Viking Therapeutics, Inc.
Form 10-Q
May 09, 2018

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the quarterly period ended March 31, 2018

OR

TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the transition period from to

Commission File Number 001-37355

VIKING THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware	46-1073877
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification Number)
12340 El Camino Real, Suite 250	
San Diego, California	92130
(Address of Principal Executive Offices)	(Zip Code)

(858) 704-4660

(Registrant's telephone number, including area code)

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

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

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act:

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

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes No

Indicate the number of shares outstanding of each of the issuer's classes of common stock, as of the latest practicable date:

Class	Number of Shares Outstanding as of April 30, 2018
Common stock, $0.00001 par value	50,933,195

VIKING THERAPEUTICS, INC.

FORM 10-Q FOR THE THREE MONTHS ENDED MARCH 31, 2018

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PART I. FINANCIAL INFORMATION

Item 1. Financial Statements
Viking Therapeutics, Inc.

Balance Sheets

(In thousands, except share and per share amounts)

	March 31, 2018 (Unaudited)	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 36,356	$ 8,988
Short-term investments – available for sale	41,112	11,587
Prepaid clinical trial and preclinical study costs	1,014	887
Prepaid expenses and other current assets	249	389
Total current assets	78,731	21,851
Deferred public offering and other financing costs	240	270
Total assets	$ 78,971	$ 22,121
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 658	$ 1,529
Other accrued liabilities	1,889	2,257
Accrued interest, current	34	22
Convertible notes payable, current (net of discount of $146 and $404 at March 31, 2018 and December 31, 2017, respectively)	3,721	3,451
Debt conversion feature liability, current	37	1,398
Total current liabilities	6,339	8,657
Total liabilities	6,339	8,657
Commitments and contingencies (Note 8)		
Stockholders' equity:		
Preferred stock, $0.00001 par value: 10,000,000 shares authorized at March 31, 2018 and December 31, 2017; no shares issued and outstanding at March 31, 2018 and December 31, 2017	—	—
Common stock, $0.00001 par value: 300,000,000 shares authorized at March 31, 2018 and December 31, 2017; 50,933,195 and 35,817,104 shares issued and outstanding at March 31, 2018 and December 31, 2017, respectively	1	—
Additional paid-in capital	157,146	94,339
Accumulated deficit	(84,406)	(80,855)
Accumulated other comprehensive loss	(109)	(20)

Total stockholders' equity	72,632	13,464
Total liabilities and stockholders' equity	$ 78,971	$ 22,121

See accompanying notes to the financial statements.

Viking Therapeutics, Inc.

Statements of Operations and Comprehensive Loss

(In thousands, except per share amounts)

(Unaudited)

	Three Months Ended March 31,	
	2018	2017
Revenues	$—	$—
Operating expenses:		
Research and development	3,043	3,528
General and administrative	1,762	1,441
Total operating expenses	4,805	4,969
Loss from operations	(4,805)	(4,969)
Other income (expense):		
Change in fair value of debt conversion feature liability	1,361	278
Amortization of debt discount	(258)	(431)
Amortization of financing costs	(30)	(98)
Interest income (expense), net	181	(2)
Total other income (expense), net	1,254	(253)
Net loss	(3,551)	(5,222)
Other comprehensive loss, net of tax:		
Unrealized loss on securities	(89)	(1)
Comprehensive loss	$(3,640)	$(5,223)
Net loss per common share		
Basic	$(0.08)	$(0.23)
Diluted	$(0.10)	$(0.23)
Weighted-average shares used to compute net loss per share		
Basic	44,649	22,353
Diluted	45,306	22,353

See accompanying notes to the financial statements.

Viking Therapeutics, Inc.

Statements of Cash Flows

(In thousands)

(Unaudited)

	Three Months Ended March 31,	
	2018	2017
Cash flows from operating activities		
Net loss	$ (3,551)	$ (5,222)
Adjustments to reconcile net loss to net cash used in operating activities		
Amortization of debt discount on notes payable	258	431
Amortization of investment premiums	62	31
Amortization of financing costs	30	98
Amortization of non-cash clinical trial costs	225	42
Change in fair value of debt conversion feature liability	(1,361)	(278)
Stock-based compensation	610	402
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(316)	221
Accounts payable	(832)	523
Accrued expenses	(428)	472
Net cash used in operating activities	(5,303)	(3,280)
Cash flows from investing activities		
Purchases of investments	(32,279)	(6,057)
	2,652	8,096

Proceeds from maturities of investments		
Net cash (used in) provided by investing activities	(29,627)	2,039
Cash flows from financing activities		
Proceeds from issuances of common stock, net of underwriting discounts and commissions	58,822	2,540
Public offering and financing costs	(146)	(16)
Proceeds from warrant and option exercises	3,622	—
Net cash provided by financing activities	62,298	2,524
Net increase in cash and cash equivalents	27,368	1,283
Cash and cash equivalents beginning of period	8,988	3,076
Cash and cash equivalents end of period	$ 36,356	$ 4,359
Supplemental disclosure of non-cash investing and financing transactions		
Shares issued to cover future clinical trial costs	$ —	$ 1,800
Unpaid deferred public offering and other financing costs	$ 136	$ 16

See accompanying notes to the financial statements.

Viking Therapeutics, Inc.

NOTES TO FINANCIAL STATEMENTS

(Unaudited)

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, California.

Basis of Presentation

The accompanying financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America ("GAAP"). The accompanying balance sheet as of March 31, 2018, statements of operations for the three months ended March 31, 2018 and 2017 and statements of cash flows for the three months ended March 31, 2018 and 2017 are unaudited. These unaudited financial statements have been prepared in accordance with the rules and regulations of the United States Securities and Exchange Commission ("SEC") for interim financial information. Accordingly, they do not include all of the information and footnotes required by GAAP for complete financial statements. These financial statements should be read in conjunction with the audited financial statements and the accompanying notes for the year ended December 31, 2017 contained in the Annual Report on Form 10-K filed by the Company with the SEC on March 7, 2018. The unaudited interim financial statements have been prepared on the same basis as the annual financial statements and, in the opinion of management, reflect all adjustments (consisting of normal recurring adjustments) necessary to state fairly the Company's financial position as of March 31, 2018, the results of operations for the three months ended March 31, 2018 and 2017 and cash flows for the three months ended March 31, 2018 and 2017. The December 31, 2017 balance sheet included herein was derived from the audited financial statements, but does not include all disclosures or notes required by GAAP for complete financial statements.

The financial data and other information disclosed in these notes to the financial statements related to the three months ended March 31, 2018 and 2017 are unaudited. Interim results are not necessarily indicative of results for an entire year.

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.

Recent Accounting Pronouncements

Adopted Accounting Standards

In May 2014, the FASB issued Accounting Standards Update ("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 evaluated the potential impact that these standards had on its financial position and results of operations. 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 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 did not 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.

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.

Prepaid Clinical Trial and Preclinical Study Costs

Prepaid clinical trial and preclinical study costs represent advance payments by the Company for future clinical trial and preclinical study services to be performed by the clinical research organization and other research organizations. Such amounts are recognized as research and development expense as the related clinical trial and preclinical study services are performed.

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 public sales 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 private sales 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/or royalties.

On January 1, 2018, the Company adopted ASU 2014-09, Revenue from Contracts with Customers and all related amendments ("ASC 606" or "the new revenue standard"). ASC 606 is a single comprehensive model for entities to use in accounting for revenue arising from contracts with customers and supersedes most current revenue recognition guidance, including industry-specific guidance. The new revenue standard is based on the principle that an entity

should recognize revenue to depict the transfer of goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. To achieve this core principle, ASC 606 provides that an entity should apply the following steps: (1) identify the contract(s) with a customer, (2) identify the performance obligations in the contract, (3) determine the transaction price, (4) allocate the transaction price to the performance obligations in the contract and (5) recognize revenue when (or as) the entity satisfies a performance obligation. The new revenue standard also requires additional disclosure about the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts, and costs to obtain or fulfill contracts. The Company will apply ASC 606 prospectively to all contracts.

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 Pharmaceuticals Incorporated, or 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.

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 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. 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. For restricted stock and restricted stock unit awards, the Company generally uses the straight-line 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.

The following table presents the computation of basic and diluted net loss per common share (in thousands, except per share data):

	Three Months Ended March 31,	
	2018	2017
Net Loss		
Net loss attributable to common stockholders - Basic	$(3,551)	$(5,222)
Change in fair value of debt conversion feature liability	(1,361)	—
Amortization of debt discount	258	—
Interest expense	24	—
Net loss attributable to common stockholders - Diluted	$(4,630)	$(5,222)
Basic Shares		
Weighted-average common shares outstanding	44,893,895	22,660,017
Less: Weighted-average shares subject to repurchase	(245,096)	(307,096)
Denominator for basic net loss per share	44,648,799	22,352,921
Diluted Shares		
Weighted-average common shares outstanding	44,893,895	22,660,017
Weighted-average shares subject to repurchase	(245,096)	(307,096)
Effect of dilutive shares issuable upon conversion of debt	656,716	—
Denominator for diluted net loss per share	45,305,515	22,352,921
Net loss per share:		
Basic	$(0.08)	$(0.23)
Diluted	$(0.10)	$(0.23)

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):

	As of March 31,	
	2018	2017
Common stock warrants	8,780,838	9,667,500
Restricted stock units	136,250	69,375
Common stock subject to repurchase	245,096	307,096
Common stock options	1,967,721	1,511,894
Shares issuable upon conversion of debt	—	2,860,361
	11,129,905	14,416,226

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.

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 March 31, 2018 and December 31, 2017, the Company's investments were in government money market funds, certificates of deposit and corporate debt securities. There were no sales of available-for-sale securities during the three months ended March 31, 2018 or during the year ended December 31, 2017.

Investments classified as available-for-sale as of March 31, 2018 consisted of the following (in thousands):

As of March 31, 2018	Amortized Cost	Gross Unrealized Gains [1]	Gross Unrealized Losses [1]	Aggregate Estimated Fair Value
Certificates of deposit [2]	$ 250	$ —	$ —	$ 250
Corporate debt securities [2]	40,963	—	(101)	40,862
	$ 41,213	$ —	$ (101)	$ 41,112

(1) Unrealized gains and losses on available-for-sale securities are included as a component of comprehensive loss. At March 31, 2018, there were no securities in an unrealized gain position and there were 56 securities in an unrealized loss position. These unrealized losses were less than $5,000 individually and $101,000 in the aggregate. These securities have not been in a continuous unrealized gain or 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 March 31, 2018, 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, 2017 consisted of the following (in thousands):

As of December 31, 2017	Amortized Cost	Gross Unrealized Gains [1]	Gross Unrealized Losses [1]	Aggregate Estimated Fair Value
Certificates of deposit [2]	$ 249	$ —	$ —	$ 249
Corporate debt securities [2]	11,357	—	(19)	11,338
	$ 11,606	$ —	$ (19)	$ 11,587

(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 $3,000 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.

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. Further, the Company believes the fair value of the debt approximates its carrying value based on relatively stable interest rates and short-term maturity of this instrument. 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 March 31, 2018 and December 31, 2017, all of the Company's financial assets that were subject to fair value measurements were valued using observable inputs. The Company's financial assets valued based on Level 1 inputs consist of money market funds and certificates of deposit. The Company's financial assets valued based on Level 2 inputs consist of corporate debt securities, which consist of investments in highly-rated investment-grade corporations.

The Company'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 March 31, 2018 and December 31, 2017 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 Loan and Security Agreement with Ligand (the "Loan and Security Agreement"), which amended the conversion terms of the Secured Convertible Promissory Note ("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 March 31, 2018, 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 (in thousands):

	Fair Value Measurements at March 31, 2018			
	Total	Level 1	Level 2	Level 3
Financial assets carried at fair value:				
Cash equivalents:				
Government money market funds	$21,876	$21,876	$—	$ —
Corporate debt securities, available-for-sale	15,777	—	15,777	—
Short-term investments				
Certificates of deposit, available-for-sale	250	—	250	—
Corporate debt securities, available-for-sale	40,862	—	40,862	—
Total financial assets	$78,765	$21,876	$56,889	$ —
Financial liabilities carried at fair value:				
Debt conversion feature - current	$37	$—	$—	$ 37
Total financial liabilities	$37	$—	$—	$ 37

	Fair Value Measurements at December 31, 2017			
	Total	Level 1	Level 2	Level 3
Financial assets carried at fair value:				
Cash equivalents:				
Government money market funds	$6,361	$6,361	$—	$—
Corporate debt securities, available-for-sale	1,244	—	1,244	—
Short-term investments				
Certificates of deposit, available-for-sale	249	—	249	—
Corporate debt securities, available-for-sale	11,338	—	11,338	—
Total financial assets	$19,192	$6,361	$12,831	$—
Financial liabilities carried at fair value:				
Debt conversion feature - current	$1,398	$—	$—	$1,398
Total financial liabilities	$1,398	$—	$—	$1,398

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 three months ended March 31, 2018 and 2017 (in thousands):

	Three Months Ended March 31,	
	2018	2017
Debt conversion feature:		
Beginning balance	$1,398	$731
Adjustments resulting from changes in fair value recognized in earnings	(1,361)	(278)

Ending balance	$37	$453

The following table sets forth the Company's valuation techniques and significant unobservable inputs used to determine fair value for significant Level 3 liabilities (in thousands, except for volatility and risk free rate):

	Fair Value				
	Asset	Liabilities	Valuation Technique(s)	Significant Unobservable Input	%
Debt conversion feature liability					
March 31, 2018	$ —	$ 37	Black-Scholes-Merton option valuation model	Volatility	63%
				Risk Free Rate	1.68%
December 31, 2017	$ —	$ 1,398	Black-Scholes-Merton option valuation model	Volatility	100%
				Risk Free Rate	1.46%

Level 3 Fair Value Sensitivity

Debt Conversion Feature Liability

As of March 31, 2018 and December 31, 2017, 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. Convertible Notes Payable

Convertible notes payable consisted of the following (in thousands):

	March 31, 2018	December 31, 2017
Current maturities:		
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)	$1,917	$ 1,917
Conversion payoff value	1,950	1,938
Discount on notes payable, current	(146)	(404)
Convertible notes payable, current (net of discount)	$3,721	$ 3,451

Pursuant to the Loan and Security Agreement, among other things, Ligand provided the Company with loans in the aggregate amount of $2.5 million. 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, 2018 (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 $2.0 million (the "Next Financing"), the Company was required to repay $1.5 million 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 $1.5 million 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 $1.5 million 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 $576,000 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 (the "Offering") resulting in net proceeds of $7.8 million, after deducting underwriting discounts, commissions and other offering expenses of $1.6 million. The Offering constituted a Next Financing under the Loan and Security Agreement; therefore, the Company repaid $1.5 million of the Ligand Note with $300,000 paid in cash and $1.2 million 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 to the Loan and Security Agreement (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 repay $200,000 of the Ligand Note 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. 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, 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.

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 (the “Remaining Balance”).

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 three months ended March 31, 2018, the Company recorded $24,000 of interest expense, $258,000 of amortization of debt discount, and $1.4 million as other income related to the decrease in the fair value of the debt conversion feature liability.

During the three months ended March 31, 2017, the Company recorded $24,000 of interest expense, $431,000 of amortization of debt discount and $278,000 as other income related to the decrease in the fair value of the debt conversion feature liability.

5. Stockholders' Equity

Preferred Stock

The Company is authorized to issue up to 10,000,000 shares of preferred stock, $0.00001 par value per share, with no shares outstanding as of March 31, 2018 and December 31, 2017. The Board of Directors is authorized to designate the terms and conditions of any preferred stock the Company may issue without further action by the stockholders of the Company.

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 $62,000. In October 2015, a triggering event became probable of occurrence and was deemed achieved in October 2016; therefore, the Company recorded $31,000 of stock-based compensation expense through December 31, 2016. No similar expense was recognized during the three months ended March 31, 2018 and 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 June 20, 2016, the Company entered into the Equity Distribution Agreement (the "Distribution Agreement") with Maxim Group LLC ("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 (the "Maxim Offering"). Effective July 26, 2017, the Distribution Agreement terminated automatically in accordance with the terms thereof. All shares of common stock offered and sold in the Maxim Offering were issued pursuant to the Company's 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 (the "Shelf Registration Statement") 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. 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 $1.6 million, after deducting the sales agent's commission.

On August 24, 2016, the Company entered into a common stock purchase agreement (the "Purchase Agreement"), pursuant to which Aspire Capital Fund, LLC ("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 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 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 shares of common stock as a commitment fee (the "Commitment Shares") to Aspire Capital with a fair value of $440,000, which has been

recorded as deferred financing costs, with a prorated portion 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 was no guarantee that additional shares would be sold under the Purchase Agreement. 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. 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 $337,000. 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 $2.0 million, 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 $1.8 million 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 has accounted for the $1.8 million as a prepaid expense on the balance sheet. During the three months ended March 31, 2018 and 2017, the Company recorded amortization expense of $225,000 and $42,000, respectively, in clinical study costs related to the SPA with PoC, with a remaining prepaid balance of $581,000 on the balance sheet as of March 31, 2018.

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 the Shelf Registration Statement. 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 offering expenses, were $3.9 million.

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 (the "LPC Initial Shares"), at a price of approximately $1.78 per share for an aggregate purchase price of $1.3 million, pursuant to the Shelf Registration Statement and the prospectus supplement thereto dated September 28, 2017.

On September 28, 2017, the Company also entered a purchase agreement (the "Commitment Purchase Agreement") and a registration rights agreement (the "Registration Rights Agreement") with LPC, pursuant to which the Company has the right to sell to LPC up to $15.0 million 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, (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 $1.0 million 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 (the "LPC 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 $802,000 in addition to the LPC Initial Shares and the LPC Commitment Shares. No additional shares were issued during the three months ended March 31, 2018.

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,

pursuant to the Shelf Registration Statement at a public offering price of $2.50 per share, for total net proceeds of $13.4 million after deducting underwriting discounts and commissions and other offering expenses.

On February 6, 2018, the Company completed an underwritten public offering of common stock pursuant to the Shelf Registration Statement (the "February 2018 Offering"). In the February 2018 Offering, the Company sold 12,650,000 shares of the Company's 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 net proceeds of $58.7 million, after deducting underwriting discounts, commissions and other offering expenses.

6. Stock-Based Compensation

In connection with the Company's initial public offering of common stock (the "IPO"), the Company's 2014 Equity Incentive Plan (the "2014 Plan") and the Company's 2014 Employee Stock Purchase Plan (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. From January 1, 2016 and through March 31, 2018, in accordance with the terms of the 2014 Plan, an additional 2,321,363 shares of the Company's common stock were added to the number of shares available for issuance under the 2014 Plan, respectively, and, in accordance with the terms of the ESPP, an additional 663,246 shares of the Company's common stock were added to the number of shares available for issuance under the ESPP, respectively.

The Company generally uses the straight-line 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. 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 three months ended March 31, 2018 and 2017, the Company recognized the following stock-based compensation expense (in thousands):

	Three Months Ended March 31,	
	2018	2017
Stock-based compensation expense by type of award:		
Stock options	$252	$225
Restricted stock and restricted stock units	204	163
Employee stock purchase plan	154	14
Total stock-based compensation expense included in expenses	$610	$402
Stock-based compensation expense by line item:		
Research and development expenses	$172	$117
General and administrative expenses	438	285
Total stock-based compensation expense included in expenses	$610	$402

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 (in thousands, except for years):

	As of March 31, 2018	
	Unrecognized Expense, Net of Estimated	Weighted-average Recognition Period

	Forfeitures	(in years)
Type of award:		
Stock options	$2,045	2.98
Restricted stock and restricted stock units	$599	2.16

The following table is a summary of restricted stock activity during the three months ended March 31, 2018:

	Shares of Restricted Stock	Weighted-Average Grant Date Fair Value
Unvested at December 31, 2017	245,096	$ 2.53
Granted	—	$ —
Vested	—	$ —
Forfeited	—	$ —
Unvested at March 31, 2018	245,096	$ 2.53

The following table summarizes restricted stock unit activity during the three months ended March 31, 2018:

	Shares Subject to Restricted Stock Units	Weighted-Average Grant Date Value
Unvested at December 31, 2017	46,250	$ 5.50
Granted	90,000	$ 4.65
Vested	—	$ —
Forfeited	—	$ —
Unvested March 31, 2018	136,250	$ 4.94

The following table summarizes stock option activity during the three months ended March 31, 2018:

	Shares Subject to Stock Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value
Options outstanding at December 31, 2017	1,575,864	$ 2.68		
Granted	455,000	$ 4.60		
Exercised	(44,393)	$ 1.65		157,000
Cancelled	(18,750)	$ 1.91		
Options outstanding at March 31, 2018	1,967,721	$ 3.16	8.66	3,592,000
Options exercisable at March 31, 2018	783,546	$ 3.49	8.07	1,558,000

The Company received $73,000 in cash proceeds from exercises of stock options during the three months ended March 31, 2018. There were no option exercises during the three months ended March 31, 2017.

Compensation cost 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 three months ended March 31, 2018 was $3.26.

As stock-based compensation expense recognized is based on options ultimately expected to vest, the fair value of each employee option grant during the three months ended March 31, 2018 was estimated on the date of grant using the Black-Scholes option pricing model with the following weighted average assumptions:

	Three Months Ended March 31, 2018	
Expected volatility	81.33	%
Expected term (in years)	6.10	
Risk-free interest rate	2.47	%
Expected dividend yield	0	%

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

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

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

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

Forfeitures are accounted for as actual forfeitures occur.

Since the Company had a net operating loss carryforward as of March 31, 2018, no excess tax benefits for the tax deductions related to stock-based awards were recognized in the Statements of Operations.

7. 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 an 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. The warrant also 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 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 March 31, 2018, 6,691,443 warrants were outstanding and 1,481,256 warrants were exercised during the three months ended March 31, 2018.

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 $1.2 million 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 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. 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." 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. As of March 31, 2018, 1,046,895 warrants were outstanding and 940,442 warrants were exercised during the three months ended March 31, 2018.

8. 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 (the "Premises").

Under the Sublease, the Company is responsible for certain charges for common area maintenance and other costs, 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 $62,000 for each of the three months ended March 31, 2018 and 2017. Future minimum payments pursuant to the Sublease are $107,000 in the aggregate through September 30, 2018, the remaining term of the Sublease.

9. Subsequent Events

The Company has evaluated all subsequent events through the date of filing of this Quarterly Report on Form 10-Q with the SEC, to ensure that this filing includes appropriate disclosure of events both recognized in the financial statements as of March 31, 2018, and events which occurred subsequent to March 31, 2018, but which 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.

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

This Quarterly Report on Form 10-Q 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," "m "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 Quarterly Report on Form 10-Q and in our other Securities and Exchange Commission, or SEC, filings. Furthermore, such forward-looking statements speak only as of the date of this Quarterly Report on Form 10-Q. We undertake no obligation to update any forward-looking statements to reflect events or circumstances occurring after the date of such 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 this study. 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. We expect to 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, VK2809, an orally available, tissue and receptor-subtype selective agonist of the thyroid hormone receptor beta, or TRß, 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 TRß 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 TRß 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 initiate a proof-of-concept study in patients with X-ALD in the first half of 2019.

On February 6, 2018, we completed an underwritten public offering of common stock, or the February 2018 Offering, pursuant to a registration statement on Form S-3 (File No. 333-212134), as amended, that was declared effective on July 26, 2016, or the Shelf Registration Statement. In the February 2018 Offering, we sold 12,650,000 shares of our common stock at a public offering price of $5.00 per share of common stock. Upon the closing of the February 2018 Offering, we received net proceeds of $58.7 million, after deducting underwriting discounts, commissions and other offering expenses.

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 our Annual Report on Form 10-K filed with the SEC on March 7, 2018.

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, 2017, we charged $13.7 million to research and development expense as we continued our efforts in conducting Phase 2 clinical trials for VK5211 and VK2809 and performed additional in vivo studies for VK2809 and VK0214. During the three months ended March 31, 2018, we charged $3.0 million to

research and development expense related to our continued efforts in conducting Phase 2 clinical trials for VK5211 and VK2809, preparing for the initiation of a Phase 1 clinical trial for VK2809 in patients with GSD Ia and initiating certain toxicology studies. We expect that our ongoing research and development expenses will consist of costs incurred for the development of our drug candidates, including, but not limited to:

- employee and consultant-related expenses, which will include salaries, benefits and stock-based compensation, and certain consultant fees and travel expenses;

- expenses incurred under agreements with investigative sites and contract research organizations, or CROs, which will conduct a substantial portion of our research and development activities on our behalf;

- payments to third-party manufacturers, which will produce our active pharmaceutical ingredients and finished products;

- license fees paid to third parties for use of their intellectual property; and

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

We expense all research and development costs as incurred.

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

General and Administrative Expenses

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 continuing as a public company, including expenses related to continued compliance with the rules and regulations of the SEC and The Nasdaq Stock Market LLC, additional insurance expenses, investor relations activities and other administrative 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 Secured Convertible Promissory Note, or Ligand Note, issued pursuant to the Loan and Security Agreement with Ligand, 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 our 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.

Our significant accounting policies are more fully described in Note 1 and Note 3 to our financial statements included in Part I, Item 1 of this Quarterly Report on Form 10-Q.

Results of Operations

Comparison of the Three Months Ended March 31, 2018 and 2017

Research and Development Expenses

The following table summarizes our research and development expenses for the three months ended March 31, 2018 and 2017 (in thousands, except % change).

	Three Months Ended March 31,		$ Change	% Change
	2018	2017		
Research and development expenses	$3,043	$3,528	$ (485)	(13.7)%

The decrease in research and development expenses during the three months ended March 31, 2018 as compared to the same period in 2017 was primarily due to a decrease in expenses of $502,000 related primarily to manufacturing of our drug candidates and a decrease of $205,000 in clinical trial activity primarily related to our VK5211 program offset by increases in expenses of $210,000 related to certain pre-clinical study efforts and $145,000 related to services provided by certain third party consultants.

General and Administrative Expenses

The following table summarizes our general and administrative expenses for the three months ended March 31, 2018 and 2017 (in thousands, except % change).

	Three Months Ended March 31,		$ Change	% Change
	2018	2017		
General and administrative expenses	$1,762	$1,441	$ 321	22.3 %

The increase in general and administrative expenses during the three months ended March 31, 2018 as compared to the same period in 2017 was primarily due to increases in stock-based compensation expense of $153,000, salary and benefits related expenses of $91,000 and legal expenses of $48,000.

Other income (expense)

The following table summarizes our other income (expense) for the three months March 31, 2018 and 2017 (in thousands, except % change).

	Three Months Ended March 31,		$ Change	% Change
	2018	2017		
Other income (expense)	$1,254	$(253)	$1,507	595.7 %

Other income (expense) recognized during the three months ended March 31, 2018 consisted primarily of income of $1.4 million related to the decrease in fair value of the Ligand Note's conversion feature and interest income of $205,000 offset by $258,000 of expense related to the amortization of the Ligand Note discount, $24,000 of interest expense related to the Ligand Note and $30,000 of expense relating to the amortization of certain financing costs. Other income (expense) recognized during the three months ended March 31, 2017 consisted primarily of an expense of $431,000 related to the amortization of the Ligand Note discount, $24,000 of interest expense related to the Ligand Note and $98,000 of expense relating to the amortization of financing costs relating to the Equity Distribution Agreement with Maxim Group LLC and the Purchase Agreement with Aspire Capital Fund, LLC, offset by $278,000 of income 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 March 31, 2018, we had cash, cash equivalents and short term investments of $77.5 million. As such, we believe our cash, cash

equivalents and short-term investments will be sufficient to fund our operations through at least the second quarter of 2019, which is more than one year after the date of our filing this Form 10-Q.

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 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, 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, we sold 7,500,000 shares of our common stock and warrants to purchase up to 7,500,000 shares of our 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. We granted the underwriters for the Offering a 45-day option to purchase up to an additional 1,125,000 shares of our common stock and/or warrants to purchase up to an additional 1,125,000 shares of our common stock to cover over-allotments, if any. On April 13, 2016, the underwriters partially exercised the over-allotment option for warrants to purchase an additional 1,125,000 shares of our common stock at a public offering price of $0.01 per warrant. Upon the closing of the Offering on April 13, 2016, we received net proceeds of $7.8 million, after deducting underwriting discounts, commissions and other offering expenses of $1.6 million.

On April 27, 2016, the underwriters for the Offering exercised their over-allotment option to purchase 1,125,000 shares of our common stock at a public offering price of $1.24 per share. On April 29, 2016, we sold the 1,125,000 shares to the underwriters pursuant to the over-allotment option and received additional net proceeds of $1.3 million, after deducting underwriting discounts, commissions and other offering expenses of $111,600. Through March 31, 2018, we have received $2.9 million in proceeds related to the exercise of the warrants that were issued in the Offering.

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 the Shelf Registration Statement. 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 $3.9 million. Through March 31, 2018, we have received $1.2 million in proceeds related to the exercise of the warrants that were issued as a part of this private placement.

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 $1.3 million, pursuant to the

Shelf Registration Statement 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 $15.0 million 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 $1.0 million 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. Since inception of this purchase agreement, we sold to LPC in accordance with the terms of the agreement 343,051 shares of our common stock for aggregate proceeds of $802,000.

On February 6, 2018, we completed the February 2018 Offering. In the February 2018 Offering, we sold 12,650,000 shares of our common stock at a public offering price of $5.00 per share of common stock. Upon the closing of the February 2018 Offering, we received net proceeds of $58.7 million after deducting underwriting discounts, commissions and other offering expenses.

The following table summarizes our cash flows for the periods indicated below (in thousands):

	Three Months Ended March 31,	
	2018	2017
Cash used in operating activities	$(5,303)	$(3,280)
Cash (used in) provided by investing activities	$(29,627)	$2,039
Cash provided by financing activities	$62,298	$2,524

Cash Used in Operating Activities

During the three months ended March 31, 2018, cash used in operating activities of $5.3 million primarily reflected our net losses for the period, adjusted 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, amortization of non-cash clinical trial costs, stock-based compensation and a decrease in the fair value of the debt conversion feature liability for the Ligand Note as well as changes in our working capital accounts, primarily consisting of an increase in prepaid expenses and decreases in accounts payable and accrued expenses.

During the three months ended March 31, 2017, cash used in operating activities of $3.3 million primarily reflected our net losses for the period, adjusted by non-cash charges such as amortization of discount charged to interest expense on the Ligand Note, amortization of investment premiums, stock-based compensation and a decrease in the fair value of the debt conversion feature liability for the Ligand Note as well as changes in our working capital accounts, primarily consisting of an increase in accounts payable and accrued expenses and a decrease in prepaid expenses.

Cash (Used in) Provided by Investing Activities

During the three months ended March 31, 2018, cash used in investing activities of $29.6 million resulted primarily from the purchase of investments of $32.3 million offset by proceeds of sales and maturities of investments of $2.7 million.

During the three months ended March 31, 2017, cash provided by investing activities of $2.0 million resulted primarily from the proceeds of sales and maturities of investments of $8.1 million, offset by the purchase of investments of $6.1 million.

Cash Provided by Financing Activities

During the three months ended March 31, 2018, cash provided by financing activities was $62.3 million, which consisted primarily of proceeds from the issuance of common stock, net of discount, of $58.8 million in the February 2018 Offering, proceeds from certain warrant exercises of $3.5 million and proceeds from certain stock option exercises of $73,000 offset by $146,000 in payments of certain deferred offering and financing costs.

During the three months ended March 31, 2017, cash provided by financing activities was $2.5 million, which consisted primarily of proceeds from the issuance of common stock, net of discount, of $2.5 million, offset by $16,000 in payments of certain deferred offering and financing costs.

Future Funding Requirements

As of the date of this Quarterly Report on Form 10-Q and based upon our current operating plan, we believe that we have sufficient capital to fund our operating and capital requirements for at least the next 12 months. 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.

Off-Balance Sheet Arrangements

As of March 31, 2018, we did not have any off-balance sheet arrangements as defined in Item 303(a)(4)(ii) of Regulation S-K promulgated by the SEC.

Item 3. 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 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

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

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

Changes in Internal Control

There has been no change in our internal control over financial reporting during the three months ended March 31, 2018 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II. OTHER INFORMATION

Item 1. Legal Proceedings

We are not currently party to, and none of our property is currently the subject of, any material legal proceedings.

Item 1A. Risk Factors

Investing in our common stock involves a high degree of risk. You should consider carefully the risks and uncertainties described below, together with all of the other information in this Quarterly Report on Form 10-Q, before making a decision to invest in our common stock. The risks and uncertainties described below may not be the only ones we face. If any of the risks actually occur, our business, financial condition and results of operations could be materially and adversely affected. In that event, the trading price of our common stock could decline, and you could lose part or all of your investment.

Risk factors marked with an asterisk (*) below include a change from or an update to the risk factors included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2017, filed with the SEC on March 7, 2018.

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 March 31, 2018, we had an accumulated deficit of $84.4 million. 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. Most of the intellectual property related to our drug candidates is currently owned by Ligand, and we have the rights to use such intellectual property pursuant to the Master License Agreement. Therefore, our ability to develop and commercialize our drug candidates depends entirely on the effectiveness and continuation of the Master License Agreement. If we lose the right to license any of these key compounds, our ability to develop existing and new drug

candidates would be harmed.

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

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

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

development efforts may not lead to commercial drugs, either because our drug candidates fail to be safe and effective or because we have inadequate financial or other resources to advance our drug candidates through the clinical development and approval processes. If any of our drug candidates fail to demonstrate safety or efficacy at any time or during any phase of development, we would experience potentially significant delays in, or be required to abandon, development of the drug candidate.

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

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

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

- clinical trials may produce negative or inconclusive results;

- preclinical studies conducted with drug candidates during clinical development to, among other things, evaluate their toxicology, carcinogenicity and pharmacokinetics and optimize their formulation may produce unfavorable results;

- patient recruitment and enrollment in clinical trials may be slower than we anticipate;

- costs of development may be greater than we anticipate;

- our drug candidates may cause undesirable side effects that delay or preclude regulatory approval or limit their commercial use or market acceptance, if approved;

- collaborators who may be responsible for the development of our drug candidates may not devote sufficient resources to these clinical trials or other preclinical studies of these candidates or conduct them in a timely manner; or

- we may face delays in obtaining regulatory approvals to commence one or more clinical trials.

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

We licensed all of the intellectual property related to our drug candidates from Ligand pursuant to the Master License Agreement. In late 2017, we reported positive top-line results from 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 March 31, 2018, we had cash and cash equivalents and investments totaling $77.5 million. 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;
- 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.

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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 coleselevam 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, or 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, or 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-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.

*Current 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 March 31, 2018, we had eleven 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 officer has agreed to provide us with at least 60 days' advance notice of resignation pursuant to his employment agreement 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 March 31, 2018, $1.9 million 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.

Our executive officer has limited experience in managing and operating a public company, which could have an adverse effect on his 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 our executive officer has 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.

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 March 31, 2018, 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 March 31, 2018, we in-licensed seven patents in the U.S. and several other patents in certain foreign jurisdictions. As of March 31, 2018, 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 March 31, 2018. For VK0612, as of March 31, 2018, 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;

- 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 March 31, 2018, 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;
- 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 March 31, 2018, our executive officers, directors and 5% or greater stockholders beneficially owned 24.6% 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 March 31, 2018, Ligand and its affiliates beneficially owned approximately 14.9% 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 $1.07 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.

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 1,397,046 shares of our common stock were reserved as of March 31, 2018 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 $1.8 million. 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 underwritten public offering of our common stock and warrants to purchase shares of our common stock completed on April 13, 2016, 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 $1.3 million, 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 $15.0 million 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 $1.0 million 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 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 $13.7 million 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 underwriters' full exercise of their option to purchase additional shares to cover over-allotments, at a public offering price of $5.00 per share, for net proceeds of $58.7 million after 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 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 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 $17.8 million for federal and $17.4 million 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. Additionally, we have determined that our underwritten public offering of common stock completed in February 2018 resulted in an "ownership change" of us. We performed an analysis as of both dates in 2015 and 2018 and determined that, while the amount of net operating losses available to use each year may be limited, the full amounts of the $17.8 million federal and $17.4 million 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;
- 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 2.Unregistered Sales of Equity Securities and Use of Proceeds

None

Item 3.Defaults Upon Senior Securities

Not applicable.

Item 4. Mine Safety Disclosures
Not applicable.

Item 5. Other Information
Not applicable.

Item 6.Exhibits

Exhibit Number	Description	Registrant's Form	Date Filed with the SEC	Exhibit Number
3.1	Amended and Restated Certificate of Incorporation.	S-1	7/1/2014	3.3
3.2	Amended and Restated Bylaws.	S-1	7/1/2014	3.4
4.1	Form of Common Stock Certificate.	S-1	7/1/2014	4.1
4.2	Form of Common Stock Purchase Warrant issued by Viking Therapeutics, Inc. to Laidlaw & Company (UK) Ltd.	S-1/A	4/10/2015	4.2
4.3	Form of Warrant Agreement, by and between Viking Therapeutics, Inc. and American Stock Transfer & Trust Company, LLC, including the Form of Warrant Certificate to be issued by Viking Therapeutics, Inc. in the Offering.	8-K	4/8/2016	4.1
4.4	Warrant to Purchase Common Stock, dated April 13, 2016, issued by Viking Therapeutics, Inc. to Ligand Pharmaceuticals Incorporated.	8-K	4/14/2016	4.1
4.5	Form of Common Stock Purchase Warrant issued by Viking Therapeutics, Inc. to purchasers in the June 2017 offering.	8-K	6/19/2017	4.1
31.1	Certification of the Principal Executive Officer pursuant to Rule 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934.			
31.2	Certification of the Principal Financial Officer pursuant to Rule 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934.			
32.1	Certification of the Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.			
101.INS	XBRL Instance Document.			
101.SCH	XBRL Taxonomy Extension Schema Document.			
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document.			
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 March 31, 2018 and December 31, 2017, (ii) Statements of Operations for the

three months ended March 31, 2018 and 2017, (iii) Statements of Cash Flows for the three months ended March 31, 2018 and 2017, and (v) Notes to Financial Statements.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

Viking Therapeutics, Inc.

Date: May 9, 2018 By: /s/ Brian Lian, Ph.D.
Brian Lian, Ph.D.
President, Chief Executive Officer and Director

(Principal Executive Officer)

Date: May 9, 2018 By: /s/ Michael Morneau
Michael Morneau
Vice President, Finance and Administration

(Principal Accounting and Financial Officer)