

Viking Therapeutics, Inc.  
Form 10-K  
March 07, 2018

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, 2017

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	(I.R.S. Employer
incorporation or organization)	Identification No.)
12340 El Camino Real, Suite 250	
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, a smaller reporting company or emerging growth company. See the definition of "large accelerated filer", "accelerated filer", "smaller reporting company" and "emerging growth 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

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

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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, 2017 (the last trading day of the registrant's second fiscal quarter of 2017), was

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\$21,405,993. 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 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 28, 2018 was 50,898,802.

DOCUMENTS INCORPORATED BY REFERENCE

None.

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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. Our lead clinical program, VK5211, is an orally available, 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 bone mineral density, or 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 believe that VK5211 may also have potential benefits to patients suffering from muscle loss in other settings, such as joint replacements or muscle wasting disorders.

In October 2015, we commenced enrollment in a Phase 2 proof-of-concept clinical trial in 108 patients recovering from non-elective hip fracture surgery. In November 2017, we announced positive top-line results from our 12-week, Phase 2 clinical trial of VK5211 in patients who recently suffered a hip fracture. Top-line data showed that the trial achieved its primary endpoint, demonstrating statistically significant, dose dependent increases in lean body mass, less head, following treatment with VK5211 as compared to placebo. The study also achieved certain secondary endpoints, demonstrating statistically significant increases in appendicular lean body mass and total lean body mass for all doses of VK5211, compared to placebo. VK5211 demonstrated encouraging safety and tolerability in this study, with no drug-related serious adverse events, or SAEs, reported. See the discussion under “VK5211: A Selective Androgen Receptor Modulator (SARM) for Hip Fracture” below for more details on these study results. We expect to receive follow-up data related to this study in the first half of 2018 and to then engage in further discussions with the U.S. Food and Drug Administration, or the FDA, regarding the potential further clinical development of VK5211 in hip fracture as well as other potential acute use settings.

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 an established animal model of osteoporosis, treatment with VK5211 resulted in significant increases in BMD and bone strength.

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 clinical program is VK2809, an orally available, tissue and receptor-subtype selective agonist of the thyroid hormone receptor beta, or TR $\beta$ , that is in a Phase 2 clinical trial 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 are currently conducting a Phase 2 clinical trial of VK2809 in approximately 60 patients with hypercholesterolemia and fatty liver disease and expect to report initial results from this Phase 2 clinical trial in the second half of 2018.

In October 2017, we announced positive final results from an eight-week study of VK2809 in an in vivo model of non-alcoholic steatohepatitis, or NASH. See the discussion of “Novel Selective Thyroid Hormone Receptor- $\beta$ , or TR $\beta$ , Agonists for Metabolic Disorders and Adrenoleukodystrophy” below for more details on these study results.

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 low-density lipoprotein cholesterol, or 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.

In the U.S., the number of patients with dyslipidemia was estimated to be greater than 100 million in 2013. In the U.S., 28.9% 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 3% to 12% of American adults, or 6.0 to 25.0 million people. As a result, we believe the global market opportunity for VK2809 in hypercholesterolemia or NASH exceeds \$1.0 billion.

In February 2017, we announced that we are commencing efforts to utilize VK2809 to potentially help patients who suffer from Glycogen Storage Disease type Ia, or GSD Ia. GSD Ia is a rare, genetic disease caused by a deficiency of glucose-6-phosphatase, or G6PC, an enzyme responsible for the liver's production of free glucose from glycogen and gluconeogenesis. Approximately 2,000 patients in the U.S. suffer from GSD Ia. We have conducted a proof-of-concept study utilizing VK2809 in an in vivo model of GSD Ia. Data demonstrated that treatment with VK2809 led to statistically significant reductions in key metabolic markers of GSD Ia. VK2809's potential to rapidly reduce hepatic triglyceride levels, as demonstrated in this initial evaluation in a GSD Ia model, provides support for the continued investigation of the compound in this indication. We expect to initiate a Phase 1 human proof-of-concept clinical trial to evaluate VK2809 in patients with GSD Ia in the first half of 2018.

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 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 data suggest that VK0214 stimulates ABCD2 expression in an in vitro model and reduces VLCFA levels in an in vivo model of X-ALD. We received orphan drug designation from the FDA for VK0214 for the treatment X-ALD in December 2016. Pending completion of certain toxicology studies, we expect to file an IND to conduct a proof-of-concept study in patients with X-ALD in the second half of 2018.

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. 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 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 Product Pipeline

The following table highlights our product pipeline:

Key: SARM, selective androgen receptor modulator; TR $\beta$ , thyroid receptor beta; NASH, nonalcoholic steatohepatitis, GSD Ia, Glycogen Storage Disease type Ia, X-ALD, X-linked adrenoleukodystrophy.

We also have three additional programs targeting metabolic diseases and anemia. The most advanced 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, or EPO, receptor, or EPOR, agonists for the potential treatment of anemia, and on developing tissue-selective inhibitors of diacylglycerol acyltransferase-1, or DGAT-1, for the potential treatment of obesity and dyslipidemia.

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.

In November 2017, we announced positive top-line results from our 12-week, Phase 2 clinical trial of VK5211 in patients who recently suffered a hip fracture. Top-line data showed that the trial achieved its primary endpoint, demonstrating statistically significant, dose dependent increases in lean body mass, less head, following treatment with VK5211 as compared to placebo. The study also achieved certain secondary endpoints, demonstrating statistically significant increases in appendicular lean body mass and total lean body mass for all doses of VK5211, compared to placebo. VK5211 demonstrated encouraging safety and tolerability in this study, with no drug-related SAEs reported.

The Phase 2 clinical trial was a randomized, double-blind, placebo-controlled, parallel group, international study designed to evaluate the efficacy, safety and tolerability of VK5211 in patients recovering from hip fracture surgery. A total of 108 patients were randomized to receive once-daily VK5211 doses of 0.5 mg, 1.0 mg, 2.0 mg, or placebo for 12 weeks. Top-line results include:

- All doses of VK5211 demonstrated statistically significant increases in total lean body mass, less head, the study's primary endpoint. Placebo-adjusted increases in lean body mass were 4.8% at 0.5 mg ( $p < 0.005$ ), 7.2% at 1.0 mg ( $p < 0.001$ ), and 9.1% at 2.0 mg ( $p < 0.001$ ). These corresponded to placebo-adjusted increases of 1.6 kg at 0.5 mg ( $p < 0.005$ ), 2.5 kg at 1.0 mg ( $p < 0.001$ ), and 3.1 kg at 2.0 mg ( $p < 0.001$ ).
- The proportion of patients experiencing at least a 5% increase in total lean body mass, less head, were 19% with placebo, 61% at 0.5 mg, 65% at 1.0 mg, and 75% at 2.0 mg ( $p < 0.01$  for each). The proportion of patients demonstrating at least a 2.0 kg gain in total lean body mass, less head, were 14% with placebo, 57% at 0.5 mg, 65% at 1.0 mg, and 81% at 2.0 mg ( $p < 0.01$  for each).
- All doses of VK5211 produced statistically significant increases in appendicular lean body mass, a secondary efficacy endpoint. Placebo-adjusted increases in appendicular lean body mass were 6.1% at 0.5 mg ( $p < 0.01$ ), 9.0% at 1.0 mg ( $p < 0.001$ ), and 10.2% at 2.0 mg ( $p < 0.001$ ). These corresponded to placebo-adjusted increases of 0.8 kg at 0.5 mg ( $p < 0.05$ ), 1.3 kg at 1.0 mg ( $p < 0.001$ ), and 1.4 kg at 2.0 mg ( $p < 0.001$ ).
- All doses of VK5211 produced statistically significant increases in total lean body mass, including head, a secondary efficacy endpoint. Increases in total lean body mass were 6.3% ( $p < 0.005$ ), 8.2% ( $p < 0.001$ ), and 9.9% ( $p < 0.001$ ) from baseline, corresponding to placebo-adjusted increases of 4.7% at 0.5 mg ( $p < 0.005$ ), 6.8% at 1.0 mg ( $p < 0.001$ ), and 8.3% at 2.0 mg ( $p < 0.001$ ). These corresponded to placebo-adjusted increases of 1.7 kg at 0.5 mg ( $p < 0.005$ ), 2.6 kg at 1.0 mg ( $p < 0.001$ ), and 3.1 kg at 2.0 mg ( $p < 0.001$ ).

- Patients receiving VK5211 demonstrated numerical improvements in certain exploratory assessments of functional performance, including the 6-minute walk test and short physical performance battery, compared with placebo. These endpoints were not powered for significance. Further evaluation of exploratory functional endpoints is underway.
- There were no significant differences in the rates of adverse events reported among patients receiving VK5211 compared with placebo. There were no dose-related differences in reported adverse events among various VK5211 treatment groups. No drug-related SAEs were observed in patients receiving VK5211.

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We expect to receive follow-up data for this Phase 2 clinical trial in the first half of 2018. We then expect to engage in further discussions with the FDA regarding the potential further clinical development of VK5211 in hip fracture as well as other potential acute use settings.

VK5211 has also 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.

## 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.

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## Hip Fracture and Other Muscle Wasting Market Opportunities

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.

### 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 addition to the Phase 2 clinical trial described above, VK5211 has been evaluated in three Phase 1 clinical trials. In these 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 high-density lipoprotein, or 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.

#### Development Plans

We expect to develop VK5211 for potential treatment of a wide range of diseases and disorders in both men and women. We expect to receive follow-up data for our Phase 2 clinical trial in the first half of 2018. We then expect to engage in further discussions with the FDA regarding the potential further clinical development of VK5211 in hip fracture as well as other potential acute use settings.

#### Novel Selective Thyroid Hormone Receptor- $\beta$ , or TR $\beta$ , Agonists for Metabolic Disorders and Adrenoleukodystrophy

##### Product Summary

VK2809 and VK0214 are novel, orally available, selective TR $\beta$  agonists in development for metabolic 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 thyroid hormone receptor alpha, or 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 and GSD Ia settings.

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 are conducting a Phase 2 clinical trial to evaluate both potential indications. We are targeting 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 commenced the study in the second half of 2016 and expect to report initial results from this Phase 2 clinical trial in the second half of 2018. Upon conclusion, we expect to be in a position to move forward in either hypercholesterolemia or NASH.

In addition, in February 2017, we announced that we are commencing efforts to utilize VK2809 to potentially help patients who suffer from GSD Ia. GSD Ia is a rare, genetic disease caused by a deficiency of G6PC, an enzyme

responsible for the liver's production of free glucose from glycogen and gluconeogenesis. The disease, for which there is no approved treatment, results in an excess accumulation of glycogen and lipids in the liver, potentially leading to hepatosteatosis, liver failure, development of hepatic adenomas and hepatocellular carcinoma. Increased triglyceride production and elevated hepatic triglyceride levels are characteristic of GSD Ia and associated with many manifestations of the disease. GSD Ia is estimated to occur in approximately 1 in every 50,000 – 100,000 births in the United States. As manifestations of the disease begin to present themselves at birth, a sizeable portion of GSD Ia patients are children. We conducted a proof-of-concept study utilizing VK2809 in an in vivo model of GSD Ia. Data demonstrated that treatment with VK2809 led to statistically significant reductions in key metabolic markers of GSD Ia. VK2809's potential to rapidly reduce hepatic triglyceride levels, as demonstrated in this initial evaluation in a GSD Ia model, provides support for the continued investigation of the compound in this indication. We expect to initiate a Phase 1 human proof-of-concept clinical trial to evaluate VK2809 in patients with GSD Ia in the first half of 2018.

#### VK2809 Summary

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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 are currently administrating and 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 patients was similar to placebo-treated patients. 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, or Lp(a), and apolipoprotein, or 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.

#### 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 and GSD Ia. 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 metabolic disorders, including dyslipidemia, hypercholesterolemia, NASH and GSD Ia.

#### In Vivo Study Results

In October 2017, we announced positive final results from an eight-week study of VK2809 in an in vivo model of NASH. Treatment with VK2809 resulted in: (1) statistically significant reductions in several key measures of steatosis, including liver triglyceride content and total liver lipid content, (2) fibrotic activity, including total liver fibrosis, type I collagen and hydroxyproline, relative to

vehicle controls, and (3) statistically significant changes in the expression of key genes associated with NASH development and progression, relative to vehicle control, suggesting improved lipid and cholesterol metabolism, improved lipid metabolism and insulin sensitivity and reduced fibrotic activity.

#### Hypercholesterolemia and NASH

We are currently conducting a Phase 2 clinical trial to evaluate patients with both cholesterolemia and fatty liver disease. We are targeting patients who have elevated cholesterol, elevated triglycerides, and non-alcoholic fatty liver disease. The primary endpoint of this trial will assess changes in LDL-C, with exploratory endpoints evaluating changes in liver fat content and certain biomarkers relevant to the assessment of NASH. We commenced the clinical trial in the second half of 2016 and expect to report the initial results from this Phase 2 the clinical trial in the second half of 2018. Upon conclusion, we expect to be in a position to move forward in either hypercholesterolemia or NASH.

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.

#### Glycogen Storage Disease type Ia

We are also developing VK2809 for potential treatment of GSD Ia. GSD Ia is a rare, genetic disease caused by a deficiency of G6PC, an enzyme responsible for the liver's production of free glucose from glycogen and gluconeogenesis. The disease, for which there is no approved treatment, results in an excess accumulation of glycogen and lipids in the liver, potentially leading to hepatosteatosis, liver failure, development of hepatic adenomas and hepatocellular carcinoma. Increased triglyceride production and elevated hepatic triglyceride levels are characteristic of GSD Ia and associated with many manifestations of the disease. GSD Ia is estimated to occur in approximately 1 in every 50,000 – 100,000 births in the United States. As manifestations of the disease begin to present themselves at birth, a sizeable portion of GSD Ia patients are children. We conducted a proof-of-concept study utilizing VK2809 in an in vivo model of GSD Ia. Data demonstrated that treatment with VK2809 led to statistically significant reductions in key metabolic markers of GSD Ia. Mean liver triglyceride content was reduced by more than 60% in VK2809-treated animals relative to vehicle-treated control animals, while average liver weight was reduced by more than 30% as compared to control animals. We expect to initiate a Phase 1 human proof-of-concept clinical trial to evaluate VK2809 in patients with GSD Ia in the first half of 2018.

#### X-linked Adrenoleukodystrophy

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. X-ALD is caused by mutations in a peroxisomal transporter of VLCFA known as ABCD1. As a result, transporter function is impaired and patients are unable to efficiently metabolize VLCFA. TRβ 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 data suggest that VK0214 stimulates ABCD2 expression in an in vitro model and reduces VLCFA levels in an in vivo model of X-ALD. Pending completion of certain toxicology

studies, we expect to file an IND to conduct a proof-of-concept study in patients with X-ALD in the second half of 2018.

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. 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 (CALD):** The most severe form of X-ALD is cerebral adrenoleukodystrophy, or 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

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by CALD typically experience rapid degeneration into vegetative state within 3 to 5 years, often resulting in death within 10 years of diagnosis.

- Adrenomyeloneuropathy (AMN): Adrenomyeloneuropathy, or 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.

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.

### 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 EPOR agonists for the potential treatment of anemia, and on developing 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.

#### EPO Receptor (EPOR) Agonist Program

We are developing small molecule agonists of the 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 \$7.2 billion in 2015. 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 metabolic 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 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 that 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, in 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, bone mineral density 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 Helsinn Group, Morphosys AG, Bristol-Myers Squibb, Pfizer and Eli Lilly and Company. In addition, nutritional and growth hormone-based therapies are sometimes used in patients experiencing muscle wasting.

## VK2809

While no therapies are currently approved for the treatment of non-alcoholic steatohepatitis, we are aware of numerous development-stage programs targeting this disease, including obeticholic acid from Intercept Pharmaceuticals, Inc., GFT505 from Genfit SA, aramchol from Galmed Pharmaceuticals Ltd., simtuzumab and selonsertib from Gilead Sciences, Inc., emricisan from Conatus Pharmaceuticals Inc., GR-MD-02 from Galectin Therapeutics Inc., cenicriviroc from Allergan plc (via acquisition of Tobira Therapeutics, Inc.), LJN452 from Novartis Pharmaceuticals Corporation, MSDC-0602 from Cirius Therapeutics, Inc. (formerly Octeta Therapeutics, LLC), BMS986036 from Bristol-Myers Squibb and MGL-3196 from Madrigal Pharmaceuticals, Inc. In addition, we are aware of active programs at Nitto Denko Corporation, NGM Biopharmaceuticals, Inc., Enanta Pharmaceuticals, Inc., Durect Corporation, NuSirt Biopharma, Inc., MiNA Therapeutics and AstraZeneca plc.

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.

In the U.S., there are currently no marketed pharmacologic therapies for the treatment of GSD Ia. We are aware of one competitor, Ultragenyx Pharmaceutical Inc., which is planning to commence a Phase1/2 study in the first half of 2018 utilizing a gene therapy approach with DTX401.

#### 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, bluebird bio, Inc. and NeuroVia, Inc., which may be competitive with VK0214, if approved.

#### Manufacturing and Supply

We do not have any manufacturing facilities and do not intend to develop any manufacturing capabilities. We believe that we have sufficient supplies of drug substance to allow for completion of our ongoing Phase 2 clinical trials. Bulk active pharmaceutical ingredient, or API, and certain dosage forms are currently in storage in compliance with good manufacturing practices, or 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 the European Medicines Agency, or 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 and obtaining the worldwide rights to certain technology, including VK5211, VK2809 and VK0214, and conducting certain clinical trials and preclinical studies related to these programs. 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.

## 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, April 8, 2015 and March 21, 2016, 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 Compounds, TR $\beta$  Compounds, FBPase Compounds, EPOR Compounds 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 our initial public offering of our common stock, or 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 TRB 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, January 22, 2016 and May 8, 2017, 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 Ligand Note, or May 21, 2018, 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 were 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.

On April 13, 2016, we completed an underwritten public offering of our common stock and warrants to purchase shares of our common stock, or the Offering, resulting in net proceeds of \$7,754,286, after deducting underwriting discounts, commissions and other offering expenses of \$1,631,964. The Offering constituted a Next Financing under the Loan and Security Agreement; therefore,

we repaid \$1,500,000 of the Ligand Note with \$300,000 paid in cash and \$1,200,000 paid in the form of 960,000 shares of our common stock, or the Ligand Shares, and a warrant to purchase up to 960,000 shares of our common stock, or the Ligand Warrant. The Ligand Warrant has an exercise price of \$1.50 per share of common stock, was immediately exercisable upon issuance and will expire on April 13, 2021.

In addition, following the Offering, we may now 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.

On May 8, 2017, we entered into an amendment to the Loan and Security Agreement, which, (1) extended the maturity date of the loans from May 21, 2017 to May 21, 2018, and (2) provided that we were required to make a repayment in the amount of \$200,000, or the Required Repayment. We made the Required Repayment on July 13, 2017. The Required Repayment was applied, first, to accrued and unpaid interest on the loans and, second, to the unpaid principal amount of the Loans. Each \$1.00 of value of the Required Repayment reduced the amount of accrued and unpaid interest and then unpaid principal amount on the Loans by \$0.50. As a result of this amendment, we recorded a debt modification adjustment of \$1.0 million to the debt conversion feature liability primarily due to the longer expected term, offset with an adjustment recorded to the Ligand Note discount.

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 prepayment 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.

## 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 Securities and Exchange Commission, or 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 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 our former Chief Operating Officer 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, on February 14, 2017, we filed a Registration Statement on Form S-3 under the Securities Act of 1933, as amended, or the Securities Act, covering 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 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 ensuring 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. Pursuant to the Registration Rights Agreement, on February 14, 2017, we filed a Registration Statement on Form S-3 under the Securities Act covering the resale of the full amount of the Registrable Securities.

#### 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.

## 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.

Pursuant to the Representative's Warrant, on February 14, 2017, we filed a Registration Statement on Form S-3 under the Securities covering the resale of the full amount of the Registrable Warrant Shares.

## 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.

## 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 some of 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 \$2.0 million for fiscal year 2017) 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 is also 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.

#### Orphan Designation and Exclusivity

The FDA may grant orphan drug designation to drugs intended to treat a rare disease or condition that affects fewer than 200,000 individuals in the United States, or if it affects more than 200,000 individuals in the United States and there is no reasonable expectation that the cost of developing and making the drug for this type of disease or condition will be recovered from sales in the United States.

Orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical study costs, tax advantages, and user-fee waivers. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. In addition, the first NDA or Biologics License Application, or BLA, applicant to receive orphan drug designation for a particular drug is entitled to orphan drug exclusivity, which means the FDA may not approve any other application to market the same drug for the same indication for a period of seven years in the United States, except in limited circumstances. Orphan drug exclusivity does not prevent the FDA from approving a different drug for the same disease or condition, or the same drug for a different disease or condition.

#### 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.

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, as amended by the Health Care and Education Reconciliation Act of 2010, collectively 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, 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. Further, there have been recent public announcements by members of the U.S. Congress, President Trump and his administration regarding their plans to repeal and replace the PPACA. For example, on December 22, 2017, President Trump signed into law the Tax Cuts and Jobs Act of 2017, which, among other things, eliminated the individual mandate requiring most Americans (other than those who qualify for a hardship exemption) to carry a minimum level of health coverage, effective January 1, 2019. We cannot predict the ultimate form or timing of any repeal or replacement of the PPACA or the effect such a repeal or replacement would have on our business.

#### 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

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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 February 28, 2018, are as follows:

Subject Matter/Compounds	# Pending Applications	# Issued Patents	Geographical Scope	Nominal Patent Term
VK5211 (SARM)	7	13	U.S., Europe, Chile, Argentina, Brazil, Canada, China, India, Japan, Korea, Mexico, Taiwan, and Venezuela	2025-2028
Other SARM	5	33	U.S., Canada, India, Japan, Korea, Mexico, Australia, China, New Zealand, Argentina, Brazil, Europe, and Israel	2017-2026
TR $\beta$ agonists	8	4	U.S., Australia, Canada, and PCT	2025-2038
VK0612 (FBPase inhibitor)	1	13	U.S., China, Hong Kong, Israel, Korea, Mexico, India, New Zealand, and PCT	2019-2036
FBPase Inhibitor Combinations	0	12	U.S., China, Korea, Israel, Mexico, Portugal, New Zealand, and Russia	2019-2021
DGAT-1 Inhibitors	2	4	U.S. and Europe	2030
EPOR Inhibitors	5	9	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 \$13,741,186 and \$9,000,499 for the years ended December 31, 2017 and 2016, respectively.

### Employees

As of February 28, 2018, we had eleven full-time employees and three part-time employees, four of whom hold a Ph.D. or M.D. degree. All employees are engaged in research and development, 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 next stages 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, VK2809 and VK0612, which are currently in Phase 2 clinical development, and VK0214 and the erythropoietin receptor, or EPOR, and diacylglycerol acyltransferase-1, or 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. As of December 31, 2017, we had an accumulated deficit of \$80,854,932. 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 potential partnering opportunities and/or 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 partnering or 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 our master license agreement with Ligand, 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 metabolic disorders. Selective androgen receptor modulators, or 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.

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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 reported positive top-line results for a Phase 2 clinical trial for VK5211. We are also currently conducting a Phase 2 clinical trial and certain preclinical studies for VK2809 and certain preclinical studies for VK0214. 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 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.

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, 2017, we had cash and cash equivalents and investments totaling \$20,575,956. Although in February 2018, we raised \$63,250,000 in gross proceeds, before deducting underwriting discounts and commissions and other estimated offering expenses in an underwritten public offering of our common stock, 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:

the scope, rate of progress, results and cost of our clinical trials, preclinical studies and other related activities;

our ability to establish and maintain strategic collaborations, licensing or other arrangements and the financial terms of such arrangements;

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;

- 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.

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;

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• 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. 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;
- 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.

We have relied upon and plan 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.

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;
  - 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, are better tolerated, have a more favorable safety profile or are 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 for 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, in 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, bone mineral density 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 Helsinn Group, Morphosys AG, Bristol-Myers Squibb and Eli Lilly and Company. In addition, nutritional and growth hormone-based therapies are sometimes used in patients experiencing muscle wasting.

#### VK2809

While no therapies are currently approved for the treatment of non-alcoholic steatohepatitis, we are aware of numerous development-stage programs targeting this disease, including obeticholic acid from Intercept Pharmaceuticals, Inc., GFT505 from Genfit SA, aramchol from Galmed Pharmaceuticals Ltd., simtuzumab and selonsertib from Gilead Sciences, Inc., emricisan from Conatus Pharmaceuticals Inc., GR-MD-02 from Galectin Therapeutics Inc., cenicriviroc from Allergan plc (via acquisition of Tobira Therapeutics, Inc.), LJN452 from Novartis Pharmaceuticals Corporation, MSDC-0602 from Cirius Therapeutics, Inc. (formerly Octeta Therapeutics, LLC), BMS986036 from Bristol-Myers Squibb and MGL-3196 from Madrigal Pharmaceuticals, Inc. In addition, we are aware of active programs at Nitto Denko Corporation, NGM Biopharmaceuticals, Inc., Enanta Pharmaceuticals, Inc., Durect Corporation, NuSirt Biopharma, Inc., MiNA Therapeutics and AstraZeneca plc.

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 colesselevam 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.

In the U.S., there are currently no marketed pharmacologic therapies for the treatment of Glycogen Storage Disease type Ia. We are aware of one competitor, Ultragenyx Pharmaceutical Inc., which is planning to commence a Phase 1/2 study in the first half of 2018 utilizing a gene therapy approach with DTX401.

#### VK0214

In the U.S., there are currently no marketed therapies for the treatment of X-linked adrenoleukodystrophy, or X-ALD. Hematopoietic stem cell therapy has been used to treat the most severe form of X-ALD, cerebral adrenoleukodystrophy, or 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, adrenomyeloneuropathy, or 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 Vertex Pharmaceuticals, Inc., MedDay Pharmaceuticals SAS, Minoryx Therapeutics S.L. bluebird bio, Inc. and NeuroVia, Inc., which may be competitive with VK0214, if approved.

We, or any future collaborators, may not be able to obtain orphan drug designation or orphan drug exclusivity for our product candidates.

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the United States. While we received orphan drug designation from the FDA for VK0214 for the treatment X-ALD in December 2016 and plan to seek orphan drug designation from the FDA for VK2809 for the treatment of GSD Ia, we, or any future collaborators, may not be granted orphan drug designations for our product candidates in the U.S. or in other jurisdictions.

Even if we, or any future collaborators, obtain orphan drug designation for a product candidate, we, or they, may not be able to obtain orphan drug exclusivity for that product candidate. Generally, a product with orphan drug designation only becomes entitled to orphan drug exclusivity if it receives the first marketing approval for the indication for which it has such designation, in which case the FDA or the EMA will be precluded from approving another marketing application for the same drug for that indication for the applicable exclusivity period. The applicable exclusivity period is seven years in the United States and ten years in Europe. The European exclusivity period can be reduced to six years if a drug no longer meets the criteria for orphan drug designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be lost if the FDA or the EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition.

Even if we, or any future collaborators, obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because FDA has taken the position that, under certain circumstances, another drug with the same active moiety can be approved for the same condition. Specifically, the FDA's regulations provide that it can approve another drug with the same active moiety for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care.

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 GLP 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, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, collectively the PPACA, was enacted. The PPACA was 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.” 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.

There have been recent public announcements by members of the U.S. Congress, President Trump and his administration regarding their plans to repeal and replace the PPACA and Medicare. For example, on December 22, 2017, President Trump signed into law the Tax Cuts and Jobs Act of 2017, which, among other things, eliminated the individual mandate requiring most Americans (other than those who qualify for a hardship exemption) to carry a minimum level of health coverage, effective January 1, 2019. 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.

In Europe, the United Kingdom has indicated its intent to withdraw from the European Union in the future. A significant portion of the regulatory framework in the United Kingdom is derived from the regulations of the European Union, and the EMA is currently located in the United Kingdom. We cannot predict what consequences the withdrawal of the United Kingdom from the European Union, if it occurs, might have on the regulatory frameworks of the United Kingdom or the European Union, or on our future operations, if any, in these jurisdictions.

Changes in government funding for the FDA and other government agencies could hinder their ability to hire and retain key leadership and other personnel, properly administer drug innovation, or prevent our product candidates from being developed or commercialized, which could negatively impact our business, financial condition and results of operations.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including budget and funding levels, ability to hire and retain key personnel, and statutory, regulatory and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of other agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable.

In December 2016, the 21st Century Cures Act was signed into law. This new legislation is designed to advance medical innovation and empower the FDA with the authority to directly hire positions related to drug and device development and review. However, government proposals to reduce or eliminate budgetary deficits may include reduced allocations to the FDA and other related government agencies. These budgetary pressures may result in a reduced ability by the FDA to perform their respective roles; including the related impact to academic institutions and research laboratories whose funding is fully or partially dependent on both the level and timing of funding from government sources.

Disruptions at the FDA and other agencies may also slow the time necessary for our product candidates to be reviewed or approved by necessary government agencies, which could adversely affect our business, financial condition and results of operations.

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 December 31, 2017, we had twelve full-time employees, three part-time employees 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.

On May 21, 2014, we entered into a Loan and Security Agreement with Ligand, as amended, 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. As of February 28, 2018, \$1,916,508 in principal amount is outstanding under the loans. 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 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, one of our third party manufacturers is 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;
- 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.

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## 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, 2017, we owned or co-owned seven patent applications and did not own any patents. 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 most 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 for licensed patents,

pending patent applications and potential future patent applications and patents 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, as of December 31, 2017, we in-licensed seven patents in the U.S. and several other patents in certain foreign jurisdictions. As of December 31, 2017, for each of VK2809 and VK0214, we in-licensed one patent and one patent application in the U.S. and additional patents or patent applications in certain foreign jurisdictions, and owned or co-owned and in-licensed four PCT applications and two U.S. provisional applications. We also in-licensed one additional PCT application and one additional U.S. application directed to VK0214 as of December 31, 2017. For VK0612, as of December 31, 2017, we in-licensed two patents in the U.S. and several other patents in certain foreign jurisdictions, and own one PCT patent application. 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.

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:

- 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 who 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;

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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 apply for, 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 apply for, 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. previously 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, and many of the provisions became effective in March 2013. However, it may take the courts years to interpret the provisions of the Leahy-Smith Act, and the implementation of the statute 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 a number of licensed patents and 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 regarding 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, as of December 31, 2017, we had several licensed patents and several licensed patent applications and may have limited remedies if such patents are infringed or if we are compelled to grant a license to a third party, which could materially diminish the value of such 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;

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- 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.

If no active trading market for our common stock 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, 2017, our executive officers, directors and 5% or greater stockholders beneficially owned 26.3% 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, 2017, Ligand and its affiliates beneficially owned approximately 20.9% of our outstanding common stock. In addition to the above ownership, 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 neither an "emerging growth company" nor a "smaller reporting 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 may not meet the standards required by Section 404 of the Sarbanes-Oxley Act, 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. Commencing with our Annual Report on Form 10-K for the fiscal year ending December 31, 2016, our management was required and will continue to be

required to report on the effectiveness of our internal control over financial reporting. However, 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, exercises and sales of outstanding warrants 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, including Ligand, 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.

Pursuant to our 2014 Equity Incentive Plan, or the 2014 Plan, a total of 669,698 shares of our common stock were reserved as of December 31, 2017 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.

On February 8, 2017, we entered into a Stock Purchase Agreement, or the SPA, with PoC Capital, LLC, or PoC, pursuant to which, among other things, we sold to PoC, and PoC purchased from us, 1,286,173 shares of our common stock for an aggregate issue price of \$1,800,000. The proceeds from the sale of such shares are reserved exclusively to fund certain of our clinical trials pursuant to a master services agreement with a contract research organization. Pursuant to the SPA, we also agreed to prepare and file one or more registration statements with the SEC for the purpose of registering the shares for resale. On February 14, 2017, we filed a registration statement covering the resale of the full amount of the shares issued to PoC pursuant to the SPA. Following effectiveness of such registration statement on March 27, 2017, the shares issued to PoC became eligible for sale in the public market. Sales of stock by PoC, or the perception that these sales may occur, could have a material adverse effect on the trading price of our common stock.

On June 14, 2017 and concurrent with our registered direct offering, we issued warrants to purchase an aggregate of 2,812,337 shares of our common stock. The exercise price of these warrants is \$1.30 per share, subject to adjustment as provided therein, and became exercisable beginning on December 19, 2017 through December 19, 2022. In addition, pursuant to the April 2016 Offering, we issued warrants to purchase an aggregate of 8,625,000 shares of our common stock which have an exercise price of \$1.50 per share of common stock and will expire on April 13, 2021. We have also issued to Ligand a warrant to purchase up to 960,000 shares of our common stock, which has an exercise price of \$1.50 per share of common stock and will expire on April 13, 2021, and to the representative of the underwriters for our initial public offering a warrant to purchase up to 82,500 shares of our common stock, which is exercisable for cash or on a cashless basis, has an exercise price of \$10.00 per share of common stock and will expire on April 28, 2020. To the extent that any of the foregoing warrants are exercised, our stockholders will experience additional dilution, which could have a material adverse effect on the trading price of our common stock.

On September 28, 2017, we entered into a purchase agreement, or the Registered Offering Purchase Agreement, pursuant to which, on September 29, 2017, we sold to Lincoln Park Capital Fund, LLC, or LPC, 701,282 shares of common stock, at a price of approximately \$1.78 per share for an aggregate purchase price of \$1,250,000, pursuant to our effective shelf registration statement on Form S-3 (Registration No. 333-212134), filed with the SEC on June 20, 2016, as amended by Amendment No. 1 thereto filed with the SEC on July 26, 2016, and declared effective on July 26, 2016, and the prospectus supplement thereto dated September 28, 2017.

On September 28, 2017, we also entered into a purchase agreement, or the Commitment Purchase Agreement, with LPC pursuant to which we have the right to sell to LPC up to \$15,000,000 in shares of common stock, subject to certain limitations and conditions set forth in the Commitment Purchase Agreement. Pursuant to the Commitment Purchase Agreement, we have the right, from time to time at our sole discretion over the 30-month period from and after October 26, 2017, to direct LPC to purchase up to 75,000 shares of common stock on any business day (subject to certain limitations contained in the Commitment Purchase Agreement), with such amounts increasing based on certain threshold prices set forth in the Commitment Purchase Agreement; however, not to exceed \$1,000,000 in total purchase proceeds per purchase date. The purchase price of shares of common stock that we elect to sell to LPC pursuant to the Commitment Purchase Agreement will be based on the market prices of the common stock at the time of such purchases as set forth in the Commitment Purchase Agreement. In addition to regular purchases, as described above, we may also direct LPC to purchase additional amounts as accelerated purchases or as additional purchases if the closing sale price of the common stock is not below certain threshold prices, as set forth in the Commitment

Purchase Agreement. In all instances, we may not sell shares of its common stock to LPC under the Commitment Purchase Agreement if it would result in LPC beneficially owning more than 4.99% of the common stock. To the extent that we sell shares pursuant to the Commitment Purchase Agreement, our stockholders will experience dilution, which could have a material adverse effect on the trading price of our common stock.

On December 11, 2017, we closed an underwritten public offering of 5,900,000 shares of our common stock, including 769,565 shares sold pursuant to the underwriter's full exercise of their option to purchase additional shares to cover over-allotments, at a public offering price of \$2.50 per share, for total net proceeds of \$13,717,500 after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

On February 6, 2018, we closed an underwritten public offering of 12,650,000 shares of our common stock, including 1,650,000 shares sold pursuant to the underwriter's full exercise of their option to purchase additional shares to cover over-allotments, at a public offering price of \$5.00 per share, for gross proceeds of \$63,250,000, before deducting underwriting discounts and commissions and estimated offering expenses payable by us.

If a large number of the holders of shares issued in our recent underwritten public offerings decided to sell their stock, or if there is even the perception that these sales may occur, the trading price of our common stock could be materially and adversely effected.

Our management will continue to have broad discretion over the use of the proceeds we received from our public offerings, registered direct offering and from the Loan and Security Agreement, and the proceeds we may receive pursuant to the Commitment Purchase Agreement we recently entered into with LPC, 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 recent December 2017 and February 2018 underwritten public offerings, our June 2017 registered direct offering, prior public offerings, proceeds received from the Loan and Security Agreement, and proceeds received pursuant to the Distribution Agreement, the Registered Offering Purchase Agreement and the Commitment Purchase 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 these remaining net proceeds, their ultimate use may vary substantially from their currently intended use. If we do not invest or apply these net proceeds 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, 2017, we had net operating loss carryforwards of approximately \$17,773,000 for federal and \$17,419,000 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 the full amounts of the \$17,773,000 federal and \$17,419,000 state tax net operating loss carryforwards are available for utilization as of December 31, 2017. 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;
- 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;

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- 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.

If we fail to comply with the continued listing requirements of the Nasdaq Capital Market, our common stock may be delisted and the price of our common stock and our ability to access the capital markets could be negatively impacted.

For six consecutive days in August 2017, the last reported sale price for our common stock on the Nasdaq Capital Market was less than \$1.00 per share. We must continue to satisfy the Nasdaq Capital Market's continued listing requirements, including, among other things, a minimum closing bid price requirement of \$1.00 per share for 30 consecutive business days. If a company fails for 30 consecutive business days to meet the \$1.00 minimum closing bid price requirement, Nasdaq will send a deficiency notice to the company, advising that it has been afforded a "compliance period" of 180 calendar days to regain compliance with the applicable requirements.

A delisting of our common stock from the Nasdaq Capital Market could materially reduce the liquidity of our common stock and result in a corresponding material reduction in the price of our common stock. In addition, delisting could harm our ability to raise capital through alternative financing sources on terms acceptable to us, or at all, and may result in the potential loss of confidence by investors and employees.

Item 1B. Unresolved Staff Comments.

None.

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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.



## 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. Warrants to purchase shares of our common stock issued as part of the underwritten public offering in April 2016 began trading on the Nasdaq Capital Market under the symbol "VKTXW" effective April 8, 2016. The following tables set forth the high and low sales prices per share of our common stock and warrants as reported on the Nasdaq Capital Market for the periods indicated.

## Common Stock

	Price Range	
	High	Low
Year Ended December 31, 2016		
First Quarter	\$4.24	\$1.37
Second Quarter	\$2.89	\$1.06
Third Quarter	\$1.54	\$1.22
Fourth Quarter	\$1.50	\$0.90
Year Ended December 31, 2017		
First Quarter	\$1.70	\$1.11
Second Quarter	\$1.49	\$0.96
Third Quarter	\$1.99	\$0.88
Fourth Quarter	\$4.40	\$1.69

## Warrants

	Price Range	
	High	Low
Year Ended December 31, 2016		
Second Quarter (from April 8, 2016)	\$0.53	\$0.05
Third Quarter	\$0.52	\$0.27
Fourth Quarter	\$0.52	\$0.35
Year Ended December 31, 2017		
First Quarter	\$0.65	\$0.21
Second Quarter	\$0.55	\$0.25
Third Quarter	\$0.73	\$0.25
Fourth Quarter	\$3.00	\$0.55

## Holders of Record

As of February 28, 2018, there were approximately eleven stockholders of record of our common stock and one holder of record of our publicly traded warrants. Certain shares and warrants 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

We satisfy certain U.S. federal and state tax withholding obligations due upon the vesting of restricted stock and restricted stock unit awards by automatically withholding from the shares being issued in connection with such award a number of shares of our common stock with an aggregate fair market value on the date of vesting equal to the minimum tax withholding obligations. The following table sets forth information with respect to shares of our common stock repurchased by us to satisfy certain tax withholding obligations during the three months ended December 31, 2017:

	(a) Total Number of Shares (or Units) Purchased	(b) Average Price Paid per Share (or Unit)	(c) Total Number of Shares (or Units) Purchased as Part of Publicly Announced Plans or Programs	(d) Maximum Number (or Approximate Dollar Value) of Shares (or Units) that may yet be Purchased Under the Plans or Programs
October 1, 2017 – October 31, 2017	—	—	—	—
November 1, 2017 – November 30, 2017	—	—	—	—
December 1, 2017 – December 31, 2017	1,879	(1) \$ 4.06	—	—
Total	1,879	\$ 4.06	—	—

(1) Represents shares of our common stock withheld from employees for the payment of taxes.

## 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.

## 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. Our lead clinical program, VK5211, is an orally available, 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 bone mineral density, 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 believe that VK5211 may also have potential benefits to patients suffering from muscle loss in other settings, such as joint replacements or muscle wasting disorders.

In October 2015, we commenced enrollment in a Phase 2 proof-of-concept clinical trial in 108 patients recovering from non-elective hip fracture surgery. In November 2017, we announced positive top-line results from our 12-week, Phase 2 clinical trial of VK5211 in patients who recently suffered a hip fracture. Top-line data showed that the trial achieved its primary endpoint, demonstrating statistically significant, dose dependent increases in lean body mass, less head, following treatment with VK5211 as compared to placebo. The study also achieved certain secondary endpoints, demonstrating statistically significant increases in appendicular lean body mass and total lean body mass for all doses of VK5211, compared to placebo. VK5211 demonstrated encouraging safety and tolerability in this study, with no drug-related serious adverse events, or SAEs, reported. See the discussion of "VK5211: A Selective Androgen Receptor Modulator (SARM) for Hip Fracture" for more details on these study results. We expect to receive follow-up data related to this study in the first half of 2018 and to then engage in further discussions with the U.S. Food and Drug Administration, or the FDA, regarding the potential further clinical development of VK5211 in hip fracture as well as other potential acute use settings.

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 in a Phase 2 clinical trial 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 thymomimetics. 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 are currently conducting a Phase 2 clinical trial of VK2809 in approximately 60 patients with hypercholesterolemia and fatty liver disease and expect to report initial results from this Phase 2 clinical trial in the second half of 2018. In October 2017, we announced positive final results from an eight-week study of VK2809 in an in vivo model of non-alcoholic steatohepatitis, or NASH. Treatment with VK2809 resulted in: (1) statistically significant reductions in several key measures of steatosis,

including liver triglyceride content and total liver lipid content, (2) fibrotic activity, including total liver fibrosis, type I collagen and hydroxyproline, relative to vehicle controls, and (3) statistically significant changes in the expression of key genes associated with NASH development and progression, relative to vehicle control, suggesting improved lipid and cholesterol metabolism, improved lipid metabolism and insulin sensitivity and reduced fibrotic activity.

In February 2017, we announced that we are commencing efforts to utilize VK2809 to potentially help patients who suffer from Glycogen Storage Disease type Ia, or GSD Ia. GSD Ia is a rare, orphan genetic disease caused by a deficiency of glucose-6-phosphatase, or G6PC, an enzyme responsible for the liver's production of free glucose from glycogen and gluconeogenesis. Approximately 2,000 patients in the U.S. suffer from GSD Ia. We have conducted a proof-of-concept study utilizing VK2809 in an in vivo model of GSD Ia. Data demonstrated that treatment with VK2809 led to statistically significant reductions in key metabolic markers of GSD Ia. VK2809's potential to rapidly reduce hepatic triglyceride levels, as demonstrated in this initial evaluation in a GSD Ia model, provides support for the continued investigation of the compound in this indication. We

expect to initiate a Phase 1 human proof-of-concept clinical trial to evaluate VK2809 in patients with GSD Ia in the first half of 2018.

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 data suggest that VK0214 stimulates ABCD2 expression in an in vitro model and reduces VLCFA levels in an in vivo model of X-ALD. Pending completion of certain toxicology studies, we expect to file an IND to conduct a proof-of-concept study in patients with X-ALD in the second half of 2018.

We were incorporated under the laws of the State of Delaware on September 24, 2012. Since our incorporation, we have devoted most of our efforts towards conducting certain clinical trials and preclinical studies related to our VK5211, VK2809 and VK0214 programs, as well as efforts towards raising capital and building infrastructure. We obtained exclusive worldwide rights to our VK5211, VK2809 and VK0214 programs and certain other assets 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 which we entered into on May 21, 2014 with Ligand, as amended, or the Master License Agreement. A summary of the Master License Agreement 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.

## 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

During the year ended December 31, 2016, we charged \$9,000,499 to research and development expense related to our efforts to conduct Phase 2 clinical trials for VK5211 and VK2809 and in vivo studies for VK0214. During the year ended December 31, 2017, we charged \$13,741,186 to research and development expense related to our continued efforts to conduct 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 efforts towards advancing our VK5211 and VK2809 programs and seek to advance our additional programs.

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## General and Administrative Expenses

Our general and administrative expenses have generally increased year-over-year as we have hired additional employees, issued additional equity awards, which has resulted in increased stock-based compensation expense, 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 includes the change in fair value of the debt conversion feature liability contained in 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.

## JOBS Act

We are an “emerging growth company” within the meaning of the rules under 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 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 the statements of operations.

The Financial Accounting Standards Board's, 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 is (a) 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: 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 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, 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, 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, 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.

In May 2014, we entered into the Master License Agreement, pursuant to which 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 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 are 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 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, 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 uses the fair value at grant date to value the awards. For restricted stock that vests upon the satisfaction of certain performance conditions, we recognize 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, we record a cumulative catch-up of the expense from the grant date to the current date, and we then amortize the remainder of the expense over the remaining service period.

We account 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, and then assessing at each balance sheet date the probability of the performance criteria being met. If the probability of achieving the criteria is deemed less-than-probable, then no expense is recorded. At the point where the criteria are deemed probable of being met, we record 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 accounting principles generally accepted in the United States of America, or GAAP. 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, 2017 and 2016

## Research and Development Expenses

The following table summarizes our research and development expenses for the years ended December 31, 2017 and 2016.

	Year Ended December		\$	%
	31,	31,	Change	Change
	2017	2016		
Research and development expenses	\$13,741,186	\$9,000,499	\$4,740,687	52.7 %

The increase in research and development expenses during the year ended December 31, 2017 as compared to the year ended December 31, 2016 was primarily due to an increase in expenses of \$3,672,401 related to clinical trial activity for our VK5211 and VK2809 programs and pre-clinical efforts for our VK0214 program, \$676,588 related primarily to manufacturing for our drug candidates and \$327,677 related to services provided by third party consultants, offset by a decrease in expenses of \$128,629 related to stock based compensation expense.

## General and Administrative Expenses

The following table summarizes our general and administrative expenses for the years ended December 31, 2017 and 2016.

	Year Ended December		\$	%
	31,	31,	Change	Change
	2017	2016		
General and administrative expenses	\$5,329,003	\$4,846,776	\$482,227	9.9 %

The increase in general and administrative expenses during the year ended December 31, 2017 as compared to the year ended December 31, 2016 was primarily due to increases in salaries and benefits related expenses of \$662,605, consultant expenses of \$53,479, franchise taxes of \$40,627 and patent expenses of \$35,916, offset by decreases in stock-based compensation expense of \$214,233 and legal expenses of \$142,042.

## Other Income (Expense)

The following table summarizes our other income (expense) for the years ended December 31, 2017 and 2016.

	Year Ended December		\$	%
	31,	31,	Change	Change

	2017	2016		
Other income (expense)	\$(1,507,476)	\$(884,547)	\$(622,929)	70.4 %

Other income (expense) recognized during the year ended December 31, 2017 consisted primarily of expenses of \$571,044 related to the amortization of financing costs, \$1,282,631 related to the amortization of the Ligand Note discount and \$96,872 of interest expenses related to the Ligand Note offset by income of \$344,928 related to the change in fair value of the Ligand Note's conversion feature and \$98,142 of interest income related to our short-term investments.

Other income (expense) recognized during the year ended December 31, 2016 consisted primarily of expenses of \$1,788,088 related to the amortization of the Ligand Note discount, \$105,551 of interest expenses related to the Ligand Note and \$138,701 of amortization expenses related to the Equity Distribution Agreement, dated June 20, 2016, or the Distribution Agreement, with Maxim Group LLC, or Maxim, and the Common Stock Purchase Agreement, or the Purchase Agreement, with Aspire Capital Fund, LLC, or Aspire Capital, offset by income of \$1,064,170 related to the 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. As of December 31, 2017, we had cash, cash equivalents and short term investments of \$20,575,956. In addition, in February 2018 we raised an additional \$63,250,000 in gross proceeds through a public offering of 12,650,000 shares of our common stock at \$5.00 per share. As such, we believe our cash, cash equivalents and short-term investments, including the proceeds received in February 2018,

will be sufficient to fund our operations through at least the first quarter of 2019, which is more than one year after the date our December 31, 2017 financial statements were issued.

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, VK2809 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 June 20, 2016, we entered into the Distribution Agreement with Maxim, pursuant to which we were able to offer and sell, from time to time, through Maxim, up to 3,748,726 shares of our common stock. Effective July 26, 2017, the Distribution Agreement terminated automatically in accordance with the terms thereof. All shares of our common stock offered and sold pursuant to the Distribution Agreement were issued pursuant to our registration statement on Form S-3 (File No. 333-212134) filed with the SEC on June 20, 2016 and the prospectus relating to shares issued pursuant to the Distribution Agreement that forms a part of the registration statement on Form S-3. The registration statement on Form S-3 was declared effective by the SEC on July 26, 2016. During the period from January 1, 2017 through July 26, 2017, we sold 488,388 shares pursuant the Distribution Agreement, resulting in net proceeds to us of \$670,031, after deducting the sales agent's commission. From the inception of the Distribution Agreement through July 26, 2017, we sold 1,267,237 shares of our common stock pursuant to the Distribution Agreement, resulting in net proceeds to us of \$1,626,549.

On August 24, 2016, we entered into the Purchase Agreement with Aspire Capital, pursuant to which Aspire Capital was committed to purchase up to an aggregate of \$12.5 million of shares of our common stock over the 30-month term of the Purchase Agreement. Upon execution of the Purchase Agreement, we issued and sold to Aspire Capital under the Purchase Agreement 333,333 shares of our common stock, or the Initial Shares, for an aggregate purchase price of \$500,000. Concurrently with the execution of the Purchase Agreement, and as consideration for Aspire Capital entering into the Purchase Agreement, we issued 336,116 shares of our common stock, or the Commitment Shares, to Aspire Capital as a commitment fee. Pursuant to the terms of the Purchase Agreement, we were able to, from time to time and subject to certain limitations, direct Aspire Capital to purchase shares of our common stock using pricing formulas based on average prevailing market prices around the time of each sale. Effective June 19, 2017, we terminated the Purchase Agreement, without liability to us in accordance with the terms thereof. During the period from January 1, 2017 through June 19, 2017, 1,326,991 shares were issued pursuant to the Purchase Agreement, resulting in net proceeds to us of \$1,869,512. From the inception of the Purchase Agreement through June 19, 2017, we sold 1,476,991 shares of our common stock pursuant to the Purchase Agreement, resulting in aggregate gross proceeds to us of \$2,042,762, in addition to the Initial Shares and the Commitment Shares.

On June 14, 2017, we entered into a securities purchase agreement with certain accredited investors, or the Purchasers, pursuant to which we issued and sold an aggregate of 3,749,783 shares of our common stock in a registered direct offering, or the Registered Direct Offering. The shares were offered by us pursuant to our shelf registration statement on Form S-3 (File No. 333-212134) filed with the SEC on June 20, 2016, as amended by Amendment No. 1 thereto filed with the SEC on July 26, 2016, and declared effective on July 26, 2016. In a concurrent private placement, we also agreed, pursuant to the securities purchase agreement, to issue and sell to each of the Purchasers a warrant to purchase 0.75 shares of common stock for each share of common stock purchased by a Purchaser in the Registered Direct Offering. The exercise price of the warrants is \$1.30 per share, subject to adjustment as provided therein, and



became exercisable beginning on December 19, 2017 through December 19, 2022. The combined purchase price for one share and one warrant to purchase 0.75 shares of common stock in these offerings was \$1.15. The closing of the offerings occurred on June 19, 2017. The aggregate net proceeds from the offerings, after deducting the placement agents' fees and other offering expenses, were \$3,866,202.

On September 28, 2017, we entered into a purchase agreement, or the Registered Offering Purchase Agreement, pursuant to which, on September 29, 2017, we sold to Lincoln Park Capital, LLC, or LPC, 701,282 shares of common stock, at a price of approximately \$1.78 per share for an aggregate purchase price of \$1,250,000, pursuant to our effective shelf registration statement on Form S-3 (Registration No. 333-212134), filed with the SEC on June 20, 2016, as amended by Amendment No. 1 thereto filed with the SEC on July 26, 2016, and declared effective on July 26, 2016, and the prospectus supplement thereto dated September 28, 2017.

On September 28, 2017, we also entered into a purchase agreement, or the Commitment Purchase Agreement with LPC, pursuant to which we have the right to sell to LPC up to \$15,000,000 in shares of common stock, subject to certain limitations and conditions set forth in the Commitment Purchase Agreement. Pursuant to the Commitment Purchase Agreement, we have the right, from time to time at our sole discretion over the 30-month period from and after October 26, 2017 to direct LPC to purchase up to 75,000 shares of common stock on any business day (subject to certain limitations contained in the Commitment Purchase Agreement), with such amounts increasing based on certain threshold prices set forth in the Commitment Purchase Agreement; however, not to exceed \$1,000,000 in total purchase proceeds per purchase date. The purchase price of shares of common stock that we elect to sell to LPC pursuant to the Commitment Purchase Agreement will be based on the market prices of the common stock at the time of such

purchases as set forth in the Commitment Purchase Agreement. In addition to regular purchases, as described above, we may also direct LPC to purchase additional amounts as accelerated purchases or as additional purchases if the closing sale price of the common stock is not below certain threshold prices, as set forth in the Commitment Purchase Agreement. In all instances, we may not sell shares of its common stock to LPC under the Commitment Purchase Agreement if it would result in LPC beneficially owning more than 4.99% of the Common Stock. As consideration for LPC's commitment to purchase shares of common stock pursuant to the Commitment Purchase Agreement, we issued to LPC 100,000 shares of common stock. During the fourth quarter of 2017, we sold to LPC in accordance with the terms of the agreement 343,051 shares of our common stock for aggregate proceeds of \$802,112.

On December 11, 2017, we closed an underwritten public offering of 5,900,000 shares of our common stock, including 769,565 shares sold pursuant to the underwriter's full exercise of their option to purchase additional shares to cover over-allotments, at a public offering price of \$2.50 per share, for total net proceeds of \$13,449,252 after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

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

	2017	2016
Cash used in operating activities	\$(14,757,767)	\$(11,071,263)
Cash provided by (used in) investing activities	\$(1,729,707)	\$3,029,765
Cash provided by financing activities	\$22,400,450	\$10,348,679

#### Cash Used in Operating Activities

During the year ended December 31, 2017, cash used in operating activities of \$14,757,767 primarily reflected our net losses for the period, offset by non-cash charges such as amortization of discount charged to interest expense on the Ligand Note, amortization of investment premiums, amortization of financing costs, stock-based compensation and changes in our working capital accounts, primarily consisting of an increase in accrued expenses, accounts payable offset by a decrease in the fair value of the debt conversion feature liability for the Ligand Note and a decrease in prepaid expenses.

During the year ended December 31, 2016, cash used in operating activities of \$11,071,263 primarily reflected our net losses for the period, offset by non-cash charges such as amortization of discount charged to interest expense on the Ligand Note, amortization of investment premiums, amortization of financing costs, stock-based compensation and changes in our working capital accounts, primarily consisting of an increase in accrued expenses offset by a decrease in the fair value of the debt conversion feature liability for the Ligand Note and a decrease in accounts payable and prepaid expenses.

#### Cash Provided by (Used in) Investing Activities

During the year ended December 31, 2017, cash used in investing activities of \$1,729,707 resulted primarily from the purchase of investments of \$22,525,707 offset by the proceeds of sales and maturities of investments of \$20,796,000.

During the year ended December 31, 2016, cash provided by investing activities of \$3,029,765 resulted primarily from the proceeds of sales and maturities of investments of \$19,952,000 offset by the purchase of investments of \$16,922,235.

#### Cash Provided by Financing Activities

During the year ended December 31, 2017, cash provided by financing activities was \$22,400,450, which consisted primarily of proceeds from the issuance of common stock, net of discount, of \$22,351,890 and \$647,790 of proceeds from the issuance of common stock related to warrant exercises, stock option exercises and employee stock purchase plan purchases offset by \$500,257 in payments of certain deferred offering and financing costs.

During the year ended December 31, 2016, cash provided by financing activities was \$10,348,450, which consisted primarily of proceeds from the issuance of common stock, net of discount, of \$11,548,518 offset by \$1,096,205 in payments of certain deferred offering and financing costs.

#### Future Funding Requirements

As of December 31, 2017, based upon our current operating plan and the \$63,250,000 in gross proceeds received in our February 2018 public offering, we believe that we will have sufficient cash to meet our projected operating requirements for at least the next 12 months following the issuance of the financial statements. We anticipate, however, 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.

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;
- our ability to establish and maintain strategic collaborations, licensing or other arrangements and the financial terms of such agreements;
- 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;
- 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

#### Adopted Accounting Standards

In May 2014, the Financial Accounting Standards Board, or FASB, issued Accounting Standards Update, or ASU, No. 2014-09, Revenue from Contracts with Customers (Topic 606), which supersedes all existing revenue recognition requirements, including most industry-specific guidance. The new standard requires a company to recognize revenue when it transfers goods or services to customers in an amount that reflects the consideration that the company expects to receive for those goods or services. The FASB has subsequently issued the following amendments to ASU No. 2014-09, which have the same effective date and transition date of January 1, 2018:

- In August 2015, the FASB issued ASU No. 2015-14, Revenue from Contracts with Customers (Topic 606): Deferral of the Effective Date, which delayed the effective date of the new standard from January 1, 2017 to January 1, 2018.

The FASB also agreed to allow entities to choose to adopt the standard as of the original effective date.

In March 2016, the FASB issued ASU No. 2016-08, Revenue from Contracts with Customers (Topic 606): Principal versus Agent Considerations, which clarifies the implementation guidance on principal versus agent considerations.

In April 2016, the FASB issued ASU No. 2016-10, Revenue from Contracts with Customers (Topic 606): Identifying Performance Obligations and Licensing, which clarifies certain aspects of identifying performance obligations and licensing implementation guidance.

In May 2016, the FASB issued ASU No. 2016-12, Revenue from Contracts with Customers (Topic 606): Narrow-Scope Improvements and Practical Expedients, which relates to disclosures of remaining performance obligations, as well as other amendments to guidance on collectability, non-cash consideration and the presentation of sales and other similar taxes collected from customers.

In December 2016, the FASB issued ASU No. 2016-20, Technical Corrections and Improvements to Topic 606, Revenue from Contracts with Customers, which amends certain narrow aspects of the guidance issued in ASU No. 2014-09 including guidance related to the disclosure of remaining performance obligations and prior-period performance obligations, as well as other amendments to the guidance on loan guarantee fees, contract costs, refund liabilities, advertising costs and the clarification of certain examples.

The Company will continue to evaluate the potential impact that these standards will have on its financial position and results of operations, although there is no current impact of this new guidance on its financial statements as the Company does not currently have any revenue generating arrangements.

In March 2016, FASB issued ASU No. 2016-09, Improvements to Employee Share-based Payment Accounting, or ASU 2016-09. ASU 2016-09 simplifies several aspects of the accounting for employee share-based payment transactions, including the accounting for income taxes, forfeitures, and statutory tax withholding requirements, as well as classification in the statement of cash flows. Under this guidance, a company recognizes all excess tax benefits and tax deficiencies as income tax expense or benefit in the statement of operations. This change eliminates the notion of the additional paid-in capital pool and reduces the complexity in accounting for excess tax benefits and tax deficiencies. The Company implemented ASU 2016-09 as required in the first quarter of fiscal year 2017. The Company's adoption of ASU 2016-09 did not have a material effect on its financial statements and related disclosures.

In August 2016, the FASB issued ASU No. 2016-15, Classification of Certain Cash Receipts and Cash Payments, or ASU 2016-15, which amends FASB Accounting Standards Codification, or ASC, Topic 230 to add or clarify guidance on eight classification issues related to the statement of cash flows such as debt prepayment or debt extinguishment costs, and contingent consideration payments made after a business combination. ASU 2016-15 is effective for fiscal periods beginning after December 15, 2017 and must be adopted using a retrospective transition method to each period presented but may be applied prospectively if retrospective application would be impracticable. Early adoption is permitted, including adoption in an interim period. We do not expect the adoption of ASU 2016-15 on January 1, 2018 to have a material effect on our financial statements and related disclosures.

#### Recent Accounting Pronouncements Not Yet Adopted

In February 2016, the FASB issued ASU No. 2016-02, Leases (Topic 842), or ASU 2016-02, which amends the existing accounting standards for leases. The new standard requires lessees to record a right-of-use asset and a corresponding lease liability on the balance sheet (with the exception of short-term leases). For lessees, leases will continue to be classified as either operating or financing in the statement of operations. This ASU will become

effective for us in the first quarter of fiscal year 2019 and early adoption is permitted. ASU 2016-02 is required to be applied with a modified retrospective approach and requires application of the new standard at the beginning of the earliest comparative period presented. We do not expect the adoption of ASU 2016-02 to have a material effect on our financial statements and related disclosures.

In June 2016, the FASB issued ASU No. 2016-13, Measurement of Credit Losses on Financial Instruments, or ASU 2016-13, which requires that financial assets measured at amortized cost be presented at the net amount expected to be collected. Currently, GAAP delays recognition of the full amount of credit losses until the loss is probable of occurring. Under this new standard, the statement of operations will reflect an entity's current estimate of all expected credit losses. The measurement of expected credit losses will be based upon historical experience, current conditions, and reasonable and supportable forecasts that affect the collectability of the reported amount. Credit losses relating to available-for-sale debt securities will be recorded through an allowance for credit losses rather than as a direct write-down to the security. The new standard is effective for us for fiscal years beginning after December 15, 2019, including interim periods within those fiscal years. Early adoption is permitted as of the fiscal years beginning after December 15, 2018, including interim periods within those fiscal years. We are evaluating the effect that ASU 2016-13 will have on our financial statements and related disclosures.

In July 2017, the FASB issued a two-part ASU No. 2017-11, I. Accounting for Certain Financial Instruments with Down Round Features and II. Replacement of the Indefinite Deferral for Mandatorily Redeemable Financial Instruments of Certain Nonpublic Entities and Certain Mandatorily Redeemable Noncontrolling Interests with a Scope Exception, or ASU 2017-11. ASU 2017-11 amends guidance in FASB ASC Topic 260: Earnings Per Share, FASB ASC Topic 480: Distinguishing Liabilities from Equity, and

FASB ASC Topic 815: Derivatives and Hedging. The amendments in Part I of ASU 2017-11 change the classification analysis of certain equity-linked financial instruments (or embedded features) with down round features. The amendments in Part II of ASU 2017-11 re-characterize the indefinite deferral of certain provisions of FASB ASC Topic 480 that now are presented as pending content in the ASC, to a scope exception. ASU 2017-11 is effective for us for fiscal years beginning after December 15, 2018, including interim periods within those fiscal years. Early adoption is permitted. We are evaluating the effect that ASU 2017-11 will have on our financial statements and related disclosures.

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.

None

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 and not absolute assurance of achieving the desired control objectives, and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Based on our management's evaluation (with the participation of the individuals serving as our principal executive officer and principal financial officer) of our disclosure controls and procedures as required by Rules 13a-15 and



15d-15 under the Exchange Act, each of the individuals serving as our principal executive officer and principal financial officer has concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of December 31, 2017, the end of the period covered by this report.

Management's Report on Internal Control over Financial Reporting.

Our management is responsible for establishing and maintaining adequate internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) of the Exchange Act). Internal control over financial reporting is a process designed under the supervision and with the participation of our management, including the individuals serving as our principal executive officer and principal financial officer, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with accounting principles generally accepted in the United States of America. Management conducted an assessment of the effectiveness of our internal control over financial reporting based on the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission in Internal Control-Integrated Framework (2013 Framework). Based on this assessment, our management concluded that, as of December 31, 2017, our internal control over financial reporting was effective based on those criteria.

Attestation Report on Internal Control over Financial Reporting.

This Annual Report on Form 10-K does not include an attestation report of our independent registered public accounting firm due to the deferral allowed under the JOBS Act for emerging growth 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, 2017 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information.

None.

## PART III

## Item 10. Directors, Executive Officers and Corporate Governance.

Our business and affairs are managed under the direction of our board of directors, or our Board, which currently consists of six members. The primary responsibilities of our Board are to provide oversight, strategic guidance, counseling and direction to our management.

In accordance with our amended and restated certificate of incorporation and our amended and restated bylaws, our Board is divided into three classes with staggered three-year terms. Only one class of directors will be elected at each annual meeting of our stockholders, with the other classes continuing for the remainder of their respective three-year terms. Our directors are divided among the three classes as follows:

our class III directors are Dr. Lian and Dr. Macartney and their term will expire at the annual meeting of stockholders to be held in 2018;

our class I directors are Mr. Foehr and Mr. Rowland and their term will expire at the annual meeting of stockholders to be held in 2019; and

our class II directors are Mr. Singleton and Mr. Webster and their term will expire at the annual meeting of stockholders to be held in 2020.

At each annual meeting of stockholders, the successors to the directors whose term will then expire will be elected to serve from the time of election and qualification until the third annual meeting following election. In addition, the authorized number of directors may be changed only by resolution of our Board. Any additional directorships resulting from an increase in the number of directors will be distributed among the three classes so that, as nearly as possible, each class will consist of one-third of the directors. This classification of our Board may have the effect of delaying or preventing a change of our management or a change in control.

The names of our current directors, their ages as of March 7, 2018, director class and biographies are listed below. There are no family relationships between or among any of our directors.

Name	Age	Director Class	Position(s)
Lawson Macartney, DVM, Ph.D.	60	Class III	Chairperson of the Board of Directors
Brian Lian, Ph.D. <sup>(1)</sup>	52	Class III	President and Chief Executive Officer, Director
Matthew W. Foehr	45	Class I	Director
Charles A. Rowland, Jr.	59	Class I	Director
J. Matthew Singleton	65	Class II	Director
Stephen W. Webster	56	Class II	Director

(1) Please see “Information Regarding Executive Officers” below for Dr. Lian’s biography.

Lawson Macartney, DVM, Ph.D., has served as the Chairperson of the Board since May 2015 and as a member of our Board since May 2014. Dr. Macartney serves on the Board of Dechra PLC, an international animal health pharmaceutical company based in the UK, and on the Supervisory Board of the Netherlands Translational Research Centre, a preclinical biopharmaceutical company based in The Netherlands. He served as President, Chief Executive Officer and a member of the board of directors of Ambrx Inc., a biopharmaceutical company, from February 2013 to June 2015. Prior to Ambrx, Dr. Macartney served at Shire AG, a specialty biopharmaceutical company, as Senior Vice President of the Emerging Business Unit from 2011 to 2013, where he was responsible for discovery initiatives through Phase 3 development of Shire's Specialty Pharmaceutical portfolio. Prior to joining Shire AG, he served at GSK, a pharmaceutical company, from 1999 to 2011, serving as Senior Vice President of Global Product Strategy and Project/Portfolio Management from 2007 to 2011, as Senior Vice President, Cardiovascular and Metabolic Medicine Development Center from 2004 to 2007, and as Vice President, Global Head of Cardiovascular, Metabolic and Urology Therapeutic Areas

from 1999 to 2004. Prior to joining GSK, Dr. Macartney was employed at Astra Pharmaceuticals from 1998 to 1999 in leadership roles in operations, marketing and sales, and served as Executive Director, Commercial Operations at AstraMerck, Inc., a pharmaceutical company, from 1996 to 1998. Dr. Macartney received his Ph.D. from Glasgow University in Scotland in 1982, where he was a Royal Society Research Fellow, and his B.V.M.S. (equivalent to a D.V.M.) in 1979 from Glasgow University Veterinary School. He is also trained in diagnostic pathology and is a Fellow of the Royal College of Pathologists. We believe that Dr. Macartney's extensive experience in leadership positions at numerous pharmaceutical companies qualifies him to serve on our Board.

Matthew W. Foehr has served as a member of our Board since May 2014. Since February 2015, Mr. Foehr has served as President and Chief Operating Officer of Ligand, and previously served as Executive Vice President and Chief Operating Officer of Ligand from April 2011 to February 2015. He has served on the board of directors of Ritter Pharmaceuticals, Inc. (Nasdaq: RTTR) since February 2015 and currently serves on its audit committee and is Chair of its compensation committee. Mr. Foehr has over 20 years of experience in the pharmaceutical industry, having managed global operations and research and development programs. From March 2010 to April 2011, he was Vice President and Head of Consumer Dermatology R&D, as well as Acting Chief Scientific Officer of Dermatology, in the Stiefel division of GSK. Following GSK's \$3.6 billion acquisition of Stiefel Laboratories, Inc., a pharmaceutical company, in 2009, Mr. Foehr led the R&D integration of Stiefel into GSK. At Stiefel Laboratories, Inc., Mr. Foehr served as Senior Vice President of Global R&D Operations, Senior Vice President of Product Development & Support, and Vice President of Global Supply Chain Technical Services from January 2007 to March 2010. Prior to Stiefel, Mr. Foehr held various executive roles at Connetics Corporation, a pharmaceutical company, including Senior Vice President of Technical Operations and Vice President of Manufacturing. Early in his career, Mr. Foehr managed manufacturing activities and worked in process sciences at both LXR Biotechnology Inc. and Berlex Biosciences. Mr. Foehr is the author of multiple scientific publications and is named on numerous U.S. patents. He received his BS in Biology from Santa Clara University. We believe that Mr. Foehr's past service in executive management roles for companies in the pharmaceutical industry and related experience provide him with the qualifications and skills to serve as a member of our Board. Pursuant to the management rights letter between us and Ligand, a greater than 5% holder of our outstanding shares of common stock, dated May 21, 2014, Ligand has the right to nominate one individual for election to our Board, and Mr. Foehr is the current member of our Board nominated by Ligand.

Charles A. Rowland, Jr., MBA, has served as a member of our Board since July 2017. He has served as a member of the board of directors of Blueprint Medicines Corporation since March 2015. From April 2016 to February 2017, Mr. Rowland served as president and chief executive officer of Aurinia Pharmaceuticals Inc., and as a member of the board of directors of Aurinia from July 2014 to February 2017. Mr. Rowland previously served as vice president and chief financial officer of ViroPharma Incorporated, an international biopharmaceutical company, from October 2008 until it was acquired by Shire plc in January 2014. Prior to joining ViroPharma, from 2006 to 2008, Mr. Rowland served as executive vice president and chief financial officer, as well as the interim co-chief executive officer, for Endo Pharmaceuticals Inc., a specialty pharmaceutical company with a primary focus in pain management. Mr. Rowland previously held positions of increasing responsibility at Biovail Pharmaceuticals, Inc., Breakaway Technologies, Inc., Pharmacia Corporation, Novartis AG and Bristol-Myers Squibb Co., each a biopharmaceutical company. Mr. Rowland joined the board of directors of Idenix Pharmaceuticals, Inc., a biopharmaceutical company, in June 2013 and served as a member of its audit committee until Idenix was acquired by Merck & Co., Inc. in August 2014. Mr. Rowland served as a member of the board of directors and chairman of the audit committee of Vitae Pharmaceuticals, Inc. from September 2014 until it was acquired by Allergan in September 2016. Since January 2015, he has served as a member of the supervisory board and chairman of the audit committee of Nabriva Therapeutics, AG, a biotechnology company based in Vienna, Austria. Mr. Rowland served as a member of the board of directors and chairman of the audit committee of BIND Therapeutics, Inc. from May 2014 to July 2016. Mr. Rowland received a B.S. in Accounting from Saint Joseph's University and an M.B.A. with a finance concentration from Rutgers University. We believe that Mr. Rowland's financial and business expertise in the life sciences industry provide him with the qualifications and skills to serve as a member of our Board.

J. Matthew Singleton has served as a member of our Board since May 2014. In October 2011, Mr. Singleton retired from his position as Executive Vice President and Chief Financial Officer of CitationAir (formerly CitationShares LLC), a privately held jet services company wholly-owned by Textron Inc., a public industrial conglomerate. He had served in this position since 2000. Mr. Singleton has extensive financial, accounting and transactional experience, including through his role as Managing Director, Executive Vice President and Chief Administrative Officer of CIBC World Markets, an investment banking company, for 20 years, from 1974 to 1994, at Arthur Andersen & Co., a public accounting firm, including as Partner-in-Charge of the Metro New York Audit and Business Advisory Practice, and as a Practice Fellow at the Financial Accounting Standards Board, a private organization responsible for establishing financial accounting reporting standards. From 2003 until 2014, Mr. Singleton served as a director of Cubist Pharmaceuticals Inc., and as Audit Committee Chair beginning in 2004. Mr. Singleton previously served as an independent director of Salomon Reinvestment Company Inc., a privately held investment services company. Mr. Singleton received an AB in Economics from Princeton University and his MBA from New York University with a focus in Accounting. We believe that Mr. Singleton's financial, accounting and business expertise provide him with the qualifications and skills to serve as a member of our Board, and are of particular importance as we continue to finance our operations.

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Stephen W. Webster has served as a member of our Board since May 2014. Mr. Webster has served as the Chief Financial Officer of Spark Therapeutics, Inc., a biotechnology company, since July 2014. He was previously SVP and Chief Financial Officer of Optimer Pharmaceuticals, Inc., a biotechnology company, from 2012 to 2013, until its acquisition by Cubist Pharmaceuticals, Inc. Prior to joining Optimer, Mr. Webster served as SVP and Chief Financial Officer of Adolor Corporation, a biopharmaceutical company, from June 2008 until its acquisition by Cubist Pharmaceuticals, Inc. in December 2011. From 2007 until joining Adolor Corporation in 2008, Mr. Webster served as Managing Director, Investment Banking Division, Health Care Group for Broadpoint Capital Inc. (formerly First Albany Capital). Mr. Webster previously served as co-founder, President and Chief Executive Officer for Neuronix, Inc., a biopharmaceutical company, from 2000 to 2006. From 1987 to 2000, Mr. Webster served in positions of increased responsibility, including as Director, Investment Banking Division, Health Care Group for PaineWebber Incorporated. He has served as a Director of Nabriva Therapeutics plc (Nasdaq: NBRV) since August 2016. Mr. Webster holds an AB in Economics cum laude from Dartmouth College and an MBA in Finance from The Wharton School of the University of Pennsylvania. We believe that Mr. Webster's extensive experience in the biopharmaceutical industry, and in particular his current and prior service as chief financial officer and in other executive management roles, provide him with the qualifications and skills to serve as a member of our Board.

### Information Regarding Executive Officers

Our executive officers are elected by, and serve at the discretion of, our Board. Our executive officers, their ages as of March 7, 2018, respective positions and biographies are listed below:

Name	Age	Position(s)
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Brian Lian, Ph.D.	52	President and Chief Executive Officer, Director
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Michael Morneau	52	Chief Financial Officer
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Brian Lian, Ph.D., has served as our President and Chief Executive Officer and as a Director since our inception in September 2012. Dr. Lian has over 15 years of experience in the biotechnology and financial services industries. Prior to joining Viking, he was a Managing Director and Senior Research Analyst at SunTrust Robinson Humphrey, an investment bank, from 2012 to 2013. At SunTrust Robinson Humphrey, he was responsible for coverage of small and mid-cap biotechnology companies with an emphasis on companies in the diabetes, oncology, infectious disease and neurology spaces. Prior to SunTrust Robinson Humphrey, he was Managing Director and Senior Research Analyst at Global Hunter Securities, an investment bank, from 2011 to 2012. Prior to Global Hunter Securities, he was Senior Healthcare Analyst at The Agave Group, LLC, a registered investment advisor, from 2008 to 2011. Prior to The Agave Group, he was an Executive Director and Senior Biotechnology Analyst at CIBC World Markets, an investment bank, from 2006 to 2008. Prior to CIBC, he was a research scientist in small molecule drug discovery at Amgen, a biotechnology company. Prior to Amgen, he was a research scientist at Microcide Pharmaceuticals, a biotechnology company. Dr. Lian holds an MBA in accounting and finance from Indiana University, an MS and Ph.D. in organic chemistry from The University of Michigan, and a BA in chemistry from Whitman College. We believe that Dr. Lian's experience in the biotechnology industry, as well as his extensive investment banking and other experience in the financial services industry, provide him with the qualifications and skills to serve as a member of our Board and bring relevant strategic and operational guidance to our Board.

Michael Morneau has served as our Chief Financial Officer since May 2014. Mr. Morneau has over 25 years of accounting and financial experience at public and private companies in the biotechnology and accounting industries. Prior to Viking, from 2009 to 2014, he was VP of Finance and Chief Accounting Officer at Trius Therapeutics, Inc., a subsidiary of Cubist Pharmaceuticals, Inc., a pharmaceutical company, following Cubist's acquisition of Trius in September 2013. Prior to Trius, from 2008 to 2009, he was Director of Lilly Research Labs Finance at Eli Lilly and Company, a pharmaceutical company. Prior to Eli Lilly, from 2006 to 2008, he was Director of Finance and Accounting at SGX Pharmaceuticals, Inc., a biotechnology company, which was acquired by Eli Lilly. Prior to SGX, from 2004 to 2006, he was Controller at Momenta Pharmaceuticals, Inc., a biotechnology company. Prior to Momenta, from 2002 to 2004, he was Controller at Gensym Corporation, a software company.

Prior to Gensym, from 1992 to 2001, he worked at PricewaterhouseCoopers, LLP in the Audit Assurance Group, the last five years of which he was Manager of Business Assurance. Mr. Morneau earned his MBA and MA in accounting from New Hampshire College, and a BA in mathematics from the University of New Hampshire.

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

#### Section 16(a) Beneficial Ownership Reporting Compliance

Section 16(a) of the Exchange Act requires our directors and executive officers, and persons who beneficially own more than ten percent of a registered class of our equity securities, to file with the SEC initial reports of ownership and reports of changes in ownership of common stock and our other equity securities. Officers, directors and greater than ten percent stockholders are required by SEC regulations to furnish us with copies of all Section 16(a) forms they file.



To our knowledge, based solely on a review of the copies of such reports furnished to us and written representations that no other reports were required, during the fiscal year ended December 31, 2017, our officers, directors and greater than ten percent beneficial owners complied with all Section 16(a) filing requirements applicable to them.

#### Security Holder Nominations

We have not made any material changes to the procedures by which our stockholders may recommend nominees to our Board since we last disclosed the procedures by which stockholders may nominate director candidates under the caption “Security Holder Nominations” in our proxy statement for our 2017 annual meeting of stockholders filed with the SEC on April 7, 2017.

#### Code of Conduct and Ethics

Our Board has adopted a Code of Conduct and Ethics that applies to all of our employees, officers and directors, including our Chief Executive Officer, Chief Financial Officer and other executive and senior officers. We have posted the Code of Conduct and Ethics on our website at <http://ir.vikingtherapeutics.com/governance-highlights> under “Governance Documents”. The Code of Conduct and Ethics can only be amended by the approval of the Audit Committee and any waiver to the Code of Conduct and Ethics for an executive officer or director may only be granted by our Audit Committee and must be timely disclosed as required by applicable law. We expect that any amendments to the Code of Conduct and Ethics, or any waivers of its requirements, will be disclosed on our website.

#### Audit Committee

We have a separately-designated standing Audit Committee established in accordance with Section 3(a)(58)(A) of the Exchange Act. Our Audit Committee is comprised of Dr. Macartney and Messrs. Singleton, Rowland and Webster, with Mr. Singleton serving as Chairperson of the committee. Each member of the Audit Committee must be independent as defined under the applicable rules and listings standards of The Nasdaq Stock Market LLC, or the Nasdaq Rules, and SEC rules and financially literate under the Nasdaq Rules. Our Board has determined that each member of the Audit Committee is “independent” and “financially literate” under the Nasdaq Rules and the SEC rules and that Mr. Singleton is an “audit committee financial expert” under the rules of the SEC. The responsibilities of the Audit Committee are included in a written charter. The Audit Committee acts on behalf of our Board in fulfilling our Board’s oversight responsibilities with respect to our corporate accounting and financial reporting processes, the systems of internal control over financial reporting and audits of financial statements, and also assists our Board in its oversight of the quality and integrity of our financial statements and reports and the qualifications, independence and performance of our independent registered public accounting firm. For this purpose, the Audit Committee performs several functions. The Audit Committee’s responsibilities include:

- appointing, determining the compensation of, retaining, overseeing and evaluating our independent registered public accounting firm and any other registered public accounting firm engaged for the purpose of performing other review or attest services for us;

- determining the engagement of our independent registered public accounting firm;

- prior to commencement of the audit engagement, reviewing and discussing with the independent registered public accounting firm a written disclosure by the prospective independent registered public accounting firm of all relationships between us, or persons in financial oversight roles, and such independent registered public accounting firm or their affiliates;

determining and approving engagements of the independent registered public accounting firm, prior to commencement of the engagement, and the scope of and plans for the audit;

monitoring the rotation of partners of the independent registered public accounting firm on our audit engagement;

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• reviewing with management and the independent registered public accounting firm any fraud that includes management or employees who have a significant role in our internal control over financial reporting and any significant changes in internal controls;

• establishing and overseeing procedures for the receipt, retention and treatment of complaints regarding accounting, internal accounting controls or other auditing matters and the confidential and anonymous submission by employees of concerns regarding questionable accounting or auditing matters;

• reviewing management’s efforts to monitor compliance with our policies designed to ensure compliance with laws and rules; and

• reviewing and discussing with management and the independent registered public accounting firm the results of the annual audit and the independent registered public accounting firm’s assessment of the quality and acceptability of our accounting principles and practices and all other matters required to be communicated to the Audit Committee by the independent registered public accounting firm under generally accepted accounting standards, the results of the independent registered public accounting firm’s review of our quarterly financial information prior to public disclosure and our disclosures in our periodic reports filed with the SEC.

The Audit Committee reviews, discusses and assesses its own performance and composition at least annually. The Audit Committee also periodically reviews and assesses the adequacy of its charter, including its role and responsibilities as outlined in its charter, and recommends any proposed changes to our Board for its consideration and approval.

Typically, the Audit Committee meets at least quarterly and with greater frequency if necessary. Our Board has adopted a written charter of the Audit Committee that is available to stockholders on our internet website at <http://ir.vikingtherapeutics.com/governance-highlights> under “Committee Charters”.

#### Item 11. Executive Compensation.

Our named executive officers for the year ended December 31, 2017, which consist of our principal executive officer and our only other executive officer during the year ended December 31, 2017, are:

• Brian Lian, Ph.D., our Chief Executive Officer; and

• Michael Morneau, our Chief Financial Officer.

#### Summary Compensation Table

The following table sets forth certain information with respect to the compensation paid to our named executive officers for the fiscal years ended December 31, 2017 and 2016:

Name and Principal Position	Year	Salary	Non-Equity	Stock	Option	All Other	Total
		(\$)	Incentive Plan	Awards	Awards (1)	Compensation	(\$)



(1) These amounts represent the aggregate grant date fair value of option awards granted to each named executive officer, computed in accordance with authoritative accounting guidance. These amounts do not represent the actual amounts paid to or realized by the named executive officer. The value as of the grant date for stock options is recognized over the number of days of service required for the stock option to vest in full. 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, 2017 was estimated on the date of grant using the Black-Scholes option pricing model with the following weighted average assumptions: expected volatility: 85.8%; expected term (in years): 6.09; risk-free interest rate: 1.97%; and expected dividend yield: 0%.

(2) Relate to amounts earned by our named executive officers pursuant to our bonus program described below under “—2017 Bonuses”.

Narrative Disclosure to Summary Compensation Table

Employment Agreements

Employment Agreement - President and Chief Executive Officer

We entered into an employment agreement with Brian Lian, Ph.D., as our President and Chief Executive Officer, or the Lian Employment Agreement, which became effective on June 2, 2014. The Lian Employment Agreement is subject to automatic renewals for additional one-year periods following June 2, 2015, unless either party gives the other written notice of its or his election to not renew, or a Lian Non-Renewal Notice. Pursuant to the Lian Employment Agreement, we agreed to nominate Dr. Lian, and to continue to nominate him, to serve as a member of our Board, and Dr. Lian agreed to continue to serve as a member of our Board for as long as he is elected by our stockholders, until his employment with us is terminated. During fiscal year 2016, Dr. Lian’s annual base salary was \$400,000, which was increased to \$440,000 on January 1, 2017, subject to annual review by our Board or Compensation Committee and, if appropriate, increase (but not decrease except in certain limited circumstances). Additionally, the Lian Employment Agreement provides that Dr. Lian will be eligible to receive a target annual bonus in an amount equal to 50% of his base salary in effect on June 30th of each calendar year for 2016 and after, which bonus will be based on our financial performance and Dr. Lian’s individual performance, in each case as determined by our Board or Compensation Committee.

Under the Lian Employment Agreement, on May 4, 2015, Dr. Lian was granted (1) a stock option to purchase 87,500 shares of our common stock, whereby 25% of the shares subject to the option were vested upon grant and 25% of the shares subject to the option will vest on each one-year anniversary of the date of grant for the following three years, so long as Dr. Lian continues to provide service to us on each applicable vesting date; (2) an award of 87,500 shares of common stock, whereby one-third of the shares subject to the award will vest on each one year anniversary of the date of grant for the following three years, so long as Dr. Lian continues to provide service to us on each applicable vesting date, subject to withholding of shares to cover tax withholding obligations arising upon the vesting of shares subject to the award; and (3) an additional award of 16,346 shares of common stock, which were fully vested upon grant, or collectively, the Lian Awards. The Lian Awards were issued under and subject to the terms and conditions of the 2014 Equity Incentive Plan.

Dr. Lian’s employment with us is at-will, meaning either we or Dr. Lian may terminate the employment relationship at any time, with or without cause. However, Dr. Lian must provide at least 60 days’ written notice of resignation. If we terminate Dr. Lian’s employment, then, so long as Dr. Lian complies with certain obligations, including execution and delivery of a general release within a specified period of time, we will pay Dr. Lian: (1) his base salary as of the termination date for six months following the termination date, if such termination is pursuant to a Lian Non-Renewal Notice, disability or death, or for 12 months in the case of termination other than by Lian Non-Renewal Notice, for cause, disability or death; (2) six monthly payments if such termination is pursuant to a Lian Non-Renewal Notice, disability or death, or 12 monthly payments in the case of termination other than by Lian Non-Renewal Notice, for

cause, disability or death, in each case equal to 1/12 of the amount equal to Dr. Lian's target annual bonus percentage as of the termination date multiplied by Dr. Lian's base salary as of such date; and (3) subject to Dr. Lian's timely election of COBRA, the amount equal to the COBRA premiums for the lesser of (a) six months if such termination is pursuant to a Lian Non-Renewal Notice, disability or death, or 12 months in the case of termination other than by Lian Non-Renewal Notice, for cause, disability or death, or (b) until Dr. Lian becomes eligible to enroll in another employer-sponsored group health plan. Additionally, if Dr. Lian's employment is terminated by us (i) pursuant to a Lian Non-Renewal Notice, disability or death, the outstanding equity awards subject to the Lian Awards that would have vested within six months following the termination date will vest and become fully exercisable as of such termination date, and Dr. Lian will have six months from the termination date to exercise vested options under the Lian Awards (unless they terminate sooner pursuant to their terms), and (ii) other than by Lian Non-Renewal Notice, for cause, disability or death, the outstanding equity awards subject to the Lian Awards that would have vested within 12 months following the termination date will vest and become fully exercisable as of the termination date, and Dr. Lian will have 12 months from the termination date to exercise

vested options under the Lian Awards (unless they terminate sooner pursuant to their terms). In each case, all other equity awards subject to the Lian Awards will terminate without compensation therefore on the termination date. Furthermore, if Dr. Lian resigns for good reason, he will be entitled to receive the same payments and accelerated vesting as if he had been terminated other than by Lian Non-Renewal Notice, for cause, disability or death, as set forth above.

In the event of a change in control of our company, 100% of the unvested outstanding equity awards granted under the Lian Awards will vest and become fully exercisable immediately prior to the change in control. Additionally, if any vested equity awards held by Dr. Lian are not assumed or substituted for in accordance with certain conditions, we will pay cash to Dr. Lian on the change in control in exchange for the satisfaction and cancellation of the outstanding equity awards. If Dr. Lian's employment is terminated within 24 months following a change in control, subject to certain conditions, he will be entitled to receive the same payments and accelerated vesting as if he had been terminated other than by Lian Non-Renewal Notice, for cause, disability or death, as set forth above; however, he will be entitled to such payments for a period of 18 months and the vesting of the Lian Awards will be accelerated by 18 months.

#### Employment Agreement - Chief Financial Officer

We entered into an employment agreement with Michael Morneau, as our Chief Financial Officer, or the Morneau Employment Agreement, which became effective on May 21, 2014. The Morneau Employment Agreement is subject to automatic renewals for additional one-year periods following May 21, 2015, unless either party gives the other written notice of its or his election to not renew, or a Morneau Non-Renewal Notice. Pursuant to the terms of the Morneau Employment Agreement, Mr. Morneau's base salary was initially \$189,000 per year. Effective October 1, 2014, Mr. Morneau agreed to an amended base salary of \$135,000. During fiscal year 2016, Mr. Morneau's annual base salary was \$285,000, which was increased to \$300,000 on January 1, 2017, subject to annual review by our Board or Compensation Committee and, if appropriate, increase (but not decrease except in certain limited circumstances). Additionally, the Morneau Employment Agreement provides that Mr. Morneau will be eligible to receive a target annual bonus in an amount equal to 35% of his base salary in effect on June 30th of each calendar year for 2016 and after, which bonus will be based on our financial performance and Mr. Morneau's individual performance, in each case as determined by our Board or Compensation Committee.

Under the Morneau Employment Agreement, on May 4, 2015, Mr. Morneau was granted (1) a stock option to purchase 25,500 shares of our common stock, whereby 25% of the shares subject to the option were vested upon grant and 25% of the shares subject to the option will vest on each one-year anniversary of the date of grant for the following three years, so long as Mr. Morneau continues to provide service to us on each applicable vesting date; (2) an award of 67,000 shares of common stock, whereby one-third of the shares subject to the award will vest on each one-year anniversary of the date of grant for the following three years, so long as Mr. Morneau continues to provide service to us on each applicable vesting date, subject to withholding of shares to cover tax withholding obligations arising upon the vesting of shares subject to the award; and (3) an additional award of 10,404 shares of common stock, which were fully vested upon grant, or collectively, the Morneau Awards. The Morneau Awards were issued under and subject to the terms and conditions of the 2014 Equity Incentive Plan.

Mr. Morneau's employment with us is at-will, meaning either we or Mr. Morneau may terminate the employment relationship at any time, with or without cause. However, Mr. Morneau must provide at least 60 days' written notice of resignation. If we terminate Mr. Morneau's employment, then, so long as Mr. Morneau complies with certain obligations, including execution and delivery of a general release within a specified period of time, we will pay Mr. Morneau: (1) his base salary as of the termination date for three months following the termination date, if such termination is pursuant to a Morneau Non-Renewal Notice, disability or death, or for six months in the case of termination other than by Morneau Non-Renewal Notice, for cause, disability or death; (2) three monthly payments if such termination is pursuant to a Morneau Non-Renewal Notice, disability or death, or six monthly payments in the case of termination other than by Morneau Non-Renewal Notice, for cause, disability or death, in each case equal

to 1/12 of the amount equal to Mr. Morneau's target annual bonus percentage as of the termination date multiplied by Mr. Morneau's base salary as of such date; and (3) subject to Mr. Morneau's timely election of COBRA, the amount equal to the COBRA premiums for the lesser of (a) three months if such termination is pursuant to a Morneau Non-Renewal Notice, disability or death, or six months in the case of termination other than by Morneau Non-Renewal Notice, for cause, disability or death, or (b) until Mr. Morneau becomes eligible to enroll in another employer-sponsored group health plan. Additionally, if Mr. Morneau's employment is terminated by us (i) pursuant to a Morneau Non-Renewal Notice, disability or death, the outstanding equity awards subject to the Morneau Awards that would have vested within three months following the termination date will vest and become fully exercisable as of such termination date, and Mr. Morneau will have three months from the termination date to exercise vested options under the Morneau Awards (unless they terminate sooner pursuant to their terms), and (ii) other than by Morneau Non-Renewal Notice, for cause, disability or death, the outstanding equity awards subject to the Morneau Awards that would have vested within six months following the termination date will vest and become fully exercisable as of the termination date, and Mr. Morneau will have six months from the termination date to exercise vested options under the Morneau Awards (unless they terminate sooner pursuant to their terms). In each case, all other equity awards subject to the Morneau Awards will terminate without compensation therefore on the termination date. Furthermore, if



Mr. Morneau resigns for good reason, he will be entitled to receive the same payments and accelerated vesting as if he had been terminated other than by Morneau Non-Renewal Notice, for cause, disability or death, as set forth above.

In the event of a change in control of our company, 100% of the unvested outstanding equity awards granted under the Morneau Awards will vest and become fully exercisable immediately prior to the change in control. Additionally, if any vested equity awards held by Mr. Morneau are not assumed or substituted for in accordance with certain conditions, we will pay cash to Mr. Morneau on the change in control in exchange for the satisfaction and cancellation of the outstanding equity awards. If Mr. Morneau's employment is terminated within 24 months following a change in control, subject to certain conditions, he will be entitled to receive the same payments and accelerated vesting as if he had been terminated other than by Morneau Non-Renewal Notice, for cause, disability or death, as set forth above; however, he will be entitled to such payments for a period of 12 months and the vesting of the Morneau Awards will be accelerated by 12 months.

#### 2017 Bonuses

In March 2017, the Compensation Committee adopted corporate performance objectives for the 2017 bonus program for our named executive officers based on milestones that primarily included the topline data readouts for VK5211 and VK2809 Phase 2 clinical trials, completion of a proof-of-concept study in GSD1, the completion of a financing, signing partnerships for one or two of the Company's programs, expansion of infrastructure and human capital, an increase in our institutional stockholder base and completing a second financing.

The Compensation Committee determined that, on a whole, not all of the corporate goals were met during 2017. Accordingly, on January 19, 2018, the Compensation Committee approved the following bonuses for our named executive officers:

- a bonus of \$110,000 for Dr. Lian, and

- a bonus of \$52,500 for Mr. Morneau.

The bonus amount awarded to Dr. Lian for 2017 was equal to 25% of his base compensation (representing 50% of his target bonus for 2017 of 50% of his base compensation). The bonus amount awarded to Mr. Morneau for 2017 was equal to 17.5% of his base compensation (representing 50% of his target bonus for 2017 of 35% of his base compensation).

#### Potential Payments Upon Termination or Change in Control

Our executive officers will be entitled to receive certain payments and benefits upon termination of their employment or a change in control of our company, as described under the section entitled "Employment Agreements" above.

#### Perquisites, Health, Welfare and Retirement Plans and Benefits

##### Health and Welfare Benefits

Our named executive officers are eligible to participate in all of our employee benefit plans, including our medical, dental, vision, group life and disability insurance plans, in each case on the same basis as other employees.

##### Perquisites and Personal Benefits

We do not currently provide perquisites or personal benefits to our named executive officers.

Pension Benefits and Non-Qualified Deferred Compensation

Commencing as of November 30, 2015, we maintain a 401(k) defined contribution plan in which all of our employees age 21 and older and who work, on average, at least 20 hours per week, are entitled to participate. Employees contribute their own funds, as salary deductions, on a pre-tax basis. Contributions will be permitted to be made up to plan limits, subject to government limitations. We do not currently intend to provide full or partial matching contributions under the 401(k) plan.

Outstanding Equity Awards at December 31, 2017

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The following table presents the outstanding option and stock awards held by each of our named executive officers as of December 31, 2017.

Name	Grant Date	Options Awards			Stock Awards			Equity Incentive Plan Awards: Market or Payout Value of Unearned Shares, or Other Rights That Have Not Vested (\$)
		Number of Securities Underlying Unexercised Options (#)	Number of Securities Underlying Option	Exercise Price (\$)	Expiration Date	Number of Shares or Units of Stock That Have Not Vested (#)	Market Value of Shares or Units of Stock That Have Not Vested (\$)	
Brian Lian, Ph.D.	1/5/2017 <sup>(1)</sup>	—	250,000	\$ 1.23	1/5/2027	—	—	—
	1/5/2017	34,931	—	\$ 1.23	1/5/2027	—	—	—
	3/14/2016	54,336	—	\$ 1.88	3/14/2026	—	—	—
	3/4/2016 <sup>(1)</sup>	31,250	93,750	\$ 2.15	3/4/2026	—	—	—
	5/4/2015 <sup>(2)</sup>	65,625	21,875	\$ 9.49	5/4/2025	—	—	—
	5/4/2015	—	—	—	—	—	29,166 <sup>(3)</sup>	\$276,785
	2/20/2014	—	—	—	—	183,095 <sup>(5)</sup>	\$30,778 <sup>(6)</sup>	—
Michael Morneau	1/5/2017 <sup>(1)</sup>	—	95,000	\$ 1.23	1/5/2027	—	—	—
	1/5/2017	17,605	—	\$ 1.23	1/5/2027	—	—	—
	3/14/2016	19,934	—	\$ 1.88	3/14/2026	—	—	—
	3/4/2016 <sup>(1)</sup>	10,000	30,000	\$ 2.15	3/4/2016	—	—	—
	5/4/2015 <sup>(2)</sup>	19,125	6,375	\$ 9.49	5/4/2025	—	—	—
	5/4/2015	—	—	—	—	—	22,333 <sup>(3)</sup>	\$211,940

(1) The shares subject to each stock option vest over a four-year period, with 25% of the shares subject to the option vesting on each anniversary of the grant date, with partial or full vesting under certain circumstances, including upon a change in control of our company or various events specified in the named executive officer's employment agreement, if applicable. The option awards remain exercisable until they expire ten years from the date of grant subject to earlier expiration following termination of employment.

(2)

The shares subject to each stock option vest over a three-year period, with 25% of the shares subject to the option vested on the grant date and 25% of the shares subject to the option vesting on each anniversary of the grant date, with partial or full vesting under certain circumstances, including upon a change in control of our company or various events specified in the named executive officer's employment agreement, if applicable. The option awards remain exercisable until they expire ten years from the date of grant subject to earlier expiration following termination of employment.

(3) The shares subject to each restricted stock award vest over a three-year period, with one-third of the shares subject to the restricted stock award vesting on each anniversary of the grant date, with partial or full vesting under certain circumstances, including upon a change in control of our company or various events specified in the named executive officer's employment agreement, if applicable.

(4) These amounts represent the aggregate grant date fair value of restricted stock awards granted to each named executive officer. These amounts do not represent the actual amounts paid to or realized by the named executive officer. The value as of the grant date for the restricted stock awards is calculated based on the number of shares granted and the grant date market price and is recognized once the requisite service period for the restricted stock is satisfied. The grant date fair value per share was \$9.49 at May 4, 2015.

(5) These shares are subject to a repurchase option and vest upon the achievement of a milestone set forth in the Common Stock Purchase Agreement, as amended, pursuant to which these shares were issued.

(6) The amount represents the aggregate grant date fair value of a stock award granted to the named executive officer, computed in accordance with authoritative accounting guidance. This amount does not represent the actual amount paid to or realized by the named executive officer. 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.

#### Non-Employee Director Compensation

Our Board has adopted a compensation policy for our non-employee directors that consists of annual retainer fees and long-term equity awards. Under this policy, each non-employee director will receive an annual retainer of \$33,170 for serving on our Board. The Chairperson of our Board will receive an additional annual retainer of \$32,800, the chairperson of the Audit Committee will receive an additional annual retainer of \$16,650, the chairperson of the Compensation Committee will receive an additional annual retainer of \$11,350 and the chairperson of the Nominating and Corporate Governance Committee will receive an additional annual retainer of \$9,280. Each other member of the Audit Committee will receive an additional annual retainer of \$8,900, each other member of the Compensation Committee will receive an additional annual retainer of \$6,750 and each other member of the Nominating and Corporate Governance Committee will receive an additional annual retainer of \$4,900. All cash retainers will be earned on a quarterly basis based on a calendar quarter, and, if applicable, will be prorated for the portion of the calendar quarter during which such non-employee director actually serves on our Board or a committee thereof, and will be paid in arrears no later than the 30th day following the end of each calendar quarter.

In addition to cash fees, each non-employee director will be granted on the first business day of each calendar year a stock option to purchase 16,000 shares of our common stock. If a non-employee director joins our Board other than at an annual meeting of our stockholders, such non-employee director will be granted on the date such individual first becomes appointed or elected as a non-employee director (1) a stock option to purchase 32,000 shares of our common stock and (2) a stock option to purchase 16,000 shares of our common stock, reduced pro rata for each day prior to the date of grant that has elapsed since January 1st of the year in which the individual first becomes a non-employee director. Annual equity awards and equity awards granted to new non-employee directors will vest in full on the one-year anniversary of the applicable date of grant, subject to the director's continuous service through such date.

Each initial equity award and each annual equity award will have a maximum term of ten years and will be made in the form of nonstatutory stock options. For any non-employee director serving at the time of a change in control of our company (as defined in our 2014 Equity Incentive Plan), all then-outstanding and unvested compensatory equity awards granted under the non-employee director compensation policy would become fully vested and exercisable, if applicable, immediately prior to the change in control.

The following table sets forth summary information concerning compensation paid or accrued to the members of our Board for services rendered to us for the fiscal year ended December 31, 2017.

Name <sup>(1)</sup>	Fees Earned or Paid in Cash	Option Awards <sup>(2)(3)</sup>	Total
Lawson Macartney, DVM, Ph.D. <sup>(4)</sup>	\$84,150	\$ 13,278	\$97,428
Matthew W. Foehr <sup>(4)</sup>	\$33,170	\$ 13,278	\$46,448
Charles A. Rowland, Jr. <sup>(5)</sup>	\$20,126	\$ 29,616	\$49,742
J. Matthew Singleton <sup>(4)</sup>	\$56,570	\$ 13,278	\$69,848

Stephen W. Webster <sup>(4)</sup>	\$58,320	\$ 13,278	\$71,598
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(1) Brian Lian, Ph.D., our President and Chief Executive Officer and a named executive officer, is not included in this table as he is an employee of ours and therefore receives no compensation for his service as a director. Dr. Lian's compensation is included in the section entitled "—Summary Compensation Table" above.

(2) On January 3, 2017, each of our then-current non-employee directors received an unvested option to purchase 16,000 shares of our common stock, which became fully vested on January 3, 2018.

- (3) These amounts represent the aggregate grant date fair value of option awards granted to each listed director in 2017, computed in accordance with authoritative accounting guidance. These amounts do not represent the actual amounts paid to or realized by the directors during 2017. The value as of the grant date for stock options is recognized over the number of days of service required for the stock option to vest in full. 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, 2017 was estimated on the date of grant using the Black-Scholes option pricing model with the following weighted average assumptions: expected volatility: 84.03%; expected term (in years): 5.5; risk-free interest rate 1.97%; and expected dividend yield: 0%.
- (4) Total options to purchase shares of our common stock held by this director as of December 31, 2017 was 52,786.
- (5) Mr. Rowland was appointed to our Board effective July 1, 2017. In connection with his appointment to our Board, on July 1, 2017, he was granted an option to purchase 40,000 shares of our common stock, which will vest in full on July 1, 2018. As of December 31, 2017, Mr. Rowland held options to purchase an aggregate of 40,000 shares of our common stock.

#### Compensation Committee Interlocks and Insider Participation; Compensation Committee Report

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

##### Securities Authorized for Issuance Under Equity Compensation Plans

The following table sets forth additional information as of December 31, 2017 with respect to the shares of common stock that may be issued upon the exercise of options and other rights under our existing equity compensation plans and arrangements in effect as of December 31, 2017. The information includes the number of shares covered by, and the weighted average exercise price of, outstanding options and the number of shares remaining available for future grant, excluding the shares to be issued upon exercise of outstanding options.

##### Equity Compensation Plan Information

Plan Category	Number of securities to be issued upon exercise of outstanding	Weighted-average price of outstanding	Number of securities remaining available for future issuance
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	options, warrants and rights (a)	options, warrants and rights	under equity compensation plans  (excluding securities reflected in  column (a))
Equity compensation plans approved by security holders <sup>(1)</sup>	1,622,114	\$ 2.60	1,363,086 <sup>(2)</sup>
Equity compensation plans not approved by security holders <sup>(3)</sup>	—	—	—
<b>Total</b>	<b>1,622,114</b>	<b>\$ 2.60</b>	<b>1,363,086</b>

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(1) Comprised of the 2014 Equity Incentive Plan, or the 2014 EIP, and the 2014 Employee Stock Purchase Plan, or the 2014 ESPP.



(2) Comprised solely of 669,698 shares subject to awards available for future issuance under the 2014 EIP and 693,388 shares of common stock that may be issued under the 2014 ESPP, in each case as of December 31, 2017. Pursuant to the terms of the 2014 EIP, the share reserve of the 2014 EIP will automatically increase on January 1st, through fiscal 2024, by 3.5% of the total number of shares of our common stock outstanding on December 31st of the preceding calendar year. Pursuant to the terms of the 2014 ESPP, the share reserve of the 2014 ESPP will automatically increase on January 1st, through fiscal 2024, by 1% of the total number of shares of our common stock outstanding on December 31st of the preceding calendar year.

(3) As of December 31, 2017, we did not have any equity compensation plans that were not approved by our stockholders.

#### Security Ownership of Certain Beneficial Owners and Management

The following table sets forth information as of February 28, 2018 with respect to the beneficial ownership of shares of our common stock by:

• each of our directors;

• each of our named executive officers;

• all of our current directors and executive officers as a group; and

• each person or group known to us to be the beneficial owner of more than five percent of our common stock. This table is based upon information supplied by officers, directors and principal stockholders and a review of Schedules 13D and 13G, if any, filed with the SEC. Other than as set forth below, we are not aware of any other beneficial owner of more than five percent of our common stock as of February 28, 2018. Except as indicated by the footnotes below, we believe, based on the information furnished to us, that the persons and entities named in the table below have sole voting and investment power with respect to all shares of common stock that they beneficially own, subject to applicable community property laws.

Applicable percentage ownership is based on 50,898,802 shares of common stock outstanding as of February 28, 2018, adjusted as required by rules promulgated by the SEC. These rules generally attribute beneficial ownership of securities to persons who possess sole or shared voting power or investment power with respect to those securities. In addition, the rules include shares of common stock issuable pursuant to the exercise of stock options that are either immediately exercisable or exercisable on or before April 29, 2018, which is 60 days after February 28, 2018. These shares are deemed to be outstanding and beneficially owned by the person holding such options for the purpose of computing the percentage ownership of that person, but they are not treated as outstanding for the purpose of computing the percentage ownership of any other person.

Unless otherwise noted below, the address of each beneficial owner listed in the table is c/o Viking Therapeutics, Inc., 12340 El Camino Real, Suite 250, San Diego, CA 92130.

Name of Beneficial Owner	Beneficial Ownership of Common Stock	
	Number of Shares	Percent of Class <sup>(1)</sup>
Named Executive Officers and Directors:		
Brian Lian, Ph.D. <sup>(2)</sup>	1,732,990	3.4%
Michael Morneau <sup>(3)</sup>	187,065	*
Matthew W. Foehr <sup>(4)</sup>	112,036	*
Lawson Macartney, DVM, Ph.D. <sup>(5)</sup>	52,786	*
Charles A. Rowland, Jr.	—	—
Matthew Singleton <sup>(5)</sup>	52,786	*
Stephen W. Webster <sup>(5)</sup>	52,786	*
All current executive officers and directors as a group <sup>(7 persons)</sup> <sup>(6)</sup>	2,190,449	4.3%
Greater than 5% Stockholders:		
Ligand Pharmaceuticals Incorporated <sup>(7)</sup>	7,820,964	14.9%
G. Nicholas Farwell <sup>(8)</sup>	2,490,008	4.9%
Sphera Funds Management Ltd. <sup>(9)</sup>	2,145,300	4.2%
Boxer Capital, LLC <sup>(10)</sup>	3,000,000	5.9%

\*Less than one percent.

(1) For each person and group included in this table, percentage ownership is calculated by dividing the number of shares beneficially owned by such person or group by the sum of shares of common stock outstanding as of February 28, 2018, plus the number of shares of common stock that such person or group had the right to acquire within 60 days after February 28, 2018.

(2) Consists of: (a) 1,453,098 shares of common stock owned directly, of which 1,240,836 are vested or will vest within 60 days of February 28, 2018, and (b) 279,892 shares of common stock issuable upon exercise of options exercisable within 60 days of February 28, 2018.

(3) Consists of: (a) 86,651 shares of common stock owned directly, of which 64,317 are vested or will vest within 60 days of February 28, 2018, and (b) 100,414 shares of common stock issuable upon exercise of options exercisable within 60 days of February 28, 2018.

(4) Consists of: (a) 39,250 shares of common stock owned directly, (b) 52,786 shares of common stock issuable upon exercise of options exercisable within 60 days of February 28, 2018, and (c) 20,000 shares of common stock issuable upon exercise of warrants exercisable within 60 days of February 28, 2018.

- (5) Consists solely of shares of common stock issuable upon exercise of options exercisable within 60 days of February 28, 2018.
- (6) Consists of: (a) 1,578,999 shares of common stock owned directly by all of our current executive officers and directors, of which 1,344,403 are vested or will vest within 60 days of February 28, 2018, (b) 591,450 shares of common stock issuable upon exercise of options exercisable within 60 days of February 28, 2018, and (c) 20,000 shares of common stock issuable upon exercise of warrants exercisable within 60 days of February 28, 2018.

- (7) Ligand Pharmaceuticals Incorporated's address is 3911 Sorrento Valley Blvd, #110, San Diego, CA 92121. Consists of: (a) 5,418,490 shares of common stock owned directly by Ligand Pharmaceuticals Incorporated, or Ligand, (b) 882,474 shares of common stock owned by Metabasis Therapeutics, Inc., a wholly-owned subsidiary of Ligand, and (c) 1,520,000 shares of common stock issuable upon exercise of warrants held by Ligand that are exercisable within 60 days of February 28, 2018. Beneficial ownership is based solely on information contained in the Schedule 13D/A filed with the Securities and Exchange Commission on April 15, 2016 by Ligand.
- (8) The number of shares consists of: (1) 950,000 shares of Common Stock held by a family trust of which the Mr. Farwell is one of the trustees and over which Mr. Farwell shares voting and dispositive power, (2) 500,000 shares of Common Stock held directly by Mr. Farwell, (3) 1,040,008 shares of Common Stock held in the Selling Stockholder's IRA, (4) 123,750 shares of Common Stock issuable upon exercise of the Warrant held by a family trust of which Mr. Farwell is one of the trustees and over which the Selling Stockholder shares voting and dispositive power, (5) 75,000 shares of Common Stock issuable upon exercise of the Warrant held directly by Mr. Farwell, (6) 273,750 shares of Common Stock issuable upon exercise of the Warrant held in the Selling Stockholder's IRA, (7) 540,000 shares of Common Stock issuable upon exercise of an additional warrant to purchase shares of Common Stock held by a family trust of which the Selling Stockholder is one of the trustees and over which the Selling Stockholder shares voting and dispositive power, (8) 185,000 shares of Common Stock issuable upon exercise of an additional warrant to purchase shares of Common Stock held directly by Mr. Farwell, and (9) 573,750 shares of Common Stock issuable upon exercise of an additional warrant to purchase shares of Common Stock held in the Selling Stockholder's IRA, except in the cases of items (4) through (9) to the extent any such exercise is restricted by the Warrant Blocker. The business address of Mr. Farwell is 1240 Arbor Road, Menlo Park, CA 94025.
- (9) Consists of 2,081,370 shares of Common Stock held directly by Sphera Global Healthcare Master Fund, which has delegated its investment management authority to Sphera Global Healthcare Management Ltd. (the "Management Company"), and 63,930 shares of Common Stock held directly by HFR HE Sphera Global Healthcare Master Trust, which has delegated its investment management authority to the Management Company. The Management Company is managed, controlled, and operated by its general partner, Sphera Global Healthcare GP Ltd., which is controlled jointly by Sphera Funds Management Ltd. and Moshe Arkin. Beneficial ownership is based solely on information contained in the Schedule 13G filed with the Securities and Exchange Commission on December 18, 2017 by Mr. Arkin, Sphera Funds Management Ltd., Sphera Global Healthcare GP Ltd. and Sphera Global Healthcare Management LP. The business address of Mr. Arkin is 6 Hachoshlim St., Herzelia, Israel and the business address of Sphera Funds Management Ltd., Sphera Global Healthcare GP Ltd. and Sphera Global Healthcare Management LP is 21 Ha'arba'ah Street, Tel Aviv 64739, Israel.
- (10) Boxer Asset Management Inc. ("Boxer Management") is the managing member and majority owner of Boxer Capital, LLC ("Boxer Capital"). Joe Lewis is the sole indirect beneficial owner of and controls Boxer Management. Beneficial ownership is based solely on information contained in the Schedule 13G filed with the Securities and Exchange Commission on February 12, 2018 by Boxer Management, Boxer Capital and Mr. Lewis. The principal business address of Boxer Capital is: 11682 El Camino Real, Suite 320, San Diego, CA 92130. The principal business address of Boxer Management and Joe Lewis is: Cay House, EP Taylor Drive N7776, Lyford Cay, New Providence, Bahamas.

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

Policies and Procedures for Related Party Transactions

Our Board has adopted a written related person transaction policy to set forth the policies and procedures for the review and approval or ratification of related person transactions. This policy covers, with certain exceptions set forth in Item 404 of Regulation S-K under the Securities Act of 1933, as amended, or the Securities Act, any transaction, arrangement, or relationship, or any series of similar transactions, arrangements, or relationships in which we were or are to be a participant, the amount involved exceeds \$120,000, and a related person had or will have a direct or indirect material interest, including, without limitation, purchases of goods or services by or from the related person or entities in which the related person has a material interest, indebtedness, guarantees of indebtedness, and employment by us of a related person. A related person is any individual who is, or who has been since the beginning of our last fiscal year, one of our directors or executive officers, or a nominee to become one of our directors, or any person known to be the beneficial owner of more than 5% of any class of our voting securities, or any immediate family member of any of the foregoing persons. Additionally, any firm, corporation or other entity by which any of the foregoing persons is employed or in which such person is a general partner or principal, or in a similar position, or in which such person has a 10% or greater beneficial ownership interest, will also be deemed to be a related person. Transactions involving compensation for services provided to us as an employee, consultant or director are not considered related-person transactions under this policy. As provided by our audit committee charter, our Audit Committee is responsible for reviewing and approving in advance any related party transaction.

## Transactions with Related Persons

In addition to the director and executive officer compensation arrangements discussed in Part III, “Item 11. Executive Compensation” of this Annual Report on Form 10-K, the following is a summary of material provisions of transactions since January 1, 2017 that we have been a party to and in which the amount involved exceeded or will exceed the lesser of \$120,000 or one percent of the average of our total assets at year end for the last two completed fiscal years, and in which any of our directors, executive officers, beneficial owners of more than 5% of our capital stock, or their immediate family members, have had or will have a direct or indirect material interest.

### Agreements with Ligand

On May 21, 2014, we entered into the Master License Agreement with Ligand, a greater than 5% holder of our outstanding common stock, pursuant to which Ligand granted us worldwide rights under (1) patents related to SARM Compounds, TRβ Compounds, FBPase Compounds, EPOR Compounds and DGAT-1 Compounds; (2) related know-how controlled by Ligand; and (3) physical quantities of SARM, TRβ, FBPase, EPOR and DGAT-1 Compounds. In connection with the Master License Agreement, we also entered into a Loan and Security Agreement with Ligand, dated May 21, 2014, as amended on April 8, 2015 and January 22, 2016, and issued to Ligand a Secured Convertible Promissory Note, or the Ligand Note, pursuant to which Ligand loaned us an initial principal amount of \$2,500,000. 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 accrues 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. 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, or May 21, 2018, unless the loans are repaid in cash or converted into equity prior to such time. On April 13, 2016, we completed an underwritten public offering of our common stock and warrants to purchase shares of our common stock, or the April 2016 Offering. Under the terms of the Loan and Security Agreement, upon the consummation of the April 2016 Offering, we were required to repay \$1,500,000 of the Ligand Note obligation to Ligand. Accordingly, we repaid \$1,500,000 of the Ligand Note with \$300,000 paid in cash and \$1,200,000 paid in the form of 960,000 shares of our common stock and a warrant to purchase up to 960,000 shares of our common stock, or the Ligand Warrant. The Ligand Warrant has an exercise price of \$1.50 per share of common stock, was immediately exercisable upon issuance and will expire on April 13, 2021. On May 8, 2017, we entered into an amendment to the Loan and Security Agreement, which, (1) extended the maturity date of the loans from May 21, 2017 to May 21, 2018, and (2) provided that we were required to make a repayment in the amount of \$200,000, or the Required Repayment. We made the Required Repayment on July 13, 2017. The Required Repayment was applied, first, to accrued and unpaid interest on the loans and, second, to the unpaid principal amount of the Loans. Each \$1.00 of value of the Required Repayment reduced the amount of accrued and unpaid interest and then unpaid principal amount on the Loans by \$0.50. In connection with the Master License Agreement, we also entered into a Registration Rights Agreement with Ligand, pursuant to which we granted certain registration rights to Ligand with respect to the securities issued by us to Ligand under the Master License Agreement and the securities issued or issuable by us to Ligand pursuant to the Ligand Note. On February 14, 2017, we filed a Registration Statement on Form S-3 under the Securities Act covering the resale of the full amount of the securities issued or issuable by us to Ligand under the Master License Agreement and the Ligand Note.

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.

### Limitation of Liability and Indemnification of Officers and Directors

Our amended and restated certificate of incorporation and amended and restated bylaws contain provisions that limit the liability of our directors for monetary damages to the fullest extent permitted by Delaware law. Consequently, our directors are not personally liable to us or our stockholders for monetary damages for any breach of fiduciary duties as directors, except liability for the following:

- any breach of their duty of loyalty to our company or our stockholders;

- any act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;

•unlawful payments of dividends or unlawful stock repurchases or redemptions as provided in Section 174 of the General Corporation Law of the State of Delaware, or the DGCL; and

•any transaction from which they derived an improper personal benefit.

Any amendment to, or repeal of, these provisions will not eliminate or reduce the effect of these provisions in respect of any act, omission or claim that occurred or arose prior to that amendment or repeal. If the DGCL is amended to provide for further limitations on the personal liability of directors of corporations, then the personal liability of our directors will be further limited to the greatest extent permitted by the DGCL.

Our amended and restated certificate of incorporation provides that we will, under certain circumstances, indemnify our directors, officers, employees or agents, subject to any provisions contained in our amended and restated bylaws. Our amended and restated bylaws provide that we will indemnify, to the fullest extent permitted by law, any person who is or was or is made a party or is threatened to be made a party to, or is otherwise involved in, any action, suit or proceeding, whether civil, criminal, administrative or investigative, by reason of the fact that he or she, or a person for whom he or she is the legal representative, is or was one of our directors or officers, or is or was serving at our request as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise, against all expense, liability and loss (including, among other things, attorney's fees and amounts paid in settlement) reasonably incurred or suffered by such director, officer, employee or agent in connection therewith, subject to certain conditions. Our amended and restated bylaws also provide us with the power to, to the extent authorized by our Board, grant rights to indemnification and to advancement of expenses to any of our employees or agents to the fullest extent indemnification may be granted to our directors and officers. In addition, our amended and restated bylaws also provide that we must advance expenses incurred by or on behalf of a director or officer in advance of the final disposition of any action or proceeding, subject to certain exceptions.

Further, we have indemnification agreements with each of our directors and executive officers that may be broader than the specific indemnification provisions contained in the DGCL. These indemnification agreements require us, among other things, to indemnify our directors and executive officers against liabilities that may arise by reason of their status or service. These indemnification agreements also require us to advance all expenses incurred by the directors and executive officers in investigating or defending any such action, suit or proceeding, subject to certain exceptions. We believe that these agreements are necessary to attract and retain qualified individuals to serve as directors and executive officers.

The limitation of liability and indemnification provisions that are included in our amended and restated certificate of incorporation, amended and restated bylaws and in indemnification agreements that we entered into with our directors and executive officers may discourage stockholders from bringing a lawsuit against our directors and executive officers for breach of their fiduciary duties. They may also reduce the likelihood of derivative litigation against our directors and executive officers even though an action, if successful, might benefit us and other stockholders. Further, a stockholder's investment may be adversely affected to the extent that we pay the costs of settlement and damage awards against directors and executive officers as required by these indemnification provisions. At present, we are not aware of any pending litigation or proceeding involving any person who is or was one of our directors, officers, employees or other agents or is or was serving at our request as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise, for which indemnification is sought, and we are not aware of any threatened litigation that may result in claims for indemnification.

Our amended and restated bylaws provide that we may purchase and maintain insurance, at our expense, to protect us and any person who is or was a director, officer, employee or agent of us or is or was serving at our request as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise against any expense, liability or loss, whether or not we would have the power to indemnify such person against such expense, liability or loss under the DGCL. We maintain insurance under which, subject to the limitations of the insurance policies, coverage is provided to our directors and executive officers against loss arising from claims made



by reason of breach of fiduciary duty or other wrongful acts as a director or executive officer, including claims relating to public securities matters, and to us with respect to payments that may be made by us to these directors and executive officers pursuant to our indemnification obligations or otherwise as a matter of law.

Certain of our non-employee directors may, through their relationships with their employers, be insured or indemnified against certain liabilities incurred in their capacity as members of our Board.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers or persons controlling our company pursuant to the foregoing provisions, we have been informed that, in the opinion of the Securities and Exchange Commission, such indemnification is against public policy as expressed in the Securities Act and is therefore unenforceable.

#### Director Independence

Under the rules and listings standards of the Nasdaq Rules, a majority of the members of our Board must satisfy the Nasdaq criteria for “independence.” No director qualifies as independent under the Nasdaq Rules unless our Board affirmatively determines that the director does not have a relationship with us that would impair independence (directly or as a partner, stockholder or officer of an organization that has a relationship with us). Our Board has determined that Dr. Macartney and Messrs. Singleton, Rowland and Webster are independent directors as defined under the Nasdaq Rules. Dr. Lian is not independent under the Nasdaq Rules as a result of his position as our President and Chief Executive Officer. Mr. Foehr is not independent under the Nasdaq Rules in light of the Master License Agreement, as amended from time to time, and related agreements between us and Ligand, and Mr. Foehr’s position as an executive officer of Ligand.

#### Item 14. Principal Accounting Fees and Services.

##### Principal Accountant Fees and Services

The following table represents aggregate fees billed to us for the fiscal years ended December 31, 2017 and December 31, 2016 by Marcum LLP, our independent registered public accounting firm for such periods. All fees described below were approved by the Audit Committee.

	Fiscal Year Ended	
	December 31,	
	2017	2016
Audit Fees (1)	\$ 159,160	\$ 155,921
Audit-Related Fees (2)	74,572	157,230
Tax Fees	—	—
All Other Fees (3)	3,145	—
Total Fees	\$ 236,877	\$ 313,151

(1) Audit fees consist of fees billed for services rendered for the audit of our annual financial statements, including review of the interim financial statements included in quarterly reports.

(2) Audit-related fees consist of fees for assurance and related services that are traditionally performed by our independent registered public accounting firm and include fees reasonably related to the performance of the audit or review of our interim financial statements and due diligence services and not reported under the caption “Audit Fees” and includes review of our registration statement for our initial and subsequent public offerings, and related services that are normally provided in connection with statutory and regulatory filings or engagements .

(3) All Other Fees consist of fees related to a Marcum conference that Viking attended and presented at.

##### Audit Committee’s Pre-Approval Policies and Procedures

The Audit Committee has adopted a policy for the pre-approval of audit and non-audit services rendered by our independent registered public accounting firm, Marcum LLP. The policy generally pre-approves specified services in the defined categories of audit services, audit-related services and tax services up to specified amounts. Pre-approval

may also be given as part of the Audit Committee's approval of the scope of the engagement of the independent registered public accounting firm or on an individual case-by-case basis before the independent registered public accounting firm is engaged to provide each service. The pre-approval of services may be delegated to one or more of the Audit Committee's members, but the decision must be reported to the full Audit Committee at its next scheduled meeting. By the adoption of this policy, the Audit Committee has delegated the authority to pre-approve services to the Chairperson of the Audit Committee, subject to certain limitations.

The Audit Committee has determined that the rendering of the services other than audit services by Marcum LLP is compatible with maintaining the independent registered public accounting firm's independence.

## 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
	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 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			Date Filed	
Number	Description	Registrant's Form	with the SEC	Exhibit Number
3.1	<u>Amended and Restated Certificate of Incorporation.</u>	S-1	7/1/2014	3.3
3.2	<u>Amended and Restated Bylaws.</u>	S-1	7/1/2014	3.4
4.1	<u>Form of Common Stock Certificate.</u>	S-1	7/1/2014	4.1
4.2	<u>Form of Common Stock Purchase Warrant issued by Viking Therapeutics, Inc. to Laidlaw &amp; Company (UK) Ltd.</u>	S-1/A	4/10/2015	4.2
4.3	<u>Form of Warrant Agreement, by and between Viking Therapeutics, Inc. and American Stock Transfer &amp; Trust Company, LLC, including the Form of Warrant Certificate issued by Viking Therapeutics, Inc.</u>	8-K	4/8/2016	4.1
4.4		8-K	4/14/2016	4.1

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Warrant to Purchase Common Stock, dated April 13, 2016, issued by Viking Therapeutics, Inc. to Ligand Pharmaceuticals Incorporated.

4.5	<u>Form of Common Stock Warrant issued by Viking Therapeutics, Inc. to purchasers in the June 2017 offering.</u>	8-K	6/19/2017	4.1
10.1#	<u>Form of Indemnification Agreement between Viking Therapeutics, Inc. and its directors and executive officers.</u>	S-1	7/1/2014	10.1
10.2#	<u>2014 Equity Incentive Plan.</u>	S-1/A	3/2/2015	10.2
10.3#	<u>Form of Stock Option Award Agreement (2014 Equity Incentive Plan).</u>	S-1	7/1/2014	10.3
10.4#	<u>Form of Restricted Stock Unit Award Agreement (2014 Equity Incentive Plan).</u>	S-1	7/1/2014	10.4
10.5#	<u>Form of Restricted Stock Award Agreement (2014 Equity Incentive Plan).</u>	S-1/A	9/2/2014	10.23
10.6#	<u>Form of Stock Appreciation Rights Award Agreement (2014 Equity Incentive Plan).</u>	S-1	7/1/2014	10.5
10.7#	<u>2014 Employee Stock Purchase Plan.</u>	S-1/A	3/2/2015	10.22

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Exhibit Number	Description	Registrant's Form	Date Filed with the SEC	Exhibit Number
10.8#	<u>Amendment No. 1 to 2014 Employee Stock Purchase Plan.</u>	S-1	11/24/2015	10.8
10.9#	<u>Employment Agreement, effective as of June 2, 2014, by and between Viking Therapeutics, Inc. and Brian Lian, Ph.D.</u>	S-1/A	9/2/2014	10.6
10.10#	<u>First Amendment to Employment Agreement, effective as of March 14, 2016, by and between Viking Therapeutics, Inc. and Brian Lian, Ph.D.</u>	8-K	3/15/2016	10.1
10.11#	<u>Employment Agreement, effective as of May 21, 2014, by and between Viking Therapeutics, Inc. and Michael Morneau.</u>	S-1/A	9/2/2014	10.7
10.12#	<u>Amendment to Employment Agreement, effective as of September 30, 2014, by and between Viking Therapeutics, Inc. and Michael Morneau.</u>	S-1/A	3/2/2015	10.26
10.13#	<u>Second Amendment to Employment Agreement, effective as of March 14, 2016, by and between Viking Therapeutics, Inc. and Michael Morneau.</u>	8-K	3/15/2016	10.2
10.14#	<u>Non-Employee Director Compensation Policy.</u>	S-1	11/24/2015	10.16
10.15†	<u>Master License Agreement, dated May 21, 2014, by and among Viking Therapeutics, Inc., Ligand Pharmaceuticals Incorporated and Metabasis Therapeutics, Inc.</u>	S-1	7/1/2014	10.12
10.16†	<u>First Amendment to Master License Agreement, dated September 6, 2014, by and among Viking Therapeutics, Inc., Ligand Pharmaceuticals Incorporated and Metabasis Therapeutics, Inc.</u>	S-1/A	9/8/2014	10.24
10.17†	<u>Second Amendment to Master License Agreement, dated April 8, 2015, by and among Viking Therapeutics, Inc., Ligand Pharmaceuticals Incorporated and Metabasis Therapeutics, Inc.</u>	S-1/A	4/10/2015	10.30
10.18†	<u>Loan and Security Agreement, dated May 21, 2014, by and between Viking Therapeutics, Inc. and Ligand Pharmaceuticals Incorporated.</u>	S-1	7/1/2014	10.13
10.19†	<u>First Amendment to Loan and Security Agreement, dated April 8, 2015, by and between Viking Therapeutics, Inc. and Ligand Pharmaceuticals Incorporated.</u>	S-1/A	4/10/2015	10.31
10.20	<u>Second Amendment to Loan and Security Agreement, dated January 22, 2016, by and between Viking Therapeutics, Inc. and Ligand Pharmaceuticals Incorporated.</u>	8-K	1/25/2016	10.1
10.21	<u>Third Amendment to Loan and Security Agreement, dated as of May 8, 2017, by and between Viking Therapeutics, Inc. and Ligand Pharmaceuticals Incorporated.</u>	10-Q	5/10/2017	10.2

10.22	<u>Convertible Note, dated May 27, 2014, issued by Viking Therapeutics, Inc. to Ligand Pharmaceuticals Incorporated.</u>	S-1	7/1/2014	10.14
10.23	<u>Letter Agreement regarding board composition and management rights, dated May 21, 2014, by and between Viking Therapeutics, Inc. and Ligand Pharmaceuticals Incorporated.</u>	S-1	7/1/2014	10.15
10.24	<u>Registration Rights Agreement, dated May 21, 2014, by and among Viking Therapeutics, Inc., Metabasis Therapeutics, Inc. and Ligand Pharmaceuticals Incorporated.</u>	S-1	7/1/2014	10.16
10.25	<u>First Amendment to Registration Rights Agreement, dated January 22, 2016, by and between Viking Therapeutics, Inc. and Ligand Pharmaceuticals Incorporated.</u>	8-K	1/25/2016	10.2

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Exhibit Number	Description	Date Filed		
		Registrant's Form	with the SEC	Exhibit Number
10.26	<u>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.</u>	S-1	7/1/2014	10.17
10.27#	<u>Founder Common Stock Purchase Agreement, dated September 26, 2012, by and between Viking Therapeutics, Inc. and Brian Lian, Ph.D.</u>	S-1	7/1/2014	10.18
10.28#	<u>Amendment No. 1 to Founder Common Stock Purchase Agreement, dated May 4, 2015, by and between Viking Therapeutics, Inc. and Brian Lian, Ph.D.</u>	10-Q	6/12/2015	10.2
10.29#†	<u>Common Stock Purchase Agreement, dated February 20, 2014, by and between Viking Therapeutics, Inc. and Brian Lian, Ph.D.</u>	S-1	7/1/2014	10.21
10.30#	<u>Amendment No. 1 to Common Stock Purchase Agreement, dated May 4, 2015, by and between Viking Therapeutics, Inc. and Brian Lian, Ph.D.</u>	10-Q	6/12/2015	10.5
10.31#	<u>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.</u>	10-Q	6/12/2015	10.1
10.32	<u>Sublease between Fish &amp; Richardson P.C. and Viking Therapeutics, Inc. dated July 7, 2015.</u>	10-Q	11/5/2015	10.1
10.33	<u>Registration Rights Agreement by and between Viking Therapeutics, Inc. and Aspire Capital Fund, LLC, dated August 24, 2016.</u>	8-K	8/25/2016	4.1
10.34	<u>Stock Purchase Agreement by and between Viking Therapeutics, Inc. and PoC Capital, LLC, dated February 8, 2017.</u>	8-K	2/14/2017	10.1
10.35	<u>Form of Securities Purchase Agreement, dated June 14, 2017 by and between Viking Therapeutics, Inc. and purchasers in the June 2017 offering.</u>	8-K	6/19/2017	10.1
10.36	<u>Placement Agent Agreement, dated June 14, 2017 by and among Viking Therapeutics, Inc., Maxim Group LLC and Roth Capital Partners, LLC.</u>	8-K	6/19/2017	10.2
10.37	<u>Purchase Agreement, dated as of September 28, 2017, by and between the Company and Lincoln Park Capital Fund, LLC.</u>	8-K	9/29/2017	10.1
10.38	<u>Purchase Agreement, dated as of September 28, 2017, by and between the Company and Lincoln Park Capital Fund, LLC.</u>	8-K	9/29/2017	10.2
10.39		8-K	9/29/2017	10.3



Registration Rights Agreement, dated as of September 28, 2017, by and between the Company and Lincoln Park Capital Fund, LLC.

- 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.

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

Attached as Exhibit 101 to this report are the following formatted in XBRL (Extensible Business Reporting Language): (i) Balance Sheets as of December 31, 2017 and December 31, 2016, (ii) Statements of Operations for the years ended December 31, 2017 and 2016, (iii) Statements of Stockholders' Equity (Deficit) for the period from December 31, 2015 to December 31, 2017, (iv) Statements of Cash Flows for the years ended December 31, 2017 and 2016, 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.

Item 16. Form 10-K Summary.

None.

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 7, 2018 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. Brian Lian, Ph.D.	President, Chief Executive Officer and Director (Principal Executive Officer)	March 7, 2018
/s/ Michael Morneau Michael Morneau	Chief Financial Officer (Principal Accounting and Financial Officer)	March 7, 2018
/s/ Lawson Macartney, DVM, Ph.D. Lawson Macartney, DVM, Ph.D.	Director	March 7, 2018
/s/ Matthew W. Foehr Matthew W. Foehr	Director	March 7, 2018
/s/ Charles A. Rowland Jr. Charles A. Rowland Jr.	Director	March 7, 2018

/s/ Matthew Singleton  
Matthew Singleton

Director

March 7, 2018

/s/ Stephen W. Webster  
Stephen W. Webster

Director

March 7, 2018

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Report of Independent Registered Public Accounting Firm

To the Stockholders and Board of Directors of

Viking Therapeutics, Inc.

Opinion on the Financial Statements

We have audited the accompanying balance sheets of Viking Therapeutics, Inc. (the “Company”) as of December 31, 2017 and 2016, the related statements of operations and comprehensive loss, stockholders’ equity and cash flows for each of the two years in the period ended December 31, 2017, and the related notes (collectively referred to as the “financial statements”). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2017 and 2016, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2017, in conformity with accounting principles generally accepted in the United States of America.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (“PCAOB”) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audits to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting 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.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ Marcum LLP

Irvine, California

March 7, 2018

We have served as the Company's auditor since 2014.

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## Viking Therapeutics, Inc.

## Balance Sheets

	December 31,	December 31,
	2017	2016
<b>Assets</b>		
<b>Current assets:</b>		
Cash and cash equivalents	\$ 8,988,478	\$ 3,075,502
Short-term investments – available for sale	11,587,478	10,075,058
Prepaid clinical trial costs	886,873	541,603
Prepaid expenses and other current assets	388,969	282,666
Total current assets	21,851,798	13,974,829
Deferred public offering and other financing costs	269,613	521,538
Deposits	—	39,341
Total assets	\$ 22,121,411	\$ 14,535,708
<b>Liabilities, convertible notes and stockholders' equity</b>		
<b>Current liabilities:</b>		
Accounts payable	\$ 1,529,935	\$ 1,203,888
Other accrued liabilities	2,256,543	1,237,122
Accrued interest	21,960	34,894
Convertible notes payable, current portion (net of discount of \$404,437 and \$675,589 at December 31, 2017 and 2016, respectively)	3,450,540	3,269,582
Debt conversion feature liability	1,397,597	731,048
Total current liabilities	8,656,575	6,476,534
Deferred rent	—	16,307
Total long-term liabilities	—	16,307
Total liabilities	8,656,575	6,492,841
<b>Commitments and contingencies (Note 11)</b>		
<b>Stockholders' equity:</b>		
Preferred stock, \$0.00001 par value: 10,000,000 shares authorized at December 31, 2017 and 2016; no shares issued and outstanding at December 31, 2017 and 2016	—	—
Common stock, \$0.00001 par value: 300,000,000 shares authorized at December 31, 2017 and 2016; 35,817,104 shares issued and outstanding at December 31, 2017 and 20,823,873 shares issued and outstanding at December 31, 2016	358	208
Additional paid-in capital	94,339,179	68,326,818
Accumulated deficit	(80,854,932 )	(60,277,267 )
Accumulated other comprehensive loss	(19,769 )	(6,892 )
Total stockholders' equity	13,464,836	8,042,867
Total liabilities and stockholders' equity	\$ 22,121,411	\$ 14,535,708



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, 2017	2016
Revenues	\$—	\$—
Operating expenses:		
Research and development	13,741,186	9,000,499
General and administrative	5,329,003	4,846,776
Total operating expenses	19,070,189	13,847,275
Loss from operations	(19,070,189)	(13,847,275)
Other income (expense):		
Change in fair value of debt conversion feature liability	344,928	1,064,170
Amortization of debt discount	(1,282,631 )	(1,788,088 )
Amortization of financing costs	(571,044 )	(138,701 )
Interest income (expense), net	1,271	(21,928 )
Total other income (expense)	(1,507,476 )	(884,547 )
Net loss	(20,577,665)	(14,731,822)
Other comprehensive gain (loss), net of tax:		
Unrealized (loss) gain on securities	(12,877 )	478
Comprehensive loss	\$(20,590,542)	\$(14,731,344)
Basic and diluted net loss per share	\$(0.79 )	\$(0.90 )
Weighted-average shares used to compute basic		
and diluted net loss per share	25,978,098	16,278,292

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



## Viking Therapeutics, Inc.

## Statements of Stockholders' Equity

	Common Stock		Additional	Accumulated	Accumulated	
	Shares	Amount	Paid-In	Deficit	Other	Comprehensive
			Capital		Loss	Total
Balance at December 31, 2015	9,683,741	\$ 97	\$54,277,716	\$(45,545,445)	\$ (7,370)	\$8,724,998
Employee stock-based compensation, net	(43,166 )	—	1,785,326	—	—	1,785,326
Convertible loan payment issued in equity to related party	960,000	9	1,199,991	—	—	1,200,000
Sale of common stock, net of issuance costs	10,223,298	102	11,063,785	—	—	11,063,887
Unrealized gain (loss) on investments	—	—	—	—	478	478
Net loss	—	—	—	(14,731,822)	—	(14,731,822)
Balance at December 31, 2016	20,823,873	208	68,326,818	(60,277,267)	(6,892)	8,042,867
Employee stock-based compensation, net	(12,534 )	—	1,429,753	—	—	1,429,753
Issuance of common stock under employee stock plans	87,978	1	73,140	—	—	73,141
Issuance of common stock from warrant exercises	1,022,119	10	678,441	—	—	678,451
Sale of common stock, net of issuance costs	13,895,668	139	23,831,027	—	—	23,831,166
Unrealized gain (loss) on investments	—	—	—	—	(12,877)	(12,877)
Net loss	—	—	—	(20,577,665)	—	(20,577,665)
Balance at December 31, 2017	35,817,104	\$ 358	\$94,339,179	\$(80,854,932)	\$ (19,769)	\$13,464,836

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

## Viking Therapeutics, Inc.

## Statements of Cash Flows

	Year Ended	
	December 31, 2017	2016
Cash flows from operating activities		
Net loss	\$(20,577,665)	\$(14,731,822)
Adjustments to reconcile net loss to net cash used in operating activities		
Amortization of debt discount on notes payable	1,282,631	1,788,088
Amortization of investment premiums	94,645	153,735
Amortization of financing costs	571,044	138,701
Amortization of non-cash clinical trial costs	993,945	—
Change in fair value of debt conversion feature liability	(344,928 )	(1,064,170 )
Stock-based compensation	1,451,465	1,794,327
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	497,626	273,329
Deposits	—	40,659
Accounts payable	263,509	677,185
Accrued expenses	1,009,961	(141,295 )
Net cash used in operating activities	(14,757,767)	(11,071,263)
Cash flows from investing activities		
Purchases of investments	(22,525,707)	(16,922,235)
Proceeds from sales and maturities of investments	20,796,000	19,952,000
Net cash (used in) provided by investing activities	(1,729,707 )	3,029,765
Cash flows from financing activities		
Proceeds from issuances of common stock, net of underwriting discounts and commissions	22,351,890	11,548,518
Public offering and financing costs	(500,257 )	(1,096,205 )
Value of shares withheld related to employee tax withholding	(21,712 )	(31,061 )
Repurchases of common stock	—	(229 )
Repayment of convertible notes payable	(77,261 )	(94,861 )
Proceeds from stock issuance under employee stock purchase plan and warrant exercises	647,790	22,288
Net cash provided by financing activities	22,400,450	10,348,450
Net increase in cash and cash equivalents	5,912,976	2,306,952
Cash and cash equivalents beginning of period	3,075,502	768,550
Cash and cash equivalents end of period	\$8,988,478	\$3,075,502
Supplemental disclosure of cash flow information:		
Cash paid during the period for interest	\$122,739	\$205,139
Supplemental disclosure of non-cash investing and financing transactions		

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Shares issued to cover future clinical trial costs	\$1,800,000	\$—
Unpaid deferred public offering and other financing costs	\$139,585	\$—
Receivable from exercise of warrants	\$103,802	\$—
Shares issued as commitment fee in connection with Lincoln Park agreement	\$180,000	
Shares issued as commitment fee in connection with Aspire agreement	\$—	\$440,278
Shares issued in lieu of loan payment to related party	\$—	\$1,200,000

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

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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 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").

### Reclassifications

Certain reclassifications have been made to certain of the prior years' financial statements to conform to the current year presentation.

### 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 feature 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 accordance with Accounting Standards Update ("ASU") No. 2014-15, Presentation of Financial Statements – Going Concern (Subtopic 205-40), the Company's management evaluates whether there are conditions or events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date that the financial statements are issued.

On June 20, 2016, the Company entered into an Equity Distribution Agreement (the "Distribution Agreement") with Maxim Group LLC, as sales agent ("Maxim"), pursuant to which the Company was able to offer and sell, from time to time, through Maxim (the

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“Maxim Offering”), up to 3,748,726 shares of its common stock. Effective July 26, 2017, the Distribution Agreement terminated automatically in accordance with the terms thereof. During the period from January 1, 2017 through July 26, 2017, the Company sold 488,388 shares of its common stock under the Distribution Agreement, resulting in net proceeds to the Company of \$670,031 after deducting the sales agent’s commission. From the inception of the Distribution Agreement through July 26, 2017, the Company sold 1,267,237 shares of its common stock under the Distribution Agreement, resulting in net proceeds to the Company of \$1,626,549, after deducting the sales agent’s commission.

On August 24, 2016, the Company entered into a Common Stock Purchase Agreement (the “Purchase Agreement”) with Aspire Capital Fund, LLC (“Aspire Capital”), pursuant to which Aspire Capital was committed to purchase up to an aggregate of \$12.5 million of shares of the Company’s common stock over the 30-month term of the Purchase Agreement. Upon execution of the Purchase Agreement, the Company issued and sold to Aspire Capital under the Purchase Agreement 333,333 shares of common stock (the “Initial Shares”) at a price per share of \$1.50, for an aggregate purchase price of \$500,000. Concurrently with the execution of the Purchase Agreement, and as consideration for Aspire Capital entering into the Purchase Agreement, the Company issued to Aspire Capital 336,116 shares of common stock as a commitment fee (the “Commitment Shares”). Pursuant to the terms of the Purchase Agreement, the Company was able to, from time to time and subject to certain limitations, direct Aspire Capital to purchase shares of the Company’s common stock using pricing formulas based on average prevailing market prices around the time of each sale. Effective June 19, 2017, the Company terminated the Purchase Agreement, without liability to the Company, in accordance with the terms thereof. During the period from January 1, 2017 through June 19, 2017, 1,326,991 shares were issued pursuant to the Purchase Agreement, resulting in aggregate gross proceeds of \$1,869,512. From the inception of the Purchase Agreement through June 19, 2017, the Company sold 1,476,991 shares of its common stock under the Purchase Agreement resulting in aggregate gross proceeds to the Company of \$2,042,762, in addition to the Initial Shares and the Commitment Shares.

On February 8, 2017, the Company entered into a Stock Purchase Agreement (the “SPA”) with PoC Capital, LLC (“PoC”), pursuant to which, among other things, the Company issued to PoC 1,286,173 shares of its common stock. Under the terms of the SPA, PoC has agreed to fund \$1,800,000 in study costs associated with certain clinical studies, including a planned proof-of-concept trial in patients with Glycogen Storage Disease type Ia (“GSD Ia”). Any study costs in excess of that amount will be the Company’s sole responsibility. The Company accounted for the \$1,800,000 as a prepaid expense on the balance sheet. As of December 31, 2017, the remaining prepaid expense on the balance sheet was \$806,055.

On May 8, 2017, the Company entered into a Third Amendment to Loan and Security Agreement (the “Third Loan Amendment”) with Ligand Pharmaceuticals Incorporated (“Ligand”), which amended the Company’s Loan and Security Agreement that was originally entered into on May 21, 2014 and subsequently amended (such agreement, as amended by the Third Loan Amendment, the “Loan and Security Agreement”). The Third Loan Amendment, among other things, (1) extended the maturity date of the loans under the Secured Convertible Promissory Note (the “Ligand Note”) issued pursuant to the Loan and Security Agreement (the “Loans”) from May 21, 2017 to May 21, 2018, and (2) provided that the Company was required to repay \$200,000 of the Ligand Note obligation to Ligand in the third quarter of 2017, which payment had to be made solely in cash (the “Required Repayment”). The Company made the Required Repayment on July 13, 2017. The Required Repayment was applied, first, to accrued and unpaid interest on the Loans and, second, to the unpaid principal amount of the Loans. Each \$1.00 of value of the Required Repayment reduced the amount of accrued and unpaid interest and then unpaid principal amount on the Loans by \$0.50.

On June 14, 2017, the Company entered into a securities purchase agreement (the “Securities Purchase Agreement”) with certain accredited investors (the “Purchasers”) pursuant to which the Company sold an aggregate of 3,749,783 shares (the “Shares”) of its common stock in a registered direct offering (the “Registered Direct Offering”). The Shares were offered by the Company pursuant to its shelf registration statement on Form S-3 (File No. 333-212134) filed

with the SEC on June 20, 2016, as amended by Amendment No. 1 thereto filed with the SEC on July 26, 2016, and declared effective on July 26, 2016. In a concurrent private placement, the Company also agreed, pursuant to the Securities Purchase Agreement, to issue and sell to each of the Purchasers a warrant to purchase 0.75 shares of common stock (the “Warrants”) for each share of common stock purchased by a Purchaser in the Registered Direct Offering (the “Private Placement” and, together with the Registered Direct Offering, the “Offerings”). The exercise price of the Warrants is \$1.30 per share, subject to adjustment as provided therein, and will be exercisable beginning on December 19, 2017 through December 19, 2022. The combined purchase price for one Share and one Warrant to purchase 0.75 shares of common stock in the Offerings was \$1.15. The closing of the Offerings occurred on June 19, 2017. The aggregate net proceeds from the Offerings, after deducting the placement agents’ fees and other offering expenses were \$3,866,202.

On September 28, 2017, the Company entered into a purchase agreement (the “Registered Offering Purchase Agreement”), pursuant to which, on September 29, 2017, the Company sold to Lincoln Park Capital Fund, LLC (“LPC”) 701,282 shares of common stock, at a price of approximately \$1.78 per share for an aggregate purchase price of \$1,250,000, pursuant to the Company’s effective shelf registration statement on Form S-3 (Registration No. 333-212134), filed with the SEC on June 20, 2016, as amended by Amendment No. 1 thereto filed with the SEC on July 26, 2016, and declared effective on July 26, 2016, and the prospectus supplement thereto dated September 28, 2017.

On September 28, 2017, the Company also entered into a purchase agreement (the “Commitment Purchase Agreement”) and a Registration Rights Agreement (the “LPC Registration Rights Agreement”) with LPC, pursuant to which the Company has the right to sell to LPC up to \$15,000,000 in shares of common stock, subject to certain limitations and conditions set forth in the Commitment Purchase Agreement. Upon the satisfaction of the conditions in the Commitment Purchase Agreement, including that a registration statement, which the Company agreed to file with the SEC, pursuant to the LPC Registration Rights Agreement, is declared effective by the SEC and a final prospectus in connection therewith is filed with the SEC (the “Commencement”), the Company will have the right, from time to time at its sole discretion over the 30-month period from and after October 26, 2017 (the date of the Commencement), to direct LPC to purchase up to 75,000 shares of common stock on any business day (subject to certain limitations contained in the Commitment Purchase Agreement), with such amounts increasing based on certain threshold prices set forth in the Commitment Purchase Agreement; however, not to exceed \$1,000,000 in total purchase proceeds per purchase date. The purchase price of shares of common stock that the Company elects to sell to LPC pursuant to the Commitment Purchase Agreement will be based on the market prices of the common stock at the time of such purchases as set forth in the Commitment Purchase Agreement. In addition to regular purchases, as described above, the Company may also direct LPC to purchase additional amounts as accelerated purchases or as additional purchases if the closing sale price of the common stock is not below certain threshold prices, as set forth in the Commitment Purchase Agreement. In all instances, the Company may not sell shares of its common stock to LPC under the Commitment Purchase Agreement if it would result in LPC beneficially owning more than 4.99% of the Common Stock. As consideration for LPC’s commitment to purchase shares of common stock pursuant to the Commitment Purchase Agreement, the Company issued to LPC 100,000 shares of common stock (“Commitment Shares). From inception of the Commitment Purchase Agreement through December 31, 2017, 343,051 shares were issued pursuant to the Commitment Purchase Agreement, resulting in aggregate gross proceeds of \$802,112 in addition to the Initial Shares and the Commitment Shares.

On December 11, 2017, the Company closed an underwritten public offering of 5,900,000 shares of its common stock, including 769,565 shares sold pursuant to the underwriters’ full exercise of their option to purchase additional shares to cover over-allotments, at a public offering price of \$2.50 per share, for total net proceeds of \$13,449,252 after deducting underwriting discounts and commissions and estimated offering expenses payable by the Company.

Although it is difficult to predict the Company’s liquidity requirements, as of December 31, 2017, and based upon the Company’s current operating plan, the Company believes that it will have sufficient cash to meet its projected operating requirements for at least the next 12 months following the issuance of the financial statements based on the balance of cash, cash equivalents and short-term investments balance as of December 31, 2017 and the proceeds received from the Company’s February 2018 offering (see Note 12). As of December 31, 2017, the Company had an accumulated deficit of \$80,854,932. 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 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 or private sale of the Company’s common stock. Costs related to the public sale of the Company’s common stock are deferred until the completion of the applicable offering, at which time such costs are reclassified to additional paid-in-capital as a reduction of the proceeds. Costs related to the private sale of the Company’s common stock are deferred until the completion of the applicable offering, at which time such costs are amortized over the term of the applicable purchase agreement.

## 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 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.

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

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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 contract 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.

In May 2014, the Company entered into 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 are expensed as incurred in the Company's statement of operations.

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## 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 records a cumulative catch-up of the expense from the grant date to the current date, and the Company then amortizes the remainder of the expense over the remaining service period.

## 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 GAAP. 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:

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	Year Ended December 31,	
	2017	2016
Historical net loss per share		
Numerator		
Net loss attributable to common stockholders	\$(20,577,665)	\$(14,731,822)
Denominator		
Weighted-average common shares outstanding	26,244,257	16,767,235
Less: Weighted-average shares subject to repurchase	(266,159 )	(488,943 )
Denominator for basic and diluted net loss per share	25,978,098	16,278,292
Basic and diluted net loss per share	\$(0.79 )	\$(0.90 )

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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, 2017	2016
Common stock warrants	11,202,536	9,667,500
Restricted stock units	46,250	80,625
Common stock subject to repurchase	245,096	307,096
Common stock options	1,575,864	883,837
Shares issuable upon conversion of debt	1,117,273	3,519,753
	14,187,019	14,458,811

## 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

### Adopted Accounting Standards

In May 2014, the FASB issued ASU No. 2014-09, Revenue from Contracts with Customers (Topic 606), which supersedes all existing revenue recognition requirements, including most industry-specific guidance. The new standard requires a company to recognize revenue when it transfers goods or services to customers in an amount that reflects the consideration that the company expects to receive for those goods or services. The FASB has subsequently issued the following amendments to ASU No. 2014-09, which have the same effective date and transition date of January 1, 2018:

• In August 2015, the FASB issued ASU No. 2015-14, Revenue from Contracts with Customers (Topic 606): Deferral of the Effective Date, which delayed the effective date of the new standard from January 1, 2017 to January 1, 2018. The FASB also agreed to allow entities to choose to adopt the standard as of the original effective date.

• In March 2016, the FASB issued ASU No. 2016-08, Revenue from Contracts with Customers (Topic 606): Principal versus Agent Considerations, which clarifies the implementation guidance on principal versus agent considerations.

• In April 2016, the FASB issued ASU No. 2016-10, Revenue from Contracts with Customers (Topic 606): Identifying Performance Obligations and Licensing, which clarifies certain aspects of identifying performance obligations and licensing implementation guidance.

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In May 2016, the FASB issued ASU No. 2016-12, Revenue from Contracts with Customers (Topic 606): Narrow-Scope Improvements and Practical Expedients, which relates to disclosures of remaining performance obligations, as well as other amendments to guidance on collectability, non-cash consideration and the presentation of sales and other similar taxes collected from customers.

In December 2016, the FASB issued ASU No. 2016-20, Technical Corrections and Improvements to Topic 606, Revenue from Contracts with Customers, which amends certain narrow aspects of the guidance issued in ASU No. 2014-09 including guidance related to the disclosure of remaining performance obligations and prior-period performance obligations, as well as other amendments to the guidance on loan guarantee fees, contract costs, refund liabilities, advertising costs and the clarification of certain examples.

The Company will continue to evaluate the potential impact that these standards will have on its financial position and results of operations, although there is no current impact of this new guidance on its financial statements as the Company does not currently have any revenue generating arrangements.

In March 2016, the Financial Accounting Standards Board (“FASB”) issued Accounting Standards Update (“ASU”) No. 2016-09, Improvements to Employee Share-based Payment Accounting (“ASU 2016-09”). ASU 2016-09 simplifies several aspects of the

accounting for employee share-based payment transactions, including the accounting for income taxes, forfeitures, and statutory tax withholding requirements, as well as classification in the statement of cash flows. Under this guidance, a company recognizes all excess tax benefits and tax deficiencies as income tax expense or benefit in the statement of operations. This change eliminates the notion of the additional paid-in capital pool and reduces the complexity in accounting for excess tax benefits and tax deficiencies. The Company implemented ASU 2016-09 as required in the first quarter of fiscal year 2017. The Company's adoption of ASU 2016-09 did not have a material effect on its financial statements and related disclosures.

In August 2016, the FASB issued ASU No. 2016-15, Classification of Certain Cash Receipts and Cash Payments ("ASU 2016-15"), which amends ASC Topic 230 to add or clarify guidance on eight classification issues related to the statement of cash flows such as debt prepayment or debt extinguishment costs, and contingent consideration payments made after a business combination. ASU 2016-15 is effective for fiscal periods beginning after December 15, 2017 and must be adopted using a retrospective transition method to each period presented but may be applied prospectively if retrospective application would be impracticable. Early adoption is permitted, including adoption in an interim period. The Company's adoption of ASU 2016-15 on January 1, 2018 is not expected to have a material effect on its financial statements and related disclosures.

#### Recent Accounting Pronouncements Not Yet Adopted

In February 2016, the FASB issued ASU No. 2016-02, Leases (Topic 842) ("ASU 2016-02"), which amends the existing accounting standards for leases. The new standard requires lessees to record a right-of-use asset and a corresponding lease liability on the balance sheet (with the exception of short-term leases). For lessees, leases will continue to be classified as either operating or financing in the statement of operations. This ASU becomes effective for the Company in the first quarter of fiscal year 2019 and early adoption is permitted. ASU 2016-02 is required to be applied with a modified retrospective approach and requires application of the new standard at the beginning of the earliest comparative period presented. The Company does not expect the adoption of ASU 2016-02 to have a material effect on its financial statements and related disclosures.

In June 2016, the FASB issued ASU No. 2016-13, Measurement of Credit Losses on Financial Instruments ("ASU 2016-13"), which requires that financial assets measured at amortized cost be presented at the net amount expected to be collected. Currently, GAAP delays recognition of the full amount of credit losses until the loss is probable of occurring. Under this new standard, the statement of operations will reflect an entity's current estimate of all expected credit losses. The measurement of expected credit losses will be based upon historical experience, current conditions, and reasonable and supportable forecasts that affect the collectability of the reported amount. Credit losses relating to available-for-sale debt securities will be recorded through an allowance for credit losses rather than as a direct write-down to the security. The new standard is effective for the Company for fiscal years beginning after December 15, 2019, including interim periods within those fiscal years. Early adoption is permitted as of the fiscal years beginning after December 15, 2018, including interim periods within those fiscal years. The Company is evaluating the effect that ASU 2016-13 will have on its financial statements and related disclosures.

In July 2017, the FASB issued a two-part ASU No. 2017-11, I. Accounting for Certain Financial Instruments with Down Round Features and II. Replacement of the Indefinite Deferral for Mandatorily Redeemable Financial Instruments of Certain Nonpublic Entities and Certain Mandatorily Redeemable Noncontrolling Interests with a Scope Exception (“ASU 2017-11”). ASU 2017-11 amends guidance in FASB ASC Topic 260: Earnings Per Share, FASB ASC Topic 480: Distinguishing Liabilities from Equity, and FASB ASC Topic 815: Derivatives and Hedging. The amendments in Part I of ASU 2017-11 change the classification analysis of certain equity-linked financial instruments (or embedded features) with down round features. The amendments in Part II of ASU 2017-11 re-characterize the indefinite deferral of certain provisions of FASB ASC Topic 480 that now are presented as pending content in the ASC, to a scope exception. ASU 2017-11 is effective for the Company for fiscal years beginning after December 15, 2018, including interim periods within those fiscal years. Early adoption is permitted. The Company is evaluating the effect that ASU 2017-11 will have on its financial statements and related disclosures.

## 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. As of December 31, 2017 and 2016, 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 years ended December 31, 2017 and 2016.

Investments classified as available-for-sale as of December 31, 2017 consisted of the following:

	Amortized	Gross Unrealized	Gross Unrealized	Aggregate Estimated
	Cost	Gains <sup>(1)</sup>	Losses <sup>(1)</sup>	Fair Value
Certificates of deposit (2)	\$249,227	\$ —	\$ —	\$249,227
Corporate debt securities (2)	11,357,248	—	(18,997 )	11,338,251
	\$11,606,475	\$ —	\$(18,997 )	\$11,587,478

- (1) Unrealized gains and losses on available-for-sale securities are included as a component of comprehensive loss. At December 31, 2017, there were 29 securities in an unrealized loss position. These unrealized losses were less than \$2,700 individually and \$19,000 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, 2017, 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.

Investments classified as available-for-sale as of December 31, 2016 consisted of the following:

	Amortized	Gross Unrealized	Gross Unrealized	Aggregate Estimated
	Cost	Gains <sup>(1)</sup>	Losses <sup>(1)</sup>	Fair Value
Corporate debt securities (2)	\$10,081,950	\$ —	\$(6,892 )	\$10,075,058

- (1) Unrealized gains and losses on available-for-sale securities are included as a component of comprehensive loss. At December 31, 2016, there were 25 securities in an unrealized loss position. These unrealized losses were less than \$1,000 individually and \$7,000 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, 2016, 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, 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, 2017 and 2016, 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.

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The Company's financial liability that was subject to fair value measurement consisted of a debt conversion feature that has been recorded as a liability based on Level 3 unobservable inputs.

The fair value of the debt conversion feature, current as of December 31, 2017 and 2016, required management to estimate fair value based on the Black-Scholes-Merton option valuation model. The Black-Scholes-Merton option valuation model was adopted as a result of the Company's entry in January 2016 into the second amendment to the Company's Loan and Security Agreement with Ligand, dated May 21, 2014, as amended (as so amended, the "Loan and Security Agreement"), which amended the conversion terms of the Secured Convertible Promissory Note (the "Ligand Note") issued pursuant to the Loan and Security Agreement.

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 Company is required to mark to market the value of the conversion feature liability.

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. As of December 31, 2017, the Company's investments were in government money market funds, certificates of deposit and corporate debt securities.

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

	Total	Fair Value Measurements at December 31, 2017		
		Level 1	Level 2	Level 3
<b>Financial assets carried at fair value:</b>				
<b>Cash equivalents:</b>				
Money market funds	\$6,360,683	\$6,360,683	\$—	\$—
Corporate debt securities, available-for-sale	1,244,217	—	1,244,217	—
<b>Short-term investments</b>				
Certificates of deposit	249,227	—	249,227	—
Corporate debt securities, available-for-sale	11,338,251	—	11,338,251	—
<b>Total financial assets</b>	<b>\$19,192,378</b>	<b>\$6,360,683</b>	<b>\$12,831,695</b>	<b>\$—</b>
<b>Financial liabilities carried at fair value:</b>				
Debt conversion feature - long-term	\$1,397,597	\$—	\$—	\$1,397,597
<b>Total financial liabilities</b>	<b>\$1,397,597</b>	<b>\$—</b>	<b>\$—</b>	<b>\$1,397,597</b>

	Total	Fair Value Measurements at December 31, 2016		
		Level 1	Level 2	Level 3
<b>Financial assets carried at fair value:</b>				
<b>Cash equivalents:</b>				
Money market funds	\$2,519,322	\$2,519,322	\$—	\$—
<b>Short-term investments</b>				
Corporate debt securities, available-for-sale	10,075,058	—	10,075,058	—
<b>Total financial assets</b>	<b>\$12,594,380</b>	<b>\$2,519,322</b>	<b>\$10,075,058</b>	<b>\$—</b>
<b>Financial liabilities carried at fair value:</b>				
Debt conversion feature - current	\$731,048	\$—	\$—	\$731,048
<b>Total financial liabilities</b>	<b>\$731,048</b>	<b>\$—</b>	<b>\$—</b>	<b>\$731,048</b>

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The table below presents a summary of changes in the Company's debt conversion feature liability measured at fair value on a recurring basis using significant unobservable inputs (Level 3) for the years ended December 31, 2017 and 2016:

	Year Ended December 31,	
	2017	2016
Debt conversion feature liability:		
Beginning balance	\$731,048	\$2,370,903
Adjustments resulting from modification of debt	1,011,477	(575,685 )
Adjustments resulting from changes in fair value recognized in earnings	(344,928 )	(1,064,170)
Ending balance	\$1,397,597	\$731,048

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

	Fair Value Assesses	Valuation Technique(s)	Significant Unobservable Input	Range
Debt conversion feature liability				
December 31, 2017	\$—\$1,397,597	Black-Scholes-Merton option valuation model	Volatility Risk Free Rate	100% 1.46%
December 31, 2016	\$—\$731,048	Black-Scholes-Merton option valuation model	Volatility Risk Free Rate	61% 0.57%

#### Level 3 Fair Value Sensitivity

##### Debt conversion feature liability

The fair value of the debt conversion feature liability includes the estimated volatility and risk free rate. The higher/lower the estimated volatility, the higher/lower the value of the debt conversion feature liability. The higher/lower the risk free interest rate, 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, as amended (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, erythropoietin

receptor (“EPOR”) and diacylglycerol acyltransferase-1 (“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 Company’s initial public offering (“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 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 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 (the "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 (the “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 (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 of 1933, as amended (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.

On January 22, 2016, the Company 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.

On February 14, 2017, the Company filed a Registration Statement on Form S-3 under the Securities Act covering the resale of the full amount of the Registrable Securities.

#### 5. Convertible Notes Payable

Convertible notes payable consisted of the following at:

	December 31,	
	2017	2016
<b>Current maturities:</b>		
Convertible note payable issued during 2014 to Ligand pursuant to the Master License Agreement with a 5% annual interest rate, due during 2016 (amended in January 2016 to a 2.5% annual interest rate with maturity extended to 2017; and subsequently amended in May 2017 with maturity extended to May 21, 2018)	\$ 1,916,508	\$ 1,955,139
Conversion payoff value	1,938,469	1,990,032

Discount on notes payable, current	(404,437 )	(675,589 )
Convertible notes payable, current portion (net of discount)	\$3,450,540	\$3,269,582

Pursuant to the Loan and Security Agreement, among other things, Ligand provided the Company with loans in the aggregate amount of \$2.5 million as of December 31, 2014. Up until 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% or 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% or 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% or the maximum interest rate permitted by law. The loans are evidenced by 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, or May 21, 2017 (the "Maturity Date"), unless the loans are repaid in cash or 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.

Additionally, pursuant to the terms of the Loan and Security Agreement, upon the consummation of the Company's first bona fide capital financing transaction occurring after January 22, 2016, but prior to the Maturity Date, with aggregate net proceeds to the Company of at least \$2,000,000 (the "Next Financing"), the Company was 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 the Company in its sole and absolute discretion), and the balance of the \$1,500,000 payment (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). Each \$1.00 of the \$1,500,000 payment reduced the amount of accrued and unpaid interest and then unpaid principal amount of the loans under the Ligand Note by \$0.50.

As a result of an amendment to the Loan and Security Agreement entered into on January 22, 2016, which amended the conversion terms of the Ligand Note, beginning with the quarter ended March 31, 2016, management adopted the Black-Scholes-Merton option valuation model for the Ligand Note, resulting in a debt modification adjustment of \$575,685 to the debt conversion feature liability offset with an adjustment recorded to the Ligand Note discount and conversion payoff value.

On April 13, 2016, the Company completed an underwritten public offering of its common stock and warrants to purchase shares of its common stock, resulting in net proceeds of \$7,754,286, after deducting underwriting discounts, commissions and other offering expenses of \$1,631,964. This offering constituted a Next Financing under the Loan and Security Agreement; therefore, the Company repaid \$1,500,000 of the Ligand Note with \$300,000 paid in cash and \$1,200,000 paid in the form of 960,000 shares of its common stock (the "Ligand Shares") and a warrant to purchase up to 960,000 shares of Company's common stock (the "Ligand Warrant"). The Ligand Warrant has an exercise price of \$1.50 per share of common stock, was immediately exercisable upon issuance and will expire on April 13, 2021.

On May 8, 2017, the Company entered into the Third Loan Amendment, which, (1) extended the maturity date of the Loans from May 21, 2017 to May 21, 2018, and (2) provided that the Company was required to make the \$200,000 Required Repayment. The Company made the Required Repayment on July 13, 2017. The Required Repayment was applied, first, to accrued and unpaid interest on the Loans and, second, to the unpaid principal amount of the Loans. Each \$1.00 of value of the Required Repayment reduced the amount of accrued and unpaid interest and then unpaid principal amount on the Loans by \$0.50. As a result of the Third Loan Amendment, the Company recorded a debt modification adjustment of \$1.0 million to the debt conversion feature liability primarily due to the longer expected term, offset with an adjustment recorded to the Ligand Note discount.

In addition, following the April 2016 underwritten public offering of the Company's common stock, the Company 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 (the "Additional Repayment Notice"), specifying the amount that the Company wishes to repay (the "Additional Payment Amount"). Ligand will 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 form of the Additional Payment Amount will be at the Company's 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 the Company's 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 the Company's 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 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). 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 the Company's 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) the Company wishes to repay the full amount owed under the Ligand Note prior to the Maturity Date, the Company 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.

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In either case, the Company may, at its sole election and discretion, elect to pay the Remaining Balance solely in cash. If the Company does not elect to repay the Remaining Balance in cash, the form of payment and mix of cash and shares of the Company's 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 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 (1) 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, and (2) \$8.00 (subject to adjustment for stock dividends, splits, combinations or similar transactions).

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.

During the year ended December 31, 2017, the Company recorded \$96,872 of interest expense, \$1,282,631 of amortization of debt discount, offset with income of \$344,928 related to the change in the fair value of the debt conversion feature liability. During the year ended December 31, 2016, the Company recorded \$105,551 of interest expense, \$1,788,088 of amortization of debt discount, offset with income of \$1,064,170 related to the change in the fair value of the debt conversion feature liability.

## 6. Stockholders' Equity

### Preferred Stock

The Company is authorized to issue up to 10,000,000 shares of \$0.00001 par value preferred stock, with no shares of preferred stock outstanding as of December 31, 2017 and 2016. 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.

In February 2014, the Company entered into a stock purchase agreement with one of its founders. The agreement provided 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 were subject to a repurchase option and were to vest in two tranches of 500,000 shares each, upon achievement of the performance target or upon a triggering event as defined.

The Company determined that the fair value of the unrecognized expense was \$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. The pro rata grant date fair value of the

unrecognized expense is \$61,556. In October 2015, a triggering event became probable of occurrence and was deemed achieved in October 2016; therefore, the Company recorded \$30,778 of stock-based compensation expense through December 31, 2016, of which \$12,371 and \$18,407 was recorded as expense during the years ended December 31, 2016 and 2015, respectively. No similar expense was recognized during the year ended December 31, 2017. The Company will continue to reassess at each reporting period whether it is probable that the performance target will be achieved, and if and when it is 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 target will actually be achieved.

On April 13, 2016, the Company completed an underwritten public offering of its common stock and warrants to purchase shares of common stock (the "Offering") pursuant to a registration statement on Form S-1 (File No. 333-208182) that was declared effective on April 7, 2016, and a registration statement on Form S-1MEF (File No. 333-210650) filed pursuant to Rule 462(b) of the Securities Act. In the Offering, the Company sold 7,500,000 shares of its common stock and warrants to purchase up to 7,500,000 shares of its common stock at a public offering price of \$1.25 per share of common stock and related warrant. The warrants have an exercise price of \$1.50 per share of common stock, were immediately exercisable upon issuance and will expire on April 13, 2021. The Company granted the underwriters for the Offering a 45-day option to purchase up to an additional 1,125,000 shares of its common stock and/or warrants to purchase up to an additional 1,125,000 shares of its common stock to cover over-allotments, if any. Ligand, a related party to the Company, purchased 560,000 shares of the Company's common stock and warrants to purchase up to 560,000 shares of Company's common stock in the Offering. On April 13, 2016, the underwriters partially exercised the over-allotment option for warrants to purchase an additional 1,125,000 shares of the Company's common stock at a public offering price of \$0.01 per warrant.

Upon the closing of the Offering on April 13, 2016, the Company received net proceeds of \$7,754,286, after deducting underwriting discounts, commissions and other offering expenses of \$1,631,964.

On April 13, 2016, pursuant to the terms of the Loan and Security Agreement, the Company issued the Ligand Shares and the Ligand Warrant to Ligand. The Ligand Warrant has an exercise price of \$1.50 per share of the Company's common stock, was immediately exercisable upon issuance (subject to a limitation on exercise to the extent that any exercise thereof would increase Ligand's beneficial ownership of the Company's common stock to greater than 49.9%) and expires on April 13, 2021. The Ligand Shares and the Ligand Warrant were issued to Ligand as a repayment of \$1,200,000 of the Company's obligation under the Ligand Note.

On April 27, 2016, the underwriters for the Offering exercised their over-allotment option to purchase 1,125,000 shares of the Company's common stock at a public offering price of \$1.24 per share. On April 29, 2016, the Company sold the 1,125,000 shares to the underwriters pursuant to the over-allotment option and received additional net proceeds of \$1,283,400, after deducting underwriting discounts, commissions and other offering expenses of \$111,600.

On June 20, 2016, the Company entered into the Distribution Agreement with Maxim pursuant to which the Company was able to offer and sell, from time to time, through Maxim up to 3,748,726 shares of its common stock. All shares of common stock offered and sold in the Maxim Offering were issued pursuant to the Company's registration statement on Form S-3 (File No. 333-212134) filed with the SEC on June 20, 2016 and the prospectus relating to the Maxim Offering that forms a part of the registration statement on Form S-3. The registration statement on Form S-3 was declared effective by the SEC on July 26, 2016. Effective July 26, 2017, the Distribution Agreement terminated automatically in accordance with the terms thereof. During the period from January 1, 2017 through July 26, 2017, the Company sold 488,388 shares of its common stock, under the Distribution Agreement, resulting in net proceeds to the Company of \$670,031, after deducting the sales agent's commission. From the inception of the Distribution Agreement through July 26, 2017, the Company sold 1,267,237 shares of its common stock under the Distribution Agreement, resulting in net proceeds to the Company of \$1,626,549, after deducting the sales agent's commission.

On August 24, 2016, the Company entered into the Purchase Agreement, pursuant to which Aspire Capital is committed to purchase up to an aggregate of \$12.5 million of shares of the Company's common stock over the 30-month term of the Purchase Agreement. Upon execution of the Purchase Agreement, the Company issued and sold 333,333 of the Initial Shares at a price per share of \$1.50, for an aggregate purchase price of \$500,000 to Aspire Capital under the Purchase Agreement. Concurrently with the execution of the Purchase Agreement, and as consideration for Aspire Capital entering into the Purchase Agreement, the Company issued 336,116 of the Commitment Shares to Aspire Capital with a fair value of \$440,278, which has been recorded as deferred financing costs and is included within other assets in the accompanying balance sheet as of December 31, 2016. The deferred financing costs were proratably charged to additional paid-in-capital for the Initial Shares, with the remainder of the costs amortized over the term of the Purchase Agreement as there is no guarantee that additional shares will be sold under the Purchase Agreement. Pursuant to the terms of the Purchase Agreement, the Company may, from time to time and subject to certain limitations, direct Aspire Capital to purchase shares of the Company's common stock using pricing formulas based on average prevailing market prices around the time of each sale. Additionally, the Company and Aspire may not effect any sales of shares of the Company's common stock under the Purchase Agreement during the continuance of an event of default or on any trading day that the closing sale price of its common stock is less than \$0.40 per share (subject to adjustment for stock dividends, splits, combinations or similar transactions or events). Effective June 19, 2017, the Company terminated the Purchase Agreement, resulting in a write off of the remaining capitalized deferred financing costs relating to the Purchase Agreement of \$336,985. During the period from January 1, 2017 through June 19, 2017, 1,326,991 shares were issued, pursuant to the Purchase Agreement, resulting in aggregate gross proceeds of \$1,869,512. From the inception of the Purchase Agreement through June 19, 2017, the Company sold 1,476,991 shares of its common stock under the Purchase Agreement resulting in aggregate

gross proceeds to the Company of \$2,042,762, in addition to the Initial Shares and the Commitment Shares.

On February 8, 2017, the Company entered into the SPA with PoC, pursuant to which, among other things, the Company issued to PoC 1,286,173 shares of its common stock. Under the terms of the SPA, PoC has agreed to fund \$1,800,000 in study costs associated with certain clinical studies, including a planned proof-of-concept trial in patients with GSD Ia. Any study costs in excess of that amount will be the Company's sole responsibility. The Company has accounted for the \$1,800,000 as a prepaid expense on the balance sheet. During the year ended December 31, 2017, the Company recorded amortization expense of \$993,945, respectively, in clinical study costs related to the SPA with PoC, with a remaining prepaid balance of \$806,055 on the balance sheet as of December 31, 2017.

On June 14, 2017, the Company entered into the Securities Purchase Agreement with the Purchasers, pursuant to which the Company sold an aggregate of 3,749,783 shares of its common stock in the Registered Direct Offering. The Shares were offered by the Company pursuant to its shelf registration statement on Form S-3 (File No. 333-212134) filed with the SEC on June 20, 2016, as amended by Amendment No. 1 thereto filed with the SEC on July 26, 2016, and declared effective on July 26, 2016. In a concurrent private placement, the Company also agreed, pursuant to the Securities Purchase Agreement, to issue and sell to each of the Purchasers a Warrant to purchase 0.75 shares of common stock for each share of common stock purchased by a Purchaser in the

Registered Direct Offering. The exercise price of the Warrants is \$1.30 per share, subject to adjustment as provided therein, and will be exercisable beginning on December 19, 2017 through December 19, 2022. The combined purchase price for one Share and one Warrant to purchase 0.75 shares of common stock in the Offerings was \$1.15. The closing of the Offerings occurred on June 19, 2017. The aggregate net proceeds from the Offerings, after deducting the placement agents' fees and offering expenses, were \$3,866,202.

On September 28, 2017, the Company entered into the Registered Offering Purchase Agreement, pursuant to which, on September 29, 2017, the Company sold to LPC 701,282 shares of common stock, at a price of approximately \$1.78 per share for an aggregate purchase price of \$1,250,000, pursuant to the Company's effective shelf registration statement on Form S-3 (Registration No. 333-212134), filed with the SEC on June 20, 2016, as amended by Amendment No. 1 thereto filed with the SEC on July 26, 2016, and declared effective on July 26, 2016, and the prospectus supplement thereto dated September 28, 2017.

On September 28, 2017, the Company also entered into the Commitment Purchase Agreement and the LPC Registration Rights Agreement with LPC, pursuant to which the Company has the right to sell to LPC up to \$15,000,000 in shares of common stock, subject to certain limitations and conditions set forth in the Commitment Purchase Agreement. Upon the Commencement, the Company will have the right, from time to time at its sole discretion over the 30-month period from and after the Commencement, to direct LPC to purchase up to 75,000 shares of common stock on any business day (subject to certain limitations contained in the Commitment Purchase Agreement), with such amounts increasing based on certain threshold prices set forth in the Commitment Purchase Agreement; however, not to exceed \$1,000,000 in total purchase proceeds per purchase date. The purchase price of shares of common stock that the Company elects to sell to LPC pursuant to the Commitment Purchase Agreement will be based on the market prices of the common stock at the time of such purchases as set forth in the Commitment Purchase Agreement. In addition to regular purchases, as described above, the Company may also direct LPC to purchase additional amounts as accelerated purchases or as additional purchases if the closing sale price of the common stock is not below certain threshold prices, as set forth in the Commitment Purchase Agreement. In all instances, the Company may not sell shares of its common stock to LPC under the Commitment Purchase Agreement if it would result in LPC beneficially owning more than 4.99% of the Common Stock. As consideration for LPC's commitment to purchase shares of common stock pursuant to the Commitment Purchase Agreement, the Company issued to LPC 100,000 shares of common stock. From inception of the Commitment Purchase Agreement through December 31, 2017, 343,051 shares were issued pursuant to the Commitment Purchase Agreement resulting in aggregate gross proceeds of \$802,112 in addition to the Initial Shares and the Commitment Shares.

On December 11, 2017, the Company closed an underwritten public offering of 5,900,000 shares of its common stock, including 769,565 shares sold pursuant to the underwriter's full exercise of their option to purchase additional shares to cover over-allotments, at a public offering price of \$2.50 per share, for total net proceeds of \$13,449,252 after deducting underwriting discounts and commissions and estimated offering expenses payable by the Company.

During the years ended December 31, 2017 and 2016, and in accordance with the Company's 2014 Employee Stock Purchase Plan (the "ESPP"), the Company issued an aggregate of 42,823 and 21,616 shares of its common stock to certain employees, respectively.

## 7. 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. On January 1, 2017 and 2016, in accordance with the terms of the 2014 Plan, an additional 728,835 and 338,930 shares of the Company's common stock were added to the number of shares reserved for issuance under the 2014 Plan, respectively, and, in accordance with the terms of the ESPP, an additional 208,238 and 96,837 shares of the Company's common stock were added to the number of shares reserved for issuance under the ESPP, respectively.

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.

During the years ended December 31, 2017 and 2016, the Company recognized the following stock-based compensation expense:

	Year Ended December	
	31,	
	2017	2016
Stock-based compensation expense by type of award:		
Stock options	\$688,766	\$921,556
Restricted stock and restricted stock units	701,395	836,964
Employee stock purchase plan	61,304	35,807
Total stock-based compensation expense included		
in expenses	\$1,451,465	\$1,794,327

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Stock-based compensation expense by line item:

Research and development expenses	\$408,379	\$537,008
General and administrative expenses	1,043,086	1,257,319
Total stock-based compensation expense included		
in expenses	\$1,451,465	\$1,794,327

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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, 2017	Unrecognized	Weighted-
Type of award:	Expense,	Net of	Recognition
	Estimated	Period	Forfeitures (in years)
Stock options	\$839,518	2.32	
Restricted stock and restricted stock units	\$384,096	0.89	

The following table is a summary of restricted shares granted during the years ended December 31, 2017 and 2016:

	Shares	Fair Value
Unvested at December 31, 2015	667,963	\$ 3.73
Granted	—	\$ —
Vested	(291,313)	\$ 2.89
Forfeited	(69,554 )	\$ 6.37
Repurchased	—	\$ —
Unvested at December 31, 2016	307,096	\$ 3.93
Granted	—	\$ —
Vested	(62,000 )	\$ 9.49
Forfeited	—	\$ —
Repurchased	—	\$ —
Unvested at December 31, 2017	245,096	\$ 2.53

The following table summarizes restricted stock units activity during the years ended December 31, 2017 and 2016:

	Weighted-	
	Average	
	Grant	
	Date	
	Shares	Value
Unvested at December 31, 2015	75,750	\$ 8.27
Granted	40,000	\$ 1.19
Vested	(28,937)	\$ 5.83
Forfeited	(6,188 )	\$ 7.25
Unvested at December 31, 2016	80,625	\$ 5.72
Granted	—	\$ —
Vested	(23,125)	\$ 5.50
Forfeited	(11,250)	\$ 7.08
Unvested December 31, 2017	46,250	\$ 5.50

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The following table summarizes stock option activity during the years ended December 31, 2017 and 2016:

	Shares	Weighted- Average Exercise Price	Weighted- Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value
Options outstanding at December 31, 2015	365,394	\$ 8.48		
Granted	620,818	\$ 2.01		
Exercised	—	\$ —		\$—
Cancelled	(102,375 )	\$ 5.55		
Options outstanding at December 31, 2016	883,837	\$ 4.28	8.87	\$—
Granted	892,534	\$ 1.25		
Exercised	(22,030 )	\$ 1.39		\$42,777
Cancelled	(178,477 )	\$ 3.55		
Options outstanding at December 31, 2017	1,575,864	\$ 2.68	8.58	\$3,346,629
Options exercisable at December 31, 2017	587,164	\$ 4.19	8.15	\$856,320

The Company received \$30,651 in cash proceeds from exercises of stock options during the year ended December 31, 2017. There were no option exercises during the year ended December 31, 2016.

The total fair value of stock options that vested during the years ended December 31, 2017 and 2016 was \$693,644 and \$1,057,749, respectively.

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, 2017 and 2016 was \$0.89 and \$1.40, respectively.

As stock-based compensation expense recognized is based on options ultimately expected to vest, the fair value of each employee option grant during the years ended December 31, 2017 and 2016 was estimated on the date of grant using the Black-Scholes option pricing model with the following weighted average assumptions:

Year ended  
December 31,

	2017	2016
Expected volatility	85.1 %	82.6 %
Expected term (in years)	5.91	5.90
Risk-free interest rate	1.97 %	1.59 %
Expected dividend yield	0 %	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 accounted for as actual forfeitures occur.

Since the Company had a net operating loss carryforward as of December 31, 2017, no excess tax benefits for the tax deductions related to stock-based awards were recognized in the Statements of Operations.

#### Common Stock Reserved for Future Issuance

Common stock reserved for future issuance as of December 31, 2017 is as follows:

Common stock warrants	11,202,536
Restricted stock units	46,250
Common stock options	1,575,864
Available for grant under the 2014 Plan	669,698
Available for issuance under Employee Stock Purchase Plan	693,388
Shares issuable upon conversion of Ligand debt	1,117,273
	15,305,009

#### 8. Warrants

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. On February 14, 2017, the Company filed a Registration Statement on Form S-3 under the Securities Act covering the resale of the 82,500 shares of the Company's common stock issuable upon exercise of the warrant.

On April 13, 2016, pursuant to the Offering, the Company sold 7,500,000 shares of its common stock and warrants to purchase up to 7,500,000 shares of its common stock at a public offering price of \$1.25 per share of common stock and related warrant. The warrants have an exercise price of \$1.50 per share of common stock, were immediately exercisable upon issuance and will expire on April 13, 2021. Additionally, on April 13, 2016, the underwriters for the Offering partially exercised the over-allotment option for warrants to purchase an additional 1,125,000 shares of the Company's common stock at a public offering price of \$0.01 per warrant to purchase a share of common stock. As of December 31, 2017, 8,172,699 warrants were outstanding and 452,301 warrants were exercised during the year ended December 31, 2017.

On April 13, 2016, pursuant to the terms of the Loan and Security Agreement, the Company issued to Ligand the Ligand Warrant to purchase up to 960,000 shares of the Company's common stock. The Ligand Warrant has an exercise price of \$1.50 per share of Company common stock, was immediately exercisable upon issuance (subject to a limitation on exercise to the extent that any exercise thereof would increase Ligand's beneficial ownership of the Company's common stock to greater than 49.9%) and expires on April 13, 2021. The Ligand Warrant was issued to Ligand as a part of the repayment of \$1,200,000 of the Company's obligation under the Ligand Note.

On June 14, 2017, pursuant to the terms of the Securities Purchase Agreement, the Company sold the 3,749,783 Shares and the Warrants to purchase up to 2,812,337 shares of its common stock to the Purchasers. The combined purchase price for one Share and one Warrant to purchase 0.75 shares of common stock in the Offerings, was \$1.15. The closing of the Offerings occurred on June 19, 2017. The Warrants have an exercise price of \$1.30 per share, subject to adjustment as provided therein, and will be exercisable beginning on December 19, 2017 through December 19, 2022. Each holder of a Warrant will not have the right to exercise any portion of its Warrant if the holder, together with its affiliates, would beneficially own in excess of 4.99% of the number of shares of common stock outstanding immediately after giving effect to such exercise (the “Beneficial Ownership Limitation”); provided, however, that upon 61 days’ prior notice to the Company, the holder may increase the Beneficial Ownership Limitation; however, in no event shall the Beneficial Ownership Limitation exceed 9.99%. The exercise price and number of shares of common stock issuable upon the exercise of the Warrants will be subject to adjustment in the event of any stock dividends and splits, reverse stock split, recapitalization, reorganization or similar transaction, as described in the Warrants. After December 19, 2017, if a registration statement covering the issuance or resale of the shares of common stock issuable upon exercise of the Warrants is not available for the issuance or resale, as applicable, the Purchasers may exercise the Warrants by means of a “cashless exercise.” As of December 31, 2017, 1,987,337 warrants were outstanding and 825,000 warrants were exercised during the year ended December 31, 2017. All warrants exercised during the year ended December 31, 2017, were exercised by means of a “cashless exercise”, resulting in the issuance of 569,818 shares of the Company’s common stock. On January 16, 2018, the resale Registration Statement on Form S-1 (File No. 333-222202) that the Company filed related to the 1,987,337 shares subject to unexercised warrants was declared effective by the SEC.

## 9. Income Taxes

Income tax expense (benefit) from continuing operations consists of the following for the years ended December 31, 2017 and 2016:

	December 31,	
	2017	2016
<b>Current:</b>		
Federal	\$—	\$—
State	—	—
	\$—	\$—
<b>Deferred:</b>		
Federal	\$(6,138,798)	\$(4,029,121)
State	(2,060,635)	(364,340)
	\$(8,199,433)	\$(4,393,461)
Change in federal tax rate	9,539,678	—
Valuation allowance	(1,340,245)	4,393,461
<b>Total income tax expense (benefit)</b>	<b>\$—</b>	<b>\$—</b>

The reconciliations of the U.S. federal statutory tax rate to the effective income tax rate for the years ended December 31, 2017 and 2016 are as follows:

	December 31,	
	2017	2016
Tax provision at U.S. Federal statutory rates	34 %	34 %
State income taxes net of federal benefit	10 %	3 %
Non-deductible permanent items	(4 )%	(7 )%
Stock options	(1 )%	—
Enactment of the Tax Cuts and Jobs Act	(46 )%	—
Change in valuation allowance	7 %	(30 )%
<b>Effective income tax rate</b>	<b>—</b>	<b>—</b>

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, 2017 and 2016 are as follows:

	December 31,	
	2017	2016
<b>Deferred tax assets:</b>		
Accrued liabilities	\$53,032	\$59,333
Intangible assets	14,696,888	16,388,591
Net operating loss carryforwards	4,948,795	4,558,964
Share-based compensation	1,030,457	1,289,957
Credits	265,588	—

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Other	6,679	3,017
Total deferred tax assets	21,001,439	22,299,862
Valuation Allowance	(20,775,747)	(22,115,992)
Total deferred tax assets, net of allowance	\$225,692	\$183,870
Deferred tax liabilities:		
Debt conversion feature	\$(225,692 )	\$(183,870 )
Total deferred tax liabilities:	\$(225,692 )	\$(183,870 )
Net deferred tax assets (liabilities):	\$—	\$—

The Tax Cuts and Jobs Act was enacted on December 22, 2017 and reduced the U.S. federal corporate rate to 21 percent, effective January 1, 2018. As such, the Company has recorded a decrease in its deferred tax assets, deferred tax liabilities and valuation allowance of \$9,645,000, \$105,000 and \$9,540,000, respectively, for the year ended December 31, 2017.

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A valuation allowance of \$20,775,747 and \$22,115,992 at December 31, 2017 and December 31, 2016, 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, 2017, the Company had federal and state net operating loss carryforwards of approximately \$17,773,000 and \$17,419,000, 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. However, the Company is able to utilize all of its net operating losses. The Company conducted a further assessment of ownership changes and determined that, through December 31, 2017, there have not been any additional ownership changes in accordance with Section 382 of the Code.

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, 2013 through December 31, 2017.

The differences between the Company's effective income tax rate and the statutory federal rate for the year ended December 31, 2017 and the year ended December 31, 2016 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, 2017 and December 31, 2016, 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.

At December 31, 2017, the Company has federal and state research and development tax credit carry-forwards of approximately \$74,000 and \$192,000, respectively. The federal credits begin to expire in 2036. The state credits do not expire.

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, 2017 or December 31, 2016.

#### 10. 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), a Management Rights Letter (see Note 4) and a Registration Rights Agreement (see Note 4). As Ligand beneficially owns 20.9% of the Company's outstanding shares as of December 31, 2017, the Company considers Ligand to be a related party.

#### 11. Commitments and Contingencies

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.

Under the Sublease, the Company is responsible for certain 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 a 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.

Rent expense was \$249,057 and \$253,316 for the years ended December 31, 2017 and 2016, respectively.

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Future minimum payments pursuant to the Sublease are as follows:

Year Ending December 31:	
2018	169,398
Total minimum lease payments	\$ 169,398

## 12. Subsequent Events

The Company evaluated subsequent events through 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, 2017, and events which occurred subsequent to December 31, 2017 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 disclosed in Note 8 (with respect to the resale Registration Statement on Form S-1 (File No. 333-222202) that the Company filed related to the 1,987,337 shares subject to unexercised warrants being declared effective by the SEC on January 16, 2018) and as follows:

On February 6, 2018, the Company completed an underwritten public offering of common stock pursuant to a registration statement on Form S-3 (File No. 333-212134), as amended, that was declared effective on July 26, 2016 (the "February 2018 Offering"). In the February 2018 Offering, the Company sold 12,650,000 shares of its common stock at a public offering price of \$5.00 per share of common stock. Upon the closing of the February 2018 Offering on February 6, 2018, the Company received gross proceeds of \$63,250,000, before deducting underwriting discounts, commissions and other offering expenses.

Additionally, from January 1, 2018 through February 28, 2018, the Company issued 2,421,698 shares of its common stock as a result of the exercise of warrants during that period for an aggregate exercise price of \$3,444,459.

The following unaudited pro forma balance sheet information as of December 31, 2017 assumes that the February 2018 Offering had been consummated as of December 31, 2017. The unaudited pro forma balance sheet information as of December 31, 2017 below gives effect to the issuance and sale of an aggregate of 12,650,000 shares of common stock at the public offering price of \$5.00 per share of common stock.

The following table summarizes certain actual balance sheet data and pro forma balance sheet data to reflect the activities related to the February 2018 Offering, as of December 31, 2017:

	December 31,	
	2017 Actual	Pro Forma
Cash, cash equivalents and investments	\$20,575,956	\$79,198,456
Deferred public offering and other financing costs	269,613	269,613
Debt	4,870,097	4,870,097
Accrued interest	21,960	21,960
Convertible notes payable	3,450,540	3,450,540

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Debt conversion feature liability	1,397,597	1,397,597
Stockholders' equity:		
Common stock	358	485
Additional paid-in capital	94,339,179	152,961,552
Accumulated deficit	(80,854,932)	(80,854,932)
Accumulated other comprehensive loss	(19,769)	(19,769)
Total stockholders' equity	\$13,464,836	\$72,087,336

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