacturer must develop methods for testing the identity, strength, quality and purity of the final drugs. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the drug candidate does not undergo unacceptable deterioration over its shelf-life.

The results of drug development, non-clinical studies and clinical trials, along with descriptions of the manufacturing process, analytical tests conducted on the chemistry of the drug, proposed labeling and other relevant information are submitted to the FDA as part of an NDA requesting approval to market the product. The FDA reviews all NDAs submitted to ensure that they are sufficiently complete for substantive review before it accepts them for filing. It may

request additional information rather than accept an NDA for filing. In this event, the NDA must be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing. The FDA has 60 days from its receipt of an NDA to conduct an initial review to determine whether the application will be accepted for filing based on the FDA's threshold determination that the application is sufficiently complete to permit substantive review. If the NDA submission is accepted for filing, the FDA reviews the NDA to determine, among other things, whether the proposed product is safe and effective for its intended use, and whether the product is being manufactured in accordance with cGMP to assure and preserve the product's identity, strength, quality and purity. The FDA has agreed to specific performance goals on the review of NDAs and seeks to review standard NDAs within 12 months from submission of the NDA. The review process may be extended by the FDA for three additional months to consider certain late submitted information or information intended to clarify information already provided in the submission. After the FDA completes its initial review of an NDA, it will communicate to the sponsor that the drug will either be approved, or it will issue a complete response letter to communicate that the NDA will not be approved in its current form and inform the sponsor of changes that must be made or additional clinical, non-clinical or manufacturing data that must be received before the application can be approved, with no implication regarding the ultimate approvability of the application or the timing of any such approval, if ever. If, or 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 to six months depending on the type of information included. The FDA may refer applications for novel drug products or drug products that present difficult questions of safety or effectiveness to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and, if so, under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Before approving an NDA, the FDA typically will inspect the facilities at which the product is manufactured. The FDA will not approve the product 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 may inspect one or more clinical sites to assure compliance with GCP regulations. If the FDA determines the application, manufacturing process or manufacturing facilities are not acceptable, it typically will outline the deficiencies and often will request additional testing or information. This may significantly delay further review of the application. If the FDA finds that a clinical site did not conduct the clinical trial in accordance with GCP regulations, the FDA may determine the data generated by the clinical site should be excluded from the primary efficacy analyses provided in the NDA. Additionally, notwithstanding the submission of any requested additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

The FDA may require, or companies may pursue, additional clinical trials after a product is approved. These so-called Phase 4 or post-approval clinical trials may be made a condition to be satisfied for continuing drug approval. The results of Phase 4 clinical trials can confirm the effectiveness of a drug candidate and can provide important safety information. In addition, the FDA now has express statutory authority to require sponsors to conduct post-marketing trials to specifically address safety issues identified by the agency. See “Post-Marketing Requirements” under Part I, “Item 1. Business” of this Annual Report on Form 10-K.

The FDA also has authority to require a Risk Evaluation and Mitigation Strategy, or a REMS, from manufacturers to ensure that the benefits of a drug outweigh its risks. A sponsor may also voluntarily propose a REMS as part of the NDA submission. The need for a REMS is determined as part of the review of the NDA. Based on statutory standards, elements of a REMS may include “dear doctor letters,” a medication guide, more elaborate targeted educational programs, and in some cases elements to assure safe use, or ETASU, which is the most restrictive REMS. ETASU can 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. These elements are negotiated as part of the NDA approval, and in some cases if consensus is not obtained until after the Prescription Drug User Fee Act of 1992, as amended, review cycle, the approval date may be delayed. Once adopted, REMS are subject to periodic assessment and modification.

Changes to some of the conditions established in an approved application, including changes in indications, labeling, manufacturing processes or facilities, require submission and FDA approval of a new NDA or NDA supplement before the change can be implemented. An NDA supplement for a new indication typically requires clinical data similar to that in the original application, and the FDA uses the same procedures and actions in reviewing NDA supplements as it does in reviewing NDAs.

Even if a drug candidate receives regulatory approval, the approval may be limited to specific disease states, patient populations and dosages, or might contain significant limitations on use in the form of warnings, precautions or contraindications, or in the form of onerous risk management plans, restrictions on distribution or post-marketing trial requirements. Further, even after regulatory approval is obtained, later discovery of previously unknown problems with a product may result in restrictions on the product or even complete withdrawal of the product from the market. Delay in obtaining, or failure to obtain, regulatory approval for our products, or obtaining approval but for significantly limited use, would harm our business. In addition, we cannot predict what adverse governmental

regulations may arise from future U.S. or foreign governmental action.

#### The Hatch-Waxman Amendments

Under the Drug Price Competition and Patent Term Restoration Act of 1984, as amended, commonly known as the Hatch-Waxman Amendments, a portion of a product's U.S. patent term that was lost during clinical development and regulatory review by the FDA may be restored. The Hatch-Waxman Amendments also provide a process for listing patents pertaining to approved products in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (commonly known as the Orange Book) and for a competitor seeking approval of an application that references a product with listed patents to make certifications pertaining to such patents. In addition, the Hatch-Waxman Amendments provide for a statutory protection, known as non-patent exclusivity, against the FDA's acceptance or approval of certain competitor applications.



### Patent Term Restoration

Patent term restoration can compensate for time lost during drug development and the regulatory review process by returning up to five years of patent life for a patent that covers a new product or its use. This period is generally one-half the time between the effective date of an IND (falling after issuance of the patent) and the submission date of an NDA, plus the time between the submission date of an NDA and the approval of that application, provided the sponsor acted with diligence. Patent term restorations, however, 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 and the extension must be applied for prior to expiration of the patent. The United States Patent and Trademark Office, or the USPTO, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration.

### Orange Book Listing

In seeking approval for a drug through an NDA, applicants are required to list with the FDA each patent whose claims cover the applicant's product. Upon approval of a drug, each of the patents listed by the NDA holder listed in the drug's application or otherwise are then published in the FDA's Orange Book. Drugs listed in the Orange Book can, in turn, be cited by potential generic competitors in support of approval of an abbreviated new drug application, or ANDA. An ANDA provides for marketing of a drug product that has the same active ingredients in the same strengths and dosage form as the listed drug and has been shown through bioequivalence testing to be therapeutically equivalent to the listed drug. Other than the requirement for bioequivalence testing, ANDA applicants are not required to conduct, or submit results of, preclinical studies or clinical trials to prove the safety or effectiveness of their drug product. Drugs approved in this way are commonly referred to as "generic equivalents" to the listed drug, and can often be substituted by pharmacists under prescriptions written for the original listed drug.

The ANDA applicant is required to certify to the FDA concerning any patents listed for the approved product in the FDA's Orange Book. Specifically, the applicant must certify that: (1) the required patent information has not been filed; (2) the listed patent has expired; (3) the listed patent has not expired, but will expire on a particular date and approval is sought after patent expiration; or (4) the listed patent is invalid or will not be infringed by the new product. The ANDA applicant may also elect to submit a Section VIII statement certifying that its proposed ANDA label does not contain (or carves out) any language regarding the patented method-of-use rather than certify to a listed method-of-use patent. If the applicant does not challenge the listed patents, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired.

A certification that the new product will not infringe the already approved product's listed patents, or that such patents are invalid, is called a Paragraph IV certification. If the ANDA 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 of the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months, expiration of the patent, settlement of the lawsuit or a decision in the infringement case that is favorable to the ANDA applicant.

An applicant submitting an NDA under Section 505(b)(2) of the FDCA, which permits the filing of an NDA where at least some of the information required for approval comes from studies not conducted by, or for, the applicant and for which the applicant has not obtained a right of reference, is required to certify to the FDA regarding any patents listed in the Orange Book for the approved product it references to the same extent that an ANDA applicant would.

### Market Exclusivity

Market exclusivity provisions under the FDCA also can delay the submission or the approval of certain applications. The FDCA provides a five-year period of non-patent marketing exclusivity within the U.S. to the first applicant to gain approval of an NDA for a new chemical entity. A drug is a new chemical entity if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, the FDA may not accept for review an ANDA or a 505(b)(2) NDA submitted by another company for another version of such drug where the applicant does not own or have a legal right of reference to all the data required for approval. However, an application may be submitted after four years if it contains a Paragraph IV certification. The FDCA also provides three years of marketing exclusivity for an NDA, 505(b)(2) NDA or supplement to an existing NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example, for new indications, dosages or strengths of an existing drug. This three-year exclusivity covers only the conditions associated with the new clinical investigations and does not prohibit the FDA from approving ANDAs for drugs containing the original active agent. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA; however, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the non-clinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

## Post-Marketing Requirements

Following approval of a new product, a pharmaceutical company and the approved product are subject to continuing regulation by the FDA, including, among other things, monitoring and record-keeping activities, reporting to the applicable regulatory authorities of adverse experiences with the product, providing the regulatory authorities with updated safety and efficacy information, product sampling and distribution requirements, and complying with promotion and advertising requirements, which include, among others, standards for direct-to-consumer advertising, restrictions on promoting drugs for uses or in patient populations that are not described in the drug's approved labeling (known as "off-label use"), limitations on industry-sponsored scientific and educational activities and requirements for promotional activities involving the internet, including social media. Although physicians may prescribe legally available drugs for off-label uses, manufacturers may not market or promote such off-label uses. Modifications or enhancements to the product or its labeling or changes of the site of manufacture are often subject to the approval of the FDA and other regulators, who may or may not grant approval, or may include in a lengthy review process.

Prescription drug advertising is subject to federal, state and foreign regulations. In the U.S., the FDA regulates prescription drug promotion, including direct-to-consumer advertising. Prescription drug promotional materials must be submitted to the FDA in conjunction with their first use. Any distribution of prescription drug products and pharmaceutical samples must comply with the U.S. Prescription Drug Marketing Act of 1987, as amended, or the PDMA, a part of the FDCA.

In the U.S., once a product is approved, its manufacture is subject to comprehensive and continuing regulation by the FDA. The FDA regulations require that products be manufactured in specific, approved facilities and in accordance with cGMP. We rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of our products in accordance with cGMP regulations. cGMP regulations require, among other things, quality control and quality assurance as well as the corresponding maintenance of records and documentation and the obligation to investigate and correct any deviations from cGMP. Drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP and other laws. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain cGMP compliance. These regulations also impose certain organizational, procedural and documentation requirements with respect to manufacturing and quality assurance activities. NDA holders using contract manufacturers, laboratories or packagers are responsible for the selection and monitoring of qualified firms and, in certain circumstances, qualified suppliers to these firms. These firms and, where applicable, their suppliers are subject to inspections by the FDA at any time, and the discovery of violative conditions, including failure to conform to cGMP, could result in enforcement actions that interrupt the operation of any such product or may result in restrictions on a product, manufacturer, or holder of an approved NDA, including, among other things, recall or withdrawal of the product from the market.

In addition, the manufacturer or sponsor under an approved NDA is subject to annual product and establishment fees, currently exceeding \$114,450 per product and \$585,200 per establishment for fiscal year 2016. These fees are typically increased annually.

The FDA also may require post-marketing testing, also known as Phase 4 testing, REMS to monitor the effects of an approved product or place conditions on an approval that could restrict the distribution or use of the product. Discovery of previously unknown problems with a product or the failure to comply with applicable FDA requirements can have negative consequences, including adverse publicity, judicial or administrative enforcement, untitled or warning letters from the FDA, mandated corrective advertising or communications with doctors, withdrawal of approval, and civil or criminal penalties, among others. Newly-discovered or developed safety or effectiveness data may require changes to a product's approved labeling, including the addition of new warnings and contraindications,

and also may require the implementation of other risk management measures. Also, new government requirements, including those resulting from new legislation, may be established, or the FDA's policies may change, which could delay or prevent regulatory approval of our products in development.

#### Reimbursement, Anti-Kickback and False Claims Laws and Other Regulatory Matters

In the U.S., the research, manufacturing, distribution, sale and promotion of drug products and medical devices are potentially subject to regulation by various federal, state and local authorities in addition to the FDA, including the Centers for Medicare & Medicaid Services, or CMS, other divisions of the U.S. Department of Health and Human Services (e.g., the Office of Inspector General), the Drug Enforcement Administration, the Consumer Product Safety Commission, the Federal Trade Commission, the Occupational Safety & Health Administration, the Environmental Protection Agency, state Attorneys General and other state and local government agencies. For example, sales, marketing and scientific/educational grant programs must comply with the federal Anti-Kickback Statute, the federal False Claims Act of 1986, as amended, or the federal False Claims Act, the privacy regulations promulgated under the Health Insurance Portability and Accountability Act of 1996, as amended, or HIPAA, and similar state laws. Pricing and rebate programs must comply with the Medicaid Drug Rebate Program requirements of the Omnibus Budget Reconciliation Act of 1990, as amended, and the Veterans Health Care Act of 1992, as amended. If products are made available to authorized users of the Federal

Supply Schedule of the General Services Administration, additional laws and requirements apply. The handling of any controlled substances must comply with the U.S. Controlled Substances Act and Controlled Substances Import and Export Act. Products must meet applicable child-resistant packaging requirements under the U.S. Poison Prevention Packaging Act. All of these activities are also potentially subject to federal and state consumer protection and unfair competition laws.

The Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or the MMA, established the Medicare Part D program to provide a voluntary prescription drug benefit to Medicare beneficiaries. Under Part D, Medicare beneficiaries may enroll in prescription drug plans offered by private entities which will provide coverage of outpatient prescription drugs. Unlike Medicare Part A and B, Part D coverage is not standardized. Part D prescription drug plan sponsors are not required to pay for all covered Part D drugs, and each drug plan can develop its own drug formulary that identifies which drugs it will cover and at what tier or level. However, Part D prescription drug formularies must include drugs within each therapeutic category and class of covered Part D drugs, though not necessarily all the drugs in each category or class. Any formulary used by a Part D prescription drug plan must be developed and reviewed by a pharmacy and therapeutic committee. Government payment for some of the costs of prescription drugs may increase demand for products for which we receive regulatory approval. However, any negotiated prices for our products covered by a Part D prescription drug plan will likely be lower than the prices we might otherwise obtain. Moreover, while the MMA applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own payment rates. Any reduction in payment that results from the MMA may result in a similar reduction in payments from non-government payors.

The distribution of pharmaceutical products is subject to additional requirements and regulations, including extensive record-keeping, licensing, storage and security requirements intended to prevent the unauthorized sale of pharmaceutical products.

The American Recovery and Reinvestment Act of 2009 provides funding for the federal government to compare the effectiveness of different treatments for the same illness. A plan for the research will be developed by the Department of Health and Human Services, the Agency for Healthcare Research and Quality and the National Institutes for Health, and periodic reports on the status of the research and related expenditures will be made to Congress. Although the results of the comparative effectiveness studies are not intended to mandate coverage policies for public or private payors, it is not clear what effect, if any, the research will have on the sales of our drug candidate, if any such product or the condition that it is intended to treat is the subject of a clinical trial. It is also possible that comparative effectiveness research demonstrating benefits in a competitor's product could adversely affect the sales of our drug candidate. If third-party payors do not consider our products to be cost-effective compared to other available therapies, they may not cover our products after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow us to sell our products on a profitable basis.

In addition, in some foreign countries, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing vary widely from country to country. For example, the European Union provides options for its member states to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. A member state may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products. Historically, products launched in the European Union do not follow price structures of the U.S. and generally tend to be priced significantly lower than in the U.S.

As noted above, in the U.S., we are subject to complex laws and regulations pertaining to healthcare “fraud and abuse,” including, but not limited to, the federal Anti-Kickback Statute, the federal False Claims Act, and other state and federal laws and regulations. The federal Anti-Kickback Statute makes it illegal for any person, including a prescription drug manufacturer, or a party acting on its behalf, to knowingly and willfully solicit, receive, offer, or pay any remuneration that is intended to induce the referral of business, including the purchase, order or prescription of a particular drug, or other good or service for which payment in whole or in part may be made under a federal healthcare program, such as Medicare or Medicaid. Violations of this law are punishable by up to five years in prison, criminal fines, administrative civil money penalties, and exclusion from participation in federal healthcare programs. In addition, many states have adopted laws similar to the federal Anti-Kickback Statute. Some of these state prohibitions apply to the referral of patients for healthcare services reimbursed by any insurer, not just federal healthcare programs such as Medicare and Medicaid. Due to the breadth of these federal and state anti-kickback laws, the absence of guidance in the form of regulations or court decisions, and the potential for additional legal or regulatory change in this area, it is possible that our future sales and marketing practices or our future relationships with medical professionals might be challenged under anti-kickback laws, which could harm us. Because we intend to commercialize products that could be reimbursed under a federal healthcare program and other governmental healthcare programs, we plan to develop a comprehensive compliance program that establishes internal controls to facilitate adherence to the rules and program requirements to which we will or may become subject.

The federal False Claims Act prohibits anyone from knowingly presenting, or causing to be presented, for payment to federal programs (including Medicare and Medicaid) claims for items or services, including drugs, that are false or fraudulent, claims for items or services not provided as claimed, or claims for medically unnecessary items or services. Although we would not submit claims directly to payors, manufacturers can be held liable under these laws if they are deemed to “cause” the submission of false or fraudulent claims by, for example, providing inaccurate billing or coding information to customers or promoting a product off-label. In addition, our future activities relating to the reporting of wholesaler or estimated retail prices for our products, the reporting of prices used to calculate Medicaid rebate information and other information affecting federal, state and third-party reimbursement for our products, and the sale and marketing of our products, are subject to scrutiny under this law. For example, pharmaceutical companies have been found liable under the federal False Claims Act in connection with their off-label promotion of drugs. Penalties for a federal False Claims Act violation include three times the actual damages sustained by the government, plus mandatory civil penalties of between \$5,500 and \$11,000 for each separate false claim, the potential for exclusion from participation in federal healthcare programs and, although the federal False Claims Act is a civil statute, conduct that results in a federal False Claims Act violation may also implicate various federal criminal statutes. If the government were to allege that we were, or convict us of, violating these false claims laws, we could be subject to a substantial fine and may suffer a decline in our stock price. In addition, private individuals have the ability to bring actions under the federal False Claims Act and certain states have enacted laws modeled after the federal False Claims Act.

There are also an increasing number of state laws that require manufacturers to make reports to states on pricing and marketing information. Many of these laws contain ambiguities as to what is required to comply with the laws. In addition, as discussed below, beginning in 2013, a similar federal requirement requires manufacturers to track and report to the federal government certain payments made to physicians and teaching hospitals in the previous calendar year. These laws may affect our sales, marketing and other promotional activities by imposing administrative and compliance burdens on us. In addition, given the lack of clarity with respect to these laws and their implementation, our reporting actions could be subject to the penalty provisions of the pertinent state, and soon federal, authorities.

The failure to comply with regulatory requirements subjects companies to possible legal or regulatory action. Depending on the circumstances, failure to meet applicable regulatory requirements can result in criminal prosecution, fines or other penalties, injunctions, recall or seizure of products, total or partial suspension of production, denial or withdrawal of product approvals, or refusal to allow a company to enter into supply contracts, including government contracts.

Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example: (1) changes to our manufacturing arrangements; (2) additions or modifications to product labeling; (3) the recall or discontinuation of our products; or (4) additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business.

#### Patient Protection and Affordable Care Act

In March 2010, the Patient Protection and Affordable Care Act, or the PPACA, was enacted, which includes measures that have or will significantly change the way healthcare is financed by both governmental and private insurers. Among the provisions of the PPACA of greatest importance to the pharmaceutical industry are the following:

- The Medicaid Drug Rebate Program requires pharmaceutical manufacturers to enter into and have in effect a national rebate agreement with the Secretary of the Department of Health and Human Services as a condition for states to receive federal matching funds for the manufacturer’s covered outpatient drugs furnished to Medicaid patients. Effective in 2010, the PPACA made several changes to the Medicaid Drug Rebate Program, including increasing pharmaceutical manufacturers’ rebate liability by raising the minimum basic Medicaid rebate on most

branded prescription drugs and biologic agents to 23.1% of the average manufacturer price, or AMP, and adding a new rebate calculation for “line extensions” ( i.e. , new formulations, such as extended release formulations) of solid oral dosage forms of branded products, as well as potentially impacting their rebate liability by modifying the statutory definition of AMP. The PPACA also expanded the universe of Medicaid utilization subject to drug rebates by requiring pharmaceutical manufacturers to pay rebates on Medicaid managed care utilization as of 2010 and by expanding the population potentially eligible for Medicaid drug benefits, to be phased-in by 2014. The CMS have proposed to expand Medicaid rebate liability to the territories of the U.S. as well. In addition, the PPACA provides for the public availability of retail survey prices and certain weighted average AMPs under the Medicaid program. The implementation of this requirement by the CMS may also provide for the public availability of pharmacy acquisition of cost data, which could negatively impact our sales.

· In order for a pharmaceutical product to receive federal reimbursement under the Medicare Part B and Medicaid programs or to be sold directly to U.S. government agencies, the manufacturer must extend discounts to entities eligible to participate in the 340B drug pricing program. The required 340B discount on a given product is calculated based on the AMP and Medicaid rebate amounts reported by the manufacturer. Effective in 2010, the PPACA expanded the types of



entities eligible to receive discounted 340B pricing, although, under the current state of the law, with the exception of children's hospitals, these newly-eligible entities will not be eligible to receive discounted 340B pricing on orphan drugs when used for the orphan indication. In addition, as 340B drug pricing is determined based on AMP and Medicaid rebate data, the revisions to the Medicaid rebate formula and AMP definition described above could cause the required 340B discount to increase.

- Effective in 2011, the PPACA imposed a requirement on manufacturers of branded drugs and biologic agents to provide a 50% discount off the negotiated price of branded drugs dispensed to Medicare Part D patients in the coverage gap (i.e., "donut hole").
- Effective in 2011, the PPACA imposed an annual, nondeductible fee on any entity that manufactures or imports certain branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs, although this fee would not apply to sales of certain products approved exclusively for orphan indications.
- Effective in 2012, the PPACA required pharmaceutical manufacturers to track certain financial arrangements with physicians and teaching hospitals, including any "transfer of value" made or distributed to such entities, as well as any investment interests held by physicians and their immediate family members. Manufacturers are required to track this information and were required to make their first reports in March 2014. The information reported is publicly available on a searchable website.
- As of 2010, a new Patient-Centered Outcomes Research Institute was established pursuant to the PPACA to oversee, identify priorities in and conduct comparative clinical effectiveness research, along with funding for such research. The research conducted by the Patient-Centered Outcomes Research Institute may affect the market for certain pharmaceutical products.
- The PPACA created the Independent Payment Advisory Board, which has the authority to recommend certain changes to the Medicare program to reduce expenditures by the program that could result in reduced payments for prescription drugs. Under certain circumstances, these recommendations will become law unless Congress enacts legislation that will achieve the same or greater Medicare cost savings.
  - The PPACA established the Center for Medicare and Medicaid Innovation within CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending. Funding has been allocated to support the mission of the Center for Medicare and Medicaid Innovation from 2011 to 2019.

Many of the details regarding the implementation of the PPACA are yet to be determined, and, at this time, the full effect of the PPACA on our business remains unclear.

#### Pediatric Exclusivity and Pediatric Use

Under the Best Pharmaceuticals for Children Act, or the BPCA, certain drugs may obtain an additional six months of exclusivity if the sponsor submits information requested in writing by the FDA, or a Written Request, relating to the use of the active moiety of the drug in children. Conditions for exclusivity include the FDA's determination that information relating to the use of a new drug in the pediatric population may produce health benefits in that population, the FDA making a written request for pediatric studies and the applicant agreeing to perform, and reporting on, the requested studies within the statutory timeframe. The FDA may not issue a Written Request for studies on unapproved or approved indications or where it determines that information relating to the use of a drug in a pediatric population, or part of the pediatric population, may not produce health benefits in that population. Applications under the BPCA are treated as priority applications, with all of the benefits that designation confers.

We have not received a Written Request for such pediatric studies, although we may ask the FDA to issue a Written Request for such studies in the future. To receive the six-month pediatric market exclusivity, we would need to receive a Written Request from the FDA, conduct the requested studies in accordance with a written agreement with the FDA or, if there is no written agreement, in accordance with commonly accepted scientific principles, and submit reports of the studies. A Written Request may include studies for indications that are not currently in the labeling if the

FDA determines that such information will benefit the public health. The FDA will accept the reports upon its determination that the studies were conducted in accordance with, and are responsive to, the original Written Request or commonly accepted scientific principles, as appropriate, and that the reports comply with the FDA's filing requirements.

Under the Pediatric Research Equity Act of 2003, or the PREA, 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. The PREA also authorizes the FDA to require holders of approved NDAs for marketed drugs to conduct pediatric studies under certain circumstances. With the enactment of the Food and Drug Administration Safety and Innovation Act, or 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 the FDASIA. Unless otherwise required by regulation, the pediatric data requirements do not apply to products with orphan designation.

### Intellectual Property

We have in-licensed from Ligand patents and patent applications that contain claims that recite our compounds, as set forth below. We plan to file additional patent applications in the U.S., E.U. and other foreign jurisdictions on our clinical and preclinical programs. Information regarding the issued patents and pending patent applications, as of January 31, 2016, are as follows:

| Subject Matter/Compounds      | # Pending Applications | # Issued Patents | Geographical Scope  | Nominal Patent Term |
|-------------------------------|------------------------|------------------|---|---------------------|
| VK5211 (SARM)                 | 10                     | 11               | U.S., Europe, Chile, Argentina, Brazil, Canada, China, India, Japan, Korea, Mexico, Taiwan, and Venezuela       | 2025-2028           |
| Other SARM                    | 9                      | 32               | U.S., Canada, India, Japan, Korea, Mexico, Australia, China, New Zealand, Argentina, Brazil, Europe, and Israel | 2017-2026           |
| VK0214 (TR $\beta$ )          | 0                      | 1                | U.S.  | 2024                |
| Other TR $\beta$ agonist      | 2                      | 1                | Australia and Canada  | 2026                |
| VK0612 (FBPase inhibitor)     | 0                      | 14               | U.S., China, Hong Kong, Israel, Korea, Mexico, India, Indonesia, New Zealand                                    | 2019-2020           |
| FBPase Inhibitor Combinations | 0                      | 12               | U.S., China, Korea, Israel, Mexico, Portugal, New Zealand, and Russia   | 2019-2021           |
| DGAT-1 Inhibitors             | 1                      | 2                | U.S. and Europe   | 2030                |
| EPOR Inhibitors               | 12                     | 3                | U.S., Australia, Canada, China, Europe, India, Japan, and Korea   | 2030-2031           |

### Corporate Information

We were incorporated under the laws of the State of Delaware on September 24, 2012. Our principal executive offices are located at 12340 El Camino Real, San Diego, CA 92130, and our telephone number is (858) 704-4660. Our website address is [www.vikingtherapeutics.com](http://www.vikingtherapeutics.com). We do not incorporate the information on, or accessible through, our website into this Annual Report on Form 10-K, and you should not consider any information on, or accessible

through, our website as part of this Annual Report on Form 10-K. We have included our website address in this Annual Report on Form 10-K solely as an inactive textual reference.

#### Research and Development Expenses

Our research and development expenses were \$6,966,842 and \$22,223,073 for the years ended December 31, 2015 and 2014, respectively.

#### Employees

As of February 29, 2016, we had nine full-time employees and one part-time employee, four of whom hold a Ph.D. or M.D. degree. All employees are engaged in research and development, project management, business development and finance. None of our employees is subject to a collective bargaining agreement. We have never experienced a material work stoppage or disruption to our business relating to employee matters. We consider our relationship with our employees to be good.

Item 1A. Risk Factors.

You should consider carefully the following information about the risks described below, together with the other information contained in this Annual Report on Form 10-K and in our other public filings in evaluating our business. If any of the following risks actually occurs, our business, financial condition, results of operations and future growth prospects would likely be materially and adversely affected. In these circumstances, the market price of our common stock would likely decline.

Risks Relating to Our Business

We are a clinical-stage company, have a very limited operating history and are expected to incur significant operating losses during the early stage of our corporate development.

We are a clinical-stage company. We were incorporated in, and have only been conducting operations since, September 2012. Our operations to date have been limited to raising capital, building infrastructure, obtaining the worldwide rights to certain technology from Ligand Pharmaceuticals Incorporated, or Ligand, and planning, preparing and conducting preclinical studies and clinical trials of our drug candidates, including VK5211 and VK0612, which are currently in Phase 2 clinical development, VK2809, which has completed Phase 1 clinical development and VK0214 and the EPOR and DGAT-1 programs, which are each currently in preclinical development. As a result, we have no meaningful historical operations upon which to evaluate our business and prospects and have not yet demonstrated an ability to obtain marketing approval for any of our drug candidates or successfully overcome the risks and uncertainties frequently encountered by companies in the biopharmaceutical industry. We also have not generated any revenue to date, and we continue to incur significant research and development and other expenses. Our net loss for the years ended December 31, 2015 and 2014 was \$23,403,988 and \$21,884,183, respectively. As of December 31, 2015, we had an accumulated deficit of \$45,545,445. For the foreseeable future, we expect to continue to incur losses, which will increase significantly from historical levels as we expand our drug development activities, seek regulatory approvals for our drug candidates and begin to commercialize them if they are approved by the U.S. Food and Drug Administration, or the FDA, the European Medicines Agency, or EMA, or comparable foreign authorities. Even if we succeed in developing and commercializing one or more drug candidates, we may never become profitable. If we fail to achieve or maintain profitability, it would adversely affect the value of our common stock.

We are substantially dependent on technologies we licensed from Ligand, and if we lose the license to such technologies or the Master License Agreement is terminated for any reason, our ability to develop existing and new drug candidates would be harmed, and our business, financial condition and results of operations would be materially and adversely affected.

Our business is substantially dependent upon technology licensed from Ligand. Pursuant to the Master License Agreement, we have been granted exclusive worldwide rights to VK5211, VK2809, VK0214, VK0612 and preclinical programs for anemia and lipid disorders. SARMS, such as our lead program VK5211, are key compounds used by us in the development and commercialization of our drug candidates. All of the intellectual property related to our drug candidates is currently owned by Ligand, and we have the rights to use such intellectual property pursuant to the Master License Agreement. Therefore, our ability to develop and commercialize our drug candidates depends entirely on the effectiveness and continuation of the Master License Agreement. If we lose the right to license any of these key compounds, our ability to develop existing and new drug candidates would be harmed.

Ligand has the right to terminate the Master License Agreement under certain circumstances, including, but not limited to: (1) in the event of our insolvency or bankruptcy, (2) if we do not pay an undisputed amount owing under the Master License Agreement when due and fail to cure such default within a specified period of time, or (3) if we default on certain of our material obligations and fail to cure the default within a specified period of time.

We are dependent on the success of one or more of our current drug candidates and we cannot be certain that any of them will receive regulatory approval or be commercialized.

We have spent significant time, money and effort on the licensing and development of our core metabolic and endocrine disease assets, VK5211, VK2809, VK0214, VK0612 and our earlier-stage assets, the EPOR and DGAT-1 programs. To date, no pivotal clinical trials designed to provide clinically and statistically significant proof of efficacy, or to provide sufficient evidence of safety to justify approval, have been completed with any of our drug candidates. All of our drug candidates will require additional development, including clinical trials as well as further preclinical studies to evaluate their toxicology, carcinogenicity and pharmacokinetics and optimize their formulation, and regulatory clearances before they can be commercialized. Positive results obtained during early development do not necessarily mean later development will succeed or that regulatory clearances will be obtained. Our drug development efforts may not lead to commercial drugs, either because our drug candidates fail to be safe and effective or because we have inadequate financial or other resources to advance our drug candidates through the clinical development and approval processes.

If any of our drug candidates fail to demonstrate safety or efficacy at any time or during any phase of development, we would experience potentially significant delays in, or be required to abandon, development of the drug candidate.

We do not anticipate that any of our current drug candidates will be eligible to receive regulatory approval from the FDA, EMA or comparable foreign authorities and begin commercialization for a number of years, if ever. Even if we ultimately receive regulatory approval for any of these drug candidates, we or our potential future partners, if any, may be unable to commercialize them successfully for a variety of reasons. These include, for example, the availability of alternative treatments, lack of cost-effectiveness, the cost of manufacturing the product on a commercial scale and competition with other drugs. The success of our drug candidates may also be limited by the prevalence and severity of any adverse side effects. If we fail to commercialize one or more of our current drug candidates, we may be unable to generate sufficient revenues to attain or maintain profitability, and our financial condition and stock price may decline.

If development of our drug candidates does not produce favorable results, we and our collaborators, if any, may be unable to commercialize these products.

To receive regulatory approval for the commercialization of our core metabolic and endocrine disease assets, VK5211, VK2809, VK0214, VK0612 and our earlier-stage assets, the EPOR and DGAT-1 programs, or any other drug candidates that we may develop, adequate and well-controlled clinical trials must be conducted to demonstrate safety and efficacy in humans to the satisfaction of the FDA, EMA and comparable foreign authorities. In order to support marketing approval, these agencies typically require successful results in one or more Phase 3 clinical trials, which our current drug candidates have not yet reached and may never reach. The development process is expensive, can take many years and has an uncertain outcome. Failure can occur at any stage of the process. We may experience numerous unforeseen events during, or as a result of, the development process that could delay or prevent commercialization of our current or future drug candidates, including the following:

- clinical trials may produce negative or inconclusive results;
  - preclinical studies conducted with drug candidates during clinical development to, among other things, evaluate their toxicology, carcinogenicity and pharmacokinetics and optimize their formulation may produce unfavorable results;
  - patient recruitment and enrollment in clinical trials may be slower than we anticipate;
  - costs of development may be greater than we anticipate;
  - our drug candidates may cause undesirable side effects that delay or preclude regulatory approval or limit their commercial use or market acceptance, if approved;
  - collaborators who may be responsible for the development of our drug candidates may not devote sufficient resources to these clinical trials or other preclinical studies of these candidates or conduct them in a timely manner;
- or
- we may face delays in obtaining regulatory approvals to commence one or more clinical trials.

Success in early development does not mean that later development will be successful because, for example, drug candidates in later-stage clinical trials may fail to demonstrate sufficient safety and efficacy despite having progressed through initial clinical trials.

We licensed all of the intellectual property related to our drug candidates from Ligand pursuant to the Master License Agreement. We recently completed a Phase 1 clinical trial and initiated a Phase 2 clinical trial for VK5211. All other clinical trials, preclinical studies and other analyses performed to date with respect to our drug candidates have been conducted by Ligand. Therefore, as a company, we have limited experience in conducting clinical trials for our drug candidates. Since our experience with our drug candidates is limited, we will need to train our existing personnel and hire additional personnel in order to successfully administer and manage our clinical trials and other studies as planned, which may result in delays in completing such planned clinical trials and preclinical studies. Moreover, to date our drug candidates have been tested in less than the number of patients that will likely need to be studied to

obtain regulatory approval. The data collected from clinical trials with larger patient populations may not demonstrate sufficient safety and efficacy to support regulatory approval of these drug candidates.

We currently do not have strategic collaborations in place for clinical development of any of our current drug candidates. Therefore, in the future, we or any potential future collaborative partner will be responsible for establishing the targeted endpoints and goals for development of our drug candidates. These targeted endpoints and goals may be inadequate to demonstrate the safety and efficacy levels required for regulatory approvals. Even if we believe data collected during the development of our drug candidates are promising, such data may not be sufficient to support marketing approval by the FDA, EMA or comparable foreign authorities. Further, data generated during development can be interpreted in different ways, and the FDA, EMA or comparable foreign authorities may interpret such data in different ways than us or our collaborators. Our failure to adequately demonstrate the safety and efficacy of

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our drug candidates would prevent our receipt of regulatory approval, and ultimately the potential commercialization of these drug candidates.

Since we do not currently possess the resources necessary to independently develop and commercialize our drug candidates, including our core metabolic and endocrine disease assets, VK5211, VK2809, VK0214, VK0612 and our earlier-stage assets, the EPOR and DGAT-1 programs, or any other drug candidates that we may develop, we may seek to enter into collaborative agreements to assist in the development and potential future commercialization of some or all of these assets as a component of our strategic plan. However, our discussions with potential collaborators may not lead to the establishment of collaborations on acceptable terms, if at all, or it may take longer than expected to establish new collaborations, leading to development and potential commercialization delays, which would adversely affect our business, financial condition and results of operations.

We expect to continue to incur significant research and development expenses, which may make it difficult for us to attain profitability.

We expect to expend substantial funds in research and development, including preclinical studies and clinical trials of our drug candidates, and to manufacture and market any drug candidates in the event they are approved for commercial sale. We also may need additional funding to develop or acquire complementary companies, technologies and assets, as well as for working capital requirements and other operating and general corporate purposes. Moreover, our planned increases in staffing will dramatically increase our costs in the near and long-term.

However, our spending on current and future research and development programs and drug candidates for specific indications may not yield any commercially viable products. Due to our limited financial and managerial resources, we must focus on a limited number of research programs and drug candidates and on specific indications. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities.

Because the successful development of our drug candidates is uncertain, we are unable to precisely estimate the actual funds we will require to develop and potentially commercialize them. In addition, we may not be able to generate sufficient revenue, even if we are able to commercialize any of our drug candidates, to become profitable.

Our independent registered public accounting firm has expressed substantial doubt about our ability to continue as a going concern.

We are a clinical-stage company, and the development and commercialization of our drug candidates is uncertain and expected to require substantial expenditures. We have not yet generated any revenues from our operations to fund our activities, and are therefore dependent upon external sources for financing our operations. The audit report issued by our independent registered public accounting firm for our financial statements for the fiscal year ended December 31, 2015 states that our independent registered public accounting firm has expressed substantial doubt in our ability to continue as a going concern due to the risk that we may not have sufficient cash and liquid assets at December 31, 2015 to cover our operating and capital requirements for the next 12 months; and in the event that sufficient cash cannot be obtained, we would have to substantially alter, or possibly even discontinue, operations. Although it is difficult to predict our liquidity requirements, as of December 31, 2015, and based upon our current operating plan, we do not believe that we will have sufficient cash to meet our projected operating requirements for at least the next 12 months unless we raise additional capital. Our financial statements and related notes thereto included elsewhere in this Annual Report on Form 10-K do not include any adjustments that might result from the outcome of this uncertainty.

Given our lack of current cash flow, we will need to raise additional capital; however, it may be unavailable to us or, even if capital is obtained, may cause dilution or place significant restrictions on our ability to operate our business.

Since we will be unable to generate sufficient, if any, cash flow to fund our operations for the foreseeable future, we will need to seek additional equity or debt financing to provide the capital required to maintain or expand our operations. As of December 31, 2015, we had cash and cash equivalents and investments totaling \$14,104,049.

There can be no assurance that we will be able to raise sufficient additional capital on acceptable terms or at all. If such additional financing is not available on satisfactory terms, or is not available in sufficient amounts, we may be required to delay, limit or eliminate the development of business opportunities and our ability to achieve our business objectives, our competitiveness, and our business, financial condition and results of operations may be materially adversely affected. In addition, we may be required to grant rights to develop and market drug candidates that we would otherwise prefer to develop and market ourselves. Our inability to fund our business could lead to the loss of your investment.

Our future capital requirements will depend on many factors, including, but not limited to:

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- the scope, rate of progress, results and cost of our clinical trials, preclinical studies and other related activities;
- the timing of, and the costs involved in, obtaining regulatory approvals for any of our current or future drug candidates;
- the number and characteristics of the drug candidates we seek to develop or commercialize;
- the cost of manufacturing clinical supplies, and establishing commercial supplies, of our drug candidates;
- the cost of commercialization activities if any of our current or future drug candidates are approved for sale, including marketing, sales and distribution costs;
- the expenses needed to attract and retain skilled personnel;
- the costs associated with being a public company;
- our ability to establish and maintain strategic collaborations, licensing or other arrangements and the financial terms of such arrangements;
- the amount of revenue, if any, received from commercial sales of our drug candidates, should any of our drug candidates receive marketing approval; and
- the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing possible patent claims, including litigation costs and the outcome of any such litigation.

If we raise additional capital by issuing equity securities, the percentage ownership of our existing stockholders may be reduced, and accordingly these stockholders may experience substantial dilution. We may also issue equity securities that provide for rights, preferences and privileges senior to those of our common stock. Given our need for cash and that equity issuances are the most common type of fundraising for companies like ours, the risk of dilution is particularly significant for stockholders of our company.

Our drug candidates may cause undesirable side effects that could delay or prevent their regulatory approval or commercialization or have other significant adverse implications on our business, financial condition and results of operations.

Undesirable side effects observed in clinical trials or in supportive preclinical studies with our drug candidates could interrupt, delay or halt their development and could result in the denial of regulatory approval by the FDA, EMA or comparable foreign authorities for any or all targeted indications or adversely affect the marketability of any such drug candidates that receive regulatory approval. In turn, this could eliminate or limit our ability to commercialize our drug candidates.

Our drug candidates may exhibit adverse effects in preclinical toxicology studies and adverse interactions with other drugs. There are also risks associated with additional requirements the FDA, EMA or comparable foreign authorities may impose for marketing approval with regard to a particular disease.

Our drug candidates may require a risk management program that could include patient and healthcare provider education, usage guidelines, appropriate promotional activities, a post-marketing observational study, and ongoing safety and reporting mechanisms, among other requirements. Prescribing could be limited to physician specialists or physicians trained in the use of the drug, or could be limited to a more restricted patient population. Any risk management program required for approval of our drug candidates could potentially have an adverse effect on our business, financial condition and results of operations.

Undesirable side effects involving our drug candidates may have other significant adverse implications on our business, financial condition and results of operations. For example:

- we may be unable to obtain additional financing on acceptable terms, if at all;
- our collaborators may terminate any development agreements covering these drug candidates;
- if any development agreements are terminated, we may determine not to further develop the affected drug candidates due to resource constraints and may not be able to establish additional collaborations for their further development

on acceptable terms, if at all;

- if we were to later continue the development of these drug candidates and receive regulatory approval, earlier findings may significantly limit their marketability and thus significantly lower our potential future revenues from their commercialization;
- we may be subject to product liability or stockholder litigation; and
- we may be unable to attract and retain key employees.

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In addition, if any of our drug candidates receive marketing approval and we or others later identify undesirable side effects caused by the product:

- regulatory authorities may withdraw their approval of the product, or we or our partners may decide to cease marketing and sale of the product voluntarily;
- we may be required to change the way the product is administered, conduct additional clinical trials or preclinical studies regarding the product, change the labeling of the product, or change the product's manufacturing facilities; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the affected product and could substantially increase the costs and expenses of commercializing the product, which in turn could delay or prevent us from generating significant revenues from the sale of the product.

Our efforts to discover drug candidates beyond our current drug candidates may not succeed, and any drug candidates we recommend for clinical development may not actually begin clinical trials.

We intend to use our technology, including our licensed technology, knowledge and expertise to develop novel drugs to address some of the world's most widespread and costly chronic diseases. We intend to expand our existing pipeline of core assets by advancing drug compounds from current ongoing discovery programs into clinical development. However, the process of researching and discovering drug compounds is expensive, time-consuming and unpredictable. Data from our current preclinical programs may not support the clinical development of our lead compounds or other compounds from these programs, and we may not identify any additional drug compounds suitable for recommendation for clinical development. Moreover, any drug compounds we recommend for clinical development may not demonstrate, through preclinical studies, indications of safety and potential efficacy that would support advancement into clinical trials. Such findings would potentially impede our ability to maintain or expand our clinical development pipeline. Our ability to identify new drug compounds and advance them into clinical development also depends upon our ability to fund our research and development operations, and we cannot be certain that additional funding will be available on acceptable terms, or at all.

Delays in the commencement or completion of clinical trials could result in increased costs to us and delay our ability to establish strategic collaborations.

Delays in the commencement or completion of clinical trials could significantly impact our drug development costs. For example, in December 2015 the FDA requested from us information related to the toxicity of certain metabolites of VK2809, prior to initiation of our planned Phase 2 clinical trial in hypercholesterolemia and fatty liver disease. We are in the process of providing this information and currently expect to initiate this study in the second quarter of 2016, representing a delay from our previous plan to initiate the clinical trial in the fourth quarter of 2015. We do not know whether planned clinical trials will begin on time or be completed on schedule, if at all. The commencement of clinical trials can be delayed for a variety of reasons, including, but not limited to, delays related to:

- obtaining regulatory approval to commence one or more clinical trials;
- reaching agreement on acceptable terms with prospective third-party contract research organizations, or CROs, and clinical trial sites;
- manufacturing sufficient quantities of a drug candidate or other materials necessary to conduct clinical trials;
- obtaining institutional review board approval to conduct one or more clinical trials at a prospective site;
- recruiting and enrolling patients to participate in one or more clinical trials; and
- the failure of our collaborators to adequately resource our drug candidates due to their focus on other programs or as a result of general market conditions.

In addition, once a clinical trial has begun, it may be suspended or terminated by us, our collaborators, the institutional review boards or data safety monitoring boards charged with overseeing our clinical trials, the FDA, EMA or comparable foreign authorities due to a number of factors, including:

- failure to conduct the clinical trial in accordance with regulatory requirements or clinical protocols;
- inspection of the clinical trial operations or clinical trial site by the FDA, EMA or comparable foreign authorities resulting in the imposition of a clinical hold;

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- unforeseen safety issues; or
- lack of adequate funding to continue the clinical trial.

If we experience delays in the completion or termination of any clinical trial of our product candidates, the commercial prospects of our product candidates will be harmed, and our ability to commence product sales and generate product revenues from any of our product candidates will be delayed. In addition, any delays in completing our clinical trials will increase our costs and slow down our product candidate development and approval process. Delays in completing our clinical trials could also allow our competitors to obtain marketing approval before we do or shorten the patent protection period during which we may have the exclusive right to commercialize our product candidates. Any of these occurrences may harm our business, financial condition and prospects significantly. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

Results of earlier clinical trials may not be predictive of the results of later-stage clinical trials.

The results of preclinical studies and early clinical trials of product candidates may not be predictive of the results of later-stage clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy results despite having progressed through preclinical studies and initial clinical trials. Many companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to adverse safety profiles or lack of efficacy, notwithstanding promising results in earlier studies. Similarly, our future clinical trial results may not be successful for these or other reasons.

This drug candidate development risk is heightened by any changes in the planned clinical trials compared to the completed clinical trials. As product candidates are developed through preclinical to early to late stage clinical trials towards approval and commercialization, it is customary that various aspects of the development program, such as manufacturing and methods of administration, are altered along the way in an effort to optimize processes and results. While these types of changes are common and are intended to optimize the product candidates for late stage clinical trials, approval and commercialization, such changes carry the risk that they will not achieve these intended objectives.

Any of these changes could make the results of our planned clinical trials or other future clinical trials we may initiate less predictable and could cause our product candidates to perform differently, including causing toxicities, which could delay completion of our clinical trials, delay approval of our product candidates, and/or jeopardize our ability to commence product sales and generate revenues.

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

We may not be able to initiate or continue clinical trials for our product candidates 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 other regulatory authorities. Patient enrollment, a significant factor in the timing of clinical trials, is affected by many factors, including the size and nature of the patient population, the proximity of patients to clinical sites, the eligibility criteria for the trial, the design of the clinical trial, competing clinical trials and clinicians' and patients' perceptions as to the potential advantages of the drug being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating.

If we fail to enroll and maintain the number of patients for which the clinical trial was designed, the statistical power of that clinical trial may be reduced, which would make it harder to demonstrate that the product candidate being tested in such clinical trial is safe and effective. Additionally, enrollment delays in our 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 any of our current or future clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether.

We intend to rely on third parties to conduct our preclinical studies and clinical trials and perform other tasks for us. If these third parties do not successfully carry out their contractual duties, meet expected deadlines, or comply with regulatory requirements, we may not be able to obtain regulatory approval for or commercialize our drug candidates and our business, financial condition and results of operations could be substantially harmed.

Ligand, the licensor of our development programs, has relied upon and plans to continue to rely upon third-party CROs, medical institutions, clinical investigators and contract laboratories to monitor and manage data for our licensed ongoing preclinical and clinical programs. We have relied and expect to continue to rely on these parties for execution of our preclinical studies and clinical trials, and we control only certain aspects of their activities. Nevertheless, we maintain responsibility for ensuring that each of our



clinical trials and preclinical studies is conducted in accordance with the applicable protocol, legal, regulatory, and scientific standards and our reliance on these third parties does not relieve us of our regulatory responsibilities. We and our CROs and other vendors are required to comply with current requirements on good manufacturing practices, or cGMP, good clinical practices, or GCP, and good laboratory practice, or GLP, which are a collection of laws and regulations enforced by the FDA, EMA or comparable foreign authorities for all of our drug candidates in clinical development. Regulatory authorities enforce these regulations through periodic inspections of preclinical study and clinical trial sponsors, principal investigators, preclinical study and clinical trial sites, and other contractors. If we or any of our CROs or vendors fails to comply with applicable regulations, the data generated in our preclinical studies and clinical trials may be deemed unreliable and the FDA, EMA or comparable foreign authorities may require us to perform additional preclinical studies and clinical trials before approving our marketing applications. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials comply with GCP regulations. In addition, our clinical trials must be conducted with products produced consistent with cGMP regulations. Our failure to comply with these regulations may require us to repeat clinical trials, which would delay the development and regulatory approval processes.

If any of our relationships with these third-party CROs, medical institutions, clinical investigators or contract laboratories terminate, we may not be able to enter into arrangements with alternative CROs on commercially reasonable terms, or at all. In addition, our CROs are not our employees, and except for remedies available to us under our agreements with such CROs, we cannot control whether or not they devote sufficient time and resources to our ongoing preclinical and clinical programs. If CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the data they obtain is compromised due to the failure to adhere to our protocols, regulatory requirements, or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to obtain regulatory approval for or successfully commercialize our drug candidates. CROs may also generate higher costs than anticipated. As a result, our business, financial condition and results of operations and the commercial prospects for our drug candidates could be materially and adversely affected, our costs could increase, and our ability to generate revenue could be delayed.

Switching or adding additional CROs, medical institutions, clinical investigators or contract laboratories involves additional cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences work replacing a previous CRO. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines. Though we carefully manage our relationships with our CROs, there can be no assurance that we will not encounter similar challenges or delays in the future or that these delays or challenges will not have a material adverse effect on our business, financial condition or results of operations.

Our drug candidates are subject to extensive regulation under the FDA, EMA or comparable foreign authorities, which can be costly and time consuming, cause unanticipated delays or prevent the receipt of the required approvals to commercialize our drug candidates.

The clinical development, manufacturing, labeling, storage, record-keeping, advertising, promotion, export, marketing and distribution of our drug candidates are subject to extensive regulation by the FDA and other U.S. regulatory agencies, EMA or comparable authorities in foreign markets. In the U.S., neither we nor our collaborators are permitted to market our drug candidates until we or our collaborators receive approval of a new drug application, or an NDA, from the FDA or receive similar approvals abroad. The process of obtaining these approvals is expensive, often takes many years, and can vary substantially based upon the type, complexity and novelty of the drug candidates involved. Approval policies or regulations may change and may be influenced by the results of other similar or competitive products, making it more difficult for us to achieve such approval in a timely manner or at all. For example, the FDA has released draft guidance regarding clinical trials for drug candidates treating diabetes that may result in more stringent requirements for the clinical trials and regulatory approval of such drug candidates. This and any future guidance that may result from recent FDA advisory panel discussions may make it more expensive to

develop and commercialize such drug candidates. Such increased expense could make it more difficult to obtain favorable terms in the collaborative arrangements we require to maximize the value of our programs seeking to develop new drug candidates for diabetes. In addition, as a company, we have not previously filed NDAs with the FDA or filed similar applications with other foreign regulatory agencies. This lack of experience may impede our ability to obtain FDA or other foreign regulatory agency approval in a timely manner, if at all, for our drug candidates for which development and commercialization is our responsibility.

Despite the time and expense invested, regulatory approval is never guaranteed. The FDA, EMA or comparable foreign authorities can delay, limit or deny approval of a drug candidate for many reasons, including:

- a drug candidate may not be deemed safe or effective;
- agency officials of the FDA, EMA or comparable foreign authorities may not find the data from non-clinical or preclinical studies and clinical trials generated during development to be sufficient;

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- the FDA, EMA or comparable foreign authorities may not approve our third-party manufacturers' processes or facilities; or
  - the FDA, EMA or a comparable foreign authority may change its approval policies or adopt new regulations.
- Our inability to obtain these approvals would prevent us from commercializing our drug candidates.

Even if our drug candidates receive regulatory approval in the U.S., we may never receive approval or commercialize our products outside of the U.S.

In order to market any products outside of the U.S., we must establish and comply with numerous and varying regulatory requirements of other countries regarding safety and efficacy. Approval procedures vary among countries and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries might differ from that required to obtain FDA approval. The regulatory approval process in other countries may include all of the risks detailed above regarding FDA approval in the U.S. as well as other risks. Regulatory approval in one country does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory process in others. Failure to obtain regulatory approval in other countries or any delay seeking or obtaining such approval would impair our ability to develop foreign markets for our drug candidates.

Even if any of our drug candidates receive regulatory approval, our drug candidates may still face future development and regulatory difficulties.

If any of our drug candidates receive regulatory approval, the FDA, EMA or comparable foreign authorities may still impose significant restrictions on the indicated uses or marketing of the drug candidates or impose ongoing requirements for potentially costly post-approval studies and trials. In addition, regulatory agencies subject a product, its manufacturer and the manufacturer's facilities to continual review and periodic inspections. If a regulatory agency discovers previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory agency may impose restrictions on that product, our collaborators or us, including requiring withdrawal of the product from the market. Our drug candidates will also be subject to ongoing FDA, EMA or comparable foreign authorities' requirements for the labeling, packaging, storage, advertising, promotion, record-keeping and submission of safety and other post-market information on the drug. If our drug candidates fail to comply with applicable regulatory requirements, a regulatory agency may:

- issue warning letters or other notices of possible violations;
- impose civil or criminal penalties or fines or seek disgorgement of revenue or profits;
- suspend any ongoing clinical trials;
- refuse to approve pending applications or supplements to approved applications filed by us or our collaborators;
- withdraw any regulatory approvals;
- impose restrictions on operations, including costly new manufacturing requirements, or shut down our manufacturing operations; or
- seize or detain products or require a product recall.

The FDA, EMA and comparable foreign authorities actively enforce the laws and regulations prohibiting the promotion of off-label uses.

The FDA, EMA and comparable foreign authorities strictly regulate the promotional claims that may be made about prescription products, such as our drug candidates, if approved. In particular, a product may not be promoted for uses that are not approved by the FDA, EMA or comparable foreign authorities as reflected in the product's approved labeling. If we receive marketing approval for our drug candidates for our proposed indications, physicians may nevertheless use our products for their patients in a manner that is inconsistent with the approved label, if the

physicians personally believe in their professional medical judgment that our products could be used in such manner. However, if we are found to have promoted our products for any off-label uses, the federal government could levy civil, criminal or administrative penalties, and seek fines against us. Such enforcement has become more common in the industry. The FDA, EMA or comparable foreign authorities could also request that we enter into a consent decree or a corporate integrity agreement, or seek a permanent injunction against us under which specified promotional conduct is monitored, changed or curtailed. If we cannot successfully manage the promotion of our drug candidates, if approved, we could become subject to significant liability, which would materially adversely affect our business, financial condition and results of operations.

If our competitors have drug candidates that are approved faster, marketed more effectively or demonstrated to be more effective than ours, our commercial opportunity may be reduced or eliminated.

The biopharmaceutical industry is characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. While we believe that our technology, knowledge, experience and scientific resources provide us with competitive advantages, we face potential competition from many different sources, including commercial biopharmaceutical enterprises, academic institutions, government agencies and private and public research institutions. Any drug candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future.

Many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical studies, clinical trials, regulatory approvals and marketing approved products than we do. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. Our competitors may succeed in developing technologies and therapies that are more effective, better tolerated or less costly than any which we are developing, or that would render our drug candidates obsolete and noncompetitive. Even if we obtain regulatory approval of any of our drug candidates, our competitors may succeed in obtaining regulatory approvals for their products earlier than we do. We will also face competition from these third parties in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, and in acquiring and in-licensing technologies and products complementary to our programs or advantageous to our business.

The key competitive factors affecting the success of each of our drug candidates, if approved, are likely to be its efficacy, safety, tolerability, frequency and route of administration, convenience and price, the level of branded and generic competition and the availability of coverage and reimbursement from government and other third-party payors.

#### VK5211

In the U.S., there are currently no marketed therapies for the maintenance or improvement of lean body mass, or LBM, bone mineral density, or BMD, or physical function in patients recovering from non-elective hip fracture surgery. However, VK5211, if approved, will face competition from several experimental therapies that are in various stages of development for acute rehabilitation following hip fracture surgery, including programs in development at Novartis AG and Morphosys AG. There are also several experimental therapies that are in various stages of clinical development for conditions characterized by muscle wasting by companies including GTx, Inc., Helsinn Group and Morphosys AG. In addition, nutritional and growth hormone-based therapies are sometimes used in patients experiencing muscle wasting.

#### VK2809

There are many therapies currently available and numerous others being developed for the treatment of hypercholesterolemia and dyslipidemia. If approved, VK2809 will face competition from therapies that are currently available and from therapies that may become available in the future. Generic statin therapies such as atorvastatin are widely prescribed for the initial treatment of hypercholesterolemia. Cholesterol absorption inhibitors such as Merck & Co., Inc.'s Zetia (ezetimibe), generic bile acid sequestrants such as colesevam and generic fibrates such as fenofibrate are also prescribed for the treatment of hypercholesterolemia. Various combinations of these therapies are often prescribed for patients suffering from dyslipidemia. In addition, recently-approved antibody therapies targeting the proprotein convertase subtilisin/kexin type 9 (PCSK9) gene are expected to be prescribed for patients whose low-density lipoprotein (LDL) remains elevated despite treatment with existing cholesterol-lowering agents. While no therapies are currently approved for the treatment of non-alcoholic steatohepatitis, we are aware of several

development-stage programs targeting this disease, including obeticholic acid from Intercept Pharmaceuticals, Inc., GFT505 from Genfit SA, aramchol from Galmed Pharmaceuticals Ltd., simtuzumab from Gilead Sciences, Inc., and emricisan from Conatus Pharmaceuticals Inc.

#### VK0214

In the U.S., there are currently no marketed therapies for the treatment of X-ALD. Hematopoietic stem cell therapy has been used to treat the most severe form of X-ALD, CALD. More recently, gene therapy has been shown to be effective in CALD as well. However, both treatments are invasive, requiring surgical intervention, and these do not appear to have an effect on the most pervasive form of X-ALD, AMN. High-dose biotin is under investigation for treatment of AMN. There are several experimental therapies that are in various stages of clinical development for X-ALD by companies, including MedDay Pharmaceuticals SAS and bluebird bio, Inc., which may be competitive with VK0214, if approved.

## VK0612

In the U.S., VK0612, if approved, will face competition from a variety of currently marketed oral type 2 diabetes therapies, including metformin (generic), pioglitazone (generic), glimepiride (generic), sitagliptin (Merck & Co., Inc.) and canagliflozin (Johnson & Johnson). These therapies are well-established and are widely accepted by physicians, patients, caregivers and third-party payors as the standard of care for the treatment of type 2 diabetes. Physicians, patients and third-party payors may not accept the addition of VK0612 to their current treatment regimens for a variety of potential reasons, including:

- if they do not wish to incur any potential additional costs related to VK0612; or
- if they perceive the use of VK0612 to be of limited additional benefit to patients.

In addition to the currently approved and marketed type 2 diabetes therapies, there are a number of experimental drugs that are in various stages of clinical development by companies such as Eli Lilly and Company, Takeda Pharmaceutical Company Limited and TransTech Pharma, Inc.

### Preclinical Programs Focused on EPOR Agonists and DGAT-1 Inhibitors

If any of our preclinical programs are ultimately determined safe and effective and approved for marketing, they may compete for market share with established therapies from a number of competitors, including large biopharmaceutical companies. Many therapies are currently available and numerous others are being developed for the treatment of anemia and obesity. Any products that we may develop from our preclinical programs may not be able to compete effectively with existing or future therapies.

We are subject to a multitude of manufacturing risks, any of which could substantially increase our costs and limit supply of our drug candidates.

The process of manufacturing our drug candidates is complex, highly regulated, and subject to several risks. For example, the process of manufacturing our drug candidates is extremely susceptible to product loss due to contamination, equipment failure or improper installation or operation of equipment, or vendor or operator error. Even minor deviations from normal manufacturing processes for any of our drug candidates could result in reduced production yields, product defects, and other supply disruptions. If microbial, viral, or other contaminations are discovered in our drug candidates or in the manufacturing facilities in which our drug candidates are made, such manufacturing facilities may need to be closed for an extended period of time to investigate and remedy the contamination. In addition, the manufacturing facilities in which our drug candidates are made could be adversely affected by equipment failures, labor shortages, natural disasters, power failures and numerous other factors.

In addition, any adverse developments affecting manufacturing operations for our drug candidates may result in shipment delays, inventory shortages, lot failures, withdrawals or recalls, or other interruptions in the supply of our drug candidates. We also may need to take inventory write-offs and incur other charges and expenses for drug candidates that fail to meet specifications, undertake costly remediation efforts, or seek costlier manufacturing alternatives.

We rely completely on third parties to manufacture our preclinical and clinical drug supplies, and our business, financial condition and results of operations could be harmed if those third parties fail to provide us with sufficient quantities of drug product, or fail to do so at acceptable quality levels or prices.

We do not currently have, nor do we plan to acquire, the infrastructure or capability internally to manufacture our preclinical and clinical drug supplies for use in our clinical trials, and we lack the resources and the capability to manufacture any of our drug candidates on a clinical or commercial scale. We rely on our manufacturers to purchase

from third-party suppliers the materials necessary to produce our drug candidates for our clinical trials. There are a limited number of suppliers for raw materials that we use to manufacture our drugs, and there may be a need to identify alternate suppliers to prevent a possible disruption of the manufacture of the materials necessary to produce our drug candidates for our clinical trials, and, if approved, ultimately for commercial sale. We do not have any control over the process or timing of the acquisition of these raw materials by our manufacturers. Although we generally do not begin a clinical trial unless we believe we have a sufficient supply of a drug candidate to complete such clinical trial, any significant delay or discontinuity in the supply of a drug candidate, or the raw material components thereof, for an ongoing clinical trial due to the need to replace a third-party manufacturer could considerably delay completion of our clinical trials, product testing and potential regulatory approval of our drug candidates, which could harm our business, financial condition and results of operations.



We and our contract manufacturers are subject to significant regulation with respect to manufacturing our drug candidates. The manufacturing facilities on which we rely may not continue to meet regulatory requirements.

All entities involved in the preparation of therapeutics for clinical trials or commercial sale, including our existing contract manufacturers for our drug candidates, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in late-stage clinical trials must be manufactured in accordance with cGMP. These regulations govern manufacturing processes and procedures and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of contaminants or to inadvertent changes in the properties or stability of our drug candidates that may not be detectable in final product testing. We or our contract manufacturers must supply all necessary documentation in support of an NDA or marketing authorization application, or MAA, on a timely basis and must adhere to good laboratory practice and cGMP regulations enforced by the FDA, EMA or comparable foreign authorities through their facilities inspection program. Some of our contract manufacturers may not have produced a commercially approved pharmaceutical product and therefore may not have obtained the requisite regulatory authority approvals to do so. The facilities and quality systems of some or all of our third-party contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of our drug candidates or any of our other potential products. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our drug candidates or any of our other potential products or the associated quality systems for compliance with the regulations applicable to the activities being conducted. Although we oversee the contract manufacturers, we cannot control the manufacturing process of, and are completely dependent on, our contract manufacturing partners for compliance with the regulatory requirements. If these facilities do not pass a pre-approval plant inspection, regulatory approval of the products may not be granted or may be substantially delayed until any violations are corrected to the satisfaction of the regulatory authority, if ever.

The regulatory authorities also may, at any time following approval of a product for sale, audit the manufacturing facilities of our third-party contractors. If any such inspection or audit identifies a failure to comply with applicable regulations or if a violation of our product specifications or applicable regulations occurs independent of such an inspection or audit, we or the relevant regulatory authority may require remedial measures that may be costly or time consuming for us or a third party to implement, and that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a facility. Any such remedial measures imposed upon us or third parties with whom we contract could materially harm our business, financial condition and results of operations.

If we or any of our third-party manufacturers fail to maintain regulatory compliance, the FDA, EMA or comparable foreign authorities can impose regulatory sanctions including, among other things, refusal to approve a pending application for a drug candidate, withdrawal of an approval, or suspension of production. As a result, our business, financial condition and results of operations may be materially and adversely affected.

Additionally, if supply from one manufacturer is interrupted, an alternative manufacturer would need to be qualified through an NDA supplement or MAA variation, or equivalent foreign regulatory filing, which could result in further delay. The regulatory agencies may also require additional studies or trials if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

These factors could cause us to incur higher costs and could cause the delay or termination of clinical trials, regulatory submissions, required approvals, or commercialization of our drug candidates. Furthermore, if our suppliers fail to meet contractual requirements and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical trials may be delayed or we could lose potential revenue.

Any collaboration arrangement that we may enter into in the future may not be successful, which could adversely affect our ability to develop and commercialize our current and potential future drug candidates.

We may seek collaboration arrangements with biopharmaceutical companies for the development or commercialization of our current and potential future drug candidates. To the extent that we decide to enter into collaboration agreements, we will face significant competition in seeking appropriate collaborators. Moreover, collaboration arrangements are complex and time consuming to negotiate, execute and implement. We may not be successful in our efforts to establish and implement collaborations or other alternative arrangements should we choose to enter into such arrangements, and the terms of the arrangements may not be favorable to us. If and when we collaborate with a third party for development and commercialization of a drug candidate, we can expect to relinquish some or all of the control over the future success of that drug candidate to the third party. The success of our collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborators generally have significant discretion in determining the efforts and resources that they will apply to these collaborations.

Disagreements between parties to a collaboration arrangement can lead to delays in developing or commercializing the applicable drug candidate and can be difficult to resolve in a mutually beneficial manner. In some cases, collaborations with biopharmaceutical companies and other third parties are terminated or allowed to expire by the other party. Any such termination or expiration would adversely affect our business, financial condition and results of operations.

If we are unable to develop our own commercial organization or enter into agreements with third parties to sell and market our drug candidates, we may be unable to generate significant revenues.

We do not have a sales and marketing organization, and we have no experience as a company in the sales, marketing and distribution of pharmaceutical products. If any of our drug candidates are approved for commercialization, we may be required to develop our sales, marketing and distribution capabilities, or make arrangements with a third party to perform sales and marketing services. Developing a sales force for any resulting product or any product resulting from any of our other drug candidates is expensive and time consuming and could delay any product launch. We may be unable to establish and manage an effective sales force in a timely or cost-effective manner, if at all, and any sales force we do establish may not be capable of generating sufficient demand for our drug candidates. To the extent that we enter into arrangements with collaborators or other third parties to perform sales and marketing services, our product revenues are likely to be lower than if we marketed and sold our drug candidates independently. If we are unable to establish adequate sales and marketing capabilities, independently or with others, we may not be able to generate significant revenues and may not become profitable.

The commercial success of our drug candidates depends upon their market acceptance among physicians, patients, healthcare payors and the medical community.

Even if our drug candidates obtain regulatory approval, our products, if any, may not gain market acceptance among physicians, patients, healthcare payors and the medical community. The degree of market acceptance of any of our approved drug candidates will depend on a number of factors, including:

- the effectiveness of our approved drug candidates as compared to currently available products;
- patient willingness to adopt our approved drug candidates in place of current therapies;
- our ability to provide acceptable evidence of safety and efficacy;
- relative convenience and ease of administration;
- the prevalence and severity of any adverse side effects;
- restrictions on use in combination with other products;
- availability of alternative treatments;
- pricing and cost-effectiveness assuming either competitive or potential premium pricing requirements, based on the profile of our drug candidates and target markets;
- effectiveness of our or our partners' sales and marketing strategy;
- our ability to obtain sufficient third-party coverage or reimbursement; and
- potential product liability claims.

In addition, the potential market opportunity for our drug candidates is difficult to precisely estimate. Our estimates of the potential market opportunity for our drug candidates include several key assumptions based on our industry knowledge, industry publications, third-party research reports and other surveys. Independent sources have not verified all of our assumptions. If any of these assumptions proves to be inaccurate, then the actual market for our drug candidates could be smaller than our estimates of our potential market opportunity. If the actual market for our drug candidates is smaller than we expect, our product revenue may be limited, it may be harder than expected to raise funds and it may be more difficult for us to achieve or maintain profitability. If we fail to achieve market acceptance of our drug candidates in the U.S. and abroad, our revenue will be limited and it will be more difficult to achieve profitability.



If we fail to obtain and sustain an adequate level of reimbursement for our potential products by third-party payors, potential future sales would be materially adversely affected.

There will be no viable commercial market for our drug candidates, if approved, without reimbursement from third-party payors. Reimbursement policies may be affected by future healthcare reform measures. We cannot be certain that reimbursement will be available for our current drug candidates or any other drug candidate we may develop. Additionally, even if there is a viable commercial market, if the level of reimbursement is below our expectations, our anticipated revenue and gross margins will be adversely affected.

Third-party payors, such as government or private healthcare insurers, carefully review and increasingly question and challenge the coverage of and the prices charged for drugs. Reimbursement rates from private health insurance companies vary depending on the company, the insurance plan and other factors. Reimbursement rates may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. There is a current trend in the U.S. healthcare industry toward cost containment.

Large public and private payors, managed care organizations, group purchasing organizations and similar organizations are exerting increasing influence on decisions regarding the use of, and reimbursement levels for, particular treatments. Such third-party payors, including Medicare, may question the coverage of, and challenge the prices charged for, medical products and services, and many third-party payors limit coverage of or reimbursement for newly approved healthcare products. In particular, third-party payors may limit the covered indications. Cost-control initiatives could decrease the price we might establish for products, which could result in product revenues being lower than anticipated. We believe our drugs will be priced significantly higher than existing generic drugs and consistent with current branded drugs. If we are unable to show a significant benefit relative to existing generic drugs, Medicare, Medicaid and private payors may not be willing to provide reimbursement for our drugs, which would significantly reduce the likelihood of our products gaining market acceptance.

We expect that private insurers will consider the efficacy, cost-effectiveness, safety and tolerability of our potential products in determining whether to approve reimbursement for such products and at what level. Obtaining these approvals can be a time consuming and expensive process. Our business, financial condition and results of operations would be materially adversely affected if we do not receive approval for reimbursement of our potential products from private insurers on a timely or satisfactory basis. Limitations on coverage could also be imposed at the local Medicare carrier level or by fiscal intermediaries. Medicare Part D, which provides a pharmacy benefit to Medicare patients as discussed below, does not require participating prescription drug plans to cover all drugs within a class of products. Our business, financial condition and results of operations could be materially adversely affected if Part D prescription drug plans were to limit access to, or deny or limit reimbursement of, our drug candidates or other potential products.

Reimbursement systems in international markets vary significantly by country and by region, and reimbursement approvals must be obtained on a country-by-country basis. In many countries, the product cannot be commercially launched until reimbursement is approved. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. The negotiation process in some countries can exceed 12 months. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our products to other available therapies.

If the prices for our potential products are reduced or if governmental and other third-party payors do not provide adequate coverage and reimbursement of our drugs, our future revenue, cash flows and prospects for profitability will suffer.

Recently enacted and future legislation may increase the difficulty and cost of commercializing our drug candidates and may affect the prices we may obtain if our drug candidates are approved for commercialization.

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

In the U.S., the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or the MMA, changed the way Medicare covers and pays for pharmaceutical products. Cost reduction initiatives and other provisions of this legislation could limit the coverage and reimbursement rate that we receive for any of our approved products. While the MMA only applies 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 MMA may result in a similar reduction in payments from private payors.

In March 2010, President Obama signed into law the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, collectively the PPACA, intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against healthcare fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The PPACA increased manufacturers' rebate liability under the Medicaid Drug Rebate Program by increasing the minimum rebate amount for both branded and generic drugs and revised the definition of "average manufacturer price," or AMP, which may also increase the amount of Medicaid drug rebates manufacturers are required to pay to states. The legislation also expanded Medicaid drug rebates and created an alternative rebate formula for certain new formulations of certain existing products that is intended to increase the rebates due on those drugs. The Centers for Medicare & Medicaid Services, which administers the Medicaid Drug Rebate Program, also has proposed to expand Medicaid rebates to the utilization that occurs in the territories of the U.S., such as Puerto Rico and the Virgin Islands. Further, beginning in 2011, the PPACA imposed a significant annual fee on companies that manufacture or import branded prescription drug products and required manufacturers to provide a 50% discount off the negotiated price of prescriptions filled by beneficiaries in the Medicare Part D coverage gap, referred to as the "donut hole." Although it is too early to determine the full effects of the PPACA, the law appears likely to continue the downward 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 introduced at both the state and federal level to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We are not sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our drug 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 approval testing and other requirements.

We are subject to "fraud and abuse" and similar laws and regulations, and a failure to comply with such regulations or prevail in any litigation related to noncompliance could harm our business, financial condition and results of operations.

In the U.S., we are subject to various federal and state healthcare "fraud and abuse" laws, including anti-kickback laws, false claims laws and other laws intended, among other things, to reduce fraud and abuse in federal and state healthcare programs. The federal Anti-Kickback Statute makes it illegal for any person, including a prescription drug manufacturer, or a party acting on its behalf, to knowingly and willfully solicit, receive, offer or pay any remuneration that is intended to induce the referral of business, including the purchase, order or prescription of a particular drug, or other good or service for which payment in whole or in part may be made under a federal healthcare program, such as Medicare or Medicaid. Although we seek to structure our business arrangements in compliance with all applicable requirements, these laws are broadly written, and it is often difficult to determine precisely how the law will be applied in specific circumstances. Accordingly, it is possible that our practices may be challenged under the federal Anti-Kickback Statute.

The federal False Claims Act prohibits anyone from, among other things, knowingly presenting or causing to be presented for payment to the government, including the federal healthcare programs, claims for reimbursed drugs or services that are false or fraudulent, claims for items or services that were not provided as claimed, or claims for medically unnecessary items or services. Under the Health Insurance Portability and Accountability Act of 1996, we are prohibited from knowingly and willfully executing a scheme to defraud any healthcare benefit program, including private payors, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services to obtain money or property of any healthcare benefit program. Violations of fraud and

abuse laws may be punishable by criminal or civil sanctions, including penalties, fines or exclusion or suspension from federal and state healthcare programs such as Medicare and Medicaid and debarment from contracting with the U.S. government. In addition, private individuals have the ability to bring actions on behalf of the government under the federal False Claims Act as well as under the false claims laws of several states.

Many states have adopted laws similar to the federal Anti-Kickback Statute, some of which apply to the referral of patients for healthcare services reimbursed by any source, not just governmental payors. In addition, some states have passed laws that require pharmaceutical companies to comply with the April 2003 Office of Inspector General Compliance Program Guidance for Pharmaceutical Manufacturers or the Pharmaceutical Research and Manufacturers of America's Code on Interactions with Healthcare Professionals. Several states also impose other marketing restrictions or require pharmaceutical companies to make marketing or price disclosures to the state. There are ambiguities as to what is required to comply with these state requirements and if we fail to comply with an applicable state law requirement we could be subject to penalties.



Neither the government nor the courts have provided definitive guidance on the application of fraud and abuse laws to our business. Law enforcement authorities are increasingly focused on enforcing these laws, and it is possible that some of our practices may be challenged under these laws. Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. If we are found in violation of one of these laws, we could be subject to significant civil, criminal and administrative penalties, damages, fines, exclusion from governmental funded federal or state healthcare programs and the curtailment or restructuring of our operations. If this occurs, our business, financial condition and results of operations may be materially adversely affected.

If we face allegations of noncompliance with the law and encounter sanctions, our reputation, revenues and liquidity may suffer, and any of our drug candidates that are ultimately approved for commercialization could be subject to restrictions or withdrawal from the market.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response, and could generate negative publicity. Any failure to comply with ongoing regulatory requirements may significantly and adversely affect our ability to generate revenues from any of our drug candidates that are ultimately approved for commercialization. If regulatory sanctions are applied or if regulatory approval is withdrawn, our business, financial condition and results of operations will be adversely affected. Additionally, if we are unable to generate revenues from product sales, our potential for achieving profitability will be diminished and our need to raise capital to fund our operations will increase.

If we fail to retain current members of our senior management and scientific personnel, or to attract and keep additional key personnel, we may be unable to successfully develop or commercialize our drug candidates.

Our success depends on our continued ability to attract, retain and motivate highly qualified management and scientific personnel. As of February 29, 2016, we had nine full-time employees, one part-time employee and a small number of consultants, which may make us more reliant on our individual employees than companies with a greater number of employees. The loss of any of our key personnel could delay or prevent the development of our drug candidates. These personnel are “at-will” employees and may terminate their employment with us at any time; however, our current executive officers have agreed to provide us with at least 60 days’ advance notice of resignation pursuant to their employment agreements with us. The replacement of key personnel likely would involve significant time and costs, and may significantly delay or prevent the achievement of our business objectives. We do not maintain “key person” insurance on any of our employees.

From time to time, our management seeks the advice and guidance of certain scientific advisors and consultants regarding clinical and regulatory development programs and other customary matters. These scientific advisors and consultants are not our employees and may have commitments to, or consulting or advisory contracts with, other entities that may limit their availability to us. In addition, our scientific advisors may have arrangements with other companies to assist those companies in developing products or technologies that may compete with ours.

Competition for qualified personnel is intense, especially in the greater San Diego, California area where we have a substantial presence and need for highly skilled personnel. We may not be successful in attracting qualified personnel to fulfill our current or future needs. Competitors and others have in the past attempted, and are likely in the future to attempt, to recruit our employees. While our employees are required to sign standard agreements concerning confidentiality and ownership of inventions, we generally do not have employment contracts or non-competition agreements with any of our personnel. The loss of the services of any of our key personnel, the inability to attract or retain highly qualified personnel in the future or delays in hiring such personnel, particularly senior management and other technical personnel, could materially and adversely affect our business, financial condition and results of operations.

We will need to increase the size of our organization and may not successfully manage our growth.

We are a clinical-stage biopharmaceutical company with a small number of employees, and our management systems currently in place are not likely to be adequate to support our future growth plans. Our ability to grow and to manage our growth effectively will require us to hire, train, retain, manage and motivate additional employees and to implement and improve our operational, financial and management systems. These demands also may require the hiring of additional senior management personnel or the development of additional expertise by our senior management personnel. Hiring a significant number of additional employees, particularly those at the management level, would increase our expenses significantly. Moreover, if we fail to expand and enhance our operational, financial and management systems in conjunction with our potential future growth, it could have a material adverse effect on our business, financial condition and results of operations.

We are party to a loan and security agreement that contains operating and financial covenants that may restrict our business and financing activities.

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On May 21 2014, we entered into a Loan and Security Agreement with Ligand, as amended on April 8, 2015 and January 22, 2016, or the Loan and Security Agreement, pursuant to which, among other things, Ligand agreed to provide us with loans in the aggregate amount of up to \$2.5 million. Each of the loans under the Loan and Security Agreement is evidenced by a Secured Convertible Promissory Note, or the Ligand Note. Under the Loan and Security Agreement and the Ligand Note, we are subject to affirmative and negative covenants. We agreed to, among other things, deliver financial statements, forecasts and budget information to Ligand. In addition, we agreed to use the proceeds from the loans solely as working capital and to fund our general business requirements in accordance with our forecast and budget, and not to take certain actions without Ligand's consent, including, but not limited to, declaring or paying dividends, incurring or repaying certain indebtedness or engaging in certain related party transactions. The operating covenants, restrictions and obligations in our Loan and Security Agreement, as well as any future financing arrangements that we may enter into, may restrict our ability to finance our operations, engage in business activities or expand or fully pursue our business strategies. Our ability to comply with these covenants may be affected by events beyond our control, and we may not be able to meet all of our covenants under the Loan and Security Agreement.

Additionally, we may be required to repay the outstanding indebtedness under the Loan and Security Agreement and the Ligand Note if an event of default occurs. An event of default under the Loan and Security Agreement will be deemed to occur or exist upon the termination of the Master License Agreement; in the event we fail to make principal or interest payments under the Ligand Note when due; if we become insolvent or breach and fail to cure within a specified period of time any representation, warranty, covenant or agreement in the Loan and Security Agreement, the Master License Agreement, the Option Agreement, dated September 27, 2012, by and between us and Ligand, as amended, the Voting Agreement, dated May 21, 2014, by and among us, Ligand, Brian Lian, Ph.D., and Michael Dinerman, M.D., our former Chief Operating Officer, or our Management Rights Letter with Ligand, dated May 21, 2014; or upon the occurrence of certain other events. We may not have enough available cash or be able to raise additional funds through equity or debt financings to repay such indebtedness at the time any such event of default occurs. In this case, we may be required to delay, limit, reduce or terminate our product development or commercialization efforts or grant to others rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves. If any of these events occur, our business, financial condition and results of operations may be materially adversely affected.

Our management's relative lack of public company experience could put us at greater risk of incurring fines or regulatory actions for failure to comply with federal securities laws and could put us at a competitive disadvantage, and could require our management to devote additional time and resources to ensure compliance with applicable corporate governance requirements.

Some of our executive officers have limited experience in managing and operating a public company, which could have an adverse effect on their ability to quickly respond to problems or adequately address issues and matters applicable to public companies. Any failure to comply with federal securities laws, rules or regulations could subject us to fines or regulatory actions, which may materially adversely affect our business, financial condition and results of operations. Further, since some of our executive officers have minimal public company experience, we may have to dedicate additional time and resources to comply with legally mandated corporate governance policies relative to our competitors whose management teams have more public company experience.

We are exposed to product liability, non-clinical and clinical liability risks which could place a substantial financial burden upon us, should lawsuits be filed against us.

Our business exposes us to potential product liability and other liability risks that are inherent in the testing, manufacturing and marketing of pharmaceutical formulations and products. In addition, the use in our clinical trials of pharmaceutical products and the subsequent sale of these products by us or our potential collaborators may cause us to

bear a portion of or all product liability risks. A successful liability claim or series of claims brought against us could have a material adverse effect on our business, financial condition and results of operations.

We currently maintain product liability insurance; however, there can be no assurance that we will be able to continue to maintain such insurance, and we may be unable to obtain replacement product liability insurance on commercially reasonable terms or in adequate amounts. On occasion, large judgments have been awarded in class action lawsuits based on drugs that had unanticipated adverse effects. A successful product liability claim or series of claims brought against us could cause our stock price to decline and, if judgments exceed our insurance coverage, could adversely affect our results of operations and business.

Our research and development activities involve the use of hazardous materials, which subject us to regulation, related costs and delays and potential liabilities.

Our research and development activities involve the controlled use of hazardous materials, chemicals and various radioactive compounds, and we will need to develop additional safety procedures for the handling and disposing of hazardous materials. If an accident occurs, we could be held liable for resulting damages, which could be substantial. We are also subject to numerous

environmental, health and workplace safety laws and regulations, including those governing laboratory procedures, exposure to blood-borne pathogens and the handling of biohazardous materials. Additional federal, state and local laws and regulations affecting our operations may be adopted in the future. We may incur substantial costs to comply with, and substantial fines or penalties if we violate any of these laws or regulations.

We rely significantly on information technology and any failure, inadequacy, interruption or security lapse of that technology, including any cybersecurity incidents, could harm our ability to operate our business effectively.

Despite the implementation of security measures, our internal computer systems and those of third parties with which we contract are vulnerable to damage from cyber-attacks, computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. System failures, accidents or security breaches could cause interruptions in our operations, and could result in a material disruption of our drug development and clinical activities and business operations, in addition to possibly requiring substantial expenditures of resources to remedy. The loss of drug development or clinical trial data could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and our development programs and the development of our drug candidates could be delayed.

Our employees and consultants may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.

We are exposed to the risk of employee or consultant fraud or other misconduct. Misconduct by our employees or consultants could include intentional failures to comply with FDA regulations, provide accurate information to the FDA, comply with manufacturing standards, comply with federal and state healthcare fraud and abuse laws and regulations, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commissions, customer incentive programs and other business arrangements. Employee and consultant misconduct also could involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter such misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a material adverse effect on our business, financial condition and results of operations, and result in the imposition of significant fines or other sanctions against us.

Business disruptions such as natural disasters could seriously harm our future revenues and financial condition and increase our costs and expenses.

Our corporate headquarters are located in greater San Diego, California, a region known for seismic activity. In addition, our third party manufacturers are located in the southeastern part of the United States, an area subject to hurricanes and related natural disasters. Our suppliers may also experience a disruption in their business as a result of natural disasters. A significant natural disaster, such as an earthquake, hurricane, flood or fire, could severely damage or destroy our headquarters or facilities or the facilities of our manufacturers or suppliers, which could have a material and adverse effect on our business, financial condition and results of operations. In addition, terrorist acts or acts of war targeted at the U.S., and specifically the greater San Diego, California region, could cause damage or disruption to us, our employees, facilities, partners and suppliers, which could have a material adverse effect on our business,

financial condition and results of operations.

We may engage in strategic transactions that could impact our liquidity, increase our expenses and present significant distractions to our management.

From time to time, we may consider strategic transactions, such as acquisitions of companies, asset purchases and out-licensing or in-licensing of products, drug candidates or technologies. Additional potential transactions that we may consider include a variety of different business arrangements, including spin-offs, strategic partnerships, joint ventures, restructurings, divestitures, business combinations and investments. Any such transaction may require us to incur non-recurring or other charges, may increase our near- and long-term expenditures and may pose significant integration challenges or disrupt our management or business, which could adversely affect our business, financial condition and results of operations. For example, these transactions may entail numerous operational and financial risks, including:

- exposure to unknown liabilities;

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- disruption of our business and diversion of our management's time and attention in order to develop acquired products, drug candidates or technologies;
- incurrence of substantial debt or dilutive issuances of equity securities to pay for any of these transactions;
- higher-than-expected transaction and integration costs;
- write-downs of assets or goodwill or impairment charges;
- increased amortization expenses;
- difficulty and cost in combining the operations and personnel of any acquired businesses or product lines with our operations and personnel;
- impairment of relationships with key suppliers or customers of any acquired businesses or product lines due to changes in management and ownership; and
- inability to retain key employees of any acquired businesses.

Accordingly, although there can be no assurance that we will undertake or successfully complete any transactions of the nature described above, any transactions that we do complete may be subject to the foregoing or other risks, and could have a material adverse effect on our business, financial condition and results of operations.

Our employment agreements with each of our executive officers may require us to pay severance benefits to any of those persons who are terminated in connection with a change in control of our company, which could harm our financial condition or results.

All of our executive officers are parties to employment agreements that contain change in control and severance provisions in the event of a termination of employment in connection with a change in control of our company providing for cash payments for severance and other benefits and acceleration of vesting of stock options and shares of restricted stock. The accelerated vesting of options and shares of restricted stock could result in dilution to our existing stockholders and lower the market price of our common stock. The payment of these severance benefits could harm our financial condition and results. In addition, these potential severance payments may discourage or prevent third parties from seeking a business combination with us.

#### Risks Relating to Our Intellectual Property

We may not be successful in obtaining or maintaining necessary rights to our drug candidates through acquisitions and in-licenses.

We currently have intellectual property rights to develop our drug candidates through a license from Ligand. As of December 31, 2015, we did not own any patents or have any patent applications pending. Because our programs require the use of proprietary rights held by Ligand, the growth of our business will likely depend in part on our ability to maintain and exploit these proprietary rights. In addition, we may need to acquire or in-license additional intellectual property in the future. We may be unable to acquire or in-license any compositions, methods of use, processes or other intellectual property rights from third parties that we identify as necessary for our drug candidates. We face competition with regard to acquiring and in-licensing third-party intellectual property rights, including from a number of more established companies. These established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license intellectual property rights to us. We also may be unable to acquire or in-license third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment.

We may enter into collaboration agreements with U.S. and foreign academic institutions to accelerate development of our current or future preclinical drug candidates. Typically, these agreements include an option for the company to negotiate a license to the institution's intellectual property rights resulting from the collaboration. Even with such an option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to

us. If we are unable to license rights from a collaborating institution, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue our desired program.

If we are unable to successfully obtain required third-party intellectual property rights or maintain our existing intellectual property rights, we may need to abandon development of the related program and our business, financial condition and results of operations could be materially and adversely affected.



If we fail to comply with our obligations in the agreements under which we in-license intellectual property and other rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose intellectual property rights that are important to our business.

The Master License Agreement is important to our business and we expect to enter into additional license agreements in the future. The Master License Agreement imposes, and we expect that future license agreements will impose, various diligence, milestone payment, royalty and other obligations on us. If we fail to comply with our obligations under these agreements, or if we file for bankruptcy, we may be required to make certain payments to the licensor, we may lose the exclusivity of our license, or the licensor may have the right to terminate the license, in which event we would not be able to develop or market products covered by the license. Additionally, the milestone and other payments associated with these licenses could materially and adversely affect our business, financial condition and results of operations.

Pursuant to the terms of the Master License Agreement, Ligand may terminate the Master License Agreement under certain circumstances, including, but not limited to: (1) in the event of our insolvency or bankruptcy, (2) if we do not pay an undisputed amount owing under the Master License Agreement when due and fail to cure such default within a specified period of time, or (3) if we default on certain of our material obligations and fail to cure the default within a specified period of time. If the Master License Agreement is terminated in its entirety or with respect to a specific licensed program for any reason, among other consequences, all licenses granted to us under the Master License Agreement (or with respect to the specific licensed program) will terminate and we may be requested to assign and transfer to Ligand certain regulatory documentation and regulatory approvals related to the licensed programs (or those related to the specific licensed program), and we may be required to wind down any ongoing clinical trials with respect to the licensed programs (or those related to the specific licensed program). Additionally, Ligand may require us to assign to Ligand the trademarks owned by us relating to the licensed programs (or those related to the specific licensed program), and we would be obligated to grant to Ligand a license under any patent rights and know-how controlled by us to the extent necessary to make, have made, import, use, offer to sell and sell the licensed programs (or those related to the specific licensed program) anywhere in the world at a royalty rate in the low single digits.

In some cases, patent prosecution of our licensed technology may be controlled solely by the licensor. If our licensor fails to obtain and maintain patent or other protection for the proprietary intellectual property we in-license, then we could lose our rights to the intellectual property or our exclusivity with respect to those rights, and our competitors could market competing products using the intellectual property. In certain cases, we may control the prosecution of patents resulting from licensed technology. In the event we breach any of our obligations related to such prosecution, we may incur significant liability to our licensing partners. Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues. Disputes may arise regarding intellectual property subject to a licensing agreement, including, but not limited to:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our collaborators; and
- the priority of invention of patented technology.

If disputes over intellectual property and other rights that we have in-licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected drug candidates. If we fail to comply with any such obligations to our licensor, such licensor may terminate their licenses to us, in which case we would not be able to market products covered by these licenses. The

loss of our license with Ligand, and potentially other licenses that we enter into in the future, would have a material adverse effect on our business.

We may be required to pay milestones and royalties to Ligand in connection with our use of the licensed technology under the Master License Agreement, which could adversely affect the overall profitability for us of any products that we may seek to commercialize.

Under the terms of the Master License Agreement, we may be obligated to pay Ligand up to an aggregate of approximately \$1.54 billion in development, regulatory and sales milestones. We will also be required to pay Ligand single-digit royalties on future worldwide net product sales. These royalty payments could adversely affect the overall profitability for us of any products that we may seek to commercialize.

We may not be able to protect our proprietary or licensed technology in the marketplace.

We depend on our ability to protect our proprietary or licensed technology. We rely on trade secret, patent, copyright and trademark laws, and confidentiality, licensing and other agreements with employees and third parties, all of which offer only limited protection. Our success depends in large part on our ability, Ligand's and any future licensor's or licensee's ability to obtain and maintain patent protection in the U.S. and other countries with respect to our proprietary or licensed technology and products. We currently in-license all of our intellectual property rights to develop our drug candidates and may in-license additional intellectual property rights in the future. Under the terms of the Master License Agreement, Ligand has the first right to file, prosecute and maintain the patents subject to the Master License Agreement in its name. We cannot be certain that patent enforcement activities by our current or future licensors have been or will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents or other intellectual property rights. We also cannot be certain that our current or future licensors will allocate sufficient resources or prioritize their or our enforcement of such patents. Even if we are not a party to these legal actions, an adverse outcome could prevent us from continuing to license intellectual property that we may need to operate our business, which would have a material adverse effect on our business, financial condition and results of operations.

We believe we will be able to obtain, through prosecution of patent applications covering technology licensed from others, adequate patent protection for our proprietary drug technology, including those related to our in-licensed intellectual property. If we are compelled to spend significant time and money protecting or enforcing our licensed patents and future patents we may own, designing around patents held by others or licensing or acquiring, potentially for large fees, patents or other proprietary rights held by others, our business, financial condition and results of operations may be materially and adversely affected. If we are unable to effectively protect the intellectual property that we own or in-license, other companies may be able to offer the same or similar products for sale, which could materially adversely affect our business, financial condition and results of operations. The patents of others from whom we may license technology, and any future patents we may own, may be challenged, narrowed, invalidated or circumvented, which could limit our ability to stop competitors from marketing the same or similar products or limit the length of term of patent protection that we may have for our products.

Obtaining and maintaining patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents and/or patent applications will be due to be paid to the U.S. Patent and Trademark Office, or the USPTO, and various governmental patent agencies outside of the U.S. in several stages over the lifetime of the applicable patent and/or patent application. The USPTO and various non-U.S. governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. In many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. However, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. If this occurs with respect to our in-licensed patents or patent applications we may file in the future, our competitors might be able to use our technologies, which would have a material adverse effect on our business, financial condition and results of

operations.

The patent positions of pharmaceutical products are often complex and uncertain. The breadth of claims allowed in pharmaceutical patents in the U.S. and many jurisdictions outside of the U.S. is not consistent. For example, in many jurisdictions, the support standards for pharmaceutical patents are becoming increasingly strict. Some countries prohibit method of treatment claims in patents. Changes in either the patent laws or interpretations of patent laws in the U.S. and other countries may diminish the value of our licensed or owned intellectual property or create uncertainty. In addition, publication of information related to our current drug candidates and potential products may prevent us from obtaining or enforcing patents relating to these drug candidates and potential products, including without limitation composition-of-matter patents, which are generally believed to offer the strongest patent protection.

Our intellectual property includes licenses covering issued patents and pending patent applications for composition of matter and method of use. For VK5211, we in-license five patents in the U.S. and several other patents in certain foreign jurisdictions. For each of VK2809 and VK0214, we in-license a patent in the U.S. and, for VK2809, additional patents in certain foreign jurisdictions. For VK0612, we in-license two patents in the U.S. and several other patents in certain foreign jurisdictions. With respect to our other current drug candidates, we have a license covering several issued patents and pending patent applications both in the U.S. and in certain foreign jurisdictions. See “Intellectual Property” under Part I, “Item 1. Business” of this Annual Report on Form 10-K.

Patents that we currently license and patents that we may own or license in the future do not necessarily ensure the protection of our licensed or owned intellectual property for a number of reasons, including, without limitation, the following:

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- the patents may not be broad or strong enough to prevent competition from other products that are identical or similar to our drug candidates;
- there can be no assurance that the term of a patent can be extended under the provisions of patent term extension afforded by U.S. law or similar provisions in foreign countries, where available;
- the issued patents and patents that we may obtain or license in the future may not prevent generic entry into the U.S. market for our drug candidates;
- we do not at this time license or own a granted European patent or national phase patents in any European jurisdictions that would prevent generic entry into the European market for one of our primary drug candidates, VK2809;
- we, or third parties from whom we in-license or may license patents, may be required to disclaim part of the term of one or more patents;
- there may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim;
- there may be prior art of which we are aware, which we do not believe affects the validity or enforceability of a patent claim, but which, nonetheless, ultimately may be found to affect the validity or enforceability of a patent claim;
- there may be other patents issued to others that will affect our freedom to operate;
- if the patents are challenged, a court could determine that they are invalid or unenforceable;
- there might be a significant change in the law that governs patentability, validity and infringement of our licensed patents or any future patents we may own that adversely affects the scope of our patent rights;
- a court could determine that a competitor's technology or product does not infringe our licensed patents or any future patents we may own; and
- the patents could irretrievably lapse due to failure to pay fees or otherwise comply with regulations or could be subject to compulsory licensing.

If we encounter delays in our development or clinical trials, the period of time during which we could market our potential products under patent protection would be reduced.

Our competitors may be able to circumvent our licensed patents or future patents we may own by developing similar or alternative technologies or products in a non-infringing manner. Our competitors may seek to market generic versions of any approved products by submitting abbreviated new drug applications to the FDA in which our competitors claim that our licensed patents or any future patents we may own are invalid, unenforceable or not infringed. Alternatively, our competitors may seek approval to market their own products similar to or otherwise competitive with our products. In these circumstances, we may need to defend or assert our licensed patents or any future patents we may own, including by filing lawsuits alleging patent infringement. In any of these types of proceedings, a court or other agency with jurisdiction may find our licensed patents or any future patents we may own invalid or unenforceable. We may also fail to identify patentable aspects of our research and development before it is too late to obtain patent protection. Even if we own or in-license valid and enforceable patents, these patents still may not provide protection against competing products or processes sufficient to achieve our business objectives.

The issuance of a patent is not conclusive as to its inventorship, scope, ownership, priority, validity or enforceability. In this regard, third parties may challenge our licensed patents or any future patents we may own in the courts or patent offices in the U.S. and abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and potential products. In addition, given the amount of time required for the development, testing and regulatory review of new drug candidates, patents protecting such drug candidates might expire before or shortly after such drug candidates are commercialized.

We may infringe the intellectual property rights of others, which may prevent or delay our drug development efforts and prevent us from commercializing or increase the costs of commercializing our products.

Our commercial success depends significantly on our ability to operate without infringing the patents and other intellectual property rights of third parties. For example, there could be issued patents of which we are not aware that our current or potential future drug candidates infringe. There also could be patents that we believe we do not infringe, but that we may ultimately be found to infringe.

Moreover, patent applications are in some cases maintained in secrecy until patents are issued. The publication of discoveries in the scientific or patent literature frequently occurs substantially later than the date on which the underlying discoveries were made and patent applications were filed. Because patents can take many years to issue, there may be currently pending applications of which we are unaware that may later result in issued patents that our drug candidates or potential products infringe. For example, pending applications may exist that claim or can be amended to claim subject matter that our drug candidates or potential products infringe. Competitors may file continuing patent applications claiming priority to already issued patents in the form of continuation, divisional, or continuation-in-part applications, in order to maintain the pendency of a patent family and attempt to cover our drug candidates.

Third parties may assert that we are employing their proprietary technology without authorization and may sue us for patent or other intellectual property infringement. These lawsuits are costly and could adversely affect our business, financial condition and results of operations and divert the attention of managerial and scientific personnel. If we are sued for patent infringement, we would need to demonstrate that our drug candidates, potential products or methods either do not infringe the claims of the relevant patent or that the patent claims are invalid, and we may not be able to do this. Proving invalidity is difficult. For example, in the U.S., proving invalidity requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. Even if we are successful in these proceedings, we may incur substantial costs and the time and attention of our management and scientific personnel could be diverted in pursuing these proceedings, which could have a material adverse effect on us. In addition, we may not have sufficient resources to bring these actions to a successful conclusion. If a court holds that any third-party patents are valid, enforceable and cover our products or their use, the holders of any of these patents may be able to block our ability to commercialize our products unless we acquire or obtain a license under the applicable patents or until the patents expire.

We may not be able to enter into licensing arrangements or make other arrangements at a reasonable cost or on reasonable terms. Any inability to secure licenses or alternative technology could result in delays in the introduction of our products or lead to prohibition of the manufacture or sale of products by us. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. We could be forced, including by court order, to cease commercializing the infringing technology or product. In addition, in any such proceeding or litigation, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing our drug candidates or force us to cease some of our business operations, which could materially and adversely affect our business, financial condition and results of operations. Any claims by third parties that we have misappropriated their confidential information or trade secrets could have a similar material and adverse effect on our business, financial condition and results of operations. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations.

Any claims or lawsuits relating to infringement of intellectual property rights brought by or against us will be costly and time consuming and may adversely affect our business, financial condition and results of operations.

We may be required to initiate litigation to enforce or defend our licensed and owned intellectual property. For example, we are currently aware of at least two third-party companies that are selling products in the U.S. bearing the name "LGD-4033", which is the name previously used by Ligand to refer to VK5211, without authority from either us or Ligand, and we may experience other potential intellectual property infringement in the future. Lawsuits to protect our intellectual property rights can be very time consuming and costly. There is a substantial amount of litigation involving patent and other intellectual property rights in the biopharmaceutical industry generally. Such litigation or proceedings could substantially increase our operating expenses and reduce the resources available for development activities or any future sales, marketing or distribution activities.

In any infringement litigation, any award of monetary damages we receive may not be commercially valuable. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during litigation. Moreover, there can be no assurance that we will have sufficient financial or other resources to file and pursue such infringement claims, which typically last for years before they are resolved. Further, any claims we assert against a perceived infringer could provoke these parties to assert counterclaims against us alleging that we have infringed their patents. 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.

In addition, our licensed patents and patent applications, and patents and patent applications that we may own or license in the future, could face other challenges, such as interference proceedings, opposition proceedings, re-examination proceedings and other forms of post-grant review. Any of these challenges, if successful, could result in the invalidation of, or in a narrowing of the scope of, any of our licensed patents and patent applications and patents and patent applications that we may own or license in the future subject to challenge. Any of these challenges, regardless of their success, would likely be time consuming and expensive to defend and resolve and would divert our management and scientific personnel's time and attention.



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 material adverse effect on the market price of our common stock.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products.

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involves both technological and legal complexity and is costly, time-consuming and inherently uncertain. For example, the U.S. has recently enacted and is currently implementing wide-ranging patent reform legislation. Specifically, on September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law and included a number of significant changes to U.S. patent law. These included changes in the way patent applications will be prosecuted, including a transition to a “first-to-file” system for deciding which party should be granted a patent when two or more patent applications are filed by different parties claiming the same invention, and may also affect patent litigation. Under a “first-to-file” system, a third party that files a patent application with the USPTO before us could be awarded a patent covering an invention of ours even if we made the invention before it was made by the third party. The USPTO has developed new and untested regulations and procedures to govern the full implementation of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the “first-to-file” provisions, became effective in March 2013. The Leahy-Smith Act has also introduced procedures that may make it easier for third parties to challenge issued patents, as well as to intervene in the prosecution of patent applications. Finally, the Leahy-Smith Act contains new statutory provisions that still require the USPTO to issue new regulations for their implementation, and it may take the courts years to interpret the provisions of the new statute. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on our business, the cost of prosecuting our licensed and future patent applications, our ability to obtain patents based on our licensed and future patent applications and our ability to enforce or defend our licensed or future issued patents. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our licensed and future patent applications and the enforcement or defense of our licensed and future patents, all of which could have a material adverse effect on our business, financial condition and results of operations.

In addition, the U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce patents that we might obtain in the future.

We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting and defending patents on drug candidates throughout the world would be prohibitively expensive. Competitors may use our licensed and owned technologies in jurisdictions where we have not licensed or obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we may obtain or license patent protection, but where patent enforcement is not as strong as that in the U.S. These products may compete with our products in jurisdictions where we do not have any issued or licensed patents and any future patent claims or other intellectual property rights may not be effective or sufficient to prevent them from so competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents and other intellectual property protection, particularly those relating to biopharmaceuticals, which could make it difficult for us to stop the infringement of our licensed patents and future patents we may own, or marketing of competing products in violation of our proprietary rights generally. Further, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the U.S. As a result, we may encounter significant problems in protecting and defending our licensed and owned intellectual property both in the U.S. and abroad. For example, China, where we currently have seven licensed patents and three licensed patent applications, currently affords less protection to a company's intellectual property than some other jurisdictions. As such, the lack of strong patent and other intellectual property protection in China may significantly increase our vulnerability as regards unauthorized disclosure or use of our intellectual property and undermine our competitive position. Proceedings to enforce our future patent rights, if any, in foreign jurisdictions could result in substantial cost and divert our efforts and attention from other aspects of our business.

Many countries, including European Union countries, India, Japan and China, have compulsory licensing laws under which a patent owner may be compelled under certain circumstances to grant licenses to third parties. In those countries, we currently have an aggregate of 19 licensed patents and 13 licensed patent applications and may have limited remedies if patents are infringed or if we are

compelled to grant a license to a third party, which could materially diminish the value of those patents. This could limit our potential revenue opportunities. Accordingly, our efforts to enforce intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we own or license.

We may be unable to adequately prevent disclosure of trade secrets and other proprietary information.

In order to protect our proprietary and licensed technology and processes, we rely in part on confidentiality agreements with our corporate partners, employees, consultants, manufacturers, outside scientific collaborators and sponsored researchers and other advisors. These agreements may not effectively prevent disclosure of our confidential information and may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. In addition, others may independently discover our trade secrets and proprietary information. Failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties.

We employ individuals who were previously employed at other biopharmaceutical companies. Although we have no knowledge of any such claims against us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed confidential information of our employees' former employers or other third parties. Litigation may be necessary to defend against these claims. There is no guarantee of success in defending these claims, and even if we are successful, litigation could result in substantial cost and be a distraction to our management and other employees. To date, none of our employees have been subject to such claims.

We may be subject to claims challenging the inventorship of our licensed patents, any future patents we may own and other intellectual property.

Although we are not currently experiencing any claims challenging the inventorship of our licensed patents or our licensed or owned intellectual property, we may in the future be subject to claims that former employees, collaborators or other third parties have an interest in our licensed patents or other licensed or owned intellectual property as an inventor or co-inventor. For example, we may have inventorship disputes arise from conflicting obligations of consultants or others who are involved in developing our drug candidates. Litigation may be necessary to defend against these and other claims challenging inventorship. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business, financial condition and results of operations. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

If we do not obtain additional protection under the Hatch-Waxman Amendments and similar foreign legislation extending the terms of our licensed patents and any future patents we may own, our business, financial condition and results of operations may be materially and adversely affected.

Depending upon the timing, duration and specifics of FDA regulatory approval for our drug candidates, one or more of our licensed U.S. patents or future U.S. patents that we may license or own may be eligible for limited patent term restoration under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during drug development and the FDA regulatory review process. This period is generally one-half the time between the effective date of an investigational new drug application, or IND (falling after

issuance of the patent), and the submission date of an NDA, plus the time between the submission date of an NDA and the approval of that application. Patent term restorations, however, cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval by the FDA.

The application for patent term extension is subject to approval by the USPTO, in conjunction with the FDA. It takes at least six months to obtain approval of the application for patent term extension. We may not be granted an extension because of, for example, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or restoration or the term of any such extension is less than we request, the period during which we will have the right to exclusively market our product will be shortened and our competitors may obtain earlier approval of competing products, and our ability to generate revenues could be materially adversely affected.

## Risks Relating to Ownership of Our Common Stock

The market price of our common stock may be highly volatile.

The trading price of our common stock is likely to be volatile. Our stock price could be subject to wide fluctuations in response to a variety of factors, including the following:

- any delay in filing an NDA for any of our drug candidates and any adverse development or perceived adverse development with respect to the FDA's review of that NDA;
- adverse results or delays in clinical trials, if any;
- significant lawsuits, including patent or stockholder litigation;
- inability to obtain additional funding;
- failure to successfully develop and commercialize our drug candidates;
- changes in laws or regulations applicable to our drug candidates;
- inability to obtain adequate product supply for our drug candidates, or the inability to do so at acceptable prices;
- unanticipated serious safety concerns related to any of our drug candidates;
- adverse regulatory decisions;
- introduction of new products or technologies by our competitors;
- failure to meet or exceed drug development or financial projections we provide to the public;
- failure to meet or exceed the estimates and projections of the investment community;
- the perception of the biopharmaceutical industry by the public, legislatures, regulators and the investment community;
- announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us or our competitors;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our licensed and owned technologies;
- additions or departures of key scientific or management personnel;
- changes in the market valuations of similar companies;
- general economic and market conditions and overall fluctuations in the U.S. equity market;
- sales of our common stock by us or our stockholders, including Ligand, in the future; and
- trading volume of our common stock.

In addition, the stock market, in general, and small biopharmaceutical companies, in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. Further, a decline in the financial markets and related factors beyond our control may cause our stock price to decline rapidly and unexpectedly.

An active trading market for our common stock may not be sustained, and you may not be able to resell your common stock at a desired market price.

Our shares of common stock began trading on the Nasdaq Capital Market on April 29, 2015. If no active trading market for our common stock develops or is sustained, you may be unable to sell your shares when you wish to sell them or at a price that you consider attractive or satisfactory. The lack of an active market may also adversely affect our ability to raise capital by selling securities in the future, or impair our ability to acquire or in-license other drug candidates, businesses or technologies using our shares as consideration.

Our management owns a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.

As of December 31, 2015, our executive officers, directors and 5% or greater stockholders beneficially owned 73.2% of our common stock. Therefore, our executive officers, directors and 5% or greater stockholders have the ability to influence us through this ownership position.

This significant concentration of stock ownership may adversely affect the trading price for our common stock because investors often perceive disadvantages in owning stock in companies with controlling stockholders. As a result, these stockholders, if they acted together, could significantly influence all matters requiring approval by our stockholders, including the election of directors and the approval of mergers or other business combination transactions. These stockholders may be able to determine all matters requiring stockholder approval. The interests of these stockholders may not always coincide with our interests or the interests of other stockholders. This may also prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders and they may act in a manner that advances their best interests and not necessarily those of other stockholders, including seeking a premium value for their common stock, and might affect the prevailing market price for our common stock.

Ligand is our largest stockholder, which may limit the ability of our stockholders to influence corporate matters and may give rise to conflicts of interest.

As of December 31, 2015, Ligand and its affiliates beneficially owned approximately 49.4% of our outstanding common stock. In addition to the above ownership, upon the consummation of our first bona fide capital financing transaction occurring after January 22, 2016, but prior to May 21, 2017, with aggregate net proceeds to us of at least \$2,000,000, we will be obligated to repay \$1,500,000 to Ligand under that certain Secured Convertible Promissory Note held by Ligand, or the Ligand Note, with at least \$300,000 of such payment to be paid in cash and the balance to be paid in shares of our common stock. Furthermore, we may in the future elect or be obligated to repay amounts outstanding under the Ligand Note to Ligand in shares of our common stock. Accordingly, Ligand may be able to exert significant influence over us and any action requiring the approval of the holders of our common stock, including the election of directors and the approval of mergers or other business combination transactions. This concentration of voting power may make it less likely that any other holder of our common stock or our board of directors will be able to affect the way we are managed and could delay or prevent an acquisition of us on terms that other stockholders may desire.

Furthermore, the interests of Ligand may not be aligned with our other stockholders and this could lead to actions that may not be in the best interests of our other stockholders. For example, Ligand may have different tax positions or strategic plans for us, which could influence its decisions regarding whether and when we should dispose of assets or incur new or refinance existing indebtedness. In addition, Ligand's significant ownership in us may discourage someone from making a significant equity investment in us, or could discourage transactions involving a change in control, including transactions in which our stockholders might otherwise receive a premium for their shares over the then-current market price.

Pursuant to the management rights letter between us and Ligand, dated May 21, 2014, Ligand has the right to nominate one individual for election to our board of directors. Matthew W. Foehr, Ligand's President and Chief Operating Officer, is the current member of our board of directors nominated by Ligand. As a result of our relationship with Ligand, there may be transactions between us and Ligand that could present an actual or perceived conflict of interest. These conflicts of interest may lead Mr. Foehr to recuse himself from deliberation and voting as a member of our board of directors with respect to any transactions involving Ligand or its affiliates.

In addition, if Ligand obtains a majority of our common stock, Ligand would be able to control a number of matters submitted to our stockholders for approval, as well as our management and affairs. For example, Ligand would be able to control the election of directors, and may be able to control amendments to our organizational documents and approvals of any merger, consolidation, sale of all or substantially all of our assets or other business combination or reorganization. In addition, if Ligand obtains a majority of our common stock, we would be deemed a “controlled company” within the meaning of the rules and listing standards of The Nasdaq Stock Market LLC. Under the rules and listing standards of The Nasdaq Stock Market LLC, a company of which more than 50% of the voting power is held by another person or group of persons acting together is a “controlled company” and may elect not to comply with certain rules and listing standards of The Nasdaq Stock Market LLC regarding corporate governance, including: (1) the requirement that a majority of our board of directors consist of independent directors, (2) the requirement that the compensation of our officers be determined or recommended to our board of directors by a compensation committee that is composed entirely of independent directors, and (3) the requirement that director nominees be selected or recommended to our board of directors by a majority of independent directors or a nominating committee that is composed entirely of independent directors.

We are an “emerging growth company” within the meaning of the Securities Act of 1933, as amended, or the Securities Act, and if we decide to take advantage of certain exemptions from various reporting requirements applicable to emerging growth companies, our common stock could be less attractive to investors.

For as long as we remain an “emerging growth company”, as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, we will have the option to take advantage of certain exemptions from various reporting and other requirements that are applicable to other public companies that are not “emerging growth companies,” including, but not limited to, not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, as amended, or the Sarbanes-Oxley Act, 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. We may take advantage of these and other exemptions until we are no longer an “emerging growth company”.

The JOBS Act provides that an emerging growth company can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act for complying with new or revised accounting standards. However, we have chosen to “opt out” of such extended transition period, and as a result, we will comply with new or revised accounting standards on the relevant dates on which adoption of such standards is required for non-emerging growth companies. Our decision to “opt out” of the extended transition period is irrevocable.

We will remain an emerging growth company until the earliest of (1) the last day of the fiscal year during which we have total annual gross revenues of \$1.0 billion or more, (2) December 31, 2020 (the last day of the fiscal year following the fifth anniversary of the completion of our initial public offering), (3) the date on which we have, during the previous three-year period, issued more than \$1.0 billion in non-convertible debt, and (4) the date on which we are deemed to be a “large accelerated filer” under the Securities Exchange Act of 1934, as amended, or the Exchange Act (i.e., the first day of the fiscal year after we have (a) more than \$700,000,000 in outstanding common equity held by our non-affiliates, measured each year on the last day of our second fiscal quarter, and (b) been public for at least 12 months).

Even after we no longer qualify as an emerging growth company, we may still qualify as a “smaller reporting company,” which would allow us to take advantage of many of the same exemptions from disclosure requirements including exemption from compliance with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. We cannot predict if investors will find our common stock less attractive because we may 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.

Our internal control over financial reporting does not currently meet the standards required by Section 404 of the Sarbanes-Oxley Act, and we have previously identified a material weakness, and failure to achieve and maintain effective internal control over financial reporting in accordance with Section 404 of the Sarbanes-Oxley Act, could have a material adverse effect on our business and share price.

As a privately held company, we were not required to evaluate our internal control over financial reporting in a manner that meets the standards of publicly traded companies required by Section 404 of the Sarbanes-Oxley Act, or Section 404. In the course of auditing our financial statements as of and for the year ended December 31, 2013, our independent registered public accounting firm identified a material weakness in our internal control over financial reporting relating to our failure to perform periodic reconciliations on various accounts. We remediated this material weakness in the year ended December 31, 2014.



Commencing with our Annual Report on Form 10-K for the fiscal year ending December 31, 2016, our management will be required to report on the effectiveness of our internal control over financial reporting. Additionally, under the JOBS Act, our independent registered public accounting firm will not be required to attest to the effectiveness of our internal control over financial reporting pursuant to Section 404 until we are no longer an “emerging growth company.” The rules governing the standards that must be met for our management to assess our internal control over financial reporting are complex and require significant documentation, testing and possible remediation.

In connection with the implementation of the necessary procedures and practices related to internal control over financial reporting, we may identify deficiencies or material weaknesses that we may not be able to remediate in time to meet the deadline imposed by the Sarbanes-Oxley Act for compliance with the requirements of Section 404. In addition, we may encounter problems or delays in completing the implementation of any requested improvements and receiving a favorable attestation in connection with the attestation provided by our independent registered public accounting firm. Failure to achieve and maintain an effective internal control environment could have a material adverse effect on our business, financial condition and results of operations and could limit our ability to report our financial results accurately and in a timely manner.

We will incur significant increased costs as a result of operating as a public company, our management has limited experience managing a public company, and our management will be required to devote substantial time to new compliance initiatives.

As a public company and particularly after we cease to be an “emerging growth company,” we will incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act of 2010, or the Dodd-Frank Act, as well as rules subsequently implemented by the Securities and Exchange Commission, or the SEC, and The Nasdaq Stock Market LLC have imposed various requirements on public companies. There are significant corporate governance and executive compensation related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact (in ways we cannot currently anticipate) the manner in which we operate our business. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain our current levels of such insurance coverage.

As a publicly traded company, we have incurred and will incur legal, accounting and other expenses associated with the SEC reporting requirements applicable to a company whose securities are registered under the Exchange Act, as well as corporate governance requirements, including those under the Sarbanes-Oxley Act, the Dodd-Frank Act and other rules implemented by the SEC and The Nasdaq Stock Market LLC. In addition, we expect that we will need to hire additional personnel in our finance department to help us comply with the various requirements applicable to public companies. The expenses incurred by public companies generally to meet SEC reporting, finance and accounting and corporate governance requirements have been increasing in recent years as a result of changes in rules and regulations and the adoption of new rules and regulations applicable to public companies.

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

The trading market for our common stock depends, in part, on the research and reports that securities or industry analysts publish about us or our business. If one or more of the analysts who cover us downgrade our stock or publish inaccurate or unfavorable research about our business, our stock price would likely decline. In addition, if our operating results fail to meet the forecast of analysts, our stock price would likely decline. If one or more of these analysts cease coverage of our company or fail to publish reports on us regularly, demand for our common stock could decrease, which might cause our stock price and trading volume to decline.

Sales of a substantial number of shares of our common stock in the public market by our existing stockholders, or future issuances of our common stock or rights to purchase our common stock, could cause our stock price to fall.

Sales of a substantial number of shares of our common stock by our existing stockholders in the public market, or the perception that these sales might occur, could depress the market price of our common stock and could impair our ability to raise capital through the sale of additional equity securities. We are unable to predict the effect that such sales may have on the prevailing market price of our common stock.

Ligand is subject to a lock-up agreement with us that restricts its ability to transfer shares of our common stock until January 23, 2017. Beginning on October 25, 2015, shares held by our executive officers, directors, 5% or greater stockholders (other than Ligand) and their affiliates and family members were released from lock-up agreements with

the underwriters of our initial public offering and, subject to certain limitations, including sales volume limitations, became eligible for sale in the public market. Sales of stock by these stockholders, or the perception that these sales may occur, could have a material adverse effect on the trading price of our common stock.

Certain holders of our securities are entitled to rights with respect to the registration of their shares under the Securities Act, subject to the lock-up arrangement described above. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act. Any sales of securities by these stockholders, or the perception that these sales may occur, could have a material adverse effect on the trading price of our common stock.

Pursuant to our 2014 Equity Incentive Plan, or the 2014 Plan, a total of 1,214,773 shares of our common stock were reserved as of December 31, 2015 for issuance to our employees, directors and consultants. Under the terms of the 2014 Plan, the number of shares available for issuance under the 2014 Plan is, unless otherwise determined by our Board of Directors or the Compensation Committee of our Board of Directors, automatically increased on January 1st of each year in an amount equal to 3.5% of the total number of shares of our common stock outstanding on December 31st of the preceding calendar year. To the extent new equity awards are

granted and exercised or we issue additional shares of common stock in the future, our stockholders may experience additional dilution, which could cause our stock price to fall.

Our management will continue to have broad discretion over the use of the proceeds we received in our initial public offering and from the Loan and Security Agreement and might not apply the proceeds in ways that increase the value of your investment.

Our management will continue to have broad discretion to use the net proceeds from our initial public offering and the proceeds from the Loan and Security Agreement and you will be relying on the judgment of our management regarding the application of these proceeds. Our management might not apply the proceeds in ways that ultimately increase the value of your investment and the failure by our management to apply these proceeds effectively could harm our business. Because of the number and variability of factors that will determine our use of the remaining net proceeds from our initial public offering and the proceeds from the Loan and Security Agreement, their ultimate use may vary substantially from their currently intended use. If we do not invest or apply the net proceeds from our initial public offering or the proceeds from the Loan and Security Agreement in ways that enhance stockholder value, we may fail to achieve the expected financial results, which could cause our stock price to decline.

We are at risk of securities class action litigation.

In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because biopharmaceutical companies have experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion of management's attention and resources, which could harm our business, financial condition and results of operations.

Our ability to use our net operating loss carryforwards may be subject to certain limitations.

At December 31, 2015, we had net operating loss carryforwards of approximately \$5,890,000 for federal and \$6,629,000 for state tax purposes, both of which will begin to expire in 2032. Our ability to utilize our federal net operating loss carryforwards may be limited under Section 382 of the Internal Revenue Code of 1986, as amended, or the Code. In the event of an "ownership change," Section 382 imposes an annual limitation on the amount of post-ownership change taxable income that may be offset with pre-ownership change net operating losses of the loss corporation experiencing the ownership change. An "ownership change" is defined by Section 382 as a cumulative change in ownership of our company of more than 50% within a three-year period. Our initial public offering in May 2015 resulted in an "ownership change" of us. We performed an analysis and determined that \$4,258,000 of the federal and \$5,155,000 of the state tax net operating loss carryforwards are available for utilization as of December 31, 2015. The full amounts of the \$5,890,000 federal and \$6,629,000 state tax net operating loss carryforwards will be available for utilization as of December 31, 2016. In addition, current or future changes in our stock ownership may trigger an "ownership change," some of which may be outside our control. Accordingly, our ability to utilize our net operating loss carryforwards to offset federal taxable income, if any, will likely be limited by Section 382, which could potentially result in increased future tax liability to us.

We may never pay dividends on our common stock so any returns would be limited to the appreciation of our stock.

We have never declared or paid any cash dividend on our common stock. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. In addition, under the Loan and Security Agreement with Ligand, we may not declare or pay dividends in respect of our common stock without Ligand's prior written consent. Any return to stockholders will therefore be limited to the appreciation of their stock.

Provisions in our amended and restated certificate of incorporation and bylaws, as well as provisions of Delaware law, could make it more difficult or expensive for a third party to acquire us or change our board of directors or current management.

Some provisions of our charter documents and Delaware law may have anti-takeover effects that could discourage an acquisition of us by others, even if an acquisition would be beneficial to our stockholders, and may prevent attempts by our stockholders to replace or remove our current management. These provisions include:

- authorizing the issuance of “blank check” preferred stock, the terms of which may be established and shares of which may be issued without stockholder approval;
- limiting the removal of directors by the stockholders;
- creating a classified board of directors;

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- providing that no stockholder is permitted to cumulate votes at any election of directors;
- allowing the authorized number of our directors to be changed only by resolution of our board of directors;
- prohibiting stockholder action by written consent, thereby requiring all stockholder actions to be taken at a meeting of our stockholders;
- requiring the approval of the holders of at least 66 2/3% of the votes that all our stockholders would be entitled to cast to amend or repeal specified provisions of our charter documents;
- eliminating the ability of stockholders to call a special meeting of stockholders; and
- establishing advance notice requirements for nominations for election to our board of directors or for proposing matters that can be acted upon at stockholder meetings.

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, which is responsible for appointing the members of our management. In addition, we are subject to Section 203 of the General Corporation Law of the State of Delaware, or the DGCL, which generally prohibits a Delaware corporation from engaging in any of a broad range of business combinations with an interested stockholder for a period of three years following the date on which the stockholder became an interested stockholder, unless such transactions are approved in advance by our board of directors or ratified by our board of directors and certain of our stockholders. This provision could have the effect of delaying or preventing a change in control, whether or not it is desired by or beneficial to our stockholders. Further, other provisions of Delaware law may also discourage, delay or prevent someone from acquiring us or merging with us.

Our amended and restated bylaws designate the Court of Chancery of the State of Delaware as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or other employees.

Our amended and restated bylaws provide that, unless we consent in writing to an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for (1) any derivative action or proceeding brought on our behalf, (2) any action asserting a claim of breach of a fiduciary duty owed by any director, officer or other employee to us or our stockholders, (3) any action asserting a claim against us or our directors, officers or employees arising pursuant to any provision of our amended and restated bylaws, our amended and restated certificate of incorporation or the DGCL, (4) any action asserting a claim against us or our directors, officers or employees that is governed by the internal affairs doctrine, or (5) any action to interpret, apply, enforce or determine the validity of our amended and restated bylaws or our amended and restated certificate of incorporation. Any person purchasing or otherwise acquiring any interest in any shares of our capital stock shall be deemed to have notice of and to have consented to this provision of our amended and restated bylaws. This choice-of-forum provision may limit our stockholders' ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or other employees, which may discourage such lawsuits. Alternatively, if a court were to find this provision of our amended and restated bylaws inapplicable or unenforceable with respect to one or more of the specified types of actions or proceedings, we may incur additional costs associated with resolving such matters in other jurisdictions, which could materially and adversely affect our business, financial condition and results of operations.

Item 1B. Unresolved Staff Comments.

None.

Item 2. Properties.

Our facilities consist of office space in San Diego, California. We lease approximately 7,049 square feet of space for our headquarters in San Diego, California under an agreement that expires on September 30, 2018. We believe that our existing facilities are adequate to meet our current needs, and that suitable additional alternative spaces will be available in the future on commercially reasonable terms.

Item 3. Legal Proceedings.

From time to time, we may be party to lawsuits in the ordinary course of business. We are not presently a party to any legal proceedings, the outcome of which, if determined adversely to us, would individually or in the aggregate be reasonably expected to have a material adverse effect on our business, operating results or financial condition.

Item 4. Mine Safety Disclosures.

Not applicable.

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## PART II

## Item 5. Market for Registrant’s Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities.

## Market Information

Our common stock began trading on the Nasdaq Capital Market on April 28, 2015 and trades under the symbol “VKTX”. Prior to April 28, 2015, there was no public market for our common stock. The following table sets forth the high and low sales prices per share of our common stock as reported on the Nasdaq Capital Market for the period indicated.

|                                      | Price Range |        |
|--------------------------------------|-------------|--------|
|                                      | High        | Low    |
| Year Ended December 31, 2015         |             |        |
| Second Quarter (from April 29, 2015) | \$10.23     | \$6.69 |
| Third Quarter                        | \$7.75      | \$5.00 |
| Fourth Quarter                       | \$7.14      | \$1.89 |

## Holders of Record

As of February 29, 2016, there were approximately 11 stockholders of record of our common stock. Certain shares are held in “street” name and, accordingly, the number of beneficial owners of such shares is not known or included in the foregoing number.

## Dividend Policy

We have never declared or paid any dividends on our common stock, and we currently intend to retain all available funds and any future earnings, if any, for use in our business. We do not anticipate paying any cash dividends in the foreseeable future. Any future determination to pay dividends will be made at the discretion of our board of directors or any authorized committee thereof after considering our financial condition, results of operations, capital requirements, business prospects and other factors our board of directors or such committee deems relevant, and will be subject to the restrictions contained in our current or future financing instruments. In addition, under our Loan and Security Agreement with Ligand Pharmaceuticals Incorporated, or Ligand, we may not declare or pay dividends in respect of our common stock without Ligand’s prior written consent.

## Issuer Repurchases of Equity Securities

None.

## Recent Sales of Unregistered Securities

None.

#### Performance Graph

We are a smaller reporting company, as defined by Rule 12b-2 of the Securities Exchange Act of 1934, as amended, and are not required to provide a performance graph.

#### Use of Proceeds

On May 4, 2015, we sold 3,000,000 shares of our common stock in our initial public offering at a public offering price of \$8.00 per share and on May 28, 2015, we sold 450,000 shares of our common stock at a public offering price of \$8.00 per share pursuant to the full exercise of the underwriters' option to purchase additional shares for aggregate gross proceeds of \$27,600,000, before deducting underwriting discounts, commissions and other offering expenses. The offer and sale of all of the shares in the offering were registered under the Securities Act of 1933, as amended, or the Securities Act, pursuant to a registration statement on Form S-1 (File No. 333-197182), which was declared effective by the Securities and Exchange Commission, or the SEC, on April 28, 2015, and a registration statement on Form S-1MEF (File No. 333-203702) filed pursuant to Rule 462(b) of the Securities Act. The offering commenced as of April 29, 2015 and did not terminate before all of the securities registered in the registration statements were sold. Laidlaw & Company (UK) Ltd. acted as the sole book-running manager for the offering. Feltham and Company, Inc. served as co-manager for the offering. After deducting underwriting discounts, commissions and other offering expenses paid by us of \$5,273,634, the net proceeds

from the offering were \$22,326,366. No offering expenses were paid or are payable, directly or indirectly, to our directors or officers, to persons owning 10% or more of any class of our equity securities, or to any of our affiliates.

The net proceeds from our initial public offering were originally deposited into money market funds. Since then, most of the proceeds have been invested in certificates of deposit as well as certain corporate debt securities with the balance of the net proceeds invested in money market funds. These investments are in accordance with our investment policy. As of December 31, 2015, we have used \$8,244,958 of the net proceeds from our initial public offering. There has been no material change in the expected use of the net proceeds from our initial public offering as described in the final prospectus, relating to our initial public offering, dated April 28, 2015, filed by us with the SEC on April 29, 2015.

#### Item 6. Selected Financial Data.

We are a smaller reporting company, as defined by Rule 12b-2 of the Securities Exchange Act of 1934, as amended, and are not required to provide the information required under this item.

Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations.

You should read the following discussion and analysis in conjunction with Part II, "Item 8. Financial Statements and Supplementary Data" included below in this Annual Report on Form 10-K. Operating results are not necessarily indicative of results that may occur in future periods.

The following discussion and analysis of our financial condition and results of operations contains forward-looking statements that involve a number of risks, uncertainties and assumptions. Actual events or results may differ materially from our expectations. Important factors that could cause actual results to differ materially from those stated or implied by our forward-looking statements include, but are not limited to, those set forth in Part I, "Item 1A. Risk Factors" in this Annual Report on Form 10-K. All forward-looking statements included in this Annual Report on Form 10-K are based on information available to us as of the time we file this Annual Report on Form 10-K and, except as required by law, we undertake no obligation to update publicly or revise any forward-looking statements.

Overview

We are a clinical-stage biopharmaceutical company focused on the development of novel, first-in-class or best-in-class therapies for metabolic and endocrine disorders. We have exclusive worldwide rights to a portfolio of five drug candidates in clinical trials or preclinical studies, which are based on small molecules licensed from Ligand Pharmaceuticals Incorporated, or Ligand. Our lead clinical program is VK5211, an orally available drug candidate, currently in a Phase 2 clinical trial for acute rehabilitation following non-elective hip fracture surgery. Hip fracture is a common injury among persons aged 60 and older. The acute recovery period post-injury is characterized by significant and rapid declines in bone mineral density, or BMD, and lean body mass, or LBM, which contributes to substantial morbidity and mortality in these patients. VK5211 is a non-steroidal selective androgen receptor modulator, or SARM. A SARM is designed to selectively interact with a subset of receptors that have a normal physiologic role of interacting with naturally-occurring hormones called androgens. Broad activation of androgen receptors with drugs, such as exogenous testosterone, can stimulate muscle growth and improve BMD, but often results in unwanted side effects such as prostate growth, hair growth and acne. VK5211 is expected to selectively produce the therapeutic benefits of testosterone in muscle and bone tissue, potentially accelerating rehabilitation and improving patient outcomes. VK5211 is also expected to have improved safety, tolerability and patient acceptance relative to testosterone. We commenced the Phase 2 clinical trial of VK5211 in October 2015 and expect to complete enrollment in the trial in the second half of 2016 and complete the trial in the first quarter of 2017.

Our second clinical program is VK2809, an orally available, tissue and receptor-subtype selective agonist of the thyroid hormone receptor beta, or TR $\beta$ , that is entering Phase 2 development for the treatment of patients with hypercholesterolemia and fatty liver disease. VK2809 belongs to a family of novel prodrugs which are cleaved in vivo to release potent thyromimetics. Selective activation of the TR $\beta$  receptor in liver tissue is believed to favorably affect cholesterol and lipoprotein levels via multiple mechanisms, including increasing the expression of low-density lipoprotein receptors and increasing mitochondrial fatty acid oxidation. We expect to commence the Phase 2 clinical trial of VK2809 in the first half of 2016 and to complete the trial in the fourth quarter of 2016 or the first quarter of 2017.

We are also developing VK0214 for X-linked adrenoleukodystrophy, or X-ALD, a rare X-linked, inherited neurological disorder characterized by a breakdown in the protective barriers surrounding brain and nerve cells. The disease, for which there is no approved treatment, is caused by mutations in a peroxisomal transporter of very long chain fatty acids, or VLCFA, known as ABCD1. As a result, transporter function is impaired and patients are unable to efficiently metabolize VLCFA. The TR $\beta$  receptor is known to regulate expression of an alternative VLCFA transporter, known as ABCD2. Various preclinical models have demonstrated that increased expression of ABCD2 can lead to normalization of VLCFA metabolism. Preliminary in vitro data suggest that VK0214 stimulates ABCD2

expression. We are conducting studies of VK0214 in an in vivo model of disease. Pending completion of this work, we expect in 2016 to commence work directed toward filing an Investigational New Drug Application, or IND.

We were incorporated under the laws of the State of Delaware on September 24, 2012. Since our incorporation, we have devoted substantially all of our efforts to raising capital, building infrastructure and obtaining the worldwide rights to certain technology, including VK5211, VK2809 and VK0214, pursuant to an exclusive license agreement with Ligand Pharmaceuticals Incorporated, or Ligand. The terms of this license agreement are detailed in the Master License Agreement, or the Master License Agreement, with Ligand, which we entered into on May 21, 2014 and amended on each of September 6, 2014 and April 8, 2015. A summary of the Master License Agreement, as amended, can be found under the heading “Agreements with Ligand—Master License Agreement” under Part I, “Item 1. Business” of this Annual Report on Form 10-K.

On May 4, 2015, we completed our initial public offering of our common stock, or the IPO, pursuant to a Registration Statement on Form S-1 that was declared effective on April 28, 2015. In the IPO, we sold 3,000,000 shares of our common stock at an initial public offering price of \$8.00 per share. The underwriters for the IPO had 30 days to exercise an over-allotment option to purchase up to an additional 450,000 shares at the initial public offering price, less the underwriting discount. Upon the closing of the IPO, on May 4, 2015, we raised a total of \$19,100,500 in net proceeds after deducting underwriting discounts, commissions and other offering expenses of \$4,899,500.

On May 26, 2015, the underwriters of the IPO exercised their full over-allotment option to purchase an additional 450,000 shares of our common stock. On May 28, 2015, we sold the 450,000 shares to the underwriters pursuant to the over-allotment option and received additional net proceeds of \$3,225,866 after deducting underwriting discounts, commissions and other offering expenses of \$374,134.

Although it is difficult to predict our liquidity requirements, as of December 31, 2015, and based upon our current operating plan, we do not believe that we will have sufficient cash to meet our projected operating requirements for at least the next 12 months unless we raise additional capital. As of December 31, 2015, we had an accumulated deficit of \$45,545,445. These losses have resulted principally from research and development costs incurred in connection with acquiring the exclusive worldwide rights to the portfolio of five drug candidates discussed above and the related non-cash interest expense recorded for increases in the deemed fair market value for the license fees payable to Ligand, research and development expenses related to the manufacturing of clinical drug product and clinical development of VK5211, VK2809 and VK0214, consulting fees and general and administrative expenses. We anticipate that we will continue to incur net losses for the foreseeable future as we continue the development of our clinical drug candidates and preclinical programs and incur additional costs associated with being a public company.

## Financial Operations Overview

### Revenues

To date, we have not generated any revenue. We do not expect to receive any revenue from any drug candidates that we develop unless and until we obtain regulatory approval for, and commercialize, our drug candidates or enter into collaborative agreements with third parties.

### Research and Development Expenses

We had limited operating expenses related to research and development activities prior to May 2014. In May 2014, we acquired certain rights to a number of research and development programs from Ligand and charged \$21,687,576 to research and development expense during the year ended December 31, 2014 as a cost of acquiring these assets. During the year ended December 31, 2015, we charged \$6,966,842 to research and development expense as following the IPO, we began manufacturing certain drug supply and initiated efforts related to conducting Phase 2 clinical trials for VK5211 and VK2809 and in vivo studies for VK0214. We expect that our ongoing research and development expenses will consist of costs incurred for the development of our drug candidates, including, but not limited to:

- employee and consultant-related expenses, which will include salaries, benefits and stock-based compensation, and certain consultant fees and travel expenses;
- expenses incurred under agreements with investigative sites and contract research organizations, or CROs, which will conduct a substantial portion of our research and development activities on our behalf;
- payments to third-party manufacturers, which will produce our active pharmaceutical ingredients and finished products;
- license fees paid to third parties for use of their intellectual property; and
-

facilities, depreciation and other allocated expenses, which will include direct and allocated expenses for rent and maintenance of facilities and equipment, depreciation of leasehold improvements, equipment and laboratory and other supplies.

We expense all research and development costs as incurred.

The process of conducting the necessary clinical research to obtain regulatory approval is costly and time consuming and the successful development of our drug candidates is highly uncertain. Our future research and development expenses will depend on the clinical success of each of our drug candidates, as well as ongoing assessments of the commercial potential of such drug candidates. In addition, we cannot forecast with any degree of certainty which drug candidates may be subject to future collaborations, when such arrangements will be secured, if at all, and to what degree such arrangements would affect our development plans and capital requirements. We expect to incur increased research and development expenses in the future as we continue our Phase 2 clinical trial for VK5211, commence our Phase 2 clinical trial for VK2809 and seek to advance our additional programs.

#### General and Administrative Expenses

Prior to the consummation of the IPO, our general and administrative expenses consisted primarily of salaries and related benefits paid to our employees in executive, operational and finance functions, including stock-based compensation and fees paid to certain consultants to help commence and continue our operations. Following the consummation of the IPO, our general and administrative expenses have increased as we have hired additional employees, issued additional equity awards, which has resulted in increased stock-based compensation expense, moved to a larger office to accommodate the increase in headcount, implemented certain systems to increase efficiency, and incurred additional costs for insurance, legal and accounting related to operating as a public company. We expect that our general and administrative expenses will continue to increase in the future in order to support our expected increase in research and development activities, including increased salaries and other related costs, stock-based compensation and consulting fees for executive, finance, accounting and business development functions. We also expect general and administrative expenses to increase as a result of additional costs associated with being a public company, including expenses related to compliance with the rules and regulations of the SEC and The Nasdaq Stock Market LLC, additional insurance expenses, investor relations activities and other administration and professional services. Other significant costs are expected to include legal fees relating to patent and corporate matters, facility costs not otherwise included in research and development expenses, and fees for accounting and other consulting services.

#### Other Expense

Other expense, following the IPO, includes the change in fair value of the debt conversion feature liability contained in the Secured Convertible Promissory Note issued to Ligand on May 21, 2014, or the Ligand Note, and its related interest expense, as well as the non-cash amortization of debt discount cost associated with the Ligand Note, offset by interest income earned from our cash and short-term investments. Prior to the consummation of the IPO, other expense also included the change in fair value of the debt conversion feature liability contained in our outstanding convertible promissory notes issued from September 2012 through June 2013, or the Convertible Notes, and the interest expense and non-cash amortization of debt discount cost associated with the Convertible Notes, and the change in fair value at each reporting period of the accrued license fees payable to Ligand under the Master License Agreement with Ligand, as amended, or the Master License Agreement. Upon the consummation of the IPO, the Convertible Notes, and the accrued interest and related debt conversion feature liability under the Convertible Notes, and the accrued license fees payable to Ligand under the Master License Agreement were converted to equity and, therefore, changes in their fair values are no longer recorded as expense.

#### JOBS Act

We are an “emerging growth company” within the meaning of the rules under the Securities Act of 1933, as amended, or the Securities Act, and we utilize certain exemptions from various reporting requirements that are applicable to public companies that are not emerging growth companies. For example, as an emerging growth company, we are not required to provide an auditor’s attestation report on our internal control over financial reporting in this and future



annual reports on Form 10-K as otherwise required by Section 404(b) of the Sarbanes-Oxley Act of 2002, as amended. In addition, Section 107 of the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, provides that an emerging growth company can utilize the extended transition period provided in Section 7(a)(2)(B) of the Securities Act for complying with new or revised accounting standards. Thus, an emerging growth company can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected to “opt out” of the extended transition period for complying with new or revised accounting standards pursuant to Section 107(b) of the JOBS Act. As a result, we are complying with new or revised accounting standards on the relevant dates on which adoption of such standards is required for non-emerging growth companies.

## Critical Accounting Policies and Estimates

Our management's discussion and analysis of financial condition and results of operations is based on our financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported revenues and expenses during the reporting periods. On an ongoing basis, we evaluate our estimates and judgments related to the fair value of the debt conversion liability, preclinical, nonclinical and clinical development costs and drug manufacturing costs. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are more fully described in Note 1 to our financial statements included elsewhere in this

Annual Report on Form 10-K, we believe that the following accounting policies will be critical to understanding our historical and future performance, as these policies relate to the significant areas involving management's judgments and estimates in the preparation of our financial statements.

### Revenue Recognition

We have not recorded any revenues since our inception. However, in the future we may enter into collaborative research and licensing agreements, under which we could be eligible for payments made in the form of upfront license fees, research funding, cost reimbursement, contingent event-based payments and royalties.

Revenue from upfront, nonrefundable license fees is recognized over the period that any related services are to be provided by us. Amounts received for research funding are recognized as revenue as the research services that are the subject of such funding are performed. Revenue derived from reimbursement of research and development costs in transactions where we act as a principal are recorded as revenue for the gross amount of the reimbursement, and the costs associated with these reimbursements are reflected as a component of research and development expense in our statements of operations.

Financial Accounting Standards Board, or FASB, Accounting Standards Codification, or ASC, Topic 605-28, Revenue Recognition – Milestone Method, or ASC 605-28, established the milestone method as an acceptable method of revenue recognition for certain contingent event-based payments under research and development arrangements. Under the milestone method, a payment that is contingent upon the achievement of a substantive milestone is recognized in its entirety in the period in which the milestone is achieved. A milestone is an event (1) that can be achieved based in whole or in part on either our performance or on the occurrence of a specific outcome resulting from our performance, (2) for which there is substantive uncertainty at the date the arrangement is entered into that the event will be achieved, and (3) that would result in additional payments being due to us. The determination that a milestone is substantive is subject to management's judgment and is made at the inception of the arrangement. Milestones are considered substantive when the consideration earned from the achievement of the milestone (a) is commensurate with either our performance to achieve the milestone or the enhancement of value of the item delivered as a result of a specific outcome resulting from our performance to achieve the milestone, (b) relates solely to past performance, and (c) is reasonable relative to all deliverables and payment terms in the arrangement.

Other contingent event-based payments received for which payment is either contingent solely upon the passage of time or the results of a collaborative partner's performance are not considered milestones under ASC 605-28. In accordance with ASC Topic 605-25, Revenue Recognition – Multiple-Element Arrangements, or ASC 605-25, such

payments will be recognized as revenue when all of the following criteria are met: (1) persuasive evidence of an arrangement exists, (2) delivery has occurred or services have been rendered, (3) price is fixed or determinable, and (4) collectability is reasonably assured. Revenues recognized for royalty payments, if any, are based upon actual net sales of the licensed compounds, as provided by the collaboration arrangement, in the period the sales occur. Any amounts received prior to satisfying our revenue recognition criteria are recorded as deferred revenue on our balance sheets.

#### Research and Development

All costs of research and development are expensed in the period incurred. Research and development costs primarily consist of fees paid to CROs and clinical trial sites; employee and consultant related expenses, which include salaries, benefits and stock-based compensation for research and development personnel; external research and development expenses incurred pursuant to agreements with third-party manufacturing organizations; license fees paid to third parties for use of their intellectual property; facilities costs; travel costs; dues and subscriptions; depreciation; and materials used in preclinical studies, clinical trials and research and development.

We estimate our preclinical study and clinical trial expenses based on the services we received pursuant to contracts with research institutions and CROs that conduct and manage preclinical studies and clinical trials on our behalf. Clinical trial-related contracts vary significantly in length and may be for a fixed amount, based on milestones or deliverables, a variable amount based on actual costs incurred, capped at a certain limit, or for a combination of these elements. We accrue service fees based on work performed, which relies on estimates of total costs incurred based on milestones achieved, patient enrollment and other events. The majority of our service providers invoice us in arrears and, to the extent that amounts invoiced differ from our estimates of expenses incurred, we accrue for additional costs. The financial terms of these agreements vary from contract to contract and may result in uneven expenses and payment flows. Preclinical study and clinical trial expenses include:

- fees paid to CROs, laboratories and consultants in connection with preclinical studies;
- fees paid to CROs, clinical trial sites, investigators and consultants in connection with clinical trials; and
- fees paid to contract manufacturers and service providers in connection with the production, testing and packaging of active pharmaceutical ingredients and drug materials for preclinical studies and clinical trials.

Payments under some of these agreements depend on factors such as the milestones accomplished, including enrollment of certain numbers of patients, site initiation and the completion of clinical trial milestones. To date, we have not experienced any events requiring us to make material adjustments to our accruals for service fees. If we do not identify costs that we have begun to incur or if we underestimate or overestimate the level of services performed or the costs of these services, our actual expenses could differ from our estimates, which could materially affect our results of operations. Adjustments to our accruals are recorded as changes in estimates become evident. Furthermore, based on amounts invoiced to us by our service providers, we may also record payments made to those providers as prepaid expenses that will be recognized as expense in future periods as services are rendered.

Our historical research and development expenses have primarily related to obtaining certain licensed compounds and related intellectual property rights from Ligand. In May 2014, we acquired certain rights to a number of research and development programs from Ligand. In doing so, we updated our policy on research and development to include the purchase of rights to intangible assets. In accordance with ASC Topic 730, Research and Development, intangible assets that are acquired and have an alternative future use, as defined, should be capitalized and reported as an intangible asset; however, the cost of acquired intangible assets that do not have alternative future uses should be reported as research and development expense as incurred. We note that intangible assets acquired that are in the preclinical or clinical stages of development when acquired, and that are not approved by the U.S. Food and Drug Administration, or the FDA, are deemed to have not satisfied the definition of having an alternative future use, as defined. Accordingly, assets acquired in the preclinical and clinical stages of development should be expensed as incurred in our statement of operations.

#### Patent Costs

Costs related to filing and pursuing patent applications are expensed as incurred to general and administrative expense, as recoverability of such expenditures is uncertain.

#### Stock-Based Compensation

We generally use the straight-line or graded vesting method to allocate compensation cost to reporting periods over each optionee's requisite service period, which is generally the vesting period, and estimate the fair value of stock-based awards or restricted stock units to employees and directors using the Black-Scholes option-valuation model. For options with a graded vesting schedule, we use the graded vesting schedule to allocate compensation cost to reporting periods. The Black-Scholes model requires the input of subjective assumptions, including volatility, the expected term and the fair value of the underlying common stock on the date of grant, among other inputs. Stock options granted to non-employees are accounted for using the fair value approach. Stock options granted to

non-employees are subject to periodic revaluation over their vesting terms. For restricted stock and restricted stock unit awards, we generally use the straight-line or graded vesting method to allocate compensation cost to reporting periods over the holder's requisite service period, which is generally the vesting period, and use the fair value at grant date to value the awards.

Prior to the consummation of the IPO, we accounted for stock-based compensation by measuring and recognizing compensation expense for all stock-based payments made to employees and directors based on estimated award date fair values, which estimates were highly complex and subjective in nature. We used the straight-line or graded vesting method to allocate compensation cost to reporting periods over each restricted award's requisite service period, which was generally the vesting period, and estimated the fair value of restricted stock-based awards to employees and consultants using a Monte Carlo market approach simulation method and performed an allocation of value to common stock based on the estimated time to a liquidity event. In addition, we accounted for performance-based restricted stock awards to employees by determining the fair value of the restricted stock award at the date of issuance by using the Probability Weighted Expected Return Method, or PWERM, and then assessing at each balance sheet date the

probability of the performance criteria being met. If the probability of achieving the criteria was deemed less-than-probable, then no expense was recorded. At the point where the criteria are deemed probable of being met, we will begin recording stock-based compensation with a cumulative catch-up expense in the period first recognized and then on a straight-line basis over the remaining period for which the performance criteria are expected to be completed.

#### Income Taxes

We account for our income taxes using the liability method whereby deferred tax assets and liabilities are determined based on temporary differences between the basis used for financial reporting and income tax reporting purposes. Deferred income taxes are provided based on the enacted tax rates in effect at the time such temporary differences are expected to reverse. A valuation allowance is provided for deferred tax assets if it is more likely than not that we will not realize those tax assets through future operations.

ASC Topic 740-10, Income Taxes, clarifies the accounting for uncertainty in income taxes recognized in our financial statements in accordance with generally accepted accounting principles. Income tax positions must meet a more-likely-than-not recognition threshold to be recognized. Income tax positions that previously failed to meet the more-likely-than-not threshold are recognized in the first subsequent financial reporting period in which that threshold is met. Previously recognized tax positions that no longer meet the more-likely-than-not threshold are derecognized in the first subsequent financial reporting period in which that threshold is no longer met.

Our policy is to recognize interest and penalties accrued on any unrecognized tax benefits as a component of income tax expense.

#### Net Loss per Common Share

Basic net loss per share is calculated by dividing the net loss attributable to common stockholders by the weighted-average number of common shares outstanding for the period, without consideration for common stock equivalents. Diluted net loss per share is computed by dividing the net loss attributable to common stockholders by the weighted-average number of common share equivalents outstanding for the period determined using the treasury-stock method. For purposes of this calculation, we currently do not have any deemed common share equivalents; therefore, our basic and diluted net loss per share calculations are the same.

#### Segments

We operate in only one segment. Management uses cash flows as the primary measure to manage our business and does not segment our business for internal reporting or decision making purposes.

#### Results of Operations

##### Comparison of the Years Ended December 31, 2015 and 2014

##### Research and Development Expenses

The following table summarizes our research and development expenses for the years ended December 31, 2015 and 2014.

|                                   | Year Ended December |              | \$             | %        |
|-----------------------------------|---------------------|--------------|----------------|----------|
|                                   | 31,                 | 31,          | Change         | Change   |
|                                   | 2015                | 2014         |                |          |
| Research and development expenses | \$6,966,842         | \$22,223,073 | \$(15,256,231) | (68.7 )% |

The decrease in research and development expenses during the year ended December 31, 2015 as compared to the year ended December 31, 2014 was primarily due to a decrease in license fees of \$21,687,576 in the year ended December 31, 2015. These fees were recorded during the year ended December 31, 2014 and related to the estimated license fee liability payable to Ligand under the Master License Agreement. This decrease was offset by increases during the year ended December 31, 2015 in research and development expenses of \$2,016,994 related to clinical manufacturing for our drug candidates, \$1,016,469 related to salaries and benefits, \$849,557 related to stock-based compensation expense, \$1,768,162 related to clinical trial activity for our VK5211 and VK2809 programs and \$565,356 related to services provided by certain third party consultants.

## General and Administrative Expenses

The following table summarizes our general and administrative expenses for the years ended December 31, 2015 and 2014.

|                                     | Year Ended December 31, |             | \$          | %       |
|-------------------------------------|-------------------------|-------------|-------------|---------|
|                                     | 2015                    | 2014        | Change      | Change  |
| General and administrative expenses | \$5,029,636             | \$1,244,910 | \$3,784,726 | 304.0 % |

The increase in general and administrative expenses during the year ended December 31, 2015 as compared to the year ended December 31, 2014 was primarily due to increases in stock-based compensation expense of \$1,745,633, salaries and related benefits of \$1,012,486, insurance premiums expense of \$336,658, board of director fees of \$154,807, legal and patent expenses of \$178,252, investor relations expenses of \$83,526 and consulting expenses of \$26,683.

## Other Income (Expense)

The following table summarizes our other income (expense) for the years ended December 31, 2015 and 2014.

|                        | Year Ended December 31, |             | \$             | %        |
|------------------------|-------------------------|-------------|----------------|----------|
|                        | 2015                    | 2014        | Change         | Change   |
| Other income (expense) | \$(11,407,510)          | \$1,583,800 | \$(12,991,310) | (820.3)% |

Other income (expense) increased during the year ended December 31, 2015 primarily due to the establishment in May 2014 of a license fee liability in accordance with the Master License Agreement, which requires the license fee liability to be marked to market at each reporting period. The increase in other income (expense) for the year ended December 31, 2015 as compared to the year ended December 31, 2014 is primarily due to the recording of an increase in the value of the license fee liability of \$9,381,848 during the year ended December 31, 2015, as compared to a decrease in the value of the license fee liability of \$1,821,713 during the year ended December 31, 2014. In addition, during the year ended December 31, 2015, we recorded additional amounts to other expense related to the Ligand Note, including an incremental expense of \$354,993 related to the amortization of the Ligand Note discount and an expense of \$1,358,284 related to the incremental change in fair value of the Ligand Note's conversion feature.

## Liquidity and Capital Resources

We have incurred losses and negative cash flows from operations and have not generated any revenues since our inception. The audit report issued by our independent registered public accounting firm for our financial statements for the fiscal year ended December 31, 2015 included in this Annual Report on Form 10-K states that our independent registered public accounting firm has substantial doubt in our ability to continue as a going concern due to the risk that



we may not have sufficient cash and liquid assets at December 31, 2015 to cover our operating and capital requirements for the next 12 months. In the event we cannot obtain sufficient funding, we may have to substantially alter, or possibly even discontinue, operations. Our financial statements and related notes thereto included elsewhere in this Annual Report on Form 10-K do not include any adjustments that might result from the outcome of this uncertainty.

Our primary use of cash is to fund operating expenses, which to date have consisted of the cost to obtain the license of intellectual property from Ligand, certain research and development expenses related to furthering the development of VK5211, VK2089 and VK0214 efforts and general and administrative expenses. Since we have not generated any revenues to date, we have incurred operating losses since our inception. Cash used to fund operating expenses is impacted by the timing of payment of these expenses, as reflected in the change in our outstanding accounts payable and accrued expenses.

On May 4, 2015, prior to the completion of the IPO, we repurchased an aggregate of 3,802,859 shares of our common stock from our stockholders at a price of \$0.00001 per share for an aggregate purchase price of \$38. Pursuant to the Master License Agreement, upon the closing of the IPO, on May 4, 2015, we issued an aggregate of 3,427,859 shares of our common stock to Ligand and Metabasis Therapeutics, Inc., a wholly-owned subsidiary of Ligand, or Metabasis.

On May 4, 2015, we consummated the IPO, pursuant to which we sold 3,000,000 shares of our common stock at an initial public offering price of \$8.00 per share. The underwriters for the IPO had 30 days to exercise an over-allotment option to purchase up to an additional 450,000 shares at the initial public offering price, less the underwriting discount. Upon the closing of the IPO, on May 4, 2015, we raised a total of \$19,100,500 in net proceeds after deducting underwriting discounts, commissions and other offering expenses of \$4,899,500. Costs directly associated with the IPO were capitalized and recorded as deferred IPO costs prior to the closing of the IPO. These costs have been recorded as a reduction of the proceeds received in arriving at the amount to be recorded in additional paid-in capital.

On May 26, 2015, the underwriters of the IPO exercised their full over-allotment option to purchase an additional 450,000 shares of our common stock at the initial public offering price of \$8.00 per share, less the underwriting discount. On May 28, 2015, we sold the 450,000 shares to the underwriters pursuant to the over-allotment option and received additional net proceeds of \$3,225,866 after deducting underwriting discounts, commissions and other offering expenses of \$374,134. In connection with the underwriters' exercise of the over-allotment option, on May 28, 2015, we issued an aggregate of 228,105 additional shares of our common stock to Ligand and Metabasis in accordance with the terms of the Master License Agreement.

The following table summarizes our cash flows for the periods indicated below:

|                                       | Year Ended December 31, |               |
|---------------------------------------|-------------------------|---------------|
|                                       | 2015                    | 2014          |
| Cash used in operating activities     | \$(8,731,494 )          | \$(1,591,423) |
| Cash used in investing activities     | \$(13,470,042)          | \$—           |
| Cash provided by financing activities | \$22,214,229            | \$2,167,661   |

#### Cash Used in Operating Activities

During the year ended December 31, 2015, cash used in operating activities of \$8,731,494 primarily reflected our net losses for the period, offset by non-cash charges such as an increase in the fair value of accrued license fees and debt conversion feature liability, amortization of discount charged to interest expense on the Convertible Notes and the Ligand Note, stock-based compensation and changes in our working capital accounts, primarily consisting of an increase in prepaid expenses, accounts payable and accrued expenses.

During the year ended December 31, 2014, cash used in operating activities was \$1,591,423. Cash used in operating activities primarily reflected our net losses for the period, offset by non-cash charges such as stock-based compensation, amortization of discount charged to interest expense on the Convertible Notes and changes in fair value of debt conversion feature liability and accrued license fees, as well as changes in our working capital accounts, primarily consisting of an increase in accounts payable, accrued expenses, accrued license fees, debt conversion feature liability and deferred initial public offering financing costs.

#### Cash Used in Investing Activities

During the year ended December 31, 2015, cash used in investing activities of \$13,470,042 resulted from the investment of the proceeds from the IPO into short term investments that are available for sale. We did not engage in any investing activities during the year ended December 31, 2014.

#### Cash Provided by Financing Activities

During the year ended December 31, 2015, cash provided by financing activities of \$22,214,229 was primarily due to the net proceeds from the IPO of \$25,392,500 (after deducting underwriting discounts and commissions) offset by payment of certain other offering expenses of \$2,782,462.

During the year ended December 31, 2014, cash provided by financing activities was \$2,167,661, which consisted primarily of proceeds from the issuance of the Ligand Note in the amount of \$2,500,000, offset by the payment of certain deferred offering costs of \$332,337 and the repurchase of shares of restricted common stock from a former

service provider at par value.

#### Future Funding Requirements

As of December 31, 2015, and based upon our current operating plan, we do not believe that we will have sufficient cash to meet our projected operating requirements for at least the next 12 months unless we raise additional capital. We anticipate that we will continue to generate losses for the foreseeable future, and we expect the losses to increase materially as we continue the development of, and seek regulatory approvals for, our drug candidates, and seek to commercialize any drugs for which we receive regulatory approval. We will need to raise additional capital to fund our operations and complete our ongoing and planned clinical trials. Although we expect to finance future cash needs through public or private equity or debt offerings, funding may not be available to us on acceptable terms, or at all. If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may be required to delay, limit, reduce or terminate our drug development or future commercialization efforts or grant rights to develop and market drug candidates that we would otherwise prefer to develop and market ourselves.

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Our future capital requirements will depend on many factors, including, but not limited to:

- the scope, rate of progress, results and costs of our clinical trials, preclinical studies and other related activities;
- the timing of, and the costs involved in, obtaining regulatory approvals for any of our current or future drug candidates;
- the number and characteristics of the drug candidates we seek to develop or commercialize;
- the cost of manufacturing clinical supplies, and establishing commercial supplies, of our drug candidates;
- the cost of commercialization activities if any of our current or future drug candidates are approved for sale, including marketing, sales and distribution costs;
- the expenses needed to attract and retain skilled personnel;
- the costs associated with being a public company;
- our ability to establish and maintain strategic collaborations, licensing or other arrangements and the financial terms of such agreements;
- the amount of revenue, if any, received from commercial sales of our drug candidates, should any of our drug candidates receive marketing approval; and
- the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing possible patent claims, including litigation costs and the outcome of any such litigation.

#### Contractual Obligations and Commitments

We are a smaller reporting company, as defined by Rule 12b-2 of the Securities Exchange Act of 1934, as amended, and are not required to provide the information required under this item.

#### Off-Balance Sheet Arrangements

We do not have any off-balance sheet arrangements as defined in Item 303(a)(4)(ii) of Regulation S-K promulgated by the SEC.

#### Recent Accounting Pronouncements

In August 2014, the FASB issued Accounting Standards Update No. 2014-15, Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern, or ASU 2014-15. ASU 2014-15 provides guidance on presentation of management's plans, when conditions or events, considered in the aggregate, raise substantial doubt about an entity's ability to continue as a going concern within one year after the date that the financial statements are issued. This guidance is intended to mitigate those conditions or events that will alleviate substantial doubt about the entity's ability to continue as a going concern. ASU 2014-15 is effective for fiscal years ending after December 15, 2016. The adoption of ASU 2014-15 is not expected to have a material impact on our financial statements.

In May 2014, the FASB issued Accounting Standards Update No. 2014-9, Revenue from Contracts with Customers, or ASU 2014-9, which provides guidance for revenue recognition. The core principle of ASU 2014-9 is that a company will recognize revenue when it transfers promised goods or services to customers in an amount that reflects the consideration to which the company expects to be entitled in exchange for those goods or services. The new guidance initially was effective for us beginning January 1, 2017, but on July 9, 2015 the FASB deferred the effective date and, as a result, the new guidance is effective for us beginning January 1, 2018. Companies may adopt the new revenue standard as of the original effective date. We are currently evaluating the method of adoption and the potential impact this standard will have on our financial statements.

In November 2015, the FASB issued Accounting Standards Update No. 2015-17, Income Taxes (Topic 740): Balance Sheet Classification of Deferred Taxes, or ASU 2015-17. To simplify the presentation of deferred income taxes, the amendments in ASU 2015-17 require that deferred income tax liabilities and assets be classified as noncurrent in a classified statement of financial position. The amendment is effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2016. The adoption of ASU 2015-17 is not expected to have a material impact on our financial statements.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk.

We are a smaller reporting company, as defined by Rule 12b-2 of the Securities Exchange Act of 1934, as amended, and are not required to provide the information required under this item.

Item 8. Financial Statements and Supplementary Data.

The information required by this Item 8 is contained on the pages indicated in Part IV, Item 15(a)(1) of this Annual Report on Form 10-K.

Item 9. Changes in and Disagreements With Accountants on Accounting and Financial Disclosure.

Our board of directors appointed Marcum LLP as our independent registered public accounting firm effective April 7, 2014. There were no disagreements or reportable events related to the change in accountants requiring disclosure under Item 304(b) of Regulation S-K.

Item 9A. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures.

We maintain disclosure controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act. Disclosure controls and procedures are controls and other procedures designed to ensure that the information required to be disclosed by us in the reports that we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by us in the reports that we file or submit under the Exchange Act is accumulated and communicated to our management, including our principal executive officer and our principal financial officer, as appropriate, to allow timely decisions regarding required disclosure.

In designing and evaluating the disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives, and management is required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures. As required by Rule 13a-15(b) or Rule 15d-15(b) promulgated by the SEC under the Exchange Act, we carried out an evaluation, under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, of the effectiveness of the design and operation of our disclosure controls and procedures as of the end of the period covered by this Annual Report on Form 10-K. Based on the foregoing, our principal executive officer and principal financial officer concluded that our disclosure controls and procedures were effective as of the end of the period covered by this Annual Report on Form 10-K at the reasonable assurance level.

Management's Report on Internal Control over Financial Reporting.

This Annual Report on Form 10-K does not include a report of management's assessment regarding internal control over financial reporting or an attestation report of our independent registered public accounting firm due to a transition period established by rules of the SEC for newly public companies.

Changes in Internal Control over Financial Reporting.

There were no changes in our internal control over financial reporting during the quarter ended December 31, 2015 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information.

None.

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PART III

Item 10. Directors, Executive Officers and Corporate Governance.

The information required by this item will be set forth in the sections entitled “Board of Directors”, “Executive Officers”, “Section 16(a) Beneficial Ownership Reporting Compliance” and “Corporate Governance and Board Matters” in our Definitive Proxy Statement for our 2016 Annual Meeting of Stockholders, or our 2016 Proxy Statement, to be filed with the SEC within 120 days after the end of the fiscal year ended December 31, 2015, and is incorporated herein by reference.

Item 11. Executive Compensation.

The information required by this item will be set forth in the section entitled “Executive Compensation” in our 2016 Proxy Statement and is incorporated herein by reference.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters.

The information required by this item will be set forth in the sections entitled “Securities Authorized for Issuance Under Equity Compensation Plans” and “Security Ownership of Certain Beneficial Owners and Management” in our 2016 Proxy Statement and is incorporated herein by reference.

Item 13. Certain Relationships and Related Transactions, and Director Independence.

The information required by this item will be set forth in the sections entitled “Transactions with Related Persons, Promoters and Certain Control Persons” and “Corporate Governance and Board Matters” in our 2016 Proxy Statement and is incorporated herein by reference.



Item 14. Principal Accounting Fees and Services.

The information required by this item will be set forth in the section entitled “Audit Related Matters” in our 2016 Proxy Statement and is incorporated herein by reference.

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## PART IV

## Item 15. Exhibits, Financial Statement Schedules.

(a)(1) The Financial Statements required to be filed by Items 8 and 15(c) of this Annual Report on Form 10-K, and filed herewith, are as follows:

|   | Page Number in<br>this Annual Report on Form 10-K |
|---|---|
| Report of Independent Registered Public Accounting Firm | F-2   |
| Balance Sheets  | F-3   |
| Statement of Operations                                 | F-4   |
| Statements of Stockholders' Equity (Deficit)            | F-5   |
| Statement of Cash Flows                                 | F-6   |
| Notes to Financial Statements                           | F-7   |

(a)(2) Financial Statement Schedules have been omitted because they are either not applicable or the required information is included in the consolidated financial statements or notes thereto listed in (a)(1) above.

(a)(3) Exhibits.

The following exhibits are filed herewith or incorporated herein by reference:

| Exhibit<br>Number | Description  | Registrant's<br>Form | Date Filed<br>with the<br>SEC | Exhibit<br>Number |
|-------------------|--|----------------------|-------------------------------|-------------------|
| 3.1               | Amended and Restated Certificate of Incorporation.   | S-1                  | 7/1/2014                      | 3.3               |
| 3.2               | Amendment to Certificate of Incorporation.   | S-1/A                | 9/2/2014                      | 3.5               |
| 3.3               | Amended and Restated Bylaws.   | S-1                  | 7/1/2014                      | 3.4               |
| 4.1               | Form of Common Stock Certificate.  | S-1                  | 7/1/2014                      | 4.1               |
| 4.2               | Form of Common Stock Purchase Warrant issued by Viking Therapeutics, Inc. to Laidlaw & Company (UK) Ltd. | S-1/A                | 4/10/2015                     | 4.2               |
| 10.1#             |  | S-1                  | 7/1/2014                      | 10.1              |

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Form of Indemnification Agreement between Viking Therapeutics, Inc. and its directors and executive officers.

|       |  |       |            |       |
|-------|--|-------|------------|-------|
| 10.2# | 2014 Equity Incentive Plan.  | S-1/A | 3/2/2015   | 10.2  |
| 10.3# | Form of Stock Option Award Agreement (2014 Equity Incentive Plan).   | S-1   | 7/1/2014   | 10.3  |
| 10.4# | Form of Restricted Stock Unit Award Agreement (2014 Equity Incentive Plan).  | S-1   | 7/1/2014   | 10.4  |
| 10.5# | Form of Restricted Stock Award Agreement (2014 Equity Incentive Plan).   | S-1/A | 9/2/2014   | 10.23 |
| 10.6# | Form of Stock Appreciation Rights Award Agreement (2014 Equity Incentive Plan).                                    | S-1   | 7/1/2014   | 10.5  |
| 10.7# | 2014 Employee Stock Purchase Plan.   | S-1/A | 3/2/2015   | 10.22 |
| 10.8# | Amendment No. 1 to 2014 Employee Stock Purchase Plan.  | S-1   | 11/24/2015 | 10.8  |
| 10.9# | Employment Agreement, effective as of June 2, 2014, by and between Viking Therapeutics, Inc. and Brian Lian, Ph.D. | S-1/A | 9/2/2014   | 10.6  |

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| Exhibit Number | Description  | Registrant's with the Form | Date Filed SEC | Exhibit Number |
|----------------|--|----------------------------|----------------|----------------|
| 10.10#         | Employment Agreement, effective as of May 21, 2014, by and between Viking Therapeutics, Inc. and Michael Morneau.  | S-1/A                      | 9/2/2014       | 10.7           |
| 10.11#         | Amendment to Employment Agreement, effective as of September 30, 2014, by and between Viking Therapeutics, Inc. and Michael Morneau.   | S-1/A                      | 3/2/2015       | 10.26          |
| 10.12#         | Employment Agreement, effective as of June 2, 2014, by and between Viking Therapeutics, Inc. and Michael Dinerman, M.D.  | S-1/A                      | 9/2/2014       | 10.8           |
| 10.13#         | Amendment to Employment Agreement, effective as of September 30, 2014, by and between Viking Therapeutics, Inc. and Michael Dinerman, M.D.   | S-1/A                      | 3/2/2015       | 10.27          |
| 10.14#         | Employment Agreement, effective as of June 2, 2014, by and between Viking Therapeutics, Inc. and Rochelle Hanley, M.D.   | S-1/A                      | 9/2/2014       | 10.9           |
| 10.15#         | Amendment to Employment Agreement, effective as of September 30, 2014, by and between Viking Therapeutics, Inc. and Rochelle Hanley, M.D.  | S-1/A                      | 3/2/2015       | 10.28          |
| 10.16#         | Non-Employee Director Compensation Policy.   | S-1                        | 11/24/2015     | 10.16          |
| 10.17†         | Master License Agreement, dated May 21, 2014, by and among Viking Therapeutics, Inc., Ligand Pharmaceuticals Incorporated and Metabasis Therapeutics, Inc.                         | S-1                        | 7/1/2014       | 10.12          |
| 10.18†         | First Amendment to Master License Agreement, dated September 6, 2014, by and among Viking Therapeutics, Inc., Ligand Pharmaceuticals Incorporated and Metabasis Therapeutics, Inc. | S-1/A                      | 9/8/2014       | 10.24          |
| 10.19†         | Second Amendment to Master License Agreement, dated April 8, 2015, by and among Viking Therapeutics, Inc., Ligand Pharmaceuticals Incorporated and Metabasis Therapeutics, Inc.    | S-1/A                      | 4/10/2015      | 10.30          |
| 10.20†         | Loan and Security Agreement, dated May 21, 2014, by and between Viking Therapeutics, Inc. and Ligand Pharmaceuticals Incorporated.   | S-1                        | 7/1/2014       | 10.13          |
| 10.21†         | First Amendment to Loan and Security Agreement, dated April 8, 2015, by and between Viking Therapeutics, Inc. and Ligand Pharmaceuticals Incorporated.                             | S-1/A                      | 4/10/2015      | 10.31          |
| 10.22          | Convertible Note, dated May 27, 2014, issued by Viking Therapeutics, Inc. to Ligand Pharmaceuticals Incorporated.  | S-1                        | 7/1/2014       | 10.14          |
| 10.23          |  | S-1                        | 7/1/2014       | 10.15          |

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Letter Agreement regarding board composition and management rights, dated May 21, 2014, by and between Viking Therapeutics, Inc. and Ligand Pharmaceuticals Incorporated.

|        |   |     |          |       |
|--------|---|-----|----------|-------|
| 10.24  | Registration Rights Agreement, dated May 21, 2014, by and among Viking Therapeutics, Inc., Metabasis Therapeutics, Inc. and Ligand Pharmaceuticals Incorporated.                              | S-1 | 7/1/2014 | 10.16 |
| 10.25  | Voting Agreement, dated May 21, 2014, by and among Viking Therapeutics, Inc., Ligand Pharmaceuticals Incorporated, Metabasis Therapeutics, Inc., Brian Lian, Ph.D. and Michael Dinerman, M.D. | S-1 | 7/1/2014 | 10.17 |
| 10.26# | Founder Common Stock Purchase Agreement, dated September 26, 2012, by and between Viking Therapeutics, Inc. and Brian Lian, Ph.D.   | S-1 | 7/1/2014 | 10.18 |

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| Exhibit<br>Number | Description   | Date Filed                    |           |                   |
|-------------------|---|-------------------------------|-----------|-------------------|
|                   |   | Registrant's with the<br>Form | SEC       | Exhibit<br>Number |
| 10.27#            | Amendment No. 1 to Founder Common Stock Purchase Agreement, dated May 4, 2015, by and between Viking Therapeutics, Inc. and Brian Lian, Ph.D.   | 10-Q                          | 6/12/2015 | 10.2              |
| 10.28#            | Founder Common Stock Purchase Agreement, dated September 26, 2012, by and between Viking Therapeutics, Inc. and Michael Dinerman, M.D.  | S-1                           | 7/1/2014  | 10.19             |
| 10.29#            | Amendment No. 1 to Founder Common Stock Purchase Agreement, dated May 4, 2015, by and between Viking Therapeutics, Inc. and Michael Dinerman, M.D.  | 10-Q                          | 6/12/2015 | 10.3              |
| 10.30#            | Common Stock Purchase Agreement, dated April 15, 2013, by and between Viking Therapeutics, Inc. and Rochelle Hanley, M.D.   | S-1                           | 7/1/2014  | 10.20             |
| 10.31#            | Amendment No. 1 to Common Stock Purchase Agreement, dated May 4, 2015, by and between Viking Therapeutics, Inc. and Rochelle Hanley, M.D.   | 10-Q                          | 6/12/2015 | 10.4              |
| 10.32#†           | Common Stock Purchase Agreement, dated February 20, 2014, by and between Viking Therapeutics, Inc. and Brian Lian, Ph.D.  | S-1                           | 7/1/2014  | 10.21             |
| 10.33#            | Amendment No. 1 to Common Stock Purchase Agreement, dated May 4, 2015, by and between Viking Therapeutics, Inc. and Brian Lian, Ph.D.   | 10-Q                          | 6/12/2015 | 10.5              |
| 10.34#            | Amended and Restated Stock Repurchase Agreement, dated April 28, 2015, by and among Viking Therapeutics, Inc., Brian Lian, Ph.D., Michael Dinerman, M.D., Isabelle Dinerman and Rochelle Hanley, M.D. | 10-Q                          | 6/12/2015 | 10.1              |
| 10.35             | Research Services Agreement, dated January 27, 2015, by and between Viking Therapeutics, Inc. and Academisch Medisch Centrum.   | S-1/A                         | 4/10/2015 | 10.29             |
| 10.36             | Sublease between Fish & Richardson P.C. and Viking Therapeutics, Inc. dated July 7, 2015.   | 10-Q                          | 11/5/2015 | 10.1              |
| 10.37             | Second Amendment to Loan and Security Agreement, dated January 22, 2016, by and between Viking Therapeutics, Inc. and Ligand Pharmaceuticals Incorporated.  | 8-K                           | 1/25/2016 | 10.1              |
| 10.38             | First Amendment to Registration Rights Agreement, dated January 22, 2016, by and between Viking Therapeutics, Inc. and Ligand Pharmaceuticals Incorporated.   | 8-K                           | 1/25/2016 | 10.2              |
| 23.1              | Consent of Marcum LLP, Independent Registered Public Accounting Firm.   |                               |           |                   |

- 24.1 Power of Attorney (included on the signature page to this Annual Report on Form 10-K).
  - 31.1 Certification of the Principal Executive Officer pursuant to Rule 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934.
  - 31.2 Certification of the Principal Financial Officer pursuant to Rule 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934.
  - 32.1 Certification of the Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
- 101.INS XBRL Instance Document.
- 101.SCH XBRL Taxonomy Extension Schema Document.
- 101.CAL XBRL Taxonomy Extension Calculation Linkbase Document.

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| Exhibit |   | Date Filed            |     |         |
|---------|---|-----------------------|-----|---------|
| Number  | Description   | Registrant's with the | SEC | Exhibit |
|         |   | Form                  |     | Number  |
| 101.DEF | XBRL Taxonomy Extension Definition Linkbase Document.   |                       |     |         |
| 101.LAB | XBRL Taxonomy Extension Label Linkbase Document.        |                       |     |         |
| 101.PRE | XBRL Taxonomy Extension Presentation Linkbase Document. |                       |     |         |

Attached as Exhibit 101 to this report are the following formatted in XBRL (Extensible Business Reporting Language): (i) Balance Sheets as of December 31, 2015 and December 31, 2014, (ii) Statements of Operations for the years ended December 31, 2015 and 2014, (iii) Statements of Stockholders' Equity (Deficit) for the period from December 31, 2013 to December 31, 2015, (iv) Statements of Cash Flows for the years ended December 31, 2015 and 2014, and (v) Notes to Financial Statements.

#Indicates compensatory plan or arrangement.

€Confidential treatment has been granted with respect to certain portions of this exhibit, which portions have been omitted and filed separately with the Securities and Exchange Commission.

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SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

Viking Therapeutics, Inc.

Date: March 8, 2016 By: /s/ Brian Lian, Ph.D.  
 Brian Lian, Ph. D.  
 President and Chief Executive Officer

POWER OF ATTORNEY

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below hereby constitutes and appoints, jointly and severally, Brian Lian, Ph.D. and Michael Morneau, and each of them acting individually, as his attorney-in-fact, each with full power of substitution and resubstitution, for him in any and all capacities, to sign any and all amendments to this Annual Report on Form 10-K, and to file the same, with exhibits thereto and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact full power and authority to do and perform each and every act and thing requisite and necessary to be done in connection therewith as fully to all intents and purposes as he might or could do in person, hereby ratifying and confirming all that said attorneys-in-fact, or their substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the Registrant and in the capacities and on the dates indicated.

| Name   | Title  | Date          |
|--|--|---------------|
| /s/ Brian Lian, Ph.D.<br>Brian Lian, Ph.D.                       | President, Chief Executive Officer and Director<br><br>(Principal Executive Officer) | March 8, 2016 |
| /s/ Michael Morneau<br>Michael Morneau                           | Chief Financial Officer<br><br>(Principal Accounting and Financial Officer)          | March 8, 2016 |
| /s/ Lawson Macartney, DVM, Ph.D.<br>Lawson Macartney, DVM, Ph.D. | Director   | March 8, 2016 |
| /s/ Matthew W. Foehr<br>Matthew W. Foehr                         | Director   | March 8, 2016 |

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/s/ Matthew Singleton  
Matthew Singleton

Director

March 8, 2016

/s/ Stephen W. Webster  
Stephen W. Webster

Director

March 8, 2016

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|--|-----|
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| <u>Statements of Operations for the Years ended December 31, 2015 and 2014</u>                     | F-4 |
| <u>Statements of Stockholders' Equity (Deficit) for the Years ended December 31, 2015 and 2014</u> | F-5 |
| <u>Statements of Cash Flows for the Years ended December 31, 2015 and 2014</u>                     | F-6 |
| <u>Notes to Consolidated Financial Statements</u>  | F-7 |

Report of Independent Registered Public Accounting Firm

Board of Directors and Stockholders

of Viking Therapeutics, Inc.

We have audited the accompanying balance sheets of Viking Therapeutics, Inc. (the “Company”) as of December 31, 2015 and 2014, and the related statements of operations, stockholders’ equity (deficit) and cash flows for the years then ended. 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 in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audits to obtain reasonable assurance about whether the financial statements are free of material misstatement. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. Our audits included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Company’s internal control over financial reporting. Accordingly, we express no such opinion. An audit also 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, as well as evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of Viking Therapeutics, Inc., as of December 31, 2015 and 2014, and the results of its operations and its cash flows for the years then ended in conformity with accounting principles generally accepted in the United States of America.

The accompanying financial statements have been prepared assuming the Company will continue as a going concern. As discussed in Note 1 to the financial statements, the Company has had recurring net losses and negative cash flows from operations that raise substantial doubt about the Company’s ability to continue as a going concern. Management’s plans regarding these matters also are described in Note 1. The financial statements do not include any adjustments that might result from the outcome of this uncertainty.

/s/ Marcum LLP

Irvine, California

March 8, 2016

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## Viking Therapeutics, Inc.

## Balance Sheets

|   | December 31,  | December 31, |
|---|---------------|--------------|
|   | 2015          | 2014         |
| <b>Assets</b>   |               |              |
| <b>Current assets:</b>  |               |              |
| Cash and cash equivalents   | \$ 768,550    | \$ 755,857   |
| Short-term investments – available for sale   | 13,335,499    | —            |
| Prepaid expenses and other current assets   | 1,097,599     | 17,827       |
| Total current assets  | 15,201,648    | 773,684      |
| Deferred public offering financing costs  | 157,455       | 2,268,675    |
| Deposits  | 80,000        | 775          |
| Total assets  | \$ 15,439,103 | \$ 3,043,134 |
| <b>Liabilities, convertible notes and stockholders' equity (deficit)</b>  |               |              |
| <b>Current liabilities:</b>   |               |              |
| Accounts payable  | \$ 592,414    | \$ 1,830,724 |
| Accrued license fees  | —             | 19,865,863   |
| Other accrued liabilities   | 1,384,398     | 380,257      |
| Accrued interest  | —             | 77,222       |
| Convertible notes payable, current portion (net of discount of \$0 and \$6,076 at December 31, 2015 and 2014, respectively)   | —             | 304,274      |
| Debt conversion feature liability   | —             | 58,742       |
| Total current liabilities   | 1,976,812     | 22,517,082   |
| Accrued interest, non-current   | 183,611       | —            |
| Convertible notes payable (net of discount of \$348,460 and \$1,235,886 at December 31, 2015 and 2014, respectively)  | 2,151,540     | 1,264,114    |
| Debt conversion feature liability   | 2,370,903     | 1,390,469    |
| Deferred rent   | 31,239        | —            |
| Total long-term liabilities   | 4,737,293     | 2,654,583    |
| Total liabilities   | 6,714,105     | 25,171,665   |
| <b>Commitments and contingencies (Note 12)</b>  |               |              |
| <b>Stockholders' equity (deficit):</b>  |               |              |
| Preferred stock, \$0.00001 par value: 10,000,000 shares authorized at December 31, 2015 and no shares authorized at December 31, 2014; no shares issued and outstanding at December 31, 2015 and 2014 | —             | —            |
| Common stock, \$0.00001 par value: 300,000,000 shares authorized at December 31,  | 97            | 60           |

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2015 and 25,000,000 shares authorized at December 31, 2014; 9,683,741 shares

issued and outstanding at December 31, 2015 and 6,000,000 shares issued and

outstanding at December 31, 2014

|  |               |               |
|--|---------------|---------------|
| Additional paid-in capital                           | 54,277,716    | 12,866        |
| Accumulated deficit                                  | (45,545,445 ) | (22,141,457 ) |
| Accumulated other comprehensive loss                 | (7,370 )      | —             |
| Total stockholders' equity (deficit)                 | 8,724,998     | (22,128,531 ) |
| Total liabilities and stockholders' equity (deficit) | \$ 15,439,103 | \$ 3,043,134  |

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

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Viking Therapeutics, Inc.

## Statements of Operations and Comprehensive Loss

|   | Year Ended           |                |
|---|----------------------|----------------|
|   | December 31,<br>2015 | 2014           |
| Revenues  | \$—                  | \$—            |
| Operating expenses:   |                      |                |
| Research and development  | 6,966,842            | 22,223,073     |
| General and administrative  | 5,029,636            | 1,244,910      |
| Total operating expenses  | 11,996,478           | 23,467,983     |
| Loss from operations  | (11,996,478)         | (23,467,983)   |
| Other income (expense):   |                      |                |
| Change in fair value of accrued license fees                                    | (9,381,848 )         | 1,821,713      |
| Change in fair value of debt conversion feature liability                       | (1,043,478 )         | 390,763        |
| Amortization of debt discount   | (893,502 )           | (557,961 )     |
| Interest expense, net   | (88,682 )            | (70,715 )      |
| Total other income (expense)  | (11,407,510)         | 1,583,800      |
| Net loss  | (23,403,988)         | (21,884,183)   |
| Other comprehensive loss, net of tax:   |                      |                |
| Unrealized gain (loss) on securities  | (7,370 )             | —              |
| Comprehensive loss  | \$(23,411,358)       | \$(21,884,183) |
| Basic and diluted net loss per share  | \$(3.68 )            | \$(5.23 )      |
| Weighted-average shares used to compute basic<br>and diluted net loss per share | 6,355,869            | 4,187,415      |

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



Viking Therapeutics, Inc.

## Statements of Stockholders' Equity (Deficit)

|  | Common Stock<br>Shares | Stock<br>Amount | Additional<br>Paid-In<br>Capital | Notes<br>Receivable<br>from<br>Stockholders | Accumulated<br>Other<br>Comprehensive<br>Loss | Accumulated<br>Deficit | Total        |
|--|------------------------|-----------------|----------------------------------|---|---|------------------------|--------------|
| Balance at December 31, 2013   | 5,200,000              | \$ 52           | \$ 11,114                        | \$ (4,496 )                                 | \$ —  | \$(257,274 )           | \$(250,604 ) |
| Repurchase of common stock   | (200,000 )             | (2 )            | (1,998 )                         | 1,998                                       | —   | —                      | (2 )         |
| Issuance of performance-based common stock                           | 1,000,000              | 10              | (10 )                            | —   | —   | —                      | —            |
| Employee stock-based compensation                                    | —                      | —               | 3,760                            | —   | —   | —                      | 3,760        |
| Forgiveness of note receivable in consideration of services rendered | —                      | —               | —                                | 2,498                                       | —   | —                      | 2,498        |
| Net loss   | —                      | —               | —                                | —   | —   | (21,884,183)           | (21,884,183) |
| Balance at December 31, 2014   | 6,000,000              | 60              | 12,866                           | —   | —   | (22,141,457)           | (22,128,531) |
| Repurchase of common stock   | (3,802,859)            | (38 )           | —                                | —   | —   | —                      | (38 )        |
| Employee stock-based compensation                                    | 318,708                | 3               | 2,205,673                        | —   | —   | —                      | 2,205,676    |
| Issuance of common stock to consultant                               | 4,882                  | —               | 28,760                           | —   | —   | —                      | 28,760       |
| Conversion of debt to equity   | 57,046                 | 1               | 456,411                          | —   | —   | —                      | 456,412      |
| Issuances of common stock to related party                           | 3,655,964              | 36              | 29,247,675                       | —   | —   | —                      | 29,247,711   |
| Initial public offering, net of issuance costs                       | 3,450,000              | 35              | 22,326,331                       | —   | —   | —                      | 22,326,366   |
| Unrealized gain (loss) on investments                                | —                      | —               | —                                | —   | (7,370 )                                      | —                      | (7,370 )     |

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|                              |           |       |              |      |             |                |              |
|------------------------------|-----------|-------|--------------|------|-------------|----------------|--------------|
| Net loss                     | —         | —     | —            | —    | —           | (23,403,988)   | (23,403,988) |
| Balance at December 31, 2015 | 9,683,741 | \$ 97 | \$54,277,716 | \$ — | \$ (7,370 ) | \$(45,545,445) | \$8,724,998  |

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

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Viking Therapeutics, Inc.

## Statements of Cash Flows

|  | Year Ended           |                |
|--|----------------------|----------------|
|  | December 31,<br>2015 | 2014           |
| Cash flows from operating activities   |                      |                |
| Net loss   | \$(23,403,988)       | \$(21,884,183) |
| Adjustments to reconcile net loss to net cash used in operating activities             |                      |                |
| Amortization of debt discount on notes payable   | 893,502              | 557,961        |
| Amortization of investment premiums  | 131,899              | —              |
| Change in fair value of accrued license fees   | 9,381,848            | (1,821,713 )   |
| Change in fair value of debt conversion feature liability                              | 1,043,478            | (390,763 )     |
| Stock-based compensation   | 2,601,448            | 6,258          |
| Changes in operating assets and liabilities:   |                      |                |
| Prepaid expenses and other current assets  | (1,079,772 )         | (17,827 )      |
| Deposits   | (79,225 )            | —              |
| Accounts payable   | 219,237              | 191,008        |
| Accounts payable - related party   | —                    | (712 )         |
| Accrued license fees   | —                    | 21,687,576     |
| Accrued expenses   | 1,560,079            | 80,972         |
| Net cash used in operating activities  | (8,731,494 )         | (1,591,423 )   |
| Cash flows from investing activities   |                      |                |
| Purchases of investments   | (16,033,042)         | —              |
| Proceeds from sales and maturities of investments                                      | 2,563,000            | —              |
| Net cash used in investing activities  | (13,470,042)         | —              |
| Cash flows from financing activities   |                      |                |
| Proceeds from issuances of common stock, net of underwriting discounts and commissions | 25,392,500           | —              |
| Public offering costs  | (2,782,462 )         | (332,337 )     |
| Value of shares withheld related to employee tax withholding                           | (418,412 )           | —              |
| Repurchases of common stock  | (38 )                | (2 )           |
| Proceeds from convertible notes payable  | —                    | 2,500,000      |
| Proceeds from stock issuance under employee stock purchase plan                        | 22,641               | —              |
| Net cash provided by financing activities  | 22,214,229           | 2,167,661      |
| Net increase in cash and cash equivalents  | 12,693               | 576,238        |
| Cash and cash equivalents beginning of period  | 755,857              | 179,619        |
| Cash and cash equivalents end of period  | \$768,550            | \$755,857      |
| Supplemental disclosure of non-cash investing and financing                            |                      |                |

| transactions  |              |             |
|---|--------------|-------------|
| Shares issued in lieu of license fee payment              | \$29,247,711 | \$—         |
| Unpaid deferred public offering costs                     | \$108,791    | \$1,936,338 |
| Conversion of notes payable                               | \$456,412    | \$—         |
| Issuance of common stock to consultant                    | \$28,760     | \$—         |
| Issuance of common stock in exchange for notes receivable | \$—          | \$—         |
| Repurchases of common stock                               | \$—          | \$(1,998 )  |

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

Viking Therapeutics, Inc.

Notes to Financial Statements

## 1. Organization, Liquidity and Management's Plan, and Summary of Significant Accounting Policies The Company

Viking Therapeutics, Inc., a Delaware corporation (the "Company"), is a clinical-stage biopharmaceutical company focused on the development of novel, first-in-class or best-in-class therapies for metabolic and endocrine disorders.

The Company was incorporated under the laws of the State of Delaware on September 24, 2012 and its principal executive offices are located in San Diego, CA.

### Basis of Presentation

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

### Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the amounts reported in the accompanying financial statements. Significant estimates made in preparing these financial statements relate to determining the fair value of the debt conversion liability and accounting for certain commitments. Actual results could differ from those estimates.

### Cash and Cash Equivalents

The Company considers all highly liquid investments with maturities of three months or less from the date of purchase to be cash equivalents.

### Investments Available-for-Sale

Available-for-sale securities are carried at fair value, with the unrealized gains and losses reported in accumulated other comprehensive income (loss). The amortized cost of debt securities is adjusted for amortization of premiums and accretion of discounts to maturity. The amortization of premiums and accretion of discounts is included in interest income. Realized gains and losses and declines in value judged to be other-than-temporary, if any, on available-for-sale securities are included in other income (expense). The cost of securities sold is based on the specific identification method. Interest and dividends on securities classified as available-for-sale are included in interest income.

### Concentration of Credit Risk

Financial instruments, which potentially subject the Company to concentration of credit risk, consist primarily of cash and cash equivalents and marketable securities. The Company maintains deposits in federally insured depository institutions in excess of federally insured limits. Management believes that the Company is not exposed to significant

credit risk due to the financial position of the depository institutions in which those deposits are held. Additionally, the Company has established guidelines regarding approved investments and maturities of investments, which are designed to maintain safety and liquidity.

#### Liquidity and Management's Plan

In May 2015, the Company completed an initial public offering ("IPO"), issuing 3,450,000 shares of its common stock and raising net proceeds of \$22,326,366, including the full exercise of the over-allotment option, and after deducting underwriting discounts, commissions and other offering expenses of \$5,273,634. (See Note 7 regarding proceeds from the Company's initial public offering received in May 2015.)

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Although it is difficult to predict the Company's liquidity requirements, as of December 31, 2015, and based upon the Company's current operating plan, the Company does not believe that it will have sufficient cash to meet its projected operating requirements for at least the next 12 months unless it raises additional capital. As of December 31, 2015, the Company had an accumulated deficit of \$45,545,445. These losses have resulted principally from research and development costs incurred in connection with acquiring the exclusive worldwide rights to the portfolio of five drug candidates from Ligand Pharmaceuticals Incorporated ("Ligand") and the related non-cash interest expense recorded for increases in the deemed fair market value for the license fees payable to Ligand, research and development expenses related to the manufacturing of clinical drug product and clinical development of VK5211, VK2809 and VK0214, consulting fees and general and administrative expenses. The Company anticipates that it will continue to incur net losses for the foreseeable future as it continues the development of its clinical drug candidates and preclinical programs and incurs additional costs associated with being a public company.

#### Deferred Financing Costs

Deferred financing costs represent legal, accounting and other direct costs related to the Company's efforts to raise capital through a public sale of the Company's common stock. Costs related to the public sale of the Company's common stock will be deferred until its completion, at which time they will be reclassified to additional paid-in-capital as a reduction of the proceeds.

#### Revenue Recognition

The Company has not recorded any revenues since its inception. However, in the future the Company may enter into collaborative research and licensing agreements, under which the Company could be eligible for payments made in the form of upfront license fees, research funding, cost reimbursement, contingent event-based payments and royalties.

Revenue from upfront, nonrefundable license fees is recognized over the period that any related services are to be provided by the Company. Amounts received for research funding are recognized as revenue as the research services that are the subject of such funding are performed. Revenue derived from reimbursement of research and development costs in transactions where the Company acts as a principal are recorded as revenue for the gross amount of the reimbursement, and the costs associated with these reimbursements are reflected as a component of research and development expense in the statements of operations.

The Financial Accounting Standards Board ("FASB") Accounting Standards Codification ("ASC") Topic 605-28, Revenue Recognition – Milestone Method ("ASC 605-28"), established the milestone method as an acceptable method of revenue recognition for certain contingent event-based payments under research and development arrangements. Under the milestone method, a payment that is contingent upon the achievement of a substantive milestone is recognized in its entirety in the period in which the milestone is achieved. A milestone is an event (1) that can be achieved based in whole or in part on either the Company's performance or on the occurrence of a specific outcome resulting from the Company's performance, (2) for which there is substantive uncertainty at the date the arrangement is entered into that the event will be achieved, and (3) that would result in additional payments being due to the Company. The determination that a milestone is substantive is subject to management's judgment and is made at the inception of the arrangement. Milestones are considered substantive when the consideration earned from the achievement of the milestone is (a) commensurate with either the Company's performance to achieve the milestone or the enhancement of value of the item delivered as a result of a specific outcome resulting from the Company's performance to achieve the milestone, (b) relates solely to past performance, and (c) is reasonable relative to all deliverables and payment terms in the arrangement.

Other contingent event-based payments received for which payment is either contingent solely upon the passage of time or the results of a collaborative partner's performance are not considered milestones under ASC 605-28. In accordance with ASC Topic 605-25, Revenue Recognition – Multiple-Element Arrangements (“ASC 605-25”), such payments will be recognized as revenue when all of the following criteria are met: persuasive evidence of an arrangement exists, delivery has occurred or services have been rendered, price is fixed or determinable and collectability is reasonably assured. Revenues recognized for royalty payments, if any, are based upon actual net sales of the licensed compounds, as provided by the collaboration arrangement, in the period the sales occur. Any amounts received prior to satisfying the Company's revenue recognition criteria are recorded as deferred revenue on its balance sheets.

#### Research and Development Expenses

All costs of research and development are expensed in the period incurred. Research and development costs primarily consist of fees paid to clinical research organizations (“CROs”) and clinical trial sites, employee and consultant related expenses, which include salaries, benefits and stock-based compensation for research and development personnel, external research and development expenses incurred pursuant to agreements with third-party manufacturing organizations, facilities costs, travel costs, dues and subscriptions, depreciation and materials used in preclinical studies, clinical trials and research and development.



The Company estimates its preclinical study and clinical trial expenses based on the services it received pursuant to contracts with research institutions and CROs that conduct and manage preclinical studies and clinical trials on the Company's behalf. Clinical trial-related contracts vary significantly in length, and may be for a fixed amount, based on milestones or deliverables, a variable amount based on actual costs incurred, capped at a certain limit, or for a combination of these elements. The Company accrues service fees based on work performed, which relies on estimates of total costs incurred based on milestones achieved, patient enrollment and other events. The majority of the Company's service providers invoice the Company in arrears, and to the extent that amounts invoiced differ from its estimates of expenses incurred, the Company accrues for additional costs. The financial terms of these agreements vary from contract to contract and may result in uneven expenses and payment flows. Preclinical study and clinical trial expenses include:

- fees paid to CROs, consultants and laboratories in connection with preclinical studies;
- fees paid to CROs, clinical trial sites, investigators and consultants in connection with clinical trials; and
- fees paid to contract manufacturers and service providers in connection with the production, testing and packaging of active pharmaceutical ingredients and drug materials for preclinical studies and clinical trials.

Payments under some of these agreements depend on factors such as the milestones accomplished, including enrollment of certain numbers of patients, site initiation and the completion of clinical trial milestones. To date, the Company has not experienced any events requiring it to make material adjustments to its accruals for service fees. If the Company does not identify costs that it has begun to incur or if it underestimates or overestimates the level of services performed or the costs of these services, its actual expenses could differ from its estimates which could materially affect its results of operations. Adjustments to the Company's accruals are recorded as changes in estimates become evident. Furthermore, based on amounts invoiced to the Company by its service providers, the Company may also record payments made to those providers as prepaid expenses that will be recognized as expense in future periods as services are rendered.

The Company's historical research and development expenses primarily related to obtaining the option to license compounds and related intellectual property rights from Ligand. In May 2014, the Company entered into a master license agreement with Ligand, as amended (the "Master License Agreement"), pursuant to which it acquired certain rights to a number of research and development programs from Ligand. In doing so, the Company updated its policy on research and development to include the purchase of rights to intangible assets. In accordance with ASC Topic 730, Research and Development, intangible assets that are acquired and have an alternative future use, as defined, should be capitalized and reported as an intangible asset; however, the cost of acquired intangible assets that do not have alternative future uses should be reported as research and development expense as incurred. The Company notes that intangible assets acquired that are in the preclinical or clinical stages of development when acquired, and not approved by the U.S. Food and Drug Administration, are deemed to have not satisfied the definition of having an alternative future use, as defined. Accordingly, assets acquired in the preclinical and clinical stages of development were expensed as incurred in the Company's statement of operations.

#### Patent Costs

Costs related to filing and pursuing patent applications are expensed as incurred to general and administrative expense, as recoverability of such expenditures is uncertain.

#### Stock-Based Compensation

The Company generally uses the straight-line or graded vesting method to allocate compensation cost to reporting periods over each optionee's requisite service period, which is generally the vesting period, and estimates the fair value of stock-based awards or restricted stock units to employees and directors using the Black-Scholes option-valuation model. For options with a graded vesting schedule, the Company uses the graded vesting schedule to allocate

compensation cost to reporting periods. The Black-Scholes model requires the input of subjective assumptions, including volatility, the expected term and the fair value of the underlying common stock on the date of grant, among other inputs. Stock options granted to non-employees are accounted for using the fair value approach. Stock options granted to non-employees are subject to periodic revaluation over their vesting terms. For restricted stock and restricted stock unit awards, the Company generally uses the straight-line or graded vesting method to allocate compensation cost to reporting periods over the holder's requisite service period, which is generally the vesting period, and uses the fair value at grant date to value the awards. For restricted stock that vests upon the satisfaction of certain performance conditions, the Company recognizes stock-based compensation expense when it becomes probable that the performance conditions will be met. At the point that it becomes probable that the performance conditions will be met, the Company will record a cumulative catchup of the expense from the grant date to the current date, and the Company will then amortize the remainder of the expense over the remaining service period.

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Prior to the IPO, the Company accounted for stock-based compensation by measuring and recognizing compensation expense for all stock-based payments made to employees and directors based on estimated award date fair values, which estimates were highly complex and subjective in nature. The Company used the straight-line or graded vesting method to allocate compensation cost to reporting periods over each restricted award's requisite service period, which was generally the vesting period, and estimated the fair value of restricted stock-based awards to employees and consultants using a Monte Carlo market approach simulation method and performed an allocation of value to common stock based on the estimated time to a liquidity event. In addition, the Company accounted for performance-based restricted stock awards to employees by determining the fair value of the restricted stock award at the date of issuance by using the Probability Weighted Expected Return Method ("PWERM") and then assessing at each balance sheet date the probability of the performance criteria being met. If the probability of achieving the criteria was deemed less-than-probable, then no expense was recorded. At the point where the criteria were deemed probable of being met, then the Company began recording stock-based compensation with a cumulative catch-up expense in the period first recognized and then on a straight-line basis over the remaining period for which the performance criteria were expected to be completed.

#### Income Taxes

The Company accounts for its income taxes using the liability method whereby deferred tax assets and liabilities are determined based on temporary differences between the basis used for financial reporting and income tax reporting purposes. Deferred income taxes are provided based on the enacted tax rates in effect at the time such temporary differences are expected to reverse. A valuation allowance is provided for deferred tax assets if it is more likely than not that the Company will not realize those tax assets through future operations.

ASC Topic 740-10, Income Taxes, clarifies the accounting for uncertainty in income taxes recognized in the Company's financial statements in accordance with generally accepted accounting principles. Income tax positions must meet a more-likely-than-not recognition threshold to be recognized. Income tax positions that previously failed to meet the more-likely-than-not threshold are recognized in the first subsequent financial reporting period in which that threshold is met. Previously recognized tax positions that no longer meet the more-likely-than-not threshold are derecognized in the first subsequent financial reporting period in which that threshold is no longer met.

The Company's policy is to recognize interest and penalties accrued on any unrecognized tax benefits as a component of income tax expense.

#### Net Loss per Common Share

Basic net loss per share is calculated by dividing the net loss attributable to common stockholders by the weighted-average number of common shares outstanding for the period, without consideration for common stock equivalents. Diluted net loss per share is computed by dividing the net loss attributable to common stockholders by the weighted-average number of common share equivalents outstanding for the period determined using the treasury-stock method. For purposes of this calculation, the Company currently does not have any deemed common share equivalents; therefore, its basic and diluted net loss per share calculations are the same.

The following table presents the computation of basic and diluted net loss per common share:

|                               | Year Ended December 31, |      |
|-------------------------------|-------------------------|------|
|                               | 2015                    | 2014 |
| Historical net loss per share |                         |      |

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|  |                |                |
|--|----------------|----------------|
| Numerator  |                |                |
| Net loss attributable to common stockholders         | \$(23,403,988) | \$(21,884,183) |
| Denominator  |                |                |
| Weighted-average common shares outstanding           | 8,514,155      | 5,860,274      |
| Less: Weighted-average shares subject to repurchase  | (2,158,286 )   | (1,672,859 )   |
| Denominator for basic and diluted net loss per share | 6,355,869      | 4,187,415      |
| Basic and diluted net loss per share                 | \$(3.68 )      | \$(5.23 )      |

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Potentially dilutive securities that are not included in the calculation of diluted net loss per share because their effect is anti-dilutive are as follows (in common equivalent shares):

|   | Year Ended December |           |
|---|---------------------|-----------|
|   | 31,<br>2015         | 2014      |
| Common stock warrants                   | 82,500              | —         |
| Restricted stock units                  | 75,750              | —         |
| Common stock subject to repurchase      | 667,963             | 2,018,754 |
| Common stock options                    | 365,394             | —         |
| Shares issuable upon conversion of debt | 670,902             | 695,733   |
|   | 1,862,509           | 2,714,487 |

### Segments

The Company operates in only one segment. Management uses cash flows as the primary measure to manage its business and does not segment its business for internal reporting or decision making purposes.

### Recent Accounting Pronouncements

In August 2014, the FASB issued Accounting Standards Update No. 2014-15, Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern ("ASU 2014-15"). ASU 2014-15 provides guidance on presentation of management's plans, when conditions or events, considered in the aggregate, raise substantial doubt about the entity's ability to continue as a going concern within one year after the date that the financial statements are issued. This guidance is intended to mitigate those conditions or events that will alleviate substantial doubt about the entity's ability to continue as a going concern. ASU 2014-15 is effective for fiscal years ending after December 15, 2016. The adoption of ASU 2014-15 is not expected to have a material impact on the Company's financial statements.

In May 2014, the FASB issued Accounting Standards Update No. 2014-9, Revenue from Contracts with Customers ("ASU 2014-9"), which provides guidance for revenue recognition. The core principle of ASU 2014-9 is that a company will recognize revenue when it transfers promised goods or services to customers in an amount that reflects the consideration to which the company expects to be entitled in exchange for those goods or services. The new guidance initially was effective for the Company beginning January 1, 2017, but on July 9, 2015 the FASB deferred the effective date and, as a result, the new guidance is effective for the Company beginning January 1, 2018. Companies may adopt the new revenue standard as of the original effective date. The Company is currently evaluating the method of adoption and the potential impact this standard will have on its financial statements.

In November 2015, the FASB issued Accounting Standards Update No. 2015-17, Income Taxes (Topic 740): Balance Sheet Classification of Deferred Taxes ("ASU 2015-17"). To simplify the presentation of deferred income taxes, the amendments in ASU 2015-17 require that deferred income tax liabilities and assets be classified as noncurrent in a classified statement of financial position. The amendment is effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2016. The adoption of ASU 2015-17 is not expected to have a material

impact on the Company's financial statements.

## 2. Investments in Marketable Securities

The Company's investment strategy is focused on capital preservation. The Company invests in instruments that meet the credit quality standards outlined in the Company's investment policy. This policy also limits the amount of credit exposure to any one issue or type of instrument. The Company did not have any investments classified as available-for-sale as of December 31, 2014. As of December 31, 2015, the Company's investments were in money market funds, certificates of deposit and corporate debt securities. There were no sales of available-for-sale securities during the year ended December 31, 2015.

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Investments classified as available-for-sale as of December 31, 2015 consisted of the following:

|                               | Amortized<br>Cost | Gross<br>Unrealized<br>Gains <sup>(1)</sup> | Gross<br>Unrealized<br>Losses <sup>(1)</sup> | Aggregate<br>Estimated<br>Fair Value |
|-------------------------------|-------------------|---|--|--------------------------------------|
| Certificates of deposit (2)   | \$5,851,343       | \$ —  | \$ —   | \$5,851,343                          |
| Corporate debt securities (2) | 7,491,526         | —   | (7,370 )                                     | 7,484,156                            |
|                               | \$13,342,869      | \$ —  | — \$ (7,370 )                                | \$13,335,499                         |

- (1) Unrealized gains and losses on available-for-sale securities are included as a component of comprehensive loss. At December 31, 2015, there were 14 securities in an unrealized loss position. These unrealized losses were less than \$2,000 individually and \$8,000 in the aggregate. These securities have not been in a continuous unrealized loss position for more than 12 months. The Company does not intend to sell these investments and it is not more likely than not that the Company will be required to sell these investments before recovery of their amortized cost basis which may be at maturity. The Company reviews its investments to identify and evaluate investments that have an indication of possible other-than-temporary impairment. Factors considered in determining whether a loss is other-than-temporary include the length of time and extent to which fair value has been less than the cost basis, the financial condition and near-term prospects of the investee, and the Company's intent and ability to hold the investment for a period of time sufficient to allow for any anticipated recovery in market value.
- (2) At December 31, 2015, none of these securities were classified as cash and cash equivalents on the Company's balance sheet and none of these securities were scheduled to mature outside of one year.

### 3. Fair Value of Financial Instruments

The Company's financial instruments consist of cash and cash equivalents, investments, accounts payable, accrued license fees, debt and its related debt conversion feature liability. The carrying amounts reported in the accompanying balance sheets for cash and cash equivalents and accounts payable approximate fair value because of the short-term maturity of those instruments. Fair value measurements are classified and disclosed in one of the following three categories:

Level 1 —Quoted prices in active markets for identical assets or liabilities.

Level 2 —Inputs other than Level 1 that are observable, either directly or indirectly, such as quoted prices for similar assets or liabilities; quoted prices in markets that are not active; or other inputs that are observable or can be corroborated by observable market data for substantially the full term of the assets or liabilities.

Level 3 —Unobservable inputs that are supported by little or no market activity and that are significant to the fair value of the assets or liabilities.

As of December 31, 2015, all of the Company's financial assets that were subject to fair value measurements were valued using observable inputs. The Company's financial assets valued based on Level 1 inputs consist of money market funds and certificates of deposit. The Company's financial assets valued based on Level 2 inputs consist of corporate debt securities, which consist of investments in highly-rated investment-grade corporations. The Company did not have any assets or liabilities categorized as Level 1 or Level 2 in the fair value hierarchy as of December 31, 2014.

The Company's financial liabilities that were subject to fair value measurements consist of accrued license fees and debt conversion features that have been recorded as liabilities based on Level 3 unobservable inputs.

The fair value of the debt conversion feature, long-term as of December 31, 2015 required management to estimate fair value based on assumed timing of conversion and an assumed annual discount rate. Alternate probabilities would have resulted in increases or decreases in the fair value of the debt conversion feature liabilities.

As of December 31, 2014, the Company's accrued license fees have been recorded based on "Level 3" fair value inputs, which consist of unobservable inputs and generally reflect management's estimate of assumptions that market participants would use in pricing the assets. The fair value of the debt conversion feature liability, current at December 31, 2014 required management to make assumptions about the occurrence of an equity financing resulting in net proceeds of at least \$5,000,000 and the outstanding convertible promissory notes issued by the Company from September 2012 through June 2013 (the "Convertible Notes") converting based on the applicable conversion terms.



The fair value of the debt conversion feature, long-term at December 31, 2014 required management to make assumptions about the probability of the occurrence of the earlier of a private equity financing with aggregate net proceeds to the Company of at least \$20,000,000 or the Company's initial public offering, and the Ligand Note being converted based on the applicable conversion terms.

The fair values of the Company's financial instruments are presented below:

|   | Total               | Fair Value Measurements at<br>December 31, 2015 |                    |                    |
|---|---------------------|---|--------------------|--------------------|
|   |                     | Level 1   | Level 2            | Level 3            |
| <b>Financial assets carried at fair value:</b>      |                     |   |                    |                    |
| <b>Cash equivalents:</b>                            |                     |   |                    |                    |
| Money market funds                                  | \$548,872           | \$548,872                                       | \$—                | \$—                |
| <b>Short-term investments</b>                       |                     |   |                    |                    |
| Certificates of deposit                             | 5,851,343           | 5,851,343                                       | —                  | —                  |
| Corporate debt securities, available-for-sale       | 7,484,156           | —   | 7,484,156          | —                  |
| <b>Total financial assets</b>                       | <b>\$13,884,371</b> | <b>\$6,400,215</b>                              | <b>\$7,484,156</b> | <b>\$—</b>         |
| <b>Financial liabilities carried at fair value:</b> |                     |   |                    |                    |
| Debt conversion feature - long-term                 | \$2,370,903         | \$—   | \$—                | \$2,370,903        |
| <b>Total financial liabilities</b>                  | <b>\$2,370,903</b>  | <b>\$—</b>                                      | <b>\$—</b>         | <b>\$2,370,903</b> |

|   | Total               | Fair Value<br>Measurements at<br>December 31, 2014 |            |                     |
|---|---------------------|--|------------|---------------------|
|   |                     | Level 1  | Level 2    | Level 3             |
| <b>Financial liabilities carried at fair value:</b> |                     |  |            |                     |
| Accrued license fees                                | \$19,865,863        | \$—  | \$—        | \$19,865,863        |
| Debt conversion feature – current                   | 58,742              | —  | —          | 58,742              |
| Debt conversion feature – long-term                 | 1,390,469           | —  | —          | 1,390,469           |
| <b>Total financial liabilities</b>                  | <b>\$21,315,074</b> | <b>\$—</b>   | <b>\$—</b> | <b>\$21,315,074</b> |

The table below presents a summary of changes in the Company's accrued license fees and debt conversion feature liability measured at fair value on a recurring basis using significant unobservable inputs (Level 3) for the years ended December 31, 2015 and 2014:

|  | Year Ended December 31, |              |
|--|-------------------------|--------------|
|  | 2015                    | 2014         |
| <b>Accrued license fees:</b>               |                         |              |
| Beginning balance                          | \$19,865,863            | \$—          |
| Additions                                  | —                       | 21,687,576   |
| Adjustments resulting from changes in fair | 9,381,848               | (1,821,713 ) |

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|  |              |              |
|--|--------------|--------------|
| value recognized in earnings               |              |              |
| Issuance of shares of common stock         | (29,247,711) | —            |
| Ending balance                             | \$—          | \$19,865,863 |
| Debt conversion feature:                   |              |              |
| Beginning balance                          | \$1,449,211  | \$71,655     |
| Additions                                  | —            | 1,768,319    |
| Adjustments resulting from changes in fair |              |              |
| value recognized in earnings               | 1,043,478    | (390,763 )   |
| Issuance of shares of common stock         | (121,786 )   | —            |
| Ending balance                             | \$2,370,903  | \$1,449,211  |

The following table sets forth the Company's valuation techniques and significant unobservable inputs used to determine fair value for significant Level 3 liabilities:

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|  | Fair Value      | Valuation  | Significant   |                              |
|--|-----------------|--|---|------------------------------|
|  | Assets          | Technique(s)                                     | Unobservable Input                                  | Range                        |
| <b>Accrued license fees:</b>                     |                 |  |   |                              |
| December 31, 2014                                | \$—\$19,865,863 | Probability weighted<br>expected return<br>model | Probability weightings assigned to each<br>scenario | 30% to 40%                   |
|  |                 |  | Time assumptions to liquidation events              | 4/30/2015-<br>3/31/2017      |
|  |                 |  | Discount rates                                      | 12% to 60%                   |
| <b>Debt conversion<br/>feature<br/>liability</b> |                 |  |   |                              |
| December 31, 2015                                | \$—\$2,370,903  | Discounted cash<br>flow model                    | Timing of the events                                | 5/21/2016                    |
|  |                 |  | Probabilities of Occurrence                         | 100%                         |
|  |                 |  | Discount rate                                       | 37.5%                        |
| December 31, 2014                                | \$—\$1,449,211  | Discounted cash<br>flow model                    | Timing of the events                                | 3/31/2015<br>to<br>6/30/2015 |
|  |                 |  | Probabilities of Occurrence                         | 30% to 40%                   |
|  |                 |  | Discount rate                                       | 37.5%                        |

Level 3 Fair Value Sensitivity

Accrued license fees

For accrued license fees, generally increases (decreases) in the probability weightings assigned to each scenario would result in increases (decreases) to the fair value. In general, an increase (decrease) in discount rate tends to result in (decreases) increases to fair value.

Debt conversion features

The fair value of the debt conversion feature liabilities includes the estimated timing of the events as well as the related probabilities of occurrence. The shorter/longer the period estimated to the events, the higher/lower the value of the debt conversion feature liabilities. The higher/lower the probability of occurrence, the higher/lower the value of the debt conversion feature liability.

#### 4. Agreements with Ligand Pharmaceuticals Incorporated

In May 2014, the Company entered into a master license agreement with Ligand (the “Master License Agreement”), pursuant to which, among other things, the Company acquired the rights to a number of research and development programs under patents related to the Company’s VK5211, VK2809, VK0214, VK0612, EPOR and DGAT-1 programs, related know-how controlled by Ligand and physical quantities of VK5211, VK2809, VK0214, VK0612, EPOR and DGAT-1 compounds.

Pursuant to the terms of the Master License Agreement, the Company has the exclusive right and sole responsibility and decision-making authority for researching and developing any pharmaceutical products that contain or comprise one or any combination of the technology and compounds licensed from Ligand pursuant to the Master License Agreement (the “Licensed Products”). The Company also has the exclusive right and sole responsibility and decision-making authority to conduct all clinical trials and preclinical studies that the Company believes are appropriate to obtain the regulatory approvals necessary for commercialization of the Licensed Products, and the Company will own and maintain all regulatory filings and all regulatory approvals for the Licensed Products. Additionally, pursuant to the terms of the Master License Agreement, the Company has the sole decision-making authority and responsibility and the exclusive right to commercialize any of the Licensed Products, either by itself or, in certain circumstances, through sublicensees selected by the Company. The Company also has the exclusive right to manufacture or have manufactured any Licensed Product itself or, in certain circumstances, through sublicensees or third parties selected by the Company. The Company will own any intellectual property that it develops in connection with the license granted under the Master License Agreement.

As partial consideration for the grant of the rights and licenses to the Company under the Master License Agreement, the Company issued to Ligand at the closing of the IPO 3,655,964 shares of its common stock having an estimated aggregate value of \$29.2 million. Furthermore, as partial consideration for the grant of the rights and licenses to the Company under the Master License Agreement the Company entered into the Loan and Security Agreement with Ligand (as discussed below).

As further partial consideration for the grant of the rights and licenses to the Company by Ligand under the Master License Agreement, the Company has agreed to pay to Ligand certain one-time, non-refundable milestone payments in connection with the Licensed Products of up to \$1.54 billion in the aggregate upon the achievement of certain development, regulatory and sales milestones. The Company will also pay to Ligand royalties on aggregate annual worldwide net sales of Licensed Products by the Company, its affiliates and its sublicensees at tiered percentage rates from the low-to-upper single digits based upon net sales.

The term of the Master License Agreement will continue unless the agreement is terminated by the Company or Ligand, and each of the Company and Ligand have the right to terminate the Master License Agreement in certain circumstances, including, without limitation, if the other party defaults on certain of its obligations under the Master License Agreement.

Ligand has the right to terminate the Master License Agreement under certain circumstances, including, but not limited to: (1) in the event of the Company's insolvency or bankruptcy, (2) if the Company does not pay an undisputed amount owing under the Master License Agreement when due and fails to cure such default within a specified period of time, or (3) if the Company defaults on certain of its material and substantial obligations and fails to cure the default within a specified period of time. The Company has the right to terminate the Master License Agreement under certain circumstances, including, but not limited to: (i) if Ligand does not pay an undisputed amount owing under the Master License Agreement when due and fails to cure such default within a specified period of time, or (ii) if Ligand defaults on certain of its material and substantial obligations and fails to cure the default within a specified period of time. In addition, provisions of the Master License Agreement can be terminated on a licensed program-by-program basis under certain circumstances. In the event that the Master License Agreement is terminated in its entirety or with respect to a specific licensed program for any reason: (A) all licenses granted to the Company under the Master License Agreement (or with respect to the specific licensed program) will terminate and the Company will, upon Ligand's request (subject to Ligand assuming legal responsibility for any clinical trials of the Licensed Products then ongoing), assign and transfer to Ligand (or to such transferee as Ligand may direct), at no cost to Ligand, all regulatory documentation and all regulatory approvals prepared or obtained by the Company or on its behalf related to the Licensed Products (or those related to the specific licensed program), or, if Ligand does not make such a request, the Company will wind down any ongoing clinical trials with respect to the Licensed Products (or those related to the specific licensed program) at no cost to Ligand; (B) the Company will, upon Ligand's request, sell and transfer to Ligand (or to such transferee as Ligand may direct), at a price equal to 125% of the Company's costs of goods, any and all chemical, biological or physical materials relating to or comprising the Licensed Products (or those related to the specific licensed program); (C) the Company will have, for a period of six months following termination, the right to sell on the normal business terms in existence before such termination any finished commercial inventory of Licensed Products (or those related to the specific licensed program) which remains on hand, so long as the Company pays to Ligand the applicable royalties and sales milestones; (D) Ligand has the right to require the Company to assign to Ligand the trademarks owned by the Company relating to the Licensed Products (or those related to the specific licensed program); and (E) the Company will grant to Ligand a non-exclusive, worldwide, royalty-bearing sublicensable license under any patent rights and know-how controlled by the Company to the extent necessary to make, have made, import, use, offer to sell and sell the Licensed Products (or those related to the specific licensed program) anywhere in the world at a royalty rate in the low single digits.

Under the Master License Agreement, the Company has agreed to indemnify Ligand for claims relating to the performance of the Company's obligations under the Master License Agreement, any breach of the representations and warranties made by the Company under the Master License Agreement, clinical trials conducted by the Company and the research, development and commercialization of the Licensed Products by the Company and its affiliates, sublicensees, distributors and agents. In addition, Ligand has agreed to indemnify the Company for claims relating to the performance of its obligations under the Master License Agreement, its breach of representations and warranties under the agreement and its research and development of the licensed compounds before the effective date of the

Master License Agreement. Each party's indemnification obligations will not apply to the extent the claims result from the negligence or willful misconduct of the indemnified party or any of its employees, agents, officers or directors or from the indemnified party's breach of its representations or warranties set forth in the Master License Agreement.

On September 6, 2014, the Company and Ligand entered into an amendment to the Master License Agreement pursuant to which the parties agreed to certain modifications to the calculations used to determine the number of shares issuable to Ligand pursuant to the Master License Agreement. As a result of the modification, the Company incurred an incremental charge to research and development expense of \$517,960 and a corresponding increase in the accrued license fees. In connection with entering into the Master License Agreement with Ligand, the Company entered into a loan and security agreement with Ligand, dated May 21, 2014 (the "Loan and Security Agreement"), pursuant to which, among other things, Ligand agreed to provide the Company with loans in the aggregate amount of up to \$2.5 million.

In May 2014, the Company also entered into a Management Rights Letter with Ligand that requires the Company to expand the size of the Company's board of directors to create an additional directorship on the Company's board of directors and to allow Ligand to appoint an individual to fill the new directorship. The Management Rights Letter will terminate upon the earliest to occur of the liquidation or indefinite cessation of the Company's business operations, the execution by the Company of a general assignment for

the benefit of creditors or the appointment of a receiver or trustee to take possession of the Company's property and assets, an acquisition of the Company by means of any transaction (including, without limitation, any reorganization, merger or consolidation) if the Company's stockholders of record as constituted immediately prior to the transaction hold less than 50% of the voting power of the surviving or acquiring entity, or following the issuance of the Company's securities pursuant to the Master License Agreement, the date that Ligand ceases to beneficially own at least 7.5% of the Company's outstanding voting stock, or the date of May 21, 2024.

The Company also entered into a Registration Rights Agreement with Ligand in May 2014 for which the Company granted certain registration rights to Ligand with respect to the securities of the Company issued to Ligand pursuant to the Master License Agreement and the Ligand Note (collectively, the "Viking Securities"), the shares of the Company's common stock issued or issuable upon conversion of the Viking Securities, if applicable, the shares of the Company's common stock issued as a dividend or other distribution with respect to, in exchange for or in replacement of the Viking Securities and the shares of the Company's capital stock issued upon conversion of the Ligand Note, or collectively, the Registrable Securities.

Under the Registration Rights Agreement, the Company has agreed to file with the SEC, by no later than the first date on which the lock-up requested by the underwriters for the Company's initial public offering expires or lapses with respect to Ligand (or the first business day thereafter), a registration statement on Form S-1 under the Securities Act that covers the resale of the full amount of the Registrable Securities. The Company has agreed to use commercially reasonable efforts to have the Registration Statement declared effective by the SEC as soon as practically possible after it is filed with the SEC. There are certain cash payment penalties that the Company may need to pay to Ligand if the Company does not meet certain timelines with the SEC. The Company has also agreed to use commercially reasonable efforts to keep each Registration Statement filed pursuant to the Registration Rights Agreement effective for certain periods of time.

The Registration Rights Agreement was amended on January 22, 2016 (see Note 13 for a description of the amendments to the agreement).

In May 2014, the Company entered into a Sublease Agreement with Ligand, as sublandlord, for approximately 5,851 square feet of individual and shared space within the building located at 11119 North Torrey Pines, San Diego, California 92037. Under the Sublease Agreement, the Company was required, among other things, to pay base rent in the amount of approximately \$13,500 per month through December 31, 2014. The sublease commenced on May 21, 2014 and expired on December 31, 2014.

#### 5. Accrued License Fees and License Fees Expense

As partial consideration for the grant of the rights and licenses to the Company under the Master License Agreement, the Company issued to Ligand at the closing of the Company's IPO 3,655,964 shares of its common stock having an estimated aggregate value of \$29.2 million. Prior to this event, the Company had to determine how to account for the potential issuance of these shares to Ligand in accordance with the Master License Agreement. Under the Master License Agreement, in the event the Company consummated a firmly underwritten public offering pursuant to a Registration Statement on Form S-1 or any successor form (an "Initial Public Offering"), the Company would issue to Ligand at the closing of the Initial Public Offering a number of shares of its common stock having an estimated aggregate value of \$29.0 million, subject to adjustment based on the deemed value of the Company as of immediately prior to the Initial Public Offering and the per share price at which shares of its common stock are sold in the Initial Public Offering. In the event the Company consummated a private financing of its equity securities (a "Private

Financing”) prior to an Initial Public Offering, Ligand had the option to receive a number of shares of the same class and type of securities issued and sold by the Company in the Private Financing having an estimated aggregate value of \$29.0 million, subject to adjustment based on the deemed value of the Company as of immediately prior to the Private Financing and the per share price at which shares of the Company’s common stock are sold in the Private Financing, or, in lieu of receiving the same class and type of shares issued in the Private Financing, to defer its right to receive equity in the Company until an Initial Public Offering or subsequent private financing of the Company.

Prior to the IPO, the Company reported the expected issuance of stock to Ligand under the Master License Agreement as a liability in accordance with ASC Topic 480, Distinguishing Liabilities from Equity, as it represents a conditional obligation that the Company must settle by issuing a variable number of shares of its common stock. The Company measured, at each reporting date until settled, the estimated amount of cash that would be paid if settlement occurred on the reporting date, with any changes in that fair market value reported as other expense during the period of change. In accordance with this guidance, the Company determined its enterprise values as of May 21, 2014, the date the Master License Agreement was entered into, December 31, 2014, its most recent fiscal year end, and as of May 4, 2015, the closing date of the Company’s IPO.

The fair value of the assets was determined using a PWERM, which incorporated relevant events and expected exit scenarios for the Company. The exit scenarios included an initial public offering, a merger or acquisition, which included an assumption of a Private



Financing, and a scenario in which neither an initial public offering nor the merger or acquisition occurred. The enterprise value was based primarily on the market approach and probability-weighted expected exit values for the Company under each scenario. As of the execution of the Master License Agreement, the Company ascribed a percentage likelihood to each expected exit scenario, and each was less than 50%. However, since Ligand was entitled to receive shares under the Master License Agreement if either of these IPO or merger or acquisition scenarios were to occur, and the sum of the percentage likelihoods for the two exceeded 50%, the Company determined that a payout of shares was probable. Further, since the valuations would determine a value, the Company determined that the liability under the Master License Agreement should be reported as of the execution of the Master License Agreement. Similar publicly traded companies and merger or acquisition transactions were utilized as part of the market approach, and appropriate metrics were applied to the Company's numbers along with qualitative comparable assessments. The Company utilized a Monte Carlo simulation method to determine the weighted average per share value as of the acquisition date and in accordance with the Master License Agreement, utilized that per share value to calculate the estimated license fee liability as of May 21, 2014 to be \$21,169,616. In addition, the Company incurred an incremental charge of \$517,960 on September 6, 2014 related to the license fee liability pursuant to the amendment to the Master License Agreement.

Having determined the amount of the liability to be reported, the Company then evaluated whether to report the offset as an asset or as an expense. In accordance with ASC Topic 730, Research and Development, intangible assets that are acquired and have an alternative future use, as defined, should be capitalized and reported as an intangible asset; however, the cost of acquired intangible assets that do not have alternative future uses should be reported as research and development expense as incurred. The Company noted that the assets, the rights for which were acquired pursuant to the Master License Agreement, are in the preclinical and clinical stages, and not FDA approved. The Company has therefore interpreted the guidance to say that since these assets are not commercially readily saleable, i.e., they are still in preclinical and clinical trials, that they do not satisfy the definition of having an alternative future use, as defined. Accordingly, the Company recorded the offset to the license fee liability as research and development expense in May 2014.

The Company revalued the license fee liability as of December 31, 2014 using consistent methodology to that used for the May valuation; however, given the Company's recent refocus to certain other programs as well as some deemed market risks as of December 31, 2014, it revalued the license fee liability based upon these new programs; VK5211 to be used in hip fractures and VK0214 to be used in X-ALD; and, based upon their updated fair value, reported a decrease in the license fee liability and other expense for the year ended December 31, 2014 in the amount of \$1,821,713, to reduce the license fee liability to \$19,865,863 as of December 31, 2014. During 2015, the Company continued to revalue the license fee liability under these new assumptions with any increases in that liability being recorded as other expense. On May 4, 2015, upon the closing of the Company's IPO, the license fee liability was determined to be valued at \$29,247,711 and the incremental amount of \$9,381,848 was recorded as additional Other expense in the Company's Statement of Operations for the period from January 1, 2015 through May 4, 2015. In accordance with the Master License Agreement, the Company issued 3,655,964 shares of its common stock having an estimated aggregate value equivalent to the May 4, 2015 license fee liability of \$29,247,711 and offset this liability against its common stock and additional paid-in-capital.

#### 6. Convertible Notes Payable

Convertible notes payable consisted of the following at:

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|  | December 31, |             |
|--|--------------|-------------|
|  | 2015         | 2014        |
| <b>Current maturities:</b>                                   |              |             |
| Various convertible notes payable issued during 2013 with    |              |             |
| interest rates ranging from 2.16% to 4.68% annually,         |              |             |
| due during 2015  | \$—          | \$310,350   |
| Discount on notes payable, current                           | —            | (6,076 )    |
| Convertible notes payable, current portion (net of discount) | \$—          | \$304,274   |
| <b>Long-term maturities:</b>                                 |              |             |
| Convertible note payable issued during 2014 to Ligand as     |              |             |
| part of the Master License Agreement with a 5% annual        |              |             |
| interest rate, due during 2016 (subsequently                 |              |             |
| amended to 2017)   | \$2,500,000  | \$2,500,000 |
| Discount on notes payable, long-term                         | (348,460 )   | (1,235,886) |
| Convertible notes payable, long-term (net of discount)       | \$2,151,540  | \$1,264,114 |

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In September 2012, the Company's board of directors authorized the Company to issue and sell up to an aggregate of \$1.0 million in convertible promissory notes (the "Notes") to accredited investors in one or more closings through September 2014 (the "Note Authorization"). The notes bore interest at a rate equal to the lesser of the short-term monthly applicable federal rate as published by the Internal Revenue Service or the maximum rate permissible by law. Interest under the Notes is due and payable at maturity. Unless repaid in full, amended or converted in full, each Note matured two years from its date of purchase. In the event that any principal amount due under the Notes was not paid in full by the maturity date, such unpaid principal amount would bear interest at the lesser of 2% or the maximum rate permissible by law.

If, prior to maturity of the Notes, the Company issues capital stock resulting in net proceeds of at least \$5.0 million (a "Qualifying Financing"), the Notes will convert into shares of the capital stock issued in the Qualifying Financing. The number of shares issued upon conversion will be equal to the quotient obtained by dividing the then-outstanding loan balance by either 70% or 75%, as applicable, of the lowest purchase price paid per share paid by another investor in the Qualifying Financing. If, prior to the maturity of the Notes, the Company issues preferred stock in a financing that does not qualify as a Qualifying Financing (a "Non-Qualifying Financing"), the holders of the Notes will have the option of converting their Notes into shares of the preferred stock issued in the Non-Qualifying Financing on the same terms as the investors in the Non-Qualifying Financing. In the event the Company undergoes a change in control, as defined in the Notes, prior to the maturity date and repayment of the Notes, the holders of the Notes will have the option to either (1) convert the loan balance into shares of the Company's common stock in an amount equal to the ratio of (a) the then-outstanding loan balance over (b) the ratio of \$7,500,000 divided by the number of shares of capital stock of the Company outstanding immediately prior to the change in control, or (2) demand immediate repayment of an amount equal to 125% of the then-outstanding loan balance. If the Notes are still outstanding at their maturity date, the holders of the Notes have the option to either demand immediate repayment of all outstanding principal and interest or to convert the loan balance into shares of the Company's common stock in an amount equal to the ratio of the then-outstanding loan balance over the ratio of \$7,500,000 divided by the number of shares of capital stock of the Company outstanding immediately prior to the maturity date.

The debt conversion feature embedded in the Notes is accounted for under ASC Topic 815 – Derivatives and Hedging. At issuance, the fair value of the debt conversion feature totaled \$8,286 on the Notes issued during 2012 and \$42,747 on the Notes issued during 2013. The fair value of the debt conversion feature was allocated from the gross proceeds of the Notes with the respective discount amortized to interest expense over the original term of the Notes using the effective interest method. The valuation of the bifurcated debt conversion feature was performed using Level 3 inputs, requiring the Company to make assumptions about the probability of the occurrence of a Qualifying Financing and the Notes being converted based on the applicable conversion terms. Alternative probabilities would have resulted in increases or decreases in the value of the debt conversion feature. The Company is required to mark to market the value of the conversion feature liability. Therefore, as of December 31, 2014, the Company revalued the fair value of the debt conversion feature for each tranche and determined the conversion feature liability to be \$58,742. The Company amortized \$25,529 of the discount in 2014.

Pursuant to the terms of the Note Authorization, from September 2012 through June 2013, the Company issued a total aggregate principal amount of \$310,350 in Notes and recorded interest expense at interest rates ranging from 3.00% to 4.68% on an annual basis of \$12,104 for the year ended December 31, 2014. The cumulative accrued interest payable on the Notes as of December 31, 2014 was \$18,611.

In accordance with the provisions of the convertible notes, on May 4, 2015, at the closing of the Company's IPO, the Company converted \$310,350 of principal amounts owed plus \$24,276 of accrued but unpaid interest as of that date on the Notes into 57,046 shares of the Company's common stock. The Company eliminated the notes payable and

recorded the offsets to common stock and additional paid-in-capital.

The Company entered into a Loan and Security Agreement with Ligand on May 21, 2014, pursuant to which, among other things, Ligand agreed to provide the Company with loans in the aggregate amount of up to \$2.5 million. Pursuant to the Loan and Security Agreement, Ligand initially loaned \$1.0 million to the Company on May 27, 2014, and additional amounts of \$250,000 to the Company each month from June 2014 through and including November 2014 for a total of \$2.5 million. The principal amount outstanding under the loans accrue interest at a fixed per annum rate equal to the lesser of 5% and the maximum interest rate permitted by law. In the event the Company defaults under the loans, the loans will accrue interest at a fixed per annum rate equal to the lesser of 8% and the maximum interest rate permitted by law. The loans are and will be evidenced by a Secured Convertible Promissory Note (the "Ligand Note"). Pursuant to the terms of the Loan and Security Agreement and the Ligand Note, the loans will become due and payable upon the written demand of Ligand at any time after the earlier to occur of an event of default under the Loan and Security Agreement or the Ligand Note, and May 21, 2016, unless the loans are converted into equity prior to such time. In addition, under the Loan and Security Agreement, the Company may not declare or pay dividends in respect of its common stock without Ligand's prior written consent. Pursuant to the terms of the Loan and Security Agreement, the Company recorded interest expense of \$58,611 during the year ended December 31, 2014, which remained payable as of December 31, 2014.

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The debt conversion feature embedded in each tranche of the Ligand Note is accounted for under ASC Topic 815 – Derivatives and Hedging. At each issuance date, the fair value of the debt conversion feature was determined. The fair value of the debt conversion feature was allocated from the gross proceeds of the Ligand Note with the respective discount amortized to interest expense over the original term of the Ligand Note using the effective interest method. The valuation of the bifurcated debt conversion feature was performed using Level 3 inputs, requiring the Company to make assumptions about the probability of the occurrence of a private equity financing with aggregate net proceeds to the Company of at least \$20,000,000 or the Company's initial public offering and the Ligand Note being converted based on the applicable conversion terms. Alternative probabilities would have resulted in increases or decreases in the value of the debt conversion feature. The Company is required to mark to market the value of the conversion feature liability. Therefore, as of December 31, 2014, the Company revalued the fair value of the debt conversion feature for each tranche of the Note outstanding as of December 31, 2014, and determined the conversion feature liability to be \$1,390,469. The Company amortized \$532,433 of the discount during the year ended December 31, 2014.

On April 8, 2015, the Company and Ligand entered into a First Amendment to Loan and Security Agreement with Ligand (the "Loan Amendment"), pursuant to which the parties agreed to amend, among other things, the timing of Ligand's conversion rights under the Secured Convertible Promissory Note issued to Ligand on May 21, 2014 (the "Ligand Note") following certain Company transactions. Under the terms of the Loan Amendment, following the consummation of the earlier to occur of (1) a bona fide capital financing transaction or series of financing transactions with one or more financial non-strategic investors resulting in aggregate net proceeds to the Company of at least \$20,000,000 and pursuant to which the Company issues shares of its equity securities, (2) a firmly underwritten public offering pursuant to the Securities Act of 1933, as amended, on Form S-1 or Form S-3, or any successor forms, or (3) May 4, 2016 (the one year anniversary of the closing of the IPO), Ligand may elect to convert the Ligand Note into shares of the Company's common stock and/or cash in an amount equal to 200% of the principal amount of the loan plus all accrued and previously unpaid interest thereon. Additionally, pursuant to the Loan Amendment, Ligand has agreed that it will not, until the earlier of (A) 270 days from the date of conversion of the Ligand Note or (B) April 28, 2016 (one year following the date of the prospectus filed with the SEC relating to the IPO), sell or otherwise transfer or dispose of the shares of common stock issuable upon conversion of the Ligand Note.

As of December 31, 2015, the Ligand Note was recorded at \$2,500,000, and interest in the amount of \$183,611 was payable on the Ligand Note. During the year ended December 31, 2015, the Company also recorded \$125,000 of interest expense, \$887,426 of amortization of debt discount and \$980,434 as other expense related to increases in the fair value of the debt conversion feature liability.

In connection with the Loan and Security Agreement, the Company also granted Ligand a continuing security interest in all of its right, title and interest in and to its assets as collateral for the full, prompt, complete and final payment and performance when due of all obligations under the Loan and Security Agreement and the Ligand Note.

A second amendment to the Loan and Security Agreement was entered into on January 22, 2016 (see Note 13).

## 7. Stockholders' Equity (Deficit)

### Preferred Stock

The Company is authorized to issue up to 10,000,000 shares of \$0.00001 par value preferred stock, with no shares outstanding as of December 31, 2015 and 2014. The Board of Directors is authorized to designate the terms and conditions of any preferred stock the company issues without further action by the common stockholders.

#### Common Stock

The Company is authorized to issue up to 300,000,000 shares of common stock, \$0.00001 par value per share. On September 26, 2012, the Company issued 4,750,000 shares of common stock to its founders for the contribution of certain intellectual property and assets having a deemed fair value of \$0.01 per share. The shares of common stock issued to the founders are subject to a repurchase feature whereby the Company can repurchase the stock if the applicable founder's arrangements with the Company are terminated. The repurchase feature lapses over time and the repurchase option immediately ceases upon the occurrence of certain triggering events. The related expense is being charged over the requisite service period. At December 31, 2015, none of these shares were subject to the repurchase option. See below regarding the repurchase of certain of these shares.

During fiscal year 2013, the Company sold an additional 700,000 shares of its common stock to consultants. The purchase price of the shares of common stock was \$0.01, which approximated fair value. The Company received a total of \$7 in cash and \$6,993 in notes receivable as consideration for the issuance of the 700,000 shares of common stock. The shares of common stock were subject to

repurchase features whereby the Company can repurchase the shares from the consultants if their arrangements with the Company are terminated. The repurchase features lapse over time. In June 2013, the Company exercised its right to repurchase 250,000 shares of its common stock issued earlier in 2013 in connection with the termination of one of the consulting arrangements. The Company paid \$3 in cash to refund the cash contributed by the consultant and eliminated the \$2,497 note receivable issued by the consultant to the Company. In March 2014, the Company terminated another one of the consulting agreements. In conjunction with the termination of the consulting agreement, the Company exercised its right to repurchase 200,000 shares by repaying the \$2 in cash contributed by the consultant and eliminating the \$1,998 note receivable issued by the consultant to the Company. In May 2014, the Company forgave the remaining notes receivable balance of \$2,581, which included \$2,498 of principal and \$83 of interest from one of its consultants in consideration for services rendered by the consultant. At December 31, 2015, there was \$290 of total unrecognized compensation costs related to these shares, which are expected to be recognized over a weighted-average period of 1.25 years. During the year ended December 31, 2015, the Company recorded stock-based compensation of \$145 related to these share issuances.

In connection with the preparation of the financial statements necessary for inclusion in a registration statement, in May 2014 the Company reassessed the estimated fair value of its common stock for financial reporting purposes. The Company reassessed the estimated fair value of its common stock for each quarterly period from its inception on September 24, 2012 through December 31, 2013. Valuation analyses were performed as of September 26, 2012, April 15, 2013 and July 15, 2013 (the respective dates of stock activity noted above). The Company concluded that its shares of common stock as of each such date had a fair value less than or equal to the then estimated fair value of common stock at the date of issuance. Therefore, no additional stock expense was required to be expensed by the Company.

In February 2014, the Company entered into a stock purchase agreement with one of its founders. The agreement provides for the purchase of 1,000,000 shares of the Company's common stock at a price per share of \$0.01 in exchange for future services to be rendered to the Company as measured by certain performance criteria. The shares are subject to a repurchase option and vest in two tranches of 500,000 shares each, upon achievement of the performance target or upon a triggering event as defined.

To appropriately account for this stock purchase, the Company determined the fair value of the common stock on the date of purchase as well as the likelihood of achievement of each of the performance conditions included in the agreement. The valuation methodology utilized in determining fair value relied on the PWERM, which incorporates relevant events and expected future exit scenarios for the Company. The exit scenarios included merger and acquisition and initial public offering scenarios. The enterprise value under each scenario was based primarily on the market approach and probability-weighted expected exit values for the Company under each scenario. Similar merger and acquisition transactions and publicly traded companies were utilized within the market approach and appropriate metrics were applied to the Company along with qualitative comparable assessments. The indicated value under the market approach was used as the starting aggregate value for the valuation of these performance-based shares. The Company utilized a Monte Carlo simulation method to determine the fair value of the performance-based shares as of the issuance date. The Monte Carlo simulation method takes into consideration the expected timing of the performance milestones, probability of achieving the milestones and estimated per share common stock prices at expected vesting dates.

The Company determined that the issuance in February 2014 had a deemed fair value lower than the reassessed fair value of the common stock on the date of issuance based upon the PWERM. Since the stock issuance to the founder is tied to certain performance criteria, the Company reviewed the probability of achieving such criteria at February 20, 2014 and December 31, 2014 and determined that it was not probable that the criteria would be met. Therefore, no compensation expense was recorded for this issuance through December 31, 2014. The Company will continue to reassess at each reporting period whether it is probable that either of the two performance criteria will be met, and if

and when either are deemed probable, the Company will begin to record compensation expense using the fair value to determine stock-based compensation expense in its financial statements over the period the Company estimates the performance criteria will actually be met. The Company determined that the fair value of the unrecognized expense was approximately \$168,000 at February 20, 2014, the grant date. In May 2015, the Company repurchased 633,810 of these shares at a purchase price of \$0.00001 per share. In connection with the repurchase, the Company entered into an amendment to the stock purchase agreement to provide that the remaining 366,190 shares will continue to vest in two tranches of 183,095 shares each, upon achievement of the performance target or upon a triggering event as defined, with the pro rata fair value of the expense of \$61,566 to be recognized. In October 2015, one of the two triggering events became probable of occurrence; therefore, for the year ended December 31, 2015, the Company recorded \$18,407 of stock-based compensation expense, with the remaining expense relating to this event estimated to be recognized through April 2017.

In June 2014, the Company entered into employment agreements with each of its five employees pursuant to which, among other things, each employee agreed to be paid a lower salary during the period of time between the signing of his or her employment agreement and the date of the closing of the Company's IPO. In exchange for this agreement to receive a lower salary, the Company agreed to issue, on the date of the closing of the IPO, to each of these employees a certain amount of fully vested restricted stock equal to the quotient of (1) the product of 150% of the difference between the employee's post-IPO salary and his or her pre-IPO salary multiplied by the number of days between the date of the closing of the IPO and the date of execution of the employee's employment

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agreement, divided by (2) the product of 365 multiplied by the closing sales price of the Company's common stock on the date of the closing of the IPO. Since the likelihood of an IPO was deemed to be less than probable as of December 31, 2014, the Company determined that it was not required to record the liability, which, as of December 31, 2014, would have been \$520,476, with respect to the potential issuance of these shares of fully vested restricted stock as of December 31, 2014, as it was less than probable that the Company would be required to issue such shares of fully vested restricted stock. Upon the closing of the IPO in May 2015, the Company issued 56,997 shares, net of shares withheld for taxes, in accordance with these agreements.

In September 2014, the Company entered into a stock repurchase agreement (the "Stock Repurchase Agreement") with each of its existing stockholders. Pursuant to the Stock Repurchase Agreement, the Company agreed to repurchase prior to the completion of the IPO, on a pro rata basis from each of the holders of its outstanding common stock, shares of the Company's common stock from these stockholders at a purchase price of \$0.00001 per share. The number of shares of common stock that the Company will repurchase pursuant to the Stock Repurchase Agreement and prior to the completion of the IPO is based on a formula in accordance with the Stock Repurchase Agreement. In accordance with this agreement, on May 4, 2015, the Company repurchased an aggregate of 3,802,859 shares of its common stock from its stockholders at a price of \$0.00001 per share for an aggregate purchase price of \$38.

On April 8, 2015, the Company entered into a Second Amendment to Master License Agreement with Ligand, pursuant to which the parties agreed to revise (1) the calculations used to determine the number of securities to be issued to Ligand upon the closing of the IPO, and (2) certain of the royalty percentages payable by the Company to Ligand based on worldwide annual net sales of the products licensed under the Master License Agreement.

Further, on April 8, 2015, the Company and Ligand entered into a First Amendment to Loan and Security Agreement with Ligand (the "Loan Amendment"), pursuant to which the parties agreed to amend, among other things, the timing of Ligand's conversion rights under the Secured Convertible Promissory Note issued to Ligand on May 21, 2014 (the "Ligand Note") following certain Company transactions. Under the terms of the Loan Amendment, following the consummation of the earlier to occur of (1) a bona fide capital financing transaction or series of financing transactions with one or more financial non-strategic investors resulting in aggregate net proceeds to the Company of at least \$20,000,000 and pursuant to which the Company issues shares of its equity securities, (2) a firmly underwritten public offering pursuant to the Securities Act of 1933, as amended, on Form S-1 or Form S-3, or any successor forms, or (3) May 4, 2016 (the one year anniversary of the closing of the IPO), Ligand may elect to convert the Ligand Note into shares of the Company's common stock and/or cash in an amount equal to 200% of the principal amount of the loan plus all accrued and previously unpaid interest thereon. Additionally, pursuant to the Loan Amendment, Ligand has agreed that it will not, until the earlier of (A) 270 days from the date of conversion of the Ligand Note or (B) April 28, 2016 (one year following the date of the prospectus filed with the SEC relating to the IPO), sell or otherwise transfer or dispose of the shares of common stock issuable upon conversion of the Ligand Note.

On May 4, 2015, the Company completed the IPO pursuant to a Registration Statement on Form S-1 that was declared effective on April 28, 2015. In the IPO, the Company sold 3,000,000 shares of its common stock at an initial public offering price of \$8.00 per share. The underwriters for the IPO had 30 days to exercise an over-allotment option to purchase up to an additional 450,000 shares at the initial public offering price, less the underwriting discount. Upon the closing of the IPO, on May 4, 2015, the Company raised a total of \$19,100,500 in net proceeds after deducting underwriting discounts, commissions and other offering expenses of \$4,899,500. These costs have been recorded as a reduction of the proceeds received in arriving at the amount to be recorded in additional paid-in capital upon completion of the IPO.

On April 28, 2015, the date of the execution and delivery of the underwriting agreement for the IPO, the Company granted stock options to purchase an aggregate of 83,144 shares of the Company's common stock to the non-employee directors of the Company.

Upon the closing of the IPO in May 2015, the Company (1) converted \$27,422,872 of accrued license fees as of May 4, 2015 into an aggregate of 3,427,859 shares of the Company's common stock issued to Ligand and Metabasis Therapeutics, Inc., a wholly-owned subsidiary of Ligand ("Metabasis") pursuant to the Master License Agreement; (2) incurred a non-cash interest charge of \$4,421,338 at the time of conversion of the accrued license fees, relating to the difference between the carrying amounts of the \$24,826,374 of accrued license fees and the fair market value of the shares issued in the IPO; (3) converted \$310,350 of the Company's convertible notes payable plus interest of \$24,276 as of May 4, 2015 into 57,046 shares of the Company's common stock; (4) incurred a beneficial conversion charge of \$121,786; and (5) issued an aggregate of 422,879 shares of the Company's common stock and granted options to purchase an aggregate of 206,000 shares of the Company's common stock to its employees, directors and a consultant of the Company pursuant to employment agreements, offer letters and a consulting agreement.

On May 26, 2015, the underwriters for the IPO exercised their full over-allotment option to purchase an additional 450,000 shares of the Company's common stock at \$8.00 per share, less the underwriting discount. On May 28, 2015, the Company sold the 450,000 shares to the underwriters pursuant to the over-allotment option and received additional net proceeds of \$3,225,866 after deducting underwriting discounts, commissions and other offering expenses of \$374,134. Upon the closing of the over-allotment option,

pursuant to the Master License Agreement, on May 28, 2015, the Company issued an additional aggregate of 228,105 shares of its common stock to Ligand and Metabasis.

During the year ended December 31, 2015, and in accordance with the Company's 2014 Employee Stock Purchase Plan (the "ESPP"), the Company issued an aggregate of 5,711 shares of its common stock to certain employees.

#### 8. Stock-Based Compensation

In connection with the IPO, the Company's 2014 Equity Incentive Plan (the "2014 Plan") and the ESPP became effective on April 28, 2015, the date of the execution and delivery of the underwriting agreement for the IPO. A total of 1,527,770 shares of the Company's common stock were initially reserved for issuance under the 2014 Plan, and 458,331 shares of the Company's common stock were initially reserved for issuance under the ESPP.

The Company generally uses the straight-line or graded vesting method to allocate compensation cost to reporting periods over each optionee's requisite service period, which is generally the vesting period, and estimates the fair value of stock-based awards or restricted stock units to employees and directors using the Black-Scholes option-valuation model. For options with a graded vesting schedule, the Company uses the graded vesting schedule to allocate compensation cost to reporting periods. The Black-Scholes model requires the input of subjective assumptions, including volatility, the expected term and the fair value of the underlying common stock on the date of grant, among other inputs. Stock options granted to non-employees are accounted for using the fair value approach. Stock options granted to non-employees are subject to periodic revaluation over their vesting terms.

**2014 Plan.** The 2014 Plan provides that the compensation committee of the Company's Board of Directors (the "Compensation Committee") may grant or issue stock options, stock appreciation rights, restricted shares, restricted stock units and unrestricted shares, deferred share units, performance and cash-settled awards and dividend equivalent rights to participants under the 2014 Plan. Initially, a total of 1,527,770 shares of the Company's common stock were reserved for issuance pursuant to the 2014 Plan, which number is also the limit on shares of common stock available for awards of incentive stock options. The number of shares available for issuance under the 2014 Plan will, unless otherwise determined by the Company's Board of Directors or the Compensation Committee, be automatically increased on January 1st of each year commencing on January 1, 2016 and ending on (and including) January 1, 2024, in an amount equal to 3.5% of the total number of shares of the Company's common stock outstanding on December 31st of the preceding calendar year. The shares of common stock deliverable pursuant to awards under the 2014 Plan are authorized but unissued shares of the Company's common stock, or shares of the Company's common stock that the Company otherwise holds in treasury or in trust. Any shares of the Company's common stock underlying awards that are settled in cash or otherwise expire, or are forfeited, terminated or cancelled (including pursuant to an exchange program established by the Compensation Committee) prior to the issuance of stock will again be available for issuance under the 2014 Plan. In addition, shares of the Company's common stock that are withheld (or not issued) in payment of the exercise price or taxes relating to an award, and shares of the Company's common stock equal to the number surrendered in payment of any exercise price or withholding taxes relating to an award, will again be available for issuance under the 2014 Plan.

**ESPP.** Initially, a total of 458,331 shares of the Company's common stock were reserved for issuance pursuant to the ESPP. The number of shares available for issuance under the ESPP will, unless otherwise determined by the Company's Board of Directors or the Compensation Committee, be automatically increased on January 1st of each year commencing on January 1, 2016 and ending on (and including) January 1, 2024, in an amount equal to 1% of the total number of shares of the Company's common stock outstanding on December 31st of the preceding calendar year.

The shares of common stock available for purchase pursuant to the ESPP are authorized but unissued shares of the Company's common stock, shares of the Company's common stock that the Company otherwise holds in treasury or shares of the Company's common stock that were purchased on the open market in arms' length transactions in accordance with applicable securities laws. Shares of the Company's common stock will be offered for purchase under the ESPP as determined by the Compensation Committee through a series of successive offerings that each have a term of 24 months and consist of four consecutive purchase periods of six months each. Prior to the commencement of any future offering under the ESPP, the Compensation Committee may determine that the current offering shall end, may commence a new offering on the first day after the end of such terminal purchase period (or any desired later date), and may decide that future offerings will consist of one or more consecutive purchase periods, each to be of such duration as determined by the Compensation Committee; however, no offering will exceed 27 months and no purchase period will exceed one year. Each employee of the Company who (1) is an employee on the first date of any offering under the ESPP, (2) is customarily scheduled to work for more than 20 hours per week and more than five months per calendar year, and (3) meets such other criteria as may be determined by the Compensation Committee (consistent with Section 423 of the Internal Revenue Code of 1986, as amended), is eligible to participate in the ESPP for each purchase period within such offering. The purchase price per share of the Company's common stock under the ESPP may not be less than, and will initially be equal to, the lesser of: (1) 85% of the fair market value per share of the Company's common stock on the first day of the offering, or (2) 85% of the fair market value per share of the Company's common stock on the date the purchase right is exercised, which will be the last day of the applicable purchase period.

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During the years ended December 31, 2015 and 2014, the Company recognized the following stock-based compensation expense:

|  | Year Ended<br>December 31, |         |
|--|----------------------------|---------|
|  | 2015                       | 2014    |
| Stock-based compensation expense by type of award: |                            |         |
| Stock options                                      | \$949,955                  | \$—     |
| Restricted stock and restricted stock units        | 1,608,049                  | 6,258   |
| Employee stock purchase plan                       | 43,444                     | —       |
| Total stock-based compensation expense included    |                            |         |
| in expenses  | \$2,601,448                | \$6,258 |
| Stock-based compensation expense by line item:     |                            |         |
| Research and development expenses                  | \$852,200                  | \$2,643 |
| General and administrative expenses                | 1,749,248                  | 3,615   |
| Total stock-based compensation expense included    |                            |         |
| in expenses  | \$2,601,448                | \$6,258 |

The following table sets forth the Company's unrecognized stock-based compensation expense, net of estimated forfeitures, by type of award and the weighted-average period over which that expense is expected to be recognized:

|   | As of December 31,<br>2015 | Unrecognized | Weighted-<br>average |
|---|----------------------------|--------------|----------------------|
| Type of award:                              | Expense, Net of            | Estimated    | Period               |
|   | Forfeitures                | (in years)   |                      |
| Stock options                               | \$1,409,808                | 2.61         |                      |
| Restricted stock and restricted stock units | \$2,442,289                | 2.58         |                      |

The following table is a summary of restricted shares granted during the year ended December 31, 2015:

|                               |             | Weighted-<br>Average<br>Grant<br>Date |
|-------------------------------|-------------|---------------------------------------|
|                               | Shares      | Fair Value                            |
| Unvested at December 31, 2014 | 2,018,752   | \$ 0.08                               |
| Granted                       | 462,090     | \$ 9.49                               |
| Vested                        | (702,165 )  | \$ 1.37                               |
| Forfeited                     | (105,000 )  | \$ 9.49                               |
| Repurchased                   | (1,005,714) | \$ 0.03                               |
| Unvested at December 31, 2015 | 667,963     | \$ 3.68                               |

The following table summarizes restricted stock units activity during the year ended December 31, 2015:

|                               |          | Weighted-<br>Average<br>Grant<br>Date |
|-------------------------------|----------|---------------------------------------|
|                               | Shares   | Value                                 |
| Unvested at December 31, 2014 | —        | \$ —                                  |
| Granted                       | 84,000   | \$ 8.18                               |
| Vested                        | —        | \$ —                                  |
| Forfeited                     | (8,250 ) | \$ 7.27                               |
| Repurchased                   | —        | \$ —                                  |
| Unvested December 31, 2015    | 75,750   | \$ 8.27                               |

The following table summarizes stock option activity during the year ended December 31, 2015:

|  | Shares    | Price   | Weighted-<br>Average<br>Remaining<br>Contractual<br>Exercise<br>Term (in<br>years) |
|--|-----------|---------|--|
| Options outstanding at December 31, 2014 | —         | \$ —    | —  |
| Granted                                  | 443,894   | \$ 8.49 | —  |
| Exercised                                | —         | \$ —    | —  |
| Cancelled                                | (78,500 ) | \$ 8.54 | —  |
| Options outstanding at December 31, 2015 | 365,394   | \$ 8.48 | 9.39   |
| Options exercisable at December 31, 2015 | 40,250    | \$ 9.49 | 9.35   |

Compensation expense for stock options granted to employees is based on the estimated grant date fair value and is recognized ratably over the vesting period of the applicable option. The estimated per share weighted average fair value of stock options granted to employees during the year ended December 31, 2015 was \$6.26. The options outstanding and exercisable at December 31, 2015 had no intrinsic value in the aggregate.

As stock-based compensation expense recognized is based on options ultimately expected to vest, the fair value of each employee option grant during the year ended December 31, 2015 was estimated on the date of grant using the Black-Scholes option pricing model with the following weighted average assumptions:

|                          | Year<br>Ended<br>December<br>31, 2015 |   |
|--------------------------|---------------------------------------|---|
| Expected volatility      | 85.2                                  | % |
| Expected term (in years) | 6.49                                  |   |
| Risk-free interest rate  | 1.81                                  | % |
| Expected dividend yield  | 0                                     | % |

**Expected Volatility.** The expected volatility rate used to value stock option grants is based on volatilities of a peer group of similar companies whose share prices are publicly available. The peer group was developed based on companies in the pharmaceutical and biotechnology industry in a similar stage of development to the Company.

**Expected Term.** The Company elected to utilize the “simplified” method for “plain vanilla” options to value stock option grants. Under this approach, the weighted-average expected life is presumed to be the average of the vesting term and

the contractual term of the option.

**Risk-free Interest Rate.** The risk-free interest rate assumption was based on zero-coupon U.S. Treasury instruments that had terms consistent with the expected term of the Company's stock option grants.

**Expected Dividend Yield.** The Company has never declared or paid any cash dividends and does not presently plan to pay cash dividends in the foreseeable future.

Forfeitures are estimated at the time of grant and revised, if necessary, in subsequent periods if actual forfeitures differ from estimates. The Company estimates forfeitures based on its historical experience. Groups of employees that have similar historical forfeiture behavior are considered separately for expense recognition.

Since the Company had a net operating loss carryforward as of December 31, 2015, no excess tax benefits for the tax deductions related to stock-based awards were recognized in the Statements of Operations.

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## Common Stock Reserved for Future Issuance

Common stock reserved for future issuance as of December 31, 2015 and 2014 is as follows:

| Common Stock Reserved for Future Issuance                 | December 31, |         |
|---|--------------|---------|
|   | 2015         | 2014    |
| Common stock warrants                                     | 82,500       | —       |
| Restricted stock units                                    | 75,750       | —       |
| Common stock options                                      | 365,394      | —       |
| Available for grant under the 2014 Plan                   | 773,629      | —       |
| Available for issuance under Employee Stock Purchase Plan | 452,620      | —       |
| Shares issuable upon conversion of Ligand debt            | 670,902      | 695,733 |
|   | 2,420,795    | 695,733 |

## 9. Representative's Warrant

Upon the closing of the IPO, on May 4, 2015, the Company issued to the representative of the underwriters as additional compensation a warrant to purchase the aggregate of 82,500 shares of the Company's common stock. The warrant is exercisable for cash or on a cashless basis at a per share exercise price equal to \$10.00 commencing on April 28, 2016, one year following the date of the prospectus filed with the SEC relating to the IPO, and expiring on April 28, 2020. In addition, the warrant provides for registration rights upon request, under certain circumstances. The piggyback registration right provided in connection with the warrant will terminate on April 28, 2022.

## 10. Income Taxes

The reconciliations of the U.S. federal statutory tax rate to the effective income tax rate for the years ended December 31, 2015 and 2014 are as follows:

|   | December 31, |        |
|---|--------------|--------|
|   | 2015         | 2014   |
| Tax provision at U.S. Federal statutory rates | 34 %         | 34 %   |
| State income taxes net of federal benefit     | 8 %          | 8 %    |
| Non-deductible permanent items                | (2 )%        | (1 )%  |
| Stock options                                 | —            | —      |
| Other   | —            | —      |
| Change in valuation allowance                 | (40 )%       | (41 )% |
| Effective income tax rate                     | —            | —      |

Deferred income taxes reflect the net tax effect of temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. Significant components of the Company's deferred taxes as of December 31, 2015 and 2014 are as follows:

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|                                  | December 31, |              |
|----------------------------------|--------------|--------------|
|                                  | 2015         | 2014         |
| <b>Deferred tax assets:</b>      |              |              |
| Accrued liabilities              | \$48,982     | \$—          |
| Intangible assets                | 14,009,497   | 7,993,293    |
| Net operating loss carryforwards | 2,430,249    | 548,247      |
| Share-based compensation         | 986,534      | 6,306        |
| Share conversion feature         | 243,759      | —            |
| Other                            | 3,510        | 530          |
| Total deferred tax assets        | 17,722,531   | 8,548,376    |
| Valuation Allowance              | (17,722,531) | (8,403,620)  |
| Net deferred tax assets          | \$—          | \$144,756    |
| <b>Deferred tax liabilities:</b> |              |              |
| Debt conversion feature          | \$—          | \$(144,756 ) |
| Total:                           | \$—          | \$—          |

A valuation allowance of \$17,722,531 and \$8,403,620 at December 31, 2015 and December 31, 2014, respectively, has been recorded to offset net deferred tax assets, as the Company is unable to conclude that it is more likely than not that such deferred tax assets will be realized.

At December 31, 2015, the Company had federal and state net operating loss carryforwards of approximately \$5,890,000 and \$6,629,000, respectively. The federal and state net operating loss carryforwards will begin to expire in 2032. The Company's ability to utilize its federal net operating loss carryforwards may be limited under Section 382 of the Internal Revenue Code of 1986, as amended (the "Code"). Specifically, this limitation may arise in the event of an "ownership change," which is defined by Section 382 of the Code as a cumulative change in ownership of the Company of more than 50% within a three-year period. If the Company undergoes one or more ownership changes in connection with any future transactions in its stock, the Company's ability to utilize net operating loss carryforwards to offset federal taxable income, if any, could potentially result in increased future tax liability to the Company. An ownership change under Section 382 of the Code occurred during the year ended December 31, 2015 in connection with the Company's initial public offering. A portion of the net operating loss carryforwards are subject to an annual limitation.

The Company is subject to U.S. federal income tax as well as income tax in various state jurisdictions. The Company is currently open to audit under the statute of limitations by the Internal Revenue Service and various state agencies for the years ended December 31, 2012 through December 31, 2015.

The differences between the Company's effective income tax rate and the statutory federal rate for the year ended December 31, 2015 and the year ended December 31, 2014 relate primarily to losses incurred for which no tax benefit was recognized, due to the uncertainty of realization. The ultimate realization of deferred tax assets is dependent upon the generation of future taxable income during the period in which those temporary differences become deductible. The Company considers projected future taxable income and tax planning strategies in making this assessment. At each of December 31, 2015 and December 31, 2014, the Company provided a full valuation allowance against its deferred tax assets due to uncertainty surrounding the realization of those assets as a result of historical taxable net losses.

The Company has reviewed its operations and has not identified any material uncertain tax positions. As a result, there is no liability for uncertain tax positions in the income tax provision as of December 31, 2015 or December 31, 2014.

#### 11. Related Party Transactions

In May 2014, the Company entered into the Master License Agreement with Ligand, pursuant to which, among other things, Ligand granted the Company an exclusive worldwide license to certain clinical and preclinical programs. See Note 4 for more information related to this agreement. In connection with entering into the Master License Agreement, the Company also entered into a Loan and Security Agreement (see Note 6 and Note 13) and a sublease and services agreement (See Note 4). As Ligand owns 49.4% of the Company's outstanding shares as of December 31, 2015, the Company considers Ligand to be a related party.

## 12. Commitments and Contingencies

In May 2014, the Company entered into a master license agreement with the licensor that included a license to the products covered by the option. See Note 4 for a description of the terms of the Master License Agreement. As noted in Note 4, in connection with the Master License Agreement, the Company also entered into a Sublease Agreement with Ligand, pursuant to which the Company leased approximately 5,851 square feet of office space from Ligand for the period from May 21, 2014 through December 31, 2014. Under the terms of the Sublease Agreement, the Company was required to make minimum lease payments of approximately \$167,000.

On July 7, 2015, the Company entered into a Sublease (the “Sublease”) for approximately 7,049 rentable square feet of space located at 12340 El Camino Real, Suite 250, San Diego, California 92130. Monthly base rent payments due under the Sublease are \$19,737, subject to annual increases of 3.0% during the term of the Sublease.

Rent expense was \$142,288 and \$186,774 for the years ended December 31, 2015 and 2014, respectively.

The Company is subject to charges for common area maintenance and other costs pursuant to the Sublease, and the Sublease provides for abatement of rent during certain periods and escalating rent payments throughout the term of the Sublease. Rent expense is being recorded on straight line basis over the life of the Sublease and the difference between the rent expense and rent paid is being recorded as deferred rent.

Future minimum payments pursuant to the Sublease are as follows:

| Year Ending December 31:     |           |
|------------------------------|-----------|
| 2016                         | 180,004   |
| 2017                         | 247,611   |
| 2018                         | 190,337   |
| Total minimum lease payments | \$617,952 |

## 13. Subsequent Events

The Company evaluated subsequent events through March 8, 2016, the date of the filing of this Annual Report on Form 10-K with the SEC, to ensure that this filing includes appropriate disclosure of events both recognized in the financial statements as of December 31, 2015, and events which occurred subsequent to December 31, 2015 but were not recognized in the financial statements. The Company has determined that there were no subsequent events which required recognition, adjustment to or disclosure in the financial statements, except as follows:

On January 22, 2016, the Company entered into a Second Amendment to Loan and Security Agreement (the “Second Loan Amendment”) with Ligand, which amends the Loan and Security Agreement, as amended by the Loan Amendment (the “Original Loan Agreement”). The Second Loan Amendment amended the Original Loan Agreement (as so amended by the Second Loan Amendment, the “Amended Loan Agreement”) to, among other things, (1) extend the maturity date of the loans under the Ligand Note from May 21, 2016 to May 21, 2017 (the “Maturity Date”), (2) reduce the annual interest rate on the principal amount outstanding under the Ligand Note from 5.0% to 2.5%, and (3) extend Ligand’s lock-up period by one year such that Ligand may not, directly or indirectly, sell or otherwise transfer or dispose of any Company securities prior to January 23, 2017. The amount payable by the Company under the Amended Loan Agreement remains equal to 200% of the original principal amount of the loans under the Ligand Note and of all accrued and previously unpaid interest thereon.

Additionally, the Amended Loan Agreement provides that, upon the consummation of the Company's first bona fide capital financing transaction occurring subsequent to January 22, 2016, but prior to the Maturity Date, with aggregate net proceeds to the Company of at least \$2.0 million (the "Next Financing"), the Company will be required to repay \$1.5 million of the Ligand Note obligation to Ligand (the "Next Financing Payment"), with at least \$0.3 million of the Next Financing Payment to be paid in cash, subject to the Company's sole and absolute discretion to pay a greater amount in cash, and the remaining amount of the Next Financing Payment that will not be paid in cash (the "Balance") to be paid in the form of such number of shares of the Company's equity securities that are issued in the Next Financing as is equal to the quotient obtained by dividing the Balance by the lesser of (1) the lowest price per share paid by investors in the Next Financing (the "Financing Price"), and (2) \$8.00 (subject to adjustment for stock dividends, splits, combinations or similar transactions). Notwithstanding the foregoing, the number of shares that the Company may issue to Ligand will be reduced to the extent the issuance of shares would increase Ligand's beneficial ownership of the Company's common stock to greater than 49.9%, and any remaining amount of the Balance would have to be paid by the Company in cash (the "Share Cap").

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Under the terms of the Amended Loan Agreement, following the consummation of the Next Financing, the Company may elect to repay any portion of the outstanding principal under the Ligand Note, plus accrued and unpaid interest thereon, by delivering a notice to Ligand (the “Additional Repayment Notice”), specifying the amount that the Company wishes to repay (the “Additional Payment Amount”). Ligand will then have five days to elect to receive the Additional Payment Amount in cash, shares of the Company’s common stock or a combination of cash and shares of the Company’s common stock. If Ligand does not make an election within such five-day period, the Company will have the right, at its sole election and discretion, to elect the form of the Additional Payment Amount, subject to the Share Cap. To the extent that any portion of an Additional Payment Amount will be paid in shares of the Company’s common stock, the Amended Loan Agreement provides that the number of shares issuable will be equal to the quotient obtained by dividing the portion of the Additional Payment Amount that will be paid in shares by the lesser of (1) (a) if the Company delivers the Additional Repayment Notice within 180 days of the closing of the Next Financing, the Financing Price, or (b) if the Company delivers the Additional Repayment Notice 180 days or more after the closing of the Next Financing, the volume weighted average closing price of the Company’s common stock for the 30 days prior to the date the Company delivers the Additional Repayment Notice, and (2) \$8.00 (subject to adjustment for stock dividends, splits, combinations or similar transactions).

The Amended Loan Agreement also provides that, on or after the Maturity Date, Ligand may demand payment of the remaining amount payable under the Ligand Note (the “Remaining Balance”). In addition, the Company is permitted to, at its sole election and discretion, repay to Ligand the Remaining Balance solely in cash. However, if the Company does not elect to repay the Remaining Balance solely in cash, Ligand can set the form of payment as cash, shares of the Company’s common stock or both. To the extent that any portion of the Remaining Balance will be paid in shares of the Company’s common stock, the number of shares issuable will be equal to the quotient obtained by dividing the portion of the Remaining Balance that will be paid in shares by the lesser of (a) the volume weighted average closing price of the Company’s common stock for the 30 days prior to the date of Ligand’s demand for repayment or the date of the Company’s prepayment of the Remaining Balance in full, as applicable, and (b) \$8.00 (subject to adjustment for stock dividends, splits, combinations or similar transactions).

On January 22, 2016, the Company also entered into a First Amendment to Registration Rights Agreement (the “Registration Rights Agreement Amendment”) with Ligand, which amends the Registration Rights Agreement. The Registration Rights Agreement Amendment extends the deadline by which the Company must file with the SEC a Registration Statement on Form S-1 (the “Registration Statement”) covering the resale of certain Company securities held by Ligand, including the shares of the Company’s common stock issuable to Ligand under the Amended Loan Agreement, by one year from January 23, 2016 to January 23, 2017, and extends the applicable deadline for seeking to have such Registration Statement declared effective by the SEC by one year.

