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AQUINOX PHARMACEUTICALS, INC

FORM 10-Q
(Quarterly Report)

Filed 08/08/17 for the Period Ending 06/30/17

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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 June 30, 2017

OR

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

For the transition period from _____to _____

Commission file number: 001-36327

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

Delaware	**98-0542593**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**

450-887 Great Northern Way,
Vancouver, B.C., Canada V5T 4T5
(Address of principal executive offices, including zip code)

(Registrant's telephone number, including area code): (604) 629-9223

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 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, a smaller reporting company or an emerging growth company. See the definition of "large accelerated filer," "accelerated filer," "smaller reporting company" and "emerging growth company" in Rule 12b-2 of the Exchange Act. (Check one):

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☐ (Do not check if a 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 ☒

As of August 8, 2017, there were 23,464,712 shares of the registrant’s common stock outstanding.

Aquinox Pharmaceuticals, Inc.

Quarterly Report on Form 10-Q

For the Quarter Ended June 30, 2017

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Except as otherwise indicated herein or as the context otherwise requires, references in this report to "Aquinox," "the company," "we," "us," "our" and similar references refer to Aquinox Pharmaceuticals, Inc., a Delaware corporation, which we refer to in this report as Aquinox USA, and Aquinox Pharmaceuticals (Canada) Inc., a corporation under the Canada Business Corporations Act and a wholly owned subsidiary of Aquinox USA, which we refer to in this report as AQXP Canada. This report contains registered marks, trademarks and trade names of other companies. All other trademarks, registered marks and trade names appearing in this report are the property of their respective holders.

PART I. FINANCIAL INFORMATION

Item 1. Condensed Consolidated Financial Statements

AQUINOX PHARMACEUTICALS, INC.

Condensed consolidated balance sheets
(Unaudited)
(In thousands of U.S. dollars, except share amounts)

	JUNE 30, 2017	DECEMBER 31, 2016
Assets		
Current assets		
Cash and cash equivalents (Note 3)	$ 27,147	$ 32,301
Short-term investments (Note 8)	104,130	70,758
Receivables, prepayments and deposits	915	426
Total current assets	132,192	103,485
Property and equipment, net	1,092	849
Long-term investments (Note 8)	—	50,046
Total assets	$ 133,284	$ 154,380
Liabilities		
Current liabilities		
Accounts payable and other liabilities	$ 8,128	$ 9,519
Total current liabilities	8,128	9,519
Other liabilities (Note 4)	548	197
Total liabilities	8,676	9,716
Stockholders' equity		
Share capital: (Note 5)		
Common stock - $0.000001 par value - authorized, 50,000,000 as of June 30, 2017 and December 31, 2016; issued and outstanding, 23,464,712 as of June 30, 2017 (December 31, 2016 – 23,423,150)	—	—
Additional paid-in capital	295,214	293,111
Accumulated deficit	(170,401)	(148,278)
Accumulated other comprehensive loss	(205)	(169)
Total stockholders' equity	124,608	144,664
Total liabilities and stockholders' equity	$ 133,284	$ 154,380

The accompanying notes form an integral part of these unaudited condensed consolidated financial statements

AQUINOX PHARMACEUTICALS, INC.

Condensed consolidated statements of operations and comprehensive loss
(Unaudited)
(In thousands of U.S. dollars, except per share and share amounts)

	THREE MONTHS ENDED JUNE 30,		SIX MONTHS ENDED JUNE 30,	
	2017	2016	2017	2016
Operating expenses				
Research and development	$ 10,475	$ 9,235	$ 16,252	$ 14,116
General and administrative	3,520	1,830	6,265	3,783
Total operating expenses	13,995	11,065	22,517	17,899
Other income, net (Note 6)	229	164	435	287
Net loss	$ (13,766)	$ (10,901)	$ (22,082)	$ (17,612)
Net loss per common stock - basic and diluted (Note 7)	$ (0.59)	$ (0.63)	$ (0.94)	$ (1.02)
Basic and diluted weighted average number of common stock outstanding	23,444,150	17,212,007	23,433,708	17,211,997
Comprehensive loss:				
Net loss	$ (13,766)	$ (10,901)	$ (22,082)	$ (17,612)
Other comprehensive income (loss) – unrealized gain (loss) on available-for-sale securities	—	119	(36)	362
Comprehensive loss	$ (13,766)	$ (10,782)	$ (22,118)	$ (17,250)

The accompanying notes form an integral part of these unaudited condensed consolidated financial statements

AQUINOX PHARMACEUTICALS, INC.

Condensed consolidated statements of cash flows

(Unaudited)
(In thousands of U.S. dollars)

	SIX MONTHS ENDED JUNE 30,	
	2017	2016
Operating activities		
Net loss	$(22,082)	$(17,612)
Non-cash items:		
Change in fair value of derivative liability (Note 6)	—	(73)
Stock-based compensation (Note 5(c))	1,685	915
Unrealized foreign exchange loss and others	271	48
Changes in operating assets and liabilities:		
Receivable, prepayments and deposits	(484)	(771)
Accounts payable and other liabilities	(973)	3,705
Cash used in operating activities	(21,583)	(13,788)
Investing activities		
Purchase of investments	—	(34,557)
Proceeds from maturity of investments	16,500	31,575
Purchase of property and equipment	(452)	(320)
Cash provided by (used in) investing activities	16,048	(3,302)
Financing activities		
Proceeds from exercise of stock options	377	4
Payment on capital lease obligations	(7)	(1)
Cash provided by financing activities	370	3
Effect of exchange rate changes on cash and cash equivalents	11	31
Net change in cash and cash equivalents during the period	(5,154)	(17,056)
Cash and cash equivalents, beginning of period	32,301	39,526
Cash and cash equivalents, end of period	$ 27,147	$ 22,470
Supplemental disclosure of cash flow information:		
Interest received	$ 484	$ 249
Non-cash investing and financing activities:		
Accrued purchase of property & equipment	$ (77)	$ —

The accompanying notes form an integral part of these unaudited condensed consolidated financial statements

AQUINOX PHARMACEUTICALS, INC.

Notes to the condensed consolidated financial statements
(Unaudited)

1. Nature of operations

Aquinox Pharmaceuticals, Inc. and its subsidiary, Aquinox Pharmaceuticals (Canada) Inc., (consolidated, the "Company") is a clinical stage pharmaceutical company discovering and developing targeted therapeutics in disease areas of inflammation and immuno-oncology. The Company's primary focus is anti-inflammatory product candidates targeting SHIP1 (SH2-containing inositol-5'-phosphatase 1), which is a key regulator of an important cellular signaling pathway in immune cells, known as the PI3K pathway.

Aquinox Pharmaceuticals, Inc. was originally incorporated under the name of Aquinox Pharmaceuticals (USA) Inc. on May 31, 2007 in the State of Delaware, United States. On January 27, 2014, Aquinox Pharmaceuticals (USA) Inc. changed its name to Aquinox Pharmaceuticals, Inc. ("Aquinox USA").

Aquinox Pharmaceuticals (Canada) Inc. ("AQXP Canada") was originally incorporated under the name of 6175813 Canada Inc. on December 26, 2003 under the Canada Business Corporations Act. In May 2014, after a corporate restructuring, the name was changed to Aquinox Pharmaceuticals (Canada) Inc.

The Company operates in Vancouver, British Columbia, Canada and San Bruno, California.

2. Condensed summary of significant accounting policies

(a) Basis of presentation

The accompanying unaudited condensed consolidated financial statements are presented in United States ("U.S.") dollars and have been prepared in accordance with accounting principles generally accepted in the United States of America ("U.S. GAAP") and pursuant to the rules and regulations of the United States Securities and Exchange Commission ("SEC") for interim financial information. Accordingly, these financial statements do not include all of the information and footnotes required for complete financial statements and should be read in conjunction with the audited consolidated financial statements and accompanying notes included in the Company's Annual Report on Form 10-K for the year ended December 31, 2016 filed with the Securities and Exchange Commission on March 9, 2017.

In management's opinion, the unaudited condensed consolidated financial statements reflect all adjustments (including reclassifications and normal recurring adjustments) necessary to present fairly the financial position as of June 30, 2017, and results of operations and cash flows for all periods presented. The interim results presented are not necessarily indicative of results that can be expected for a full year.

(b) Use of estimates and assumptions

The preparation of financial statements in conformity with U.S. GAAP requires management 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 and the reported amounts of expenses during the reporting period. Significant areas requiring management estimates include valuation of stock options and warrants, amortization and depreciation, accrual of expenses, valuation allowance for deferred income taxes, and contingencies. Actual results could differ from those estimates.

(c) Short and long-term investments

Short-term investments consist of bank term deposits and U.S. government securities with initial maturities of less than a year. Long-term investments consist of U.S. government securities with initial maturities of greater than a year. Short-term investments and long-term investments are both classified as available-for-sale and carried at their estimated fair value with unrealized gains and losses recorded as a component of other comprehensive loss. Realized gains and losses are recorded in net loss. The Company periodically reviews its investments for impairment and when a decline in market value is deemed to be other than temporary, the loss is recognized in net loss.

(d) Accounting for stock-based compensation

The Company measures the cost of services received in exchange for an award of equity instruments based on the grant-date fair value of the award. The cost of such award will be recognized over the period during which services are provided in exchange for the award, generally the vesting period. All share-based payments to employees are recognized in the consolidated financial statements based upon their respective grant date fair values.

The Company estimates the fair value of options granted using the Black-Scholes option pricing model. This approximation uses assumptions regarding a number of inputs that requires management to make significant estimates and judgments. Prior to the completion of the Company's initial public offering in March 2014, the Company's common stock was not publicly traded. As a result, the expected volatility assumption is based on industry peer information due to insufficient trading history of the Company's common stock. Additionally, because the Company has no significant history to calculate the expected term, the simplified method calculation is used.

(e) Segment reporting

The Company operates in one segment, the identification and development of therapeutics for inflammatory diseases and cancer. The Company has significant Canadian operations but its assets are mostly held in the United States with an immaterial amount of long lived assets in Canada.

(f) Net loss per common stock

Basic net loss per common stock is computed by dividing loss by the weighted-average number of common stock outstanding during the period. Diluted net loss per common stock is determined using the weighted-average number of common stock outstanding during the period, adjusted for the dilutive effect of common stock equivalents, consisting of shares that might be issued upon exercise of common stock options and warrants. In periods where losses are reported, the weighted-average number of shares of common stock outstanding excludes common stock equivalents because their inclusion would be anti-dilutive.

(g) Recently issued and recently adopted accounting standards

The Company adopted FASB ASU 2015-17 "Income Taxes (Topic 740): Balance Sheet Classification of Deferred Taxes," effective January 1, 2017 which requires entities to present deferred tax assets and deferred tax liabilities as noncurrent in a classified balance sheet. The previous guidance required entities to separately present deferred tax assets and deferred tax liabilities as current or noncurrent in a classified balance sheet. This change did not have a material impact on the Company's financial statements as a full valuation allowance has been applied against the deferred tax assets.

The Company adopted FASB ASU 2016-09 "Compensation-Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting," effective January 1, 2017 which simplifies several aspects of the accounting for employee share-based payment transactions, including the accounting for income taxes, forfeitures, and statutory tax withholding requirements, as well as classification in the statement of cash flows. The Company has elected to change its accounting policy to account for forfeitures as they occur instead of on an estimated basis as this will more accurately reflect the cost of forfeitures. The change has been applied on a modified retrospective approach with a cumulative-effect adjustment to retained deficit of an immaterial amount as of January 1, 2017. The provisions relating to the accounting for income taxes has no significant impact on the consolidated financial statements as the Company applies a full valuation allowance against its deferred tax assets.

In January 2016, the FASB issued ASU 2016-01 "Financial Instruments-Overall (Subtopic 825-10): Recognition and Measurement of Financial Assets and Financial Liabilities," which revises an entity's accounting related to (1) the classification and measurement of investments in equity securities and (2) the presentation of certain fair value changes for financial liabilities measured at fair value. The ASU also amends certain disclosure requirements associated with the fair value of financial instruments. ASU 2016-01 is effective for fiscal years and interim periods within those fiscal years beginning after December 15, 2017, with early adoption permitted under certain circumstances. The Company is currently assessing the impact of ASU 2016-01 on the Company's financial statements.

In February 2016, the FASB issued ASU 2016-02 "Leases (Topic 842)", which requires the recognition of right-of-use assets and lease liabilities by lessees for those leases with a lease term of greater than 12 months. Upon the adoption of ASU 2016-02, leases will be recognized and measured at the beginning of the earliest period presented using a modified retrospective approach. The modified retrospective approach includes a number of optional practical expedients that companies may elect to apply. ASU 2016-02 is effective for fiscal years and interim periods beginning after December 15, 2018, with early adoption permitted. The Company is currently assessing the impact of ASU 2016-02 on the Company's financial statements and whether to elect to apply the optional practical expedients under the modified retrospective approach.

(h) Risks and uncertainties

The Company is subject to numerous risks and uncertainties. These risks, among others, included the following:

- the Company has no source of revenue, had an accumulated deficit of $170.4 million as of June 30, 2017, may never become profitable and may incur substantial and increasing net losses for the foreseeable future as it continues development of, seeks regulatory approvals for, and potentially begins to commercialize rosiptor, its lead product candidate, and any future product candidates;
- the Company is likely to require additional capital to finance its operations which may not be available to it on acceptable terms, or at all;
- the Company's success is primarily dependent on the successful development, regulatory approval and commercialization of rosiptor, its lead product candidate, and any future product candidates;
- SHIP1 has not been validated as a target;
- the Company is subject to regulatory approval processes that are lengthy, time consuming and inherently unpredictable; the Company may not be able to obtain approval for rosiptor or any future product candidates from the U.S. Food and Drug Administration, or FDA, or foreign regulatory authorities;
- the Company's intellectual property rights may be subject to claims by third parties and can be difficult and costly to protect;
- the Company may not be able to recruit or retain key employees, including its senior management team;
- the Company depends on the performance of third parties, including contract research organizations and third-party manufacturers; and
- the Company faces competition from other pharmaceutical and biotechnology companies, academic institutions, governmental agencies, and public and private research institutions, among others.

3. Cash and cash equivalents

(in thousands)	JUNE 30, 2017	DECEMBER 31, 2016
Cash	$ 9,421	$ 3,726
Cash equivalents	17,726	28,575
	$ 27,147	$ 32,301

4. Other liabilities

(in thousands)	JUNE 30, 2017	DECEMBER 31, 2016
Capital lease obligations	$ 40	$ 47
Deferred rent liability	508	150
	$ 548	$ 197

5. Stockholders' equity

(a) Share capital

Aquinox USA is authorized to issue two classes of stock, common and preferred. The total number of shares Aquinox USA is authorized to issue is 55,000,000 shares, comprised of 50,000,000 common stock and 5,000,000 preferred stock both with a par value of $0.000001 per share. As of June 30, 2017 and December 31, 2016, the total number of shares of common stock issued and outstanding was 23,464,712 and 23,423,150, respectively. As of June 30, 2017 and December 31, 2016, no shares of preferred stock were issued or outstanding.

(b) Stock option plan

On January 27, 2014, the stockholders of Aquinox USA approved a 2014 Equity Incentive Plan ("2014 Plan"). The 2014 Plan became effective on March 6, 2014. The 2014 Plan is the successor to and continuation of the Joint Canadian Stock Option Plan (the "2006 Plan"). No further grants will be made under the 2006 Plan. The 2014 Plan provides for the grant of stock options, stock appreciation rights, restricted stock awards, restricted stock unit awards, performance stock awards, performance cash awards, and other forms of equity awards to employees, directors, and consultants.

As at June 30, 2017, the maximum number of shares of common stock that may be issued under the 2014 Plan was 2,864,126 shares. Additionally, the number of shares of common stock reserved for issuance under the 2014 Plan will automatically increase on January 1 of each year for a period of up to 10 years, beginning on January 1, 2015 and ending on and including January 1, 2024, by 4% of the total number of shares of capital outstanding on December 31 of the preceding calendar year, or a lesser number of shares determined by the board of directors.

Stock option transactions and the number of stock options outstanding are summarized below:

	NUMBER OF SHARES	WEIGHTED AVERAGE EXERCISE PRICE	WEIGHTED AVERAGE REMAINING CONTRACTUAL LIFE (IN YEARS)	AGGREGATE INTRINSIC VALUE (IN THOUSANDS)
Outstanding at December 31, 2016	1,378,352	$ 9.38	7.81	$ 9,829
Options granted	779,040	16.97		
Options exercised	(41,562)	9.33		
Options forfeited	(59,063)	12.37		
Outstanding at June 30, 2017	2,056,767	$ 12.17	8.23	$ 6,114
Exercisable as of June 30, 2017	766,877	$ 9.28	6.53	$ 3,670

During the six months ended June 30, 2017, the Company granted 698,040 stock options to employees and 81,000 stock options to non-employee directors. The stock options granted to employees during the six months ended June 30, 2017 have exercise prices per share ranging from $13.74 to $17.35 and vest 25% one year after the beginning of the vesting period and thereafter ratably each month over the following thirty-six months. The stock options granted to non-employee directors during the six months ended June 30, 2017 have an exercise price per share of $13.74 and have vesting period of one year in equal monthly installments from the beginning of the vesting period for certain grants and vesting period of three years in equal annual installments from the beginning of the vesting period for other grants. All stock options under the 2014 Plan are subject to a 10-year expiration period.

During the three and six months ended June 30, 2017, 41,562 shares of common stock were issued upon exercise of options.

(c) Stock-based compensation

The fair value of stock options granted is estimated using the Black-Scholes option pricing model with the following weighted average assumptions:

	THREE MONTHS ENDED JUNE 30,		SIX MONTHS ENDED JUNE 30,	
	2017	2016	2017	2016
Expected volatility	90%	79%	91%	80%
Expected dividends	0%	0%	0%	0%
Expected terms (years)	6.00	6.00	6.00	6.00
Risk free rate	1.82%	1.26%	1.89%	1.27%
Weighted average grant-date fair value of stock options	$ 10.25	$ 5.27	$ 12.72	$ 5.61

The Company amortizes the fair value of the stock options on a straight-line basis over the applicable requisite service periods of the awards, which is generally the vesting period. Stock-based compensation expense charged to operating expenses was $1.1 million and $1.7 million for the three and six months ended June 30, 2017, respectively, and $0.5 million and $0.9 million for the three and six months ended June 30, 2016, respectively. Total unrecognized compensation cost for all stock-based compensation plans was $12.6 million and $4.3 million as of June 30, 2017 and June 30, 2016, respectively, which is expected to be recognized over a weighted-average period of 3.06 years (June 30, 2016 – 2.96 years)

6. Other income, net

(in thousands)	THREE MONTHS ENDED JUNE 30,		SIX MONTHS ENDED JUNE 30,	
	2017	2016	2017	2016
Change in fair value of derivative liability	$ —	$ 23	$ —	$ 73
Foreign exchange (losses) gain	(12)	6	(20)	(20)
Interest income	250	128	484	249
Miscellaneous (expenses) income	(9)	7	(29)	(15)
	$ 229	$ 164	$ 435	$ 287

7. Net loss per common stock

Basic and diluted net loss per common stock is computed by dividing net loss by the weighted average number of common stock outstanding. The Company excluded outstanding stock options and common stock warrants from the calculation of basic and diluted net loss per common stock as the effect would have been antidilutive for all periods presented.

The following have been excluded from the computation of basic and diluted net loss per common stock as their effect would have been antidilutive:

	THREE AND SIX MONTHS ENDED JUNE 30,	
	2017	2016
Outstanding stock options	2,056,767	1,251,114
Common stock warrants	—	11,363
Total	2,056,767	1,262,477

8. Financial instruments

Securities classified as available for sale

The Company's short-term investments and long-term investments consisted of available-for-sale securities as follows:

(in thousands) **June 30, 2017**	Amortized cost	Gross unrealized gains	Gross unrealized losses	Fair value
Short-term investments:				
U.S. treasury securities	$ 104,335	$ —	$ (205)	$ 104,130
Contractual maturities:				
Due within one year	$ 104,335			$ 104,130

December 31, 2016	Amortized cost	Gross unrealized gains	Gross unrealized losses	Fair value
Short-term investments:				
U.S. treasury securities	$ 70,819	$ —	$ (61)	$ 70,758
Long-term investments:				
U.S. treasury securities	$ 50,155	$ —	$ (109)	$ 50,046
Contractual maturities:				
Due within one year	$ 70,819			$ 70,758
Due after one year through two years	50,155			50,046

The aggregate estimated fair value of the Company's investments with unrealized losses are as follows:

(in thousands)	Period of continuous unrealized loss			
	12 months or less		Greater than 12 months	
June 30, 2017	Fair value	Gross unrealized losses	Fair value	Gross unrealized losses
U.S. treasury securities	$ 104,130	$ (205)	N/A	N/A
December 31, 2016				
U.S. treasury securities	$ 120,804	$ (170)	N/A	N/A

Fair value of financial instruments

Fair value is defined as the amounts that would be received upon sale of an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date (exit price). The fair value of the Company's financial instruments are determined according to a fair value hierarchy that prioritizes the inputs and assumptions used, and the valuation techniques used to measure fair value. The hierarchy gives the highest priority to unadjusted quoted prices in active markets for identical assets or liabilities (Level 1 measurements) and the lowest priority to unobservable inputs (Level 3 measurements). The three levels of the hierarchy are as follows:

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.

The determination of a financial instrument's level within the fair value hierarchy is based on an assessment of the lowest level of any input that is significant to the fair value measurement. The Company considers observable data to be market data which is readily available, regularly distributed or updated, reliable and verifiable, not proprietary, and provided by independent sources that are actively involved in the relevant market.

The carrying amounts of certain of the Company's financial instruments including cash, cash equivalents, receivables, accounts payable and other liabilities, approximate their fair values because of their nature and/or short maturities. The Company holds short and long-term investments that are classified as available-for-sale securities, which are measured at fair value determined on a recurring basis according to the fair value hierarchy.

The following tables present the fair value of our financial instruments that are measured at fair value on a recurring basis:

(in thousands)	QUOTED PRICES IN ACTIVE MARKETS FOR IDENTICAL ASSETS (LEVEL 1)	OTHER OBSERVABLE INPUTS (LEVEL 2)	SIGNIFICANT UN-OBSERVABLE INPUTS (LEVEL 3)	TOTAL
BALANCES – June 30, 2017				
Short-term investments – U.S. treasury securities	$ 104,130	$ —	$ —	$ 104,130
	$ 104,130	$ —	$ —	$ 104,130
BALANCES – December 31, 2016				
Short-term investments – U.S. treasury securities	$ 70,758	$ —	$ —	$ 70,758
Long-term investments – U.S. treasury securities	50,046	—	—	50,046
	$ 120,804	$ —	$ —	$ 120,804

Level 1 instruments, which include investments that are valued based on quoted market prices in active markets, consisted of U.S. treasury securities. The Company had no Level 2 or 3 investments as at June 30, 2017 and December 31, 2016. There were no transfers between Levels 1, 2, and 3 during the three and six months ended June 30, 2017 and the year ended December 31, 2016.

As at June 30, 2017, the Company had short-term investments consisting of available-for-sale securities of $104.1 million. Total gains for securities were $0.2 million and $0.4 million for the three and six months ended June 30, 2017, respectively and $0.1 million and $0.2 million for the three and six months ended June 30, 2016, respectively.

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

You should read the following discussion of our financial condition and results of operations in conjunction with the unaudited interim condensed consolidated financial statements and notes thereto included elsewhere in this report and our audited consolidated financial statements and notes included as part of our Annual Report on Form 10-K for the year ended December 31, 2016.

Forward-Looking Statements

The following discussion of our financial condition and results of operations contains forward-looking statements within the meaning of Section 27A of the Securities Act and Section 21E of the Securities Exchange Act of 1934. Forward-looking statements are based on our management's beliefs and assumptions and on information currently available to our management. All statements other than statements of historical facts are "forward-looking statements" for purposes of these provisions, including those relating to future events or our future financial performance and financial guidance. In some cases, you can identify forward-looking statements by terminology such as "may," "might," "will," "should," "expect," "plan," "anticipate," "project," "believe," "estimate," "predict," "potential," "intend" or "continue," the negative of terms like these or other comparable terminology, and other words or terms of similar meaning in connection with any discussion of future operating or financial performance. These statements are only predictions. All forward-looking statements included in this document are based on information available to us on the date hereof, and we assume no obligation to update any such forward-looking statements. Any or all of our forward-looking statements in this document may turn out to be wrong. Actual events or results may differ materially. Our forward-looking statements can be affected by inaccurate assumptions we might make or by known or unknown risks, uncertainties and other factors. In evaluating these statements, you should specifically consider various factors, including the risks outlined under the caption "Risk Factors" set forth in Item 1A of Part II of this quarterly report on Form 10-Q, as well as those contained from time to time in our other filings with the SEC. We caution investors that our business and financial performance are subject to substantial risks and uncertainties.

Overview

We are a clinical-stage pharmaceutical company discovering and developing targeted therapeutics in disease areas of inflammation and immuno-oncology. Our primary focus is anti-inflammatory product candidates targeting the SH2-containing inositol-5'-phosphatase 1 (SHIP1) enzyme, which is a key regulator of an important cellular signaling pathway in immune cells, known as the PI3K pathway. Our product candidate, AQX-1125, received the generic name "rosiptor" in April 2017 from the United States Adopted Name Council (USAN). This name was published by USAN on July 7, 2017. We will adopt the reference of rosiptor for appropriate use going forward while phasing out reference to AQX-1125.

Rosiptor is a small molecule activator of SHIP1 suitable for oral, once daily dosing. We are currently developing rosiptor as a treatment for interstitial cystitis/bladder pain syndrome (IC/BPS), a chronic inflammatory disease of the bladder. For rosiptor, we retain full worldwide rights and hold patents with terms through 2024 in Europe and 2028 in the United States with the possibility of further patent term extension avenues available to us.

Rosiptor is a SHIP1 activator that has demonstrated preliminary safety, broad anti-inflammatory potential and favorable drug properties in multiple preclinical studies. We have also completed seven clinical trials, exposing over 380 subjects to once daily oral administration of rosiptor. These trials have demonstrated a good tolerability profile, with over 200 patients receiving rosiptor in two Phase 2 trials for periods of 12 weeks. We believe rosiptor is the only SHIP1 activator currently in clinical trials and that no SHIP1 activator has yet received marketing approval as a treatment for disease in humans. We use a proprietary screening approach to discover new drug candidates that selectively target SHIP1 to modulate activated immune cells while minimizing their toxicity to normal cells. This approach has provided us with an extensive chemical library and several candidate compounds that target SHIP1. These compounds have both similar and distinct properties from rosiptor. Our intellectual property covers SHIP1 as a target, the C2 binding domain for screening and the composition of matter for our compounds. Our longer-term strategy is to broaden our development activities for rosiptor and to advance next generation SHIP1 activators for the treatment of additional inflammatory diseases and cancer.

We commenced operations in Canada in December 2003. Aquinox Pharmaceuticals (Canada) Inc., a corporation formed under the Canada Business Corporations Act, is a wholly owned subsidiary of Aquinox Pharmaceuticals, Inc., a Delaware corporation formed in May 2007. We currently have operations in Vancouver, British Columbia and San Bruno, California.

Since commencing operations, we have dedicated a significant portion of our resources to development efforts for our clinical-stage product candidate rosiptor. We anticipate that we will continue to incur significant operating expenses related to research and development as we continue to advance our clinical-stage product candidate and preclinical programs. We have funded our operations primarily through the sale of common stock and preferred stock. As of June 30, 2017, we had $131.3 million in cash, cash equivalents and short-term investments in liquid, high-quality securities.

Since inception, we have incurred significant operating losses. Our net loss for the six months ended June 30, 2017 was $22.1 million, compared to $17.6 million for the six months ended June 30, 2016. As of June 30, 2017, we had an accumulated deficit of $170.4 million. We expect to incur significant expenses and operating losses for the foreseeable future as we continue the development and clinical trials of, and, if successful, to potentially seek regulatory approval for rosiptor and any future product candidates we advance to clinical development. If we are able to obtain regulatory approval for any of our product candidates, we expect to incur significant commercialization expenses. For example, we do not currently have the infrastructure for the sales, marketing, manufacture and distribution of any products. We may enter into licensing and co-promotion agreements with strategic or collaborative partners for the commercialization of our products in the United States and other territories, but have not currently entered into any such arrangements. To develop a commercial infrastructure, we would have to invest considerable financial and management resources, some of which would have to be deployed prior to having any certainty of marketing approval.

Unless and until we generate sufficient revenue to be profitable, we will seek to fund our operations through public or private equity or debt financings or other sources. Additional financing may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed could have a material adverse effect on our business, results of operations, financial condition, cash flows and future prospects.

Results of Operations

Revenue

To date, we have not generated any revenue. In the future, we may generate revenue from a combination of product sales, license fees, milestone payments and royalties from the sale of products developed under licenses of our intellectual property.

Operating Expenses

The following table summarizes our operating expenses for the three and six months ended June 30, 2017 and 2016:

(in thousands)	THREE MONTHS ENDED JUNE 30,		SIX MONTHS ENDED JUNE 30,	
	2017	2016	2017	2016
Research and development	$ 10,475	$ 9,235	$ 16,252	$ 14,116
General and administrative	3,520	1,830	6,265	3,783
	$ 13,955	$ 11,065	$ 22,517	$ 17,899

Research and Development Expenses

We are currently developing rosiptor as a treatment for IC/BPS. In 2015, we completed and reported results from our Leadership 201 trial, a multicenter, randomized, double-blind, placebo-controlled, Phase 2 clinical trial investigating the ability of 200 mg oral, once daily rosiptor to reduce pain and urinary symptoms in 69 female patients with IC/BPS. Results demonstrated a positive trend in the primary endpoint and statistically significant changes on secondary endpoints. We are proceeding with further development of rosiptor in IC/BPS and have initiated the first Phase 3 clinical trials of rosiptor in IC/BPS. The first trial (Leadership 301 trial), which commenced enrollment in the third quarter of 2016, is a three-arm, multicenter, randomized, double-blind, placebo-controlled Phase 3 clinical trial, with 12 weeks dosing followed by an extension period of 52 weeks, to assess the efficacy and safety of rosiptor in both female and male IC/BPS patients. The Leadership 301 trial is anticipated to enroll a minimum of 300 female patients, up to a maximum of 600 patients including males, at clinical research centers in the United States, Canada and Europe. The Leadership 301 trial is investigating the ability of 200 mg and 100 mg oral, once daily rosiptor to reduce bladder pain in patients with IC/BPS. Patients are randomized to receive one of the two potential doses of rosiptor or placebo. The primary endpoint of the Leadership 301 trial is to measure the difference in the change from baseline in the maximum daily bladder pain score based on an 11-point numeric rating scale (NRS) at twelve weeks recorded by electronic diary. The trial also includes an extension period of 52 weeks affording all participating patients the opportunity for treatment with rosiptor. Additional endpoints will include urinary symptoms, including frequency and nighttime awakenings to void, as well as measures of quality of life.

We continue to engage with the FDA and other regulatory authorities to discuss the safety and efficacy trials and analyses required for approval of rosiptor in IC/BPS in the U.S., Europe, and other targeted countries. We continue to evaluate the design and number of additional trials that we will conduct in order to meet ICH patient exposure guidelines and demonstrate the safety and efficacy of rosiptor in support of a potential NDA filing. We expect the data from the Leadership 301 trial, as well as further discussions with regulatory authorities, will be fundamental in defining the number, design and size of these additional trials.

Our research and development expenses consist primarily of costs incurred for the development of rosiptor and other future product candidates. Research and development expenses include:

- costs associated with research, development and regulatory activities;
- employee-related expenses, including salaries, benefits, travel and stock-based compensation expense;
- expenses incurred under agreements with CROs and investigative sites that conduct our clinical trials and preclinical studies;
- the cost of acquiring and manufacturing our products, for preclinical studies and clinical trials;
- cost incurred in relation to purchase of technology licenses and patent rights; and
- facilities, and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, amortization of equipment and leasehold improvements, insurance and supplies.

Overall, research and development expenses for the three and six months ended June 30, 2017 were $10.5 million and $16.3 million, respectively, compared to $9.2 million and $14.1 million for the three and six months ended June 30, 2016, respectively. Higher expenditure during the three and six months ended June 30, 2017 was primarily driven by increased clinical activities as we continued our Leadership 301 clinical trial of rosiptor in IC/BPS. We expect to incur progressively higher research and development expenses in the foreseeable future as we continue our development of rosiptor, particularly as a result of our Leadership 301 trial and other clinical development activities in IC/BPS.

General and Administrative Expenses

General and administrative expenses consist primarily of personnel related costs (including stock-based compensation and travel expenses), facility-related costs, insurance, public company expenses and professional fees for consulting, legal and accounting services.

For the three and six months ended June 30, 2017, general and administrative expenses were $3.5 million and $6.3 million, respectively, compared to $1.8 million and $3.8 million for the three and six months ended June 30, 2016, respectively. The increase was primarily the result of higher personnel related costs and pre-commercial and market assessment activities.

Other income, net

(in thousands)	THREE MONTHS ENDED JUNE 30, 2017	THREE MONTHS ENDED JUNE 30, 2016	SIX MONTHS ENDED JUNE 30, 2017	SIX MONTHS ENDED JUNE 30, 2016
Change in fair value of derivative liability	$ —	$ 23	$ —	$ 73
Foreign exchange (losses) gain	(12)	6	(20)	(20)
Interest income	250	128	484	249
Miscellaneous (expenses) income	(9)	7	(29)	(15)
Total other income, net	$ 229	$ 164	$ 435	$ 287

There was no change in fair value of derivative liability for the three and six months ended June 30, 2017, as we did not have any derivative liability as of December 31, 2016 or June 30, 2017. The change in fair value of derivative liability for the three and six months ended June 30, 2016 resulted from the change in fair value of a warrant issued to SVB under the terms of a loan agreement. The warrant was being re-measured at each balance sheet date. In September 2016, SVB exercised the warrant on a cashless basis as provided for under the warrant agreement, and as a result we issued 3,001 shares of common stock to SVB as net settlement for the exercise of the warrant.

Foreign exchange losses and gains were insignificant as the net effect of change in foreign exchange rates on our foreign currency holdings was offset by the net effect on our foreign currency liabilities.

Interest income increased significantly during the three and six months ended June 30, 2017 compared to the same period in 2016 as a result of higher cash and investment balances for the three and six months ended June 30, 2017.

Liquidity and Capital Resources

Since our inception, we have incurred net losses and negative cash flows from our operations. Our operating activities used $21.6 million and $13.8 million of cash flows during the six months ended June 30, 2017 and 2016, respectively. As of June 30, 2017, we had an accumulated deficit of $170.4 million, working capital of $124.1 million and cash, cash equivalents and short-term investments of $131.3 million.

Cash Flows

The following table summarizes our cash flows for the six months ended June 30, 2017 and 2016:

(in thousands)	SIX MONTHS ENDED JUNE 30, 2017	SIX MONTHS ENDED JUNE 30, 2016
Net cash (used in) provided by:		
Operating activities	$ (21,583)	$ (13,788)
Investing activities	16,048	(3,302)
Financing activities	370	3
Effect of exchange rate changes on cash and cash equivalents	11	31
Net change in cash and cash equivalents	$ (5,154)	$ (17,056)

Net cash used in operating activities

Net cash used in operating activities for the six months ended June 30, 2017 increased due to higher operating expenses compared to the six months ended June 30, 2016 and timing of expenditures. During the six months ended June 30, 2017, we paid significant amounts related to expenditures previously incurred in 2016 whereas in the six months ended June 30, 2016 we incurred significant expenditures with payments occurring after June 30, 2016.

Net cash provided by (used in) investing activities

Net cash provided by investing activities for the six months ended June 30, 2017 increased compared to net cash used by investing activities for the six months ended June 30, 2016 due to investments maturing during the six months ended June 30, 2017 compared to a net purchase of investments during the six months ended June 30, 2016.

Net cash provided by financing activities

For the six months ended June 30, 2017, net cash provided by financing activities was the result of the exercise of stock options.

Operating and Capital Expenditure Requirements

We have not generated product revenue or achieved profitability since our inception and we expect to continue to incur net losses for the foreseeable future. We expect our cash expenditures to increase as we continue our development of rosiptor in IC/BPS, as well as other clinical and preclinical activities. We believe that our existing capital resources will be sufficient to fund our operations for at least the next 12 months and we anticipate that we will need to raise substantial financing in the future to fund our operations. In order to meet these additional cash requirements, we may seek to sell additional equity or convertible debt securities that may result in dilution to our stockholders. If we raise additional funds through the issuance of convertible debt securities, these securities could have rights senior to those of our common stock and could contain covenants that restrict our operations. There can be no assurance that we will be able to obtain additional equity or debt financing on terms acceptable to us, if at all. Our failure to obtain sufficient funds on acceptable terms when needed could have a negative impact on our business, results of operations, financial condition, cash flows and future prospects. Our future capital requirements will depend on many factors, including:

- our Phase 3 Leadership 301 trial of rosiptor in IC/BPS and any other future clinical trials;
- the number and characteristics of any future product candidates we develop or may acquire;
- the scope, progress, results and costs of researching and developing our product candidates or any future product candidates, and conducting preclinical studies and clinical trials;
- the timing of, and the costs involved in, obtaining regulatory approvals for rosiptor or any future product candidates;
- the cost of manufacturing rosiptor and our future product candidates and any products that may achieve regulatory approval;
- the cost of commercialization activities if rosiptor or any future product candidates are approved for sale, including marketing, sales and distribution costs;
- the timing, receipt and amount of sales of, or royalties on, future approved products, if any;
- our ability to establish and maintain strategic collaborations, licensing or other arrangements and the financial terms of such agreements;
- any product liability or other lawsuits related to our products;
- the expenses needed to attract and retain skilled personnel;
- the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing patent claims, including litigation costs and the outcome of such litigation.

Please see Item 1A of Part II of this Quarterly Report titled “Risk Factors” for additional risks associated with our substantial capital requirements.

Contractual Obligations and Commitments

Our future minimum contractual commitments were reported in our Annual Report on Form 10-K for the year ended December 31, 2016, as filed with the SEC. There have been no material changes from the contractual commitments previously discussed in that Annual Report on Form 10-K.

Critical Accounting Policies and Significant Judgments and Estimates

The preparation of these financial statements in accordance with U.S. GAAP requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenue and expenses and the disclosure of contingent assets and liabilities in our consolidated financial statements. On an ongoing basis, we evaluate our estimates and judgments, including those related to accrued liabilities and stock-based compensation. We base our estimates on historical experience, known trends and events and various other factors that we believe to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions. A summary of our significant accounting policies is presented in Part II, Item 8, of our Annual Report on Form 10-K for the year ended December 31, 2016. Apart from the adoption of new accounting pronouncements discussed below, there have been no material changes to our significant accounting policies during the three and six months ended June 30, 2017.

Recent Accounting Pronouncements

We adopted FASB ASU 2015-17 “Income Taxes (Topic 740): Balance Sheet Classification of Deferred Taxes,” effective January 1, 2017 which requires entities to present deferred tax assets and deferred tax liabilities as noncurrent in a classified balance sheet. The previous guidance required entities to separately present deferred tax assets and deferred tax liabilities as current or noncurrent in a classified balance sheet. This change did not have a material impact on our financial statements as a full valuation allowance has been applied against the deferred tax assets.

We adopted FASB ASU 2016-09 “Compensation-Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting,” effective January 1, 2017 which simplifies several aspects of the accounting for employee share-based payment transactions, including the accounting for income taxes, forfeitures, and statutory tax withholding requirements, as well as classification in the statement of cash flows. We have elected to change our accounting policy to account for forfeitures as they occur instead of on an estimated basis as this will more accurately reflect the cost of forfeitures. The change has been applied on a modified retrospective approach with a cumulative-effect adjustment to retained deficit of an immaterial amount as of January 1, 2017. The provisions relating to the accounting for income taxes has no significant impact on the consolidated financial statements as we apply a full valuation allowance against our deferred tax assets.

In January 2016, the FASB issued ASU 2016-01 “Financial Instruments-Overall (Subtopic 825-10): Recognition and Measurement of Financial Assets and Financial Liabilities,” which revises an entity’s accounting related to (1) the classification and measurement of investments in equity securities and (2) the presentation of certain fair value changes for financial liabilities measured at fair value. The ASU also amends certain disclosure requirements associated with the fair value of financial instruments. ASU 2016-01 is effective for fiscal years and interim periods within those fiscal years beginning after December 15, 2017, with early adoption permitted under certain circumstances. We are currently assessing the impact of ASU 2016-01 on our financial statements.

In February 2016, the FASB issued ASU 2016-02 “Leases (Topic 842)”, which requires the recognition of right-of-use assets and lease liabilities by lessees for those leases with a lease term of greater than 12 months. Upon the adoption of ASU 2016-02, leases will be recognized and measured at the beginning of the earliest period presented using a modified retrospective approach. The modified retrospective approach includes a number of optional practical expedients that companies may elect to apply. ASU 2016-02 is effective for fiscal years and interim periods beginning after December 15, 2018, with early adoption permitted. We are currently assessing the impact of ASU 2016-02 on our financial statements and whether to elect to apply the optional practical expedients under the modified retrospective approach.

Off-Balance Sheet Arrangements

We do not have any off-balance sheet arrangements.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

Management believes there have been no material changes to our quantitative and qualitative disclosures about market risks during the six months ended June 30, 2017, compared to those discussed in our Annual Report on Form 10-K for the year ended December 31, 2016, filed with the SEC.

Interest rate risk

Our exposure to market risk for changes in interest rates relates primarily to our investment portfolio. As of June 30, 2017, we had holdings in U.S. government securities of $121.9 million. We have estimated the effect on our investment portfolio of a hypothetical increase in interest rates by one percent (100 basis points) to be a reduction of $0.5 million in the fair value of our investment portfolio as of June 30, 2017.

Foreign Currency Risk

Our exposure to foreign currency risk relates primarily to our Canadian operations, including payments we make to vendors and suppliers. We currently do not hedge against foreign currency risk. If the Canadian dollar strengthens against the U.S. dollar, it can result in higher expenditures and have a negative impact on our financial results. We also maintain bank balances in foreign currencies such as the Canadian dollar and the Euro. If these foreign currencies decline against the U.S. dollar, it can have a negative impact on our financial positions. For the three and six months ended June 30, 2017 and 2016, foreign exchange gains (losses) were insignificant as the impact of changes in foreign exchange rates on our foreign currency portfolio was offset by its impact on our foreign currency liabilities.

Item 4. Controls and Procedures

Evaluation of disclosure controls and procedures. Under the supervision and with the participation of our principal executive officer and principal financial officer, our management conducted an evaluation of the effectiveness of the design and operation of our disclosure controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, as of the end of the period covered by this report.

In designing and evaluating our disclosure controls and procedures, management recognizes that any disclosure controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives. In addition, the design of disclosure controls and procedures must reflect the fact that there are resource constraints and that management is required to apply its judgment in evaluating the benefits of possible controls and procedures relative to their costs.

Based on management's evaluation, our principal executive officer and principal financial officer concluded that our disclosure controls and procedures are designed to, and are effective to, provide assurance at a reasonable level that the information we are required to disclose in reports that we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in SEC rules and forms, and that such information is accumulated and communicated to our management, including our Chief Executive Officer and Chief Financial Officer, as appropriate, to allow timely decisions regarding required disclosures.

Changes in internal control over financial reporting. There was no change in our internal control over financial reporting identified in connection with the evaluation required by Rule 13a-15(d) and 15d-15(d) of the Exchange Act that occurred during the period covered by this report 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 may from time to time be named as a party to legal claims, actions and complaints, including matters involving employment, intellectual property or others.

Item 1A. Risk Factors

You should carefully consider the following risk factors, in addition to the other information contained in this Quarterly Report on Form 10-Q, including our condensed consolidated financial statements and related notes. If any of the events described in the following risk factors occurs, our business, operating results and financial condition could be seriously harmed. This Quarterly Report on Form 10-Q also contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those anticipated in the forward-looking statements as a result of factors that are described below and elsewhere in this Quarterly Report on Form 10-Q. We have marked with an asterisk (*) those risk factors below that reflect significant changes from the risk factors included in our Annual Report on Form 10-K for the year ended December 31, 2016.

Risks Related to Our Financial Position and Capital Needs

We have incurred significant losses in every quarter since our inception and anticipate that we will continue to incur significant losses in the future.*

We are a clinical-stage pharmaceutical company with a limited operating history. Investment in pharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that any potential product candidate will fail to demonstrate adequate effect or an acceptable safety profile, gain regulatory approval or become commercially viable. We do not have any products approved by regulatory authorities for marketing or commercial sale and have not generated any revenue from product sales, or otherwise, to date, and we continue to incur significant research, development and other expenses related to our ongoing operations. As a result, we are not profitable and have incurred losses in every reporting period since our inception in 2003. For the years ended December 31, 2016 and 2015, and for the six months ended June 30, 2017, we reported a net loss of $37.0 million, $21.9 million and $22.1 million, respectively. As of June 30, 2017, we had an accumulated deficit since inception of $170.4 million.

We expect to continue to incur significant expenses and operating losses for the foreseeable future. We anticipate these losses to increase as we continue the research and development of, and seek regulatory approvals for, rosiptor and any of our future product candidates, and potentially begin to commercialize any products that may achieve regulatory approval. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our financial condition. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenues. Our prior losses and expected future losses have had, and will continue to have, an adverse effect on our financial condition. If rosiptor or any future product candidate fails in clinical trials or does not gain regulatory approval, or if approved, fails to achieve market acceptance, we may never become profitable. Even if we achieve profitability in the future, we may not be able to sustain profitability in subsequent periods.

We have a limited operating history, which may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

Our operations to date have been primarily limited to organizing and staffing our company, acquiring product and technology rights, discovering and developing novel small molecule drug candidates and undertaking preclinical studies and clinical trials of rosiptor. We have not yet obtained regulatory approval for rosiptor or any product candidate. Consequently, evaluating our performance, viability or possibility of future success will be more difficult than if we had a longer operating history or approved products on the market.

We currently have no source of product revenue and may never become profitable.

To date, we have not generated any revenues from commercial product sales, or otherwise. Our ability to generate revenue from product sales and achieve profitability will depend upon our ability, alone or with any future collaborators, to successfully commercialize products, including rosiptor or any future product candidates that we may develop, in-license or acquire in the future. Even if we are able to successfully achieve regulatory approval for rosiptor or any future product candidates, we do not know when any of these products will generate revenue from product sales for us, if at all. Our ability to generate revenue from product sales from rosiptor, or any of our future product candidates, also depends on a number of additional factors, including our or any future collaborators' ability to:

- complete development activities, including the necessary clinical trials;
- complete and submit new drug applications, or NDAs, to the U.S. Food and Drug Administration, or FDA, and obtain regulatory approval for indications for which there is a commercial market;
- complete and submit applications to, and obtain regulatory approval from, foreign regulatory authorities;
- set a commercially viable price for our products;
- establish and maintain supply and manufacturing relationships with third parties, and ensure adequate and legally compliant manufacturing of bulk drug substances and drug products to maintain that supply;
- develop a commercial organization capable of sales, marketing and distribution for any products for which we obtain marketing approval and intend to sell ourselves in the markets in which we choose to commercialize on our own;
- find suitable distribution partners to help us market, sell and distribute our approved products in other markets;
- obtain coverage and adequate reimbursement from third-party payors, including government and private payors;
- achieve market acceptance for our products, if any;

- establish, maintain and protect our intellectual property rights; and
- attract, hire and retain qualified personnel.

In addition, because of the numerous risks and uncertainties associated with pharmaceutical product development, including that rosiptor, or any future product candidates, may not advance through development or achieve the endpoints of applicable clinical trials, we are unable to predict the timing or amount of increased expenses, or when or if we will be able to achieve or maintain profitability. In addition, our expenses could increase beyond expectations if we decide, or are required by the FDA or foreign regulatory authorities, to perform studies or trials in addition to those that we currently anticipate. Even if we are able to complete the development and regulatory process for rosiptor or any future product candidates, we anticipate incurring significant costs associated with commercializing these products.

Even if we are able to generate revenues from the sale of rosiptor or any future product candidates that may be approved, we may not become profitable and may need to obtain additional funding to continue operations. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce our operations.

Our operating results may fluctuate significantly on a quarterly and annual basis, which may make our future operating results difficult to predict and could cause our operating results to fall below expectations or any guidance we may provide.

Our quarterly and annual operating results have varied significantly in the past and may continue to fluctuate significantly in the future from quarter-to-quarter or year-to-year due to a variety of factors, many of which are beyond our control, which may make it difficult for us to predict our future operating results. Factors that may contribute to these fluctuations include the following, as well as other factors described elsewhere in this report:

- our ability to obtain additional funding for research and development and manufacturing activities relating to rosiptor or any of our future product candidates;
- the timing and cost of research and development activities relating to rosiptor or any of our future product candidates, which may change from time to time, including the number, size and duration of clinical trials required to demonstrate safety and efficacy;
- the cost of manufacturing rosiptor or any of our future product candidates, which may vary depending on the quantity of production and the terms of our agreements with manufacturers;
- expenditures that we will or may incur to acquire or develop additional product candidates and technologies;
- the level of demand for rosiptor or any of our future product candidates, should they receive approval, which may vary significantly;
- our ability to enroll patients in clinical trials, in particular our Leadership 301 clinical trial and any other Phase 3 trials of rosiptor;
- the success or failure of clinical trials through all phases of clinical development for rosiptor or any of our future product candidates or competing product candidates, including our Leadership 301 clinical trial and any other Phase 3 trials of rosiptor, or any other change in the competitive landscape of our industry;
- any delays in regulatory review and approval of rosiptor or any of our future product candidates;
- potential side effects of rosiptor or our future product candidates that could delay or prevent commercialization or cause an approved drug to be taken off the market;
- the ability of patients or healthcare providers to obtain coverage of, or sufficient reimbursement for, rosiptor or our future product candidates and our ability to achieve acceptance among patients and physicians;
- competition from existing and potential future drugs that compete with rosiptor or our future product candidates;
- our ability to receive approval and commercialize rosiptor or our future product candidates outside of the United States;
- our dependency on third-party manufacturers to supply or manufacture our rosiptor or our future product candidates;
- our ability to establish or maintain collaborations, licensing or other arrangements;
- our ability and third parties' abilities to protect intellectual property rights;
- costs related to, and outcomes of, potential intellectual property litigation;
- costs associated with recently enacted healthcare legislation;
- our ability to adequately support future growth;
- our ability to attract and retain key personnel to manage our business effectively;
- our ability to build our finance infrastructure and improve our accounting systems and controls;

- potential product liability claims;
- potential liabilities associated with hazardous materials;
- fluctuations in foreign currency exchange rates;
- our ability to use potential future operating losses and our federal and state net operating loss carryforwards to offset taxable income;
- potential unforeseen business disruptions that increase our costs or expenses;
- our ability to maintain adequate insurance policies; and
- the changing and volatile U.S., European and global economic environments.

Investors should not rely on our quarterly or annual results as an indication of our future performance. This variability and unpredictability could also result in our failing to meet the expectations of industry or financial analysts or investors for any period. If our operating results fall below the expectations of analysts or investors or below any forecasts we may provide to the market, or if the forecasts we provide to the market are below the expectations of analysts or investors, the price of our common stock could decline substantially.

We are likely to require additional capital to finance our operations which may not be available to us on acceptable terms, or at all. If we fail to obtain necessary financing, we may be unable to complete the development and potential commercialization of rosiptor or develop future product candidates.

Developing pharmaceutical products, including conducting preclinical studies and clinical trials, is expensive. Our operations have consumed substantial amounts of cash since inception. We expect research and clinical development expenses to increase substantially in connection with our ongoing activities, particularly as we advance rosiptor or future product candidates in clinical trials and launch and commercialize any product candidates for which we receive regulatory approval, including potentially building our own commercial organization. We believe that our existing cash, cash equivalents, short-term and long-term investments will be sufficient to fund our operating requirements for at least the next 12 months. However, circumstances may cause us to consume capital more rapidly than we anticipate. We will likely require additional capital for the further development and potential commercialization of rosiptor or future product candidates and may also need to raise additional funds sooner to pursue a more accelerated development of rosiptor or future product candidates.

If we need to secure additional financing, fundraising efforts may divert our management from our day-to-day activities, which may adversely affect our ability to develop and commercialize rosiptor or future product candidates. In addition, we cannot guarantee that future financing will be available in sufficient amounts or on terms acceptable to us, if at all. If we do not raise additional capital when required or on acceptable terms, we may need to:

- significantly delay, scale back or discontinue clinical trials related to the development or commercialization of rosiptor or any of our future product candidates or cease operations altogether;
- seek strategic alliances for research and development programs at an earlier stage than we would otherwise desire or on terms less favorable than might otherwise be available; or
- relinquish, or license on unfavorable terms, our rights to rosiptor or any future product candidates that we otherwise would seek to develop or commercialize ourselves.

If we need to conduct additional fundraising activities and we do not raise additional capital in sufficient amounts or on terms acceptable to us, we may be prevented from pursuing development and commercialization efforts, which will have a material adverse effect on our business, operating results and prospects.

Our forecast of the period of time through which our financial resources will be adequate to support our operations is a forward-looking statement and involves risks and uncertainties, and actual results could vary as a result of a number of factors, including the factors discussed elsewhere in this “Risk Factors” section. We have based this estimate on assumptions that may prove to be wrong, and we could spend our available capital resources sooner than we currently expect. Our future funding requirements, both near and long-term, will depend on many factors, including, but not limited to:

- the initiation, progress, timing, costs and results of clinical trials for rosiptor and any future product candidates;
- the clinical development plans we establish for rosiptor or any future product candidates;

- the achievement of milestones and our obligation to make milestone payments under our present or any future in-licensing agreements;
- the number and characteristics of product candidates that we discover, or in-license and develop;
- the outcome, timing and cost of regulatory review by the FDA and comparable foreign regulatory authorities, including the potential for the FDA or comparable foreign regulatory authorities to require that we perform more studies than those that we currently expect;
- the cost to establish, maintain, expand and defend the scope of our intellectual property portfolio, including the amount and timing of any payments we may be required to make, or that we may receive, in connection with licensing, preparing, filing, prosecuting, defending and enforcing any patent claims and maintaining and enforcing other intellectual property rights;
- the effect of competing technological and market developments;
- the costs and timing of the implementation of commercial-scale outsourced manufacturing activities; and
- the costs and timing of establishing sales, marketing, distribution and pharmacovigilance capabilities for any product candidates for which we may receive regulatory approval in territories where we choose to commercialize products on our own.

If we are unable to expand our operations or otherwise capitalize on our business opportunities due to a lack of capital, our business, results of operations, financial condition and cash flows and future prospects could be materially adversely affected.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies, rosiptor or any future product candidates.

Until we can generate substantial revenue from product sales, if ever, we expect to finance future cash needs through a combination of private and public equity offerings, debt financings, strategic collaborations and alliances and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interests of our stockholders will be diluted, and the terms may include liquidation or other preferences that adversely affect the rights of our stockholders. Additional capital may not be available on reasonable terms, if at all. If we raise additional funds through the issuance of additional debt or equity securities that could result in dilution to our stockholders, and/or increased fixed payment obligations. Furthermore, these securities may have rights senior to those of our common stock and could contain covenants that include restrictive covenants limiting our ability to take important actions and potentially impair our competitiveness, such as limitations on our ability to incur additional debt, make capital expenditures, acquire, sell or license intellectual property rights or declare dividends. If we raise additional funds through strategic collaborations and alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, rosiptor or our future product candidates, or grant licenses on terms that are not favorable to us. If we are unable to raise additional funds through equity or debt financing when needed, we may be required to delay, limit, reduce or terminate our product development or commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

We plan to use potential future operating losses and our federal and state net operating loss, or NOL, carryforwards to offset taxable income from revenue generated from operations or corporate collaborations. However, our ability to use NOL carryforwards could be limited.

We plan to use our current year operating losses to offset taxable income from any revenue generated from operations or corporate collaborations. To the extent that our taxable income exceeds any current year operating losses, we plan to use our NOL carryforwards to offset income that would otherwise be taxable. However, under the Tax Reform Act of 1986, the amount of benefits from our NOL carryforwards may be impaired or limited if we incur a cumulative ownership change of more than 50%, as interpreted by the U.S. Internal Revenue Service, over a three-year period. As a result, our use of federal NOL carryforwards could be limited by the provisions of Section 382 of the U.S. Internal Revenue Code of 1986, as amended, as a result of historical or future equity offerings and/or other changes in our stock ownership, some of which are outside our control. State NOL carryforwards may be similarly limited. Any such disallowances may result in greater tax liabilities than we would incur in the absence of such a limitation and any increased liabilities could adversely affect our business, results of operations, financial condition and cash flow and future prospects.

The acquisition of control of AQXP Canada could result in adverse Canadian tax consequences, including limitations on AQXP Canada's ability to use non-capital loss carryforwards and other similar tax attributes to offset taxable income for Canadian tax purposes.

We underwent a reorganization immediately prior to the closing of our initial public offering in March 2014 which resulted in AQXP Canada becoming a wholly owned subsidiary of Aquinox USA through an exchange of shares. Under the Income Tax Act (Canada), referred to herein as the Tax Act, in connection with the exchange of shares of AQXP Canada for shares of Aquinox USA, there may be limitations on AQXP Canada's ability to use its non-capital loss carryforwards and other similar tax attributes following the acquisition of control. In general, an acquisition of control would result in AQXP Canada losing its net capital loss carryforwards, if any, and AQXP Canada's non-capital loss carryforwards and other similar tax attributes only being "useable" to offset income, excluding capital gains, derived from the business operated by AQXP Canada that generated such tax attributes or a business "similar" to such business and provided the business that generated the tax attributes continues to be carried on by AQXP Canada for profit or with a reasonable expectation of profit. We expect that we will continue to carry on the business of AQXP Canada for profit or with a reasonable expectation of profit and that, accordingly, its non-capital loss carryforwards and other similar tax attributes should be available to offset future income for Canadian tax purposes to the extent of income from that business or "similar" businesses, subject to expiry of such loss carryforwards over time pursuant to the provisions of the Tax Act. If our use of these non-capital loss carryforwards or other similar tax attributes is restricted as a result of an acquisition of control or otherwise, our Canadian federal income tax liability may be materially increased, which could adversely affect our business, results of operations, financial condition and cash flow and future prospects.

Fluctuations in foreign currency exchange rates could result in changes in our reported financial results.

We currently incur significant expenses denominated in foreign currencies, specifically in connection with our operations in Canada. In addition, we utilize numerous clinical trial sites as part of our clinical trials for rosiptor, many of which are located in various countries outside of the United States. These clinical trial sites invoice us in the local currency of the site. We do not engage in foreign currency hedging arrangements for our accounts payable, and, consequently, foreign currency fluctuations may adversely affect our earnings. We may decide to manage this risk by hedging our foreign currency exposure, principally through derivative contracts. Even if we decide to enter into such hedging transactions, we cannot be sure that such hedges will be effective or that the costs of such hedges will not exceed their benefits. Fluctuations in the rate of exchange between the U.S. dollar and foreign currencies, primarily the Canadian dollar, could result in material amounts of cash being required to settle the hedge transactions or could adversely affect our financial results.

Risks Related to Our Business and Industry

Our future success is dependent primarily on the regulatory approval and commercialization of rosiptor and any of our future product candidates.

We do not have any products that have gained regulatory approval. Currently, our only clinical-stage product candidate is rosiptor, which has completed three Phase 2 clinical trials. In September 2016, we initiated dosing in our Leadership 301 Phase 3 clinical trial of rosiptor for the treatment of IC/BPS.

As a result, our near-term prospects, including our ability to finance our operations and generate revenue, are substantially dependent on our ability to obtain regulatory approval for, and, if approved, to successfully commercialize rosiptor in a timely manner. Our near-term prospects are largely dependent on our ability to obtain favorable results from our Leadership 301 clinical trial in IC/BPS, as well as our anticipated additional clinical trials with rosiptor. We cannot commercialize rosiptor or our future product candidates in the United States without first obtaining regulatory approval for the product from the FDA; similarly, we cannot commercialize rosiptor or our future product candidates outside of the United States without obtaining regulatory approval from comparable foreign regulatory authorities. The FDA review process for an NDA typically takes more than a year to complete and approval is never guaranteed. Before obtaining regulatory approvals for the commercial sale of rosiptor or our future product candidates for a target indication, we must demonstrate with substantial evidence gathered in preclinical and well-controlled clinical trials, generally including at least two well-controlled Phase 3 trials, and, with respect to approval in the United States, to the satisfaction of the FDA, that the product candidate is safe and effective for use for that target indication and that the manufacturing facilities, processes and controls are adequate. We may be required to conduct more clinical trials, or clinical trials of a greater size and/or duration than currently contemplated, in order to receive regulatory approval. Obtaining regulatory approval for marketing of rosiptor or our future product candidates in one country does not ensure we will be able to obtain regulatory approval in other countries but a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory process in other countries.

Even if rosiptor or any of our future product candidates were to successfully obtain approval from the FDA and comparable foreign regulatory authorities, any approval might contain significant limitations related to use restrictions for specified age groups, gender or subpopulation of target indication, warnings, precautions or contraindications, or may be subject to burdensome post-approval study or risk management requirements. If we are unable to obtain regulatory approval for rosiptor in one or more jurisdictions, or any approval contains significant limitations, we may not be able to obtain sufficient funding or generate sufficient revenue to continue the development of any of our future product candidate that we may discover, in-license, develop or acquire in the future. Also, any regulatory approval of any of rosiptor or our future product candidates, once obtained, may be withdrawn. Furthermore, even if we obtain regulatory approval for rosiptor, the commercial success of rosiptor will depend on a number of factors, including the following:

- development of a commercial organization or establishment of a commercial collaboration with a commercial infrastructure;
- establishment of commercially viable pricing and adequate reimbursement from third-party and government payors;
- the ability of our third-party manufacturers to manufacture quantities of rosiptor using commercially sufficient processes and at a scale sufficient to meet anticipated demand and enable us to reduce our cost of manufacturing;
- our success in educating physicians and patients about the benefits, administration and use of rosiptor;
- the availability, perceived advantages, relative cost, relative safety and relative efficacy of alternative and competing treatments;
- the effectiveness of our own or our potential strategic collaborators' marketing, sales and distribution strategy and operations;
- acceptance of rosiptor as safe and effective by patients and the medical community; and
- a continued acceptable safety profile of rosiptor following approval.

Many of these factors are beyond our control. If we, or our potential commercialization collaborators, are unable to successfully commercialize rosiptor, we may not be able to earn sufficient revenues to continue our business.

Because the results of preclinical testing or earlier clinical trials are not necessarily predictive of future results, rosiptor or any future product candidate we advance into clinical trials, may not have favorable results in later clinical trials or receive regulatory approval.

Success in preclinical testing and early clinical trials does not ensure that later clinical trials will generate adequate data to demonstrate the efficacy and safety of an investigational drug. A number of companies in the pharmaceutical and biotechnology industries, including those with greater resources and experience, have suffered significant setbacks in clinical trials, even after seeing promising results in earlier clinical trials. Despite the results reported in earlier clinical trials for rosiptor, we do not know whether the Leadership 301 clinical trial, or any other clinical trials we may conduct, will demonstrate adequate efficacy and safety to result in regulatory approval to market rosiptor or any of our future product candidates in any particular jurisdiction. For example, our Phase 2 Leadership 201 clinical trial in IC/BPS, the results of which were announced in June 2015, failed to demonstrate statistically significant results in its primary endpoint. In addition, despite showing positive results in our chronic obstructive pulmonary disease, or COPD, proof-of-concept trial following a lipopolysaccharide (LPS) challenge in healthy subjects, our Phase 2 Flagship clinical trial with rosiptor, the results of which we announced in July 2015, failed to demonstrate efficacy in COPD patients with a history of frequent exacerbations. Our Phase 2 Kinship clinical trial, the results for which we announced in November 2015, failed to demonstrate efficacy in patients with mild to moderate atopic dermatitis. Even if we believe that we have adequate data to support an application for regulatory approval to market our product candidates, FDA or other applicable foreign regulatory authorities may not agree and may require we conduct additional clinical trials. If later-stage clinical trials do not produce favorable results, our ability to achieve regulatory approval for any of our product candidates may be adversely impacted.

We have not yet established the optimal dose for rosiptor. There can be no guarantee that the 200 mg dose studied in our Phase 2 clinical trial will be efficacious or, if it is, whether it will be the optimal dose to continue development with. It is possible that we may need to conduct additional clinical trials to evaluate additional dose levels of rosiptor. There cannot be any guarantee that any of these trials will be successful in determining a dose of rosiptor suitable for marketing approval.

SHIP1 has not been validated as a target.

Our primary focus is small molecule anti-inflammatory product candidates targeting SHIP1, which is a key regulator of an important cellular signaling pathway in immune cells. To date, no drug which specifically targets SHIP1 has been

demonstrated to provide clinical benefit or been approved by any regulatory authority for the treatment of disease. Therefore, SHIP1 has not been validated as a target. We are therefore pursuing development of rosiptor against a novel and unproven target. We believe rosiptor is the only SHIP1 activator currently in clinical trials. SHIP1 activators as a class of drug, including rosiptor, may ultimately prove unsuitable for treatment of human diseases, or if approved for treatment of human diseases, may be commercially unsuccessful, either of which could cause our business to fail.

Clinical drug development involves a lengthy and expensive process with an uncertain outcome.

Clinical testing is expensive, can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and early clinical trials.

We have experienced delays in site initiation and were initially behind our anticipated schedule in our ongoing Leadership 301 clinical trial and may experience delays in future clinical trials. We will not know whether planned clinical trials will begin or enroll subjects on time, need to be redesigned or will be completed on schedule, if at all. There can be no assurance that the FDA or other comparable foreign regulatory authority will not put clinical trials of rosiptor or any other of our product candidates on clinical hold in the future. Clinical trials may be delayed, suspended or prematurely terminated for a variety of reasons, such as:

- delay or failure in reaching agreement with the FDA or a comparable foreign regulatory authority on a trial design that we are able to execute;
- delay or failure in obtaining authorization to commence a trial or inability to comply with conditions imposed by a regulatory authority regarding the scope or design of a clinical trial;
- delay or failure in reaching agreement on acceptable terms with prospective contract research organizations, or CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- delay or failure in obtaining institutional review board (IRB) or ethics committee approval or the approval of other reviewing entities, including comparable foreign regulatory authorities, to conduct a clinical trial at each site;
- withdrawal of clinical trial sites from our clinical trials as a result of changing standards of care or the ineligibility of a site to participate in our clinical trials;
- delay or failure in recruiting and enrolling suitable subjects to participate in a trial;
- delay or failure in subjects completing a trial or returning for post-treatment follow-up;
- clinical sites and investigators deviating from trial protocol, failing to conduct the trial in accordance with regulatory requirements, or dropping out of a trial;
- inability to identify and maintain a sufficient number of trial sites, many of which may be engaged in other clinical trial programs, including some that may be for the same indication;
- failure of our third parties, such as CROs, to satisfy their contractual duties or meet expected deadlines;
- delay or failure in adding new clinical trial sites;
- ambiguous or negative interim results or results that are inconsistent with earlier results;
- feedback from the FDA, the IRB, data safety monitoring boards, or a comparable foreign regulatory authority, or results from earlier stage or concurrent preclinical studies and clinical trials, that might require modification to the protocol for the trial;
- decision by the FDAor a comparable foreign regulatory authority to impose a clinical hold following an inspection of our clinical trial operations or trial sites, or recommendation by a data safety monitoring board, the IRB or us, to suspend or terminate clinical trials at any time for safety issues or for any other reason;
- unacceptable risk-benefit profile, unforeseen safety issues or adverse side effects;
- failure to demonstrate a benefit from using a drug;
- delays in the testing, validation, manufacturing and delivery of the investigational or placebo products to the clinical sites;
- lack of adequate funding to continue the clinical trial, including the incurrence of unforeseen costs due to enrollment delays, requirements to conduct additional clinical trials or increased expenses associated with the services of our CROs and other third parties; or
- changes in governmental regulations or administrative actions.

As an organization, we have never completed a Phase 3 pivotal clinical trial or submitted an NDA to the FDA or other marketing applications to comparable foreign regulatory authorities before, and may be unable to do so for rosiptor or any product candidate we are developing.

The conduct of pivotal clinical trials and the submission of a successful marketing application is a complicated process. As an organization, we have not completed a Phase 3 pivotal clinical trial before, have limited experience in preparing, submitting and prosecuting regulatory filings, and have not submitted an NDA to the FDA or any other marketing application to a foreign regulatory authority before. We also have had limited interactions with the FDA and comparable foreign authorities. We may be unable to successfully and efficiently execute and complete necessary clinical trials in a way that leads to marketing application submission and approval of rosiptor or any other product candidate we are developing. We may require more time and incur greater costs than our competitors and may not succeed in obtaining regulatory approvals of products that we develop. Failure to commence or complete, or delays in, our Leadership 301 clinical trial or any other planned clinical trials, would prevent us from, or delay us in, commercializing rosiptor or any other product candidate we are developing.

If we are unable to enroll subjects in clinical trials, we will be unable to complete these trials in a timely fashion.

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 subjects to clinical sites, local standard of care, the number of clinical sites and the rate at which they can be initiated, the eligibility criteria for the trial, the design of the clinical trial, delays or failure in obtaining authorization to commence a trial or inability to comply with conditions imposed by a regulatory authority regarding the scope or design of a clinical trial, ability to obtain and maintain patient consents, risk that enrolled subjects will drop out before completion, 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. Furthermore, we rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials, and while we have agreements governing their committed activities, we have limited influence over their actual performance. For example, in our Phase 1b LPS challenge proof-of-concept trial of rosiptor, a large number of data points were lost for one part of the trial through error, rendering an analysis for efficacy uninterpretable for that part. In our Leadership 301 clinical trial, we have experienced delays in initiating clinical sites.

If we experience delays in the completion or termination of, any clinical trial of rosiptor or any future product candidates, the commercial prospects of our product candidates will be harmed, and our ability to generate revenues from any of these product candidates will be delayed. In addition, any delays in completing our clinical trials will increase our costs, slow down our product candidate development and approval process and could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, and jeopardize our ability to commence product sales, which would impair our ability to generate revenues and may harm our business, results of operations, financial condition and cash flows and future prospects. In addition, many of the factors that could cause a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of rosiptor or our future product candidates.

The regulatory approval processes of the FDA and comparable foreign regulatory authorities are lengthy, time consuming and inherently unpredictable, and if we are ultimately unable to obtain regulatory approval for rosiptor or our future product candidates, our business will be substantially harmed.

The time required to obtain approval by the FDA and comparable foreign regulatory authorities is unpredictable, but typically takes many years following the commencement of preclinical studies and clinical trials and depends upon numerous factors, including the substantial discretion of the regulatory authorities. In addition, approval policies, regulations, or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions. We have not obtained regulatory approval for any product candidate, and it is possible that neither rosiptor nor any future product candidates we may discover, in-license or acquire and seek to develop will ever obtain regulatory approval.

Our product candidates could fail to receive regulatory approval from the FDA or a comparable foreign regulatory authority for many reasons, including:

- disagreement over the design or implementation of our clinical trials or the number of phase 3 clinical trials required;
- failure to demonstrate that a product candidate is safe and effective for its proposed indication;
- failure of clinical trials to meet the level of statistical significance required for approval;

- failure to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;
- disagreement over our interpretation of data from preclinical studies or clinical trials;
- disagreement over whether to accept efficacy results from clinical trial sites outside the United States where the standard of care is potentially different from that in the United States;
- the insufficiency of data collected from clinical trials of rosiptor or our future product candidates to support the submission and filing of an NDA or other submission or to obtain regulatory approval;
- disapproval of the manufacturing processes or facilities of third-party manufacturers with whom we contract for clinical and commercial supplies; or
- changes in the approval policies or regulations that render our preclinical and clinical data insufficient for approval.

The FDA or a comparable foreign regulatory authority may require more information, including additional preclinical or clinical data to support approval, which may delay or prevent approval and our commercialization plans, or we may decide to abandon the development program altogether. Even if we do obtain regulatory approval, rosiptor or our future product candidates may be approved for fewer or more limited indications than we request, approval may be granted but contingent on the performance of costly post-marketing clinical trials, or approval with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate. In addition, if rosiptor, or our future product candidates, produce undesirable side effects or safety issues, the FDA may require the establishment of a Risk Evaluation and Mitigation Strategy, or REMS, or a comparable foreign regulatory authority may require the establishment of a similar strategy, that may restrict distribution of our products and impose burdensome implementation requirements on us. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates.

Approval by the FDA does not ensure approval by comparable foreign regulatory authorities and approval by one or more foreign regulatory authorities does not ensure approval by regulatory authorities in other countries or by the FDA. However, a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory process in others. We may not be able to file for regulatory approvals and even if we file we may not receive the necessary approvals to commercialize our products in any market.

We are conducting, and may in the future conduct, clinical trials for rosiptor or any future product candidates in sites outside the United States and the FDA may not accept data from trials conducted in such locations.

We are conducting, and may in the future choose to conduct, one or more of our clinical trials outside the United States. Although the FDA may accept data from clinical trials conducted outside the United States, acceptance of this data is subject to certain conditions imposed by the FDA. For example, the clinical trial must be well designed and conducted and performed by qualified investigators in accordance with ethical principles. The trial population must also adequately represent the U.S. population, and the data must be applicable to the U.S. population and U.S. medical practice in ways that the FDA deems clinically meaningful. Generally, the patient population for any clinical trials conducted outside of the United States must be representative of the population for whom we intend to label the product in the United States. In addition, while these clinical trials are subject to the applicable local laws, FDA acceptance of the data will be dependent upon its determination that the trials also complied with all applicable U.S. laws and regulations. There can be no assurance the FDA will accept data from trials conducted outside the United States. If the FDA does not accept the data from our clinical trials conducted outside the United States, it would likely result in the need for additional trials, which would be costly and time-consuming and delay or permanently halt our development of rosiptor or any future product candidates.

Rosiptor or our future product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial profile of an approved label, or result in significant negative consequences following any marketing approval.

Undesirable side effects caused by rosiptor or our future product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or other comparable foreign regulatory authority. Rosiptor administered orally has generally been well tolerated by patients in our clinical trials with limited adverse events, the most frequent of which were gastrointestinal disorders. However, in our animal toxicity studies certain side-effects, including severe ulcerations to the gastrointestinal tract of dogs and adverse effects to the ocular lens of some animals occurred. There can be no assurance that these toxicities in animals will not occur in humans. If these toxicities do occur in our future clinical trials they could cause delay or even discontinuance of further development of rosiptor or future product candidates, which would impair our ability to generate revenues and would have a material adverse effect on our business, results of operations, financial condition and cash flows and future prospects.

To date, the most common side-effect of rosiptor noted in clinical trials is mild gastrointestinal upset including mild diarrhea, nausea and gastric pain. There can be no assurance that side-effects from rosiptor in future clinical trials will not prompt the discontinued development of rosiptor or future product candidates. As a result of these side effects or further safety or toxicity issues that we may experience in our clinical trials in the future, we may not receive approval to market rosiptor or any future product candidates, which could prevent us from ever generating revenues or achieving profitability. Results of our trials could reveal an unacceptably high severity and prevalence of side effects. In such an event, our trials could be suspended or terminated and the FDA or comparable foreign regulatory authorities could order us to cease further development of, or deny approval of, our product candidates for any or all targeted indications. The drug-related side effects could affect patient recruitment or the ability of enrolled subjects to complete the trial or result in potential product liability claims. Any of these occurrences may have a material adverse effect on our business, results of operations, financial condition and cash flows and future prospects.

Additionally, if rosiptor or any of our future product candidates receives marketing approval, and we, or others, later identify undesirable side effects caused by such product, a number of potentially significant negative consequences could result, including:

- we may be forced to suspend marketing of such product;
- regulatory authorities may withdraw their approvals of such product;
- regulatory authorities may require additional warnings on the label that could diminish the usage or otherwise limit the commercial success of such products;
- the FDA or other regulatory bodies may issue safety alerts, Dear Healthcare Provider letters, press releases or other communications containing warnings about such product;
- the FDA may require the establishment or modification of a REMS or a comparable foreign regulatory authority may require the establishment or modification of a similar strategy that may, for instance, restrict distribution of our products and impose burdensome implementation requirements on us;
- we may be required to change the way the product is administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused to subjects or patients;
- we may be subject to litigation or product liability claims; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved.

Even if rosiptor or our future product candidates receive regulatory approval, they may still face future development and regulatory difficulties.

Even if we obtain regulatory approval for rosiptor or a future product candidate, it would be subject to ongoing requirements by the FDA and comparable foreign regulatory authorities governing the manufacture, quality control, further development, labeling, packaging, storage, distribution, safety surveillance, import, export, advertising, promotion, recordkeeping and reporting of safety and other post-market information. The safety profile of any product will continue to be closely monitored by the FDA and comparable foreign regulatory authorities after approval. If the FDA or comparable foreign regulatory authorities become aware of new safety information after approval of rosiptor or any future product candidate, they may require labeling changes or establishment of a REMS or similar strategy, impose significant restrictions on a product's indicated uses or marketing, or impose ongoing requirements for potentially costly post-approval studies or post-market surveillance. For example, the label ultimately approved for rosiptor, if it achieves marketing approval, may include restrictions on use.

In addition, manufacturers of drug products and their facilities are subject to continual review and periodic inspections by the FDA and other regulatory authorities for compliance with current good manufacturing practice (cGMP), requirements and other regulations. If we or a regulatory agency discover previously unknown problems with a product, such as 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, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing. If we, our product candidates or the manufacturing facilities for our product candidates, fail to comply with applicable regulatory requirements, a regulatory agency may:

- issue warning letters or untitled letters;
- impose restrictions on the marketing or manufacturing of the product candidates;
- mandate modifications to promotional materials or require us to provide corrective information to healthcare practitioners;
- require us or any future collaborator to enter into a consent decree, which can include imposition of various fines, reimbursements for inspection costs, required due dates for specific remediation actions and penalties for noncompliance;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve pending applications or supplements to applications filed by us;
- suspend or impose restrictions on operations, including costly new manufacturing requirements; or
- seize or detain products, refuse to permit the import or export of products, or require us to initiate a product recall.

The occurrence of any event or penalty described above may inhibit our ability to commercialize rosiptor or any future product candidates and generate revenue.

The FDA strictly regulates the advertising and promotion of drug products, and drug products may only be marketed or promoted for their FDA approved uses, consistent with the product's approved labeling. Advertising and promotion of any product candidate that obtains approval in the United States will be heavily scrutinized by the FDA, the DOJ, the Office of Inspector General of the Department of Health and Human Services, state attorneys general, members of Congress and the public. Violations, including promotion of our products for unapproved or off-label uses, are subject to enforcement letters, inquiries and investigations, and civil, criminal and/or administrative sanctions by the FDA and other enforcement authorities. Additionally, advertising and promotion of any product candidate that obtains approval outside of the United States will be heavily scrutinized by relevant foreign regulatory authorities.

In the United States, engaging in impermissible promotion of our future products for off-label uses can also subject us to false claims litigation under federal and state statutes, which can lead to numerous actions, including civil, criminal and/or administrative penalties and fines and agreements that materially restrict the manner in which we promote or distribute our drug products. These false claims statutes include the federal False Claims Act, which allows the federal government, or any individual relator or whistleblower on behalf of the federal government to bring a lawsuit against a pharmaceutical company alleging submission of false or fraudulent claims, or causing to present such false or fraudulent claims, for payment by a federal program such as Medicare or Medicaid. If the government prevails in the lawsuit, the individual relator may share in any fines or settlement funds. Since 2004, these False Claims Act lawsuits against pharmaceutical

companies have increased significantly in volume and breadth, leading to several substantial civil and criminal settlements based on certain sales practices promoting off-label drug uses. This growth in litigation has increased the risk that a pharmaceutical company will have to defend a false claim action, pay settlement fines or restitution, agree to comply with burdensome reporting and compliance obligations, and be excluded from the Medicare, Medicaid and other federal and state healthcare programs. If we do not lawfully promote our products once they have received regulatory approval, we may become subject to such litigation and, if we are not successful in defending against such actions, those actions could have a material adverse effect our business, results of operations, financial condition and cash flows and future prospects.

Existing government regulations may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of rosiptor or any future product candidates. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and/or be subject to fines or enhanced government oversight and reporting obligations, which would adversely affect our business, prospects and ability to achieve or sustain profitability.

Failure to obtain regulatory approval in international jurisdictions would prevent rosiptor or any future product candidates from being marketed outside the United States.

In order to market and sell our products in the European Union and other jurisdictions, we must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our products in certain countries. We may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. A failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory approval process in others. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our products in any market. If we are unable to obtain approval of rosiptor or any of our future product candidates by regulatory authorities in the European Union or another jurisdiction, the commercial prospects of that product candidate may be significantly diminished and our business prospects could decline.

Recently enacted and future legislation, including potentially unfavorable pricing regulations or other healthcare reform initiatives, may increase the difficulty and cost for us to obtain marketing approval of, and commercialization of, rosiptor or our future product candidates and affect the prices we may obtain.*

The regulations that govern, among other things, marketing approvals, coverage, pricing and reimbursement for new drug products vary from country to country. In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of rosiptor or our future product candidates, restrict or regulate post-approval activities and affect our ability to successfully sell any product candidates for which we obtain marketing approval.

In the United States in recent years, Congress has considered reductions in Medicare reimbursement for drugs administered by physicians. CMS, the agency that administers the Medicare program, also has the authority to revise reimbursement rates and to implement coverage restrictions for drugs. Cost reduction initiatives and changes in coverage implemented through legislation or regulation could decrease utilization of, and reimbursement for, any approved products, which in turn could affect the price we can receive for those products. While Medicare regulations apply only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in establishing their own coverage polices and reimbursement rates. Therefore, any reduction in reimbursement that results from federal legislation or regulation may result in a similar reduction in payments from private payors.

In March 2010, President Obama signed into law the Affordable Care Act in an effort to, among other things, broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on

pharmaceutical and medical device manufacturers and impose additional health policy reforms. The Affordable Care Act, among other things, also expanded manufacturers' rebate liability to include covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations, increased the minimum rebate due for innovator drugs from 15.1% of average manufacturer price, or AMP, to 23.1% of AMP and capped the total rebate amount for innovator drugs at 100% of AMP. The Affordable Care Act and subsequent legislation and regulation also revised the definition of AMP for calculating and reporting Medicaid drug rebates on outpatient prescription drug prices. This could increase the amount of Medicaid drug rebates to states. Furthermore, the Affordable Care Act imposes a significant annual, nondeductible fee on companies that manufacture or import certain branded prescription drug products. Substantial provisions affecting compliance were enacted, which may affect our business practices with healthcare practitioners, and a significant number of provisions are not yet, or have only recently become, effective. On January 20, 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities under Affordable Care Act to waive, defer, grant exemptions from, or delay the implementation of any provision of Affordable Care Act that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. Further, in May 2017, the U.S. House of Representatives passed legislation to repeal and replace major portions of the Affordable Care Act. However, this legislation requires further approval by the U.S. Senate and President, and the prospects for this legislation becoming law, or the terms of any final legislation repealing or replacing elements of the Affordable Care Act, are uncertain. Because of the continued uncertainty about the implementation of Affordable Care Act, including the potential for further legal challenges or repeal of Affordable Care Act, we cannot quantify or predict with any certainty the likely impact of the Affordable Care Act or its repeal on our business, prospects, financial condition or results of operations.

In addition, other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. On August 2, 2011, the President signed into law the Budget Control Act of 2011, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend to Congress proposals in spending reductions. The Joint Select Committee did not achieve a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions to Medicare payments to providers of up to 2% per fiscal year that went into effect on April 1, 2013 and, due to subsequent legislative amendments to the statute, will remain in effect through 2025 unless additional Congressional action is taken. On January 2, 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on our customers and accordingly, our financial operations. Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. Furthermore, there has been heightened governmental scrutiny recently over the manner in which manufacturers set prices for their marketed products. For example, there have been several recent Congressional inquiries and proposed bills designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer's patient programs, and reform government program reimbursement methodologies for drug products. We cannot be sure whether additional legislative changes will be enacted, or whether existing regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of rosiptor or our future product candidates, if any, may be.

In the United States, the European Union and other potentially significant markets for rosiptor and our future product candidates, government authorities and third-party payors are increasingly attempting to limit or regulate the price of medical products and services, particularly for new and innovative products and therapies, which has resulted in lower average selling prices. Furthermore, the increased emphasis on managed healthcare in the United States and on country and regional coverage, pricing and reimbursement controls in the European Union will put additional pressure on product coverage, pricing, reimbursement and utilization, which may adversely affect our business, results of operations, financial condition and cash flows and future prospects. These pressures can arise from various sources, including but not limited to, rules and practices of managed care groups, judicial decisions and governmental laws and regulations related to Medicare, Medicaid and healthcare reform, pharmaceutical reimbursement policies and pricing in general.

Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product candidate in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, which could negatively impact the revenues we are able to generate from the sale of the product in that particular country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates even if our product candidates obtain marketing approval.

Laws and regulations governing international operations may preclude us from developing, manufacturing and selling certain product candidates outside of the United States and Canada and require us to develop and implement costly compliance programs.

As we expand our operations outside of the United States and Canada, we must comply with numerous laws and regulations in each jurisdiction in which we plan to operate. We must also comply with U.S. laws applicable to the foreign operations of U.S. businesses and individuals, such as the Foreign Corrupt Practices Act, or FCPA, and Canadian laws applicable to the foreign operations of Canadian businesses and individuals, such as the Corruption of Foreign Public Officials Act, or CFPOA. The creation and implementation of international business practices compliance programs is costly and such programs are difficult to enforce, particularly where reliance on third parties is required.

The FCPA prohibits any U.S. individual or business from paying, offering, authorizing payment or offering anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring us to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations. The anti-bribery provisions of the FCPA are enforced primarily by the DOJ. The SEC is involved with enforcement of the books and records provisions of the FCPA.

Compliance with the FCPA is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders also restrict the use and dissemination outside the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. Our expanding presence outside the United States will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products and product candidates outside the United States, which could limit our growth potential and increase our development costs.

The failure to comply with laws governing international business practices may result in substantial penalties, including suspension or debarment from government contracting. Violation of the FCPA can result in significant civil and criminal penalties. Indictment alone under the FCPA can lead to suspension of the right to do business with the U.S. government until the pending claims are resolved. Conviction of a violation of the FCPA can result in long-term disqualification as a government contractor. The termination of a government contract or relationship as a result of our failure to satisfy any of our obligations under laws governing international business practices would have a negative impact on our operations and harm our reputation and ability to procure government contracts. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

The CFPOA prohibits Canadian businesses and individuals from giving or offering to give a benefit of any kind to a foreign public official, or any other person for the benefit of the foreign public official, where the ultimate purpose is to obtain or retain a business advantage. Furthermore, a company may be found liable for violations by not only its employees, but also by its third-party agents. Any failure to comply with the CFPOA, as well as applicable laws and regulations in foreign jurisdictions, could result in substantial penalties or restrictions on our ability to conduct business in certain foreign jurisdictions, which may have a material adverse impact on us and our share price.

Even if we are able to commercialize rosiptor or our future product candidates, the products may not receive coverage and adequate reimbursement from third-party payors, which could harm our business.

Our ability to commercialize any products successfully will depend, in part, on the extent to which coverage and adequate reimbursement for these products and related treatments will be available from government authorities, private health insurers, health maintenance organizations and third-party payors. Patients who are prescribed medications for the

treatment of their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their prescription drugs. Coverage and adequate reimbursement from government healthcare programs, such as Medicare and Medicaid, and private health insurers are critical to new product acceptance. Patients are unlikely to use rosiptor, or our future product candidates, unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our product candidates. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. As a result, government authorities and other third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. Third-party payors may also seek additional clinical evidence, beyond the data required to obtain marketing approval, demonstrating clinical benefits and value in specific patient populations before covering our products for those patients. We cannot be sure that coverage and adequate reimbursement will be available for any product that we commercialize and, if reimbursement is available, what that level of reimbursement will be. Coverage and reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. If coverage and reimbursement are not available or are available only at limited levels, we may not be able to successfully commercialize any product candidate for which we obtain marketing approval.

There may be significant delays in obtaining coverage and reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the drug is approved by the FDA or comparable foreign regulatory authorities. Moreover, obtaining coverage does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sales and distribution. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may only be temporary. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used. Reimbursement rates may also be based in part on existing reimbursement amounts for lower cost drugs or may be bundled into the payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. Coverage and reimbursement for drug products can differ significantly from payor to payor. As a result, the coverage and reimbursement determination process is often a time-consuming and costly process with no assurance that coverage and adequate reimbursement will be obtained or applied consistently. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own coverage and reimbursement policies. Our inability to promptly obtain coverage and profitable reimbursement rates from both government-funded and private payors for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

We have never marketed a drug before, and if we are unable to establish an effective sales force and marketing infrastructure, or enter into acceptable third-party sales and marketing or licensing arrangements, we may be unable to generate any revenue.

We do not currently have an infrastructure for the sales, marketing and distribution of pharmaceutical drug products and the cost of establishing and maintaining such an infrastructure may exceed the cost-effectiveness of doing so. In order to market any products that may be approved by the FDA and comparable foreign regulatory authorities, we must build our sales, marketing, managerial and other non-technical capabilities or make arrangements with third parties to perform these services. If we are unable to establish adequate sales, marketing and distribution capabilities, whether independently or with third parties, we may not be able to generate product revenue and may not become profitable. We will be competing with many companies that have extensive and well-funded sales and marketing operations. Without an internal commercial organization or the support of a third party to perform sales and marketing functions, we may be unable to compete successfully against these more established companies.

rosiptor and our future product candidates, if approved, may not achieve adequate market acceptance among physicians, patients, and healthcare payors and others in the medical community necessary for commercial success.

Even if we obtain regulatory approval for rosiptor or any of our product candidates that we may develop or acquire in the future, the product may not gain market acceptance among physicians, healthcare payors, patients or the medical community. In addition, the reported diagnosis rate for IC/BPS is significantly lower than the number of people estimated to suffer from IC/BPS, which could limit our commercial opportunities for that indication. Our commercial success also depends on coverage and adequate reimbursement of our product candidates by third-party payors, including government payors, generally, which may be difficult or time-consuming to obtain, may be limited in scope and may not be obtained in

all jurisdictions in which we may seek to market our products. Market acceptance of any of our product candidates for which we receive approval depends on a number of factors, including:

- the efficacy and safety of such product candidates as demonstrated in clinical trials;
- the clinical indications for which the product candidate is approved;
- acceptance by physicians and patients of the product candidate as a safe and effective treatment;
- the potential and perceived advantages of product candidates over alternative treatments;
- the safety of product candidates seen in a broader patient group, including a product candidate's use outside the approved indications;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA or other regulatory authorities;
- the timing of market introduction of our products as well as competitive products;
- the cost of treatment in relation to alternative treatments;
- the availability of coverage and adequate reimbursement and pricing by third-party payors and government authorities;
- relative convenience and ease of administration;
- the effectiveness of our sales and marketing efforts and those of any future collaborators; and
- unfavorable publicity relating to the product candidate.

If any of our product candidates are approved but fail to achieve market acceptance among physicians, patients, or healthcare payors, we will not be able to generate significant revenues, which would compromise our ability to become profitable.

Our relationships with healthcare professionals, principal investigators, consultants, customers (actual and potential) and third-party payors are and will be subject, directly and indirectly, to applicable anti-kickback, fraud and abuse, privacy, transparency and other healthcare laws and regulations, which could expose us to penalties, including without limitation, civil, criminal and administrative sanctions, civil penalties, damages, fines, disgorgement, individual imprisonment, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, integrity obligations, contractual damages, reputational harm, administrative burdens and diminished profits and future earnings and the curtailment or restructuring of our operations.

As a pharmaceutical company, even though we do not and will not control referrals of healthcare services or bill directly to Medicare, Medicaid or other third-party payors, federal and state healthcare laws and regulations pertaining to fraud and abuse and patients' rights are and will be applicable to our future arrangements with third-party payors and customers who are in a position to purchase, recommend and/or prescribe our product candidates for which we obtain marketing approval. These broadly applicable fraud and abuse and other healthcare laws and regulations may constrain our future business or financial arrangements and relationships with healthcare professionals, principal investigators, consultants, customers, and third-party payors and other entities, including our marketing practices, educational programs and pricing policies. Restrictions under applicable federal and state healthcare laws and regulations that may affect our ability to operate include, but are not limited to, the following:

- the federal Anti-Kickback Statute, which, among other things, prohibits persons from knowingly and willfully soliciting, offering, receiving or providing paying any remuneration (including any kickback, bribe, or rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, lease, order or recommendation of, any good, facility, item or service, for which payment may be made, in whole or in part, under a federal healthcare program such as Medicare and Medicaid;
- federal civil and criminal false claims laws, including the federal civil False Claims Act, and civil monetary penalty laws impose criminal and civil penalties, including through civil whistleblower or qui tam actions, among other things, prohibits individuals or entities from knowingly presenting, or causing to be presented, to the federal government, including the Medicare and Medicaid programs, claims for payment or approval that are false or fraudulent or from knowingly making a false statement to improperly avoid, decrease or conceal an obligation to pay money to the federal government;
- HIPAA, which, among other things, imposes criminal liability for knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or to obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the

custody or control of, any healthcare benefit program, regardless of the payor (e.g. public or private) and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of or payment for healthcare benefits, items or services relating to healthcare matters;

- HIPAA, as amended by HITECH, and its implementing regulations, which also imposes certain obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information without the appropriate authorization by entities subject to the law, such as health plans, healthcare clearinghouses and healthcare providers;
- the federal Physician Payments Sunshine Act, created under Section 6002 of the Affordable Care Act and its implementing regulations, which requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to CMS, information related to "payments or other transfers of value" made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, and applicable manufacturers and applicable group purchasing organizations to report annually to CMS ownership and investment interests held by physicians (as defined above) and their immediate family members and payments or other "transfers of value" to such physician owners and their immediate family members; and
- analogous local, state and foreign laws and regulations, including: state anti-kickback and false claims laws which may apply to our business practices, including, but not limited to, research, distribution, sales and marketing arrangements and claims involving healthcare items or services reimbursed by state governmental and non-governmental third-party payors, including private insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the applicable compliance guidance promulgated by the federal government; local, state and foreign laws that require drug manufacturers to track gifts and other remuneration and items of value provided to healthcare professionals and entities and file reports relating to pricing and marketing information and/or register their pharmaceutical sales representatives; and local, state and foreign laws that govern the privacy and security of health information in specified circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Efforts to ensure that our internal operations and business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. Recent healthcare reform legislation has also strengthened these laws. For example, the Affordable Care Act, among other things, amends the intent requirement of the federal Anti-Kickback Statute, such that a person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation. In addition, the Affordable Care Act codified case law that a claim that includes items or services resulting from a violation of the federal anti-kickback statute constitutes a false or fraudulent claim for purposes of the federal civil False Claims Act. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations, agency guidance, or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental laws and regulations that may apply to us, we may be subject to penalties, including without limitation, significant civil, criminal and administrative penalties, damages, fines, disgorgement, individual imprisonment, exclusion from government funded healthcare programs, such as Medicare and Medicaid, integrity obligations, contractual damages, reputational harm, administrative burdens and diminished profits and future earnings, and the curtailment or restructuring of our operations. If any physicians or other healthcare providers or entities with whom we expect to do business are found to not be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs. Moreover, we expect there will continue to be federal, state, local and foreign laws and regulations, proposed and implemented, that could impact our operations and business. The extent to which future legislation or regulations, if any, relating to healthcare fraud abuse laws or enforcement, may be enacted or what effect such legislation or regulation would have on our business remains uncertain.

We may form strategic alliances in the future, and we may not realize the benefits of such alliances.

We may form strategic alliances, create joint ventures or collaborations or enter into licensing arrangements with third parties that we believe will complement or augment our existing business. These relationships or those like them may require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that dilute our stockholders or disrupt our management and business. In addition, we face significant competition in seeking appropriate strategic partners and the negotiation process is time-consuming and complex. Moreover, we may not be

successful in our efforts to establish a strategic partnership or other alternative arrangements for rosiptor or any future drug candidates and programs because our research and development pipeline may be insufficient, our drug candidates and programs may be deemed to be at too early a stage of development for collaborative effort and third parties may not view our drug candidates and programs as having the requisite potential to demonstrate safety and efficacy. If we license products or businesses, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture. We cannot be certain that, following a strategic transaction or license, we will achieve the revenues or specific net income that justifies such transaction. Any delays in entering into new strategic partnership agreements related to our drug candidates could also delay the development and commercialization of our drug candidates and reduce their competitiveness even if they reach the market.

Our employees, independent contractors, principal investigators, CROs, consultants, vendors and collaboration partners may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could cause significant liability for us and harm our reputation.

We are exposed to the risk of fraud or other misconduct by our employees, independent contractors, principal investigators, CROs, consultants, vendors and collaboration partners, including intentional failures to comply with FDA regulations or similar regulations of comparable foreign regulatory authorities, provide accurate information to the FDA or comparable foreign regulatory authorities, comply with manufacturing standards we have established, comply with federal and state data privacy and security, fraud and abuse and other healthcare laws and regulations and similar laws and regulations established and enforced by comparable foreign regulatory authorities, report financial information or data accurately or disclose unauthorized activities to us. Specifically, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, 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 commission, customer incentive programs and other business arrangements. Employee misconduct could also involve the improper use or misrepresentation of information obtained in the course of clinical trials or creating fraudulent data in our pre-clinical studies or clinical trials, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of conduct for our directors, officers and employees, but it is not always possible to identify and deter misconduct by employees and other third parties, 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. Additionally, we are subject to the risk that a person or government could allege such fraud or other misconduct, even if none occurred. 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 significant impact on our business, results of operations, financial condition and cash flows from future prospects, including the imposition of significant fines or other sanctions.

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

The development and commercialization of new drug products is highly competitive. We face competition with respect to our current product candidate, rosiptor, and will face competition with respect to any future product candidates that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. There are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of the disease indications for which we are developing or may develop rosiptor or our future product candidates for. Some of these competitive products and therapies may be based on scientific approaches that are the same as or similar to our approach, and others are based on entirely different approaches. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

More established companies may have a competitive advantage over us due to their greater size, cash flows and institutional experience. Compared to us, many of our competitors may have significantly greater financial, technical and human resources.

As a result of these factors, our competitors may obtain regulatory approval of their products before we do, which will limit our ability to develop or commercialize rosiptor or any of our future product candidates. Although there are no approved therapies that specifically target SHIP1, there are currently approved therapies for treating the same diseases or indications for which our product candidates may be useful. In addition, many companies are developing new therapeutics, and we cannot predict what the standard of care will be as our product candidates progress through clinical development.

If rosiptor were approved for the treatment of IC/BPS, it could face competition from currently approved and marketed products, including Janssen Pharmaceuticals Inc.'s pentosan polysulfate sodium, marketed in the United States as Elmiron, which is off patent. Also, we believe that Gilead Sciences, Inc., Amgen Inc., and TG Therapeutics, Inc. are developing drugs that target the delta and/or gamma isoforms of PI3K. In addition, many companies are developing product candidates directed to IC/BPS, or for other diseases which we may develop rosiptor or other SHIP1 activators for in the future. Such companies include Pfizer, AbbVie, Urigen, Astellas, Allergan, and Merck.

We believe that our ability to successfully compete will depend on, among other things:

- the efficacy and safety profile of rosiptor, including relative to marketed products and product candidates in development by third parties;
- the time it takes for rosiptor or any of our future product candidates to complete clinical development and receive marketing approval;
- the ability to commercialize rosiptor and future product candidates that receive regulatory approval;
- whether coverage and adequate levels of reimbursement are available under private and governmental health insurance plans, including Medicare;
- the ability to establish, maintain and protect intellectual property rights related to our product candidates;
- the ability to manufacture commercial quantities of rosiptor and future product candidates that receive regulatory approval; and
- acceptance of rosiptor and future product candidates that receive regulatory approval by physicians and other healthcare providers.

Our failure to successfully identify, acquire, develop and commercialize additional product candidates or approved products other than rosiptor could impair our ability to grow.

Although a substantial amount of our efforts will focus on the continued clinical testing and potential approval of rosiptor, a key element of our growth strategy is to acquire, develop and/or market additional products and product candidates. All of our other potential product candidates remain in the discovery and preclinical study stages. Research programs to identify product candidates require substantial technical, financial and human resources, whether or not any product candidates are ultimately identified. Because our internal research capabilities are limited, we may be dependent upon pharmaceutical and biotechnology companies, academic scientists and other researchers to sell or license products or technology to us. The success of this strategy depends partly upon our ability to identify, select and acquire promising pharmaceutical product candidates and products. The process of proposing, negotiating and implementing a license or acquisition of a product candidate or approved product is lengthy and complex. Other companies, including some with substantially greater financial, marketing and sales resources, may compete with us for the license or acquisition of product candidates and approved products. We have limited resources to identify and execute the acquisition or in-licensing of third-party products, businesses and technologies and integrate them into our current infrastructure. Moreover, we may devote resources to potential acquisitions or in-licensing opportunities that are never completed, or we may fail to realize the anticipated benefits of such efforts. Any product candidate that we acquire may require additional development efforts prior to commercial sale, including extensive clinical testing and approval by the FDA and applicable foreign regulatory authorities. All product candidates are prone to risks of failure typical of pharmaceutical product development, including the possibility that a product candidate will not be shown to be sufficiently safe and effective for approval by regulatory authorities. In addition, we cannot provide assurance that any products that we develop or approved products that we acquire will be manufactured profitably or achieve market acceptance.

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

We face an inherent risk of product liability exposure related to the testing of rosiptor and any future product candidates in human clinical trials and will face an even greater risk if we commercially sell any products that we may develop. Product liability claims may be brought against us by subjects enrolled in our clinical trials, patients, healthcare providers or others using, administering or selling our products. If we cannot successfully defend ourselves against claims that our product

candidates or products caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or products that we may develop;
- termination of clinical trial sites or entire trial programs;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards to trial subjects or patients;
- loss of revenue;
- diversion of management and scientific resources from our business operations; and
- the inability to commercialize our product candidates.

We currently have product liability insurance coverage, which is limited to $5 million per occurrence and $10 million in the aggregate. This coverage may not be adequate to cover all liabilities that we may incur. Insurance coverage is becoming increasingly expensive and in the future we may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise. We intend to expand our insurance coverage for products to include the sale of commercial products if we obtain marketing approval for rosiptor or our future product candidates, but we may be unable to obtain commercially reasonable product liability insurance for any products approved for marketing. Large judgments have been awarded in class action lawsuits based on drugs that had unanticipated side effects. A successful product liability claim or series of claims brought against us, particularly if judgments exceed our insurance coverage, could decrease our cash and adversely affect our business.

We will need to expand our operations and grow the size of our organization, and we may experience difficulties in managing this growth.*

As of June 30, 2017, we had 48 employees. As our development and commercialization plans and strategies develop, or as a result of any future acquisitions, we will need additional managerial, operational, sales, marketing, scientific, and financial headcount and other resources. Our management, personnel and systems currently in place may not be adequate to support this future growth. Future growth would impose significant added responsibilities on members of management, including:

- managing our clinical trials effectively, which we anticipate being conducted at numerous clinical sites;
- identifying, recruiting, maintaining, motivating and integrating additional employees with the expertise and experience we will require;
- managing our internal development efforts effectively while complying with our contractual obligations to licensors, licensees, contractors and other third parties;
- manage additional relationships with various strategic partners, suppliers and other third parties;
- improving our managerial, development, operational and finance reporting systems and procedures; and
- expanding our facilities.

Our failure to accomplish any of these tasks could prevent us from successfully growing our company.

Our future success depends on our ability to attract, retain and motivate qualified personnel.

We may not be able to attract or retain qualified managerial, operational, sales, marketing, scientific and financial personnel in the future due to the intense competition for qualified personnel among biotechnology, pharmaceutical and other businesses. If we are not able to attract and retain necessary personnel to accomplish our business objectives, we may experience constraints that will significantly impede the achievement of our development objectives, our ability to raise additional capital and our ability to implement our business strategy.

We are highly dependent on the development, regulatory, commercialization and business development expertise of our executive officers. If we lose one or more of our executive officers or key employees, our ability to implement our business strategy successfully could be seriously harmed. Replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to develop, gain regulatory approval of and commercialize products successfully. Competition to hire

from this limited pool is intense, and we may be unable to hire, train, retain or motivate these additional key personnel. Many of the other pharmaceutical companies that we compete with for qualified personnel have greater financial and other resources, different risk profiles and a longer history in the industry than we do. They also may provide more diverse opportunities and better chances for career advancement. Some of these characteristics may be more appealing to high-quality candidates than what we have to offer. Further, we do not maintain "key person" insurance for any of our executives or other employees. Our failure to retain key personnel could impede the achievement of our research, development and commercialization objectives.

We may engage in future acquisitions that could disrupt our business, cause dilution to our stockholders and harm our business, results of operations, financial condition and cash flows and future prospects.

While we currently have no specific plans to acquire any other businesses, we may, in the future, make acquisitions of, or investments in, companies that we believe have products or capabilities that are a strategic or commercial fit with rosiptor or our future product candidates and business or otherwise offer opportunities for our company. In connection with these acquisitions or investments, we may:

- issue stock that would dilute our stockholders' percentage of ownership;
- incur debt and assume liabilities; and
- incur amortization expenses related to intangible assets or incur large and immediate write-offs.

We may not be able to complete acquisitions on favorable terms, if at all. If we do complete an acquisition, we cannot assure you that it will ultimately strengthen our competitive position or that it will be viewed positively by customers, financial markets or investors. Furthermore, future acquisitions could pose numerous additional risks to our operations, including:

- problems integrating the purchased business, products or technologies;
- increases to our expenses;
- the failure to discover undisclosed liabilities of the acquired asset or company;
- diversion of management's attention from their day-to-day responsibilities;
- harm to our operating results or financial condition;
- entrance into markets in which we have limited or no prior experience; and
- potential loss of key employees, particularly those of the acquired entity.

We may not be able to complete any acquisitions or effectively integrate the operations, products or personnel gained through any such acquisition.

Our business and operations would suffer in the event of computer system failures or security breaches.

In the ordinary course of our business, we collect, store and transmit confidential information, including intellectual property, proprietary business information and personal information. Despite the implementation of security measures, our internal computer systems, and those of our CROs and other third parties on which we rely, are vulnerable to damage from computer viruses, unauthorized access, cyberattacks, natural disasters, fire, terrorism, war and telecommunication and electrical failures. Cyberattacks are increasing in their frequency, sophistication and intensity. Cyberattacks could include the deployment of harmful malware, denial-of-service attacks, social engineering and other means to affect service reliability and threaten the confidentiality, integrity and availability of information. Significant disruptions of our information technology systems or security breaches could adversely affect our business operations and/or result in the loss, misappropriation, and/or unauthorized access, use or disclosure of, or the prevention of access to, confidential information (including trade secrets or other intellectual property, proprietary business information and personal information), and could result in financial, legal, business and reputational harm to us. If such disruptions were to occur and cause interruptions in our operations, it could result in a material disruption of our drug development programs. For example, the loss of clinical trial data from completed, ongoing or planned clinical trials 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 results in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development of rosiptor or our future product candidates could be delayed.

Business disruptions could seriously harm our future revenues and financial condition and increase our costs and expenses.

Our operations could be subject to earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics and other natural or manmade disasters or business interruptions, for which we are predominantly self-insured. We do not carry insurance for all categories of risk that our business may encounter. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses. We rely on third-party manufacturers to produce our product candidates. Our ability to obtain clinical supplies of product candidates could be disrupted, if the operations of these suppliers are affected by a man-made or natural disaster or other business interruption. The ultimate impact on us, our significant suppliers and our general infrastructure of being consolidated in certain geographical areas is unknown, but our operations and financial condition could suffer in the event of a major earthquake, fire or other natural disaster. Further, any significant uninsured liability may require us to pay substantial amounts, which would adversely affect our business, results of operations, financial condition and cash flows from future prospects.

Risks Related to Our Dependence on Third Parties

We rely on third parties to conduct our preclinical studies and clinical trials. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, this could substantially harm our business because we may not be able to obtain regulatory approval for or commercialize rosiptor or our future product candidates in a timely manner or at all.

We have extensively relied upon, and plan to continue to extensively rely upon, third-party CROs and other consultants to monitor and manage data for our ongoing preclinical and clinical programs. We rely on these parties for execution of our preclinical studies and clinical trials, and we control only some aspects of their activities. Nevertheless, we are responsible for ensuring that each of our studies and clinical trials is conducted in accordance with the applicable protocol and legal, regulatory and scientific standards, and our reliance on the CROs does not relieve us of our regulatory responsibilities. We also rely on third parties to assist in conducting our preclinical studies in accordance with Good Laboratory Practices, or GLP, and the Animal Welfare Act requirements. We and our CROs are required to comply with federal regulations and current Good Clinical Practices, or GCP, which are international standards meant to protect the rights and health of patients that are enforced by the FDA, the Competent Authorities of the Member States of the European Economic Area, or EEA, and comparable foreign regulatory authorities for all of our products in clinical development. Regulatory authorities enforce GCP through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of our CROs or consultants fail to comply with applicable GCP, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional 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 requirements. In addition, our clinical trials must be conducted with product produced under cGMP requirements. Failure to comply with these regulations may require us to repeat preclinical studies and clinical trials, which would delay the regulatory approval process.

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 clinical, nonclinical and preclinical programs. If CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines or if the quality or accuracy of the clinical data is compromised due to the failure to adhere to our clinical 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 rosiptor or our future product candidates. As a result, our results of operations and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenues could be delayed.

Because we have relied on third parties, our internal capacity to perform these functions is limited. Outsourcing these functions involves risk that third parties may not perform to our standards, may not produce results in a timely manner or may fail to perform at all. In addition, the use of third-party service providers requires us to disclose our proprietary information to these parties, which could increase the risk that this information will be misappropriated. We currently have a small number of employees, which limits the internal resources we have available to identify and monitor our third-party providers. To the extent we are unable to identify and successfully manage the performance of third-party service providers in the future, our business may be adversely affected. Though we carefully manage our relationships with our CROs and consultants, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, results of operations, financial condition and cash flows and future prospects.

If our relationships with CROs terminate, our drug development efforts could be delayed.

We rely on third-party vendors and CROs for preclinical studies and clinical trials related to our drug development efforts. Switching or adding additional CROs would involve additional cost and requires management time and focus. Our CROs have the right to terminate their agreements with us in the event of an uncured material breach. In addition, some of our CROs have an ability to terminate their respective agreements with us if it can be reasonably demonstrated that the safety of the subjects participating in our clinical trials warrants such termination, if we make a general assignment for the benefit of our creditors or if we are liquidated. If any of our relationships with our third-party CROs terminate, we could experience a significant delay in identifying, qualifying and managing performance of a comparable third-party service provider, which could adversely affect our development programs. In addition, there is a natural transition period when a new CRO commences work and the new CRO may not provide the same type or level of services as the original provider. We may not be able to enter into arrangements with alternative CROs or do so on commercially reasonable terms.

We have no experience manufacturing our product candidates on a large clinical or commercial scale and have no manufacturing facility. As a result, we are dependent on third-party manufacturers for the manufacture of rosiptor, as well as on third parties for our supply chain, and if we experience problems with any third parties, the manufacturing of rosiptor or our future product candidates or products could be delayed.

We do not own or operate facilities for the manufacture of our product candidates. We currently have no plans to build our own manufacturing facilities for clinical or commercial operations. We currently rely on a single source contract manufacturing organization, or CMO, for the chemical manufacture of active pharmaceutical ingredient for rosiptor, and another CMO for the production of rosiptor final product formulation and packaging for clinical trials. To meet our projected needs for clinical supplies to support our activities through regulatory approval and commercial manufacturing, the CMOs with whom we currently work will need to increase the scale of production. We may need to identify additional CMOs for continued production of supply for our product candidates. We have not yet identified alternate suppliers in the event the current CMOs we utilize are unable to scale production, or if we otherwise experience any problems with them. Although alternative third-party suppliers with the necessary manufacturing and regulatory expertise and facilities exist, it could be expensive and take a significant amount of time to arrange for alternative suppliers. We may encounter technical difficulties or delays in the transfer of rosiptor manufacturing on a commercial scale to additional third-party manufacturers. We may be unable to enter into agreements for commercial supply with third-party manufacturers, or may be unable to do so on acceptable terms. If we are unable to arrange for alternative third-party manufacturing sources, or to do so on commercially reasonable terms or in a timely manner, we may not be able to complete development of our product candidates, or market or distribute them.

Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured product candidates or products ourselves, including reliance on the third party for regulatory compliance and quality assurance, the possibility of breach of the manufacturing agreement by the third party because of factors beyond our control, including a failure to synthesize and manufacture our product candidates or any products we may eventually commercialize in accordance with our specifications, and the possibility of termination or nonrenewal of the agreement by the third party, based on its own business priorities, at a time that is costly or damaging to us. In addition, the FDA and other regulatory authorities require that our product candidates and any products that we may eventually commercialize be manufactured according to cGMP and similar foreign standards. Any failure by our third-party manufacturers to comply with cGMP or failure to scale up manufacturing processes, including any failure to deliver sufficient quantities of product candidates in a timely manner, could lead to a delay in, or failure to obtain, regulatory approval of any of our product candidates and could cause us to incur higher costs and prevent us from commercializing our product candidates successfully. In addition, such failure could be the basis for the FDA to issue a warning letter, withdraw approvals for product candidates previously granted to us, or take other regulatory or legal action, including recall or seizure of outside supplies of the product candidate, total or partial suspension of production, suspension of ongoing clinical trials, refusal to approve pending applications or supplemental applications, detention of products, refusal to permit the import or export of products, injunction, or imposing civil and criminal penalties.

Any significant disruption in our supplier relationships could harm our business. Any significant delay in the supply of a product candidate or its key materials for an ongoing clinical trial could considerably delay completion of our clinical trials, product testing and potential regulatory approval of our product candidates. If our manufacturers or we are unable to

purchase these key materials after regulatory approval has been obtained for our product candidates, the commercial launch of our product candidates would be delayed or there would be a shortage in supply, which would impair our ability to generate revenues from the sale of our product candidates. It may take several years to establish an alternative source of supply for our product candidates and to have any such new source approved by the FDA.

Risks Related to Intellectual Property

If we are unable to obtain or protect intellectual property rights, our competitive position could be harmed.

We depend on our ability to protect our proprietary 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 commercial success will depend in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary technology and products. Where we have the right to do so under our license agreements, we seek to protect our proprietary position by filing patent applications in the United States and abroad related to our novel technologies and products that are important to our business. The patent positions of biotechnology and pharmaceutical companies generally are highly uncertain, involve complex legal and factual questions and have in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patents, including those patent rights licensed to us by third parties, are highly uncertain.

The steps we have taken to protect our proprietary rights may not be adequate to preclude misappropriation of our proprietary information or infringement of our intellectual property rights, both inside and outside the United States. The rights already granted under any of our currently issued patents and those that may be granted under future issued patents may not provide us with the proprietary protection or competitive advantages we are seeking. If we are unable to obtain and maintain patent protection for our technology and products, or if the scope of the patent protection obtained is not sufficient, our competitors could develop and commercialize technology and products similar or superior to ours, and our ability to successfully commercialize our technology and products may be adversely affected.

With respect to patent rights, we do not know whether any of the pending patent applications for any of our discovered or licensed compounds will result in the issuance of patents that protect our technology or products, or if any of our issued patents will effectively prevent others from commercializing competitive technologies and products. Our pending applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Further, the examination process may require us or our future potential licensor(s) to narrow the claims for our pending patent applications, which may limit the scope of patent protection that may be obtained if these applications issue. Because the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, issued patents that we own or have licensed from third parties may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in the loss of patent protection, the narrowing of claims in such patents or the invalidity or unenforceability of such patents, 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 for our technology and products. Protecting against the unauthorized use of our patented technology, trademarks and other intellectual property rights is expensive, difficult and may in some cases not be possible. In some cases, it may be difficult or impossible to detect third-party infringement or misappropriation of our intellectual property rights, even in relation to issued patent claims, and proving any such infringement may be even more difficult.

We could be required to incur significant expenses to strengthen our intellectual property rights, and our intellectual property rights may be inadequate to protect our competitive position.

The patent prosecution process is expensive and time-consuming, and we or our future potential licensors may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we or our future potential licensors will fail to identify patentable aspects of inventions made in the course of our development and commercialization activities before it is too late to obtain patent protection on them. Further, given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours. We expect to seek extensions of patent terms in the United States and, if available, in other countries where we are prosecuting patents. In the United States, the Drug Price Competition and Patent Term Restoration Act of 1984 permits a patent term extension of up to five years beyond the expiration of the patent. However, the applicable authorities, including the FDA in the United States, and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to our patents, or

may grant more limited extensions than we request. If this occurs, our competitors may be able to take advantage of our investment in development and clinical trials by referencing our clinical and preclinical data and launch their product earlier than might otherwise be the case. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection. The laws of foreign countries may not protect our rights to the same extent as the laws of the United States, and these foreign laws may also be subject to change. For example, methods of treatment and manufacturing processes may not be patentable in certain jurisdictions. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or in some cases not at all. Therefore we cannot be certain that we or our licensors were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we or our licensors were the first to file for patent protection of such inventions.

Our patent applications and the enforcement or defense of our issued patents may be impacted by the application of or changes in U.S. and foreign standards.*

The standards that the U.S. Patent and Trademark Office and foreign patent offices use to grant patents are not always applied predictably or uniformly and can change. Consequently, our pending patent applications may not be allowed and, if allowed, may not contain the type and extent of patent claims that will be adequate to conduct our business as planned. Additionally, any issued patents we currently own or obtain in the future may have a shorter patent term than expected or may not contain claims that will permit us to stop competitors from using our technology or similar technology or from copying our product candidates. Similarly, the standards that courts use to interpret patents are not always applied predictably or uniformly and may evolve, particularly as new technologies develop. In addition, changes to patent laws in the United States or other countries may be applied retroactively to affect the validation enforceability, or term of our patent. For example, the U.S. Supreme Court has recently modified some legal standards applied by the U.S. Patent and Trademark Office in examination of U.S. patent applications, which may decrease the likelihood that we will be able to obtain patents and may increase the likelihood of challenges to patents we obtain or license. In addition, changes to the U.S. patent system have come into force under the Leahy-Smith America Invents Act, or the Leahy-Smith Act, which was signed into law in September 2011. The Leahy-Smith Act included a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. In particular, under the Leahy-Smith Act, the United States transitioned in March 2013 to a “first to file” system in which the first inventor to file a patent application will be entitled to the patent. Third parties are allowed to submit prior art before the issuance of a patent by the U.S. Patent and Trademark Office, or the USPTO, and may become involved in opposition, derivation, reexamination, inter-partes review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, which could adversely affect our competitive position.

While we cannot predict with certainty the impact the Leahy-Smith Act or any potential future changes to the U.S. or foreign patent systems will have on the operation of our business, the Leahy-Smith Act and such future changes could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business, results of operations, financial condition and cash flows and future prospects.

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

Periodic maintenance and annuity fees on any issued patent are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent. The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, 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. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we or our future potential licensors fail to maintain the patents and patent applications covering rosiptor or our future product candidates, our competitive position would be adversely affected.

We may be subject to claims by third parties claiming ownership of what we regard as our own intellectual property.

Many of our employees, including our senior management, were previously employed at universities or at other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Some of these employees, including members of our senior management, executed proprietary rights, non-disclosure and non-competition agreements, in connection with such previous employment. Although we try to ensure that our employees do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we, or these employees, have used or disclosed confidential information or intellectual property, including trade secrets or other proprietary information, of any such employee's former employer. We are not aware of any threatened or pending claims related to these matters or concerning the agreements with our senior management, but in the future litigation may be necessary to defend against such claims. In addition, third parties may from time to time make claims over what we regard as our intellectual property, or we may get into disputes with licensors or licensees of our intellectual property rights over the interpretation of the license terms. Our licensors may have the right to terminate their license agreements with us or pursue damages or other legal remedies. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel or sustain damages. Such intellectual property rights could be awarded to a third party, and we could be required to obtain a license from such third party to commercialize our technology or products. Such a license may not be available on commercially reasonable terms or at all. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

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

In addition to seeking patents for some of our technology and products, we also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. We seek to protect these trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, CMOs, consultants, advisors and other third parties. We also generally enter into confidentiality and invention or patent assignment agreements with our employees and consultants. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts both within and outside the United States may be less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such competitor from using that technology or information to compete with us, which could harm our competitive position.

Although we expect all of our employees to assign their inventions to us, and all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information or technology to enter into confidentiality agreements, we cannot provide any assurances that all such agreements have been duly executed or that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret. In addition, others may independently discover our trade secrets and proprietary information. For example, the FDA, as part of its Transparency Initiative, is currently considering whether to make additional information publicly available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA's disclosure policies may change in the future, if at all.

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

Filing, prosecuting and defending patents on rosiptor and our future product candidates throughout the world would be prohibitively expensive. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we and our licensors or future collaborators may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products, and may export otherwise infringing products to territories where we have patent protection, but where enforcement is not as strong

as that in the United States. These products may compete with our products in jurisdictions where we do not have any issued patents and our 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 patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial cost and divert our efforts and attention from other aspects of our business. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful.

The requirements for patentability may differ in certain countries, particularly developing countries. For example, unlike other countries, China has a heightened requirement for patentability, and specifically requires a detailed description of medical uses of a claimed drug. In India, unlike the United States, there is no link between regulatory approval of a drug and its patent status. Furthermore, generic or biosimilar drug manufacturers or other competitors may challenge the scope, validity or enforceability of our or our licensors' or collaborators' patents, requiring us or our licensors or collaborators to engage in complex, lengthy and costly litigation or other proceedings. Generic or biosimilar drug manufacturers may develop, seek approval for, and launch biosimilar versions of our products. In addition to India, certain countries in Europe and developing countries, including China, have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In those countries, we and our licensors or collaborators may have limited remedies if patents are infringed or if we or our licensors or collaborators are compelled to grant a license to a third party, which could materially diminish the value of those patents. This could limit our potential revenue opportunities. Accordingly, our and our licensors' or collaborators' 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.

Intellectual property rights do not necessarily address all potential threats to any competitive advantage we may have.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business, or permit us to maintain our competitive advantage. The following examples are illustrative:

- Others may be able to make compounds that are the same as or similar to rosiptor or our future product candidates but that are not covered by the claims of the patents that we own or have exclusively licensed.
- We or any of our licensors or strategic partners might not have been the first to make the inventions covered by the issued patent or pending patent application that we own or have exclusively licensed.
- We or any of our licensors or strategic partners might not have been the first to file patent applications covering certain of our inventions.
- Others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights.
- It is possible that our pending patent applications will not lead to issued patents.
- Issued patents that we own or have exclusively licensed may not provide us with any competitive advantages, or may be held invalid or unenforceable, as a result of legal challenges by our competitors.
- Our competitors might conduct research and development activities in the United States and other countries that provide a safe harbor from patent infringement claims for certain research and development activities, as well as in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets.
- We may not develop additional proprietary technologies that are patentable.
- The patents of others may have an adverse effect on our business.

Risks Related to Ownership of Our Common Stock

Our stock price has been and will likely continue to be volatile and may decline regardless of our operating performance, resulting in substantial losses for investors.

The trading price of our common stock has been, and is likely to continue to be, volatile for the foreseeable future. For example, in the year ended December 31, 2016, our common stock's sales price on The NASDAQ Global Market ranged

from a low of $6.01 to a high of $18.35. The trading price of our common stock could be subject to wide fluctuations in response to various factors, some of which are beyond our control. In addition to the factors discussed in this "Risk Factors" section and elsewhere in this report, these factors include:

- the success of competitive products or technologies;
- regulatory actions with respect to our products or our competitors' products;
- actual or anticipated changes in our growth rate relative to our competitors;
- announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures, collaborations or capital commitments;
- results of clinical trials, including both safety and efficacy, of rosiptor or any of our future product candidates or those of our competitors;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to rosiptor or any of our future product candidates or clinical development programs;
- the results of our efforts to in-license or acquire additional product candidates or products;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- fluctuations in the valuation of companies perceived by investors to be comparable to us;
- share price and volume fluctuations attributable to inconsistent trading volume levels of our shares;
- announcement or expectation of additional financing efforts;
- sales of our common stock by us, our insiders or our other stockholders;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors; and
- general economic, industry and market conditions.

In addition, the stock market in general, and pharmaceutical and biotechnology 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. The realization of any of these risks or any of a broad range of other risks, including those described in this "Risk Factors" section and elsewhere in this report, could have a dramatic and material adverse impact on the market price of our common stock.

We may be subject to securities litigation, which is expensive and could divert management attention.

The trading price of our common stock has been and will continue to be volatile. For example, on July 8, 2015, the closing price of our common stock on The NASDAQ Global Market was $6.55, and on July 9, 2015, following our announcement of negative results from our Phase 2 Flagship clinical trial in COPD, the closing price was $2.13. Similarly, on August 6, 2015, the closing price of our common stock on The NASDAQ Global Market was $1.79 and on August 7, 2015, following our announcement of positive results from secondary endpoints in our Phase 2 Leadership 201 clinical trial in IC/BPS, the closing price was $10.42 and increased to $22.13 on August 14, 2015. In the past, companies that have experienced volatility in the market price of their stock have been subject to securities class action litigation. We may be the target of this type of litigation in the future. Securities litigation against us could result in substantial costs and divert our management's attention from other business concerns, which could seriously harm our business.

Our principal stockholders, directors and management own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.*

Our executive officers, directors, holders of 5% or more of our capital stock and their respective affiliates together beneficially own a majority of our outstanding voting stock. In particular, based on information available to us, entities affiliated with Baker Bros. Advisors LP, or the Baker Entities, which together are our largest stockholders, collectively beneficially owned approximately 46.7% of our common stock as of March 1, 2017. These stockholders are able to determine the outcome of all matters requiring stockholder approval. For example, these stockholders are able to control

elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may 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. The interests of this group of stockholders may not always coincide with your interests or the interests of other 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.

We are an "emerging growth company" as that term is used in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and we have taken advantage of reduced disclosure and governance requirements applicable to emerging growth companies, which could result in our common stock being less attractive to investors and adversely affect the market price of our common stock or make it more difficult to raise capital as and when we need it.

We are an "emerging growth company" as that term is used in the JOBS Act, and we are taking advantage of certain exemptions from various reporting 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, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved and exemptions from any rules that the Public Company Accounting Oversight Board may adopt requiring mandatory audit firm rotation or a supplement to the auditor's report on the financial statements. We have taken and currently intend to take advantage of some, but not all, of the reduced regulatory and reporting requirements that are available to us under the JOBS Act, so long as we qualify as an "emerging growth company." For example, so long as we qualify as an "emerging growth company," we may elect not to provide you with certain information, including certain financial information and certain information regarding compensation of our executive officers, that we would otherwise have been required to provide in filings we make with the SEC, which may make it more difficult for investors and securities analysts to evaluate our company. We cannot predict if investors will find our common stock less attractive because we will 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. We may take advantage of these reporting exemptions until we are no longer an emerging growth company, which in certain circumstances could be for up to five years.

Because of the exemptions from various reporting requirements provided to us as an "emerging growth company" we may be less attractive to investors and it may be difficult for us to raise additional capital as and when we need it. Investors may be unable to compare our business with other companies in our industry if they believe that our financial accounting is not as transparent as other companies in our industry. If we are unable to raise additional capital as and when we need it, our business, results of operations, financial condition and cash flows and future prospects may be materially and adversely affected.

If we fail to maintain an effective system of internal control over financial reporting in the future, we may not be able to accurately report our business, results of operations, financial condition and cash flows and future prospects, which may adversely affect investor confidence in us and, as a result, the value of our common stock.

The Sarbanes-Oxley Act requires, among other things, that we maintain effective internal controls for financial reporting and disclosure controls and procedures and that we furnish a report by management on, among other things, the effectiveness of our internal control over financial reporting. This assessment needs to include disclosure of any material weaknesses identified by our management in our internal control over financial reporting. A material weakness is a deficiency, or combination of deficiencies, in internal control over financial reporting that results in more than a reasonable possibility that a material misstatement of annual or interim financial statements will not be prevented or detected on a timely basis. Section 404 of the Sarbanes-Oxley Act also generally requires an attestation from our independent registered public accounting firm on the effectiveness of our internal control over financial reporting. However, for as long as we remain an emerging growth company as defined in the JOBS Act, we intend to take advantage of the exemption permitting us not to comply with the independent registered public accounting firm attestation requirement.

Our compliance with Section 404 will require that we incur substantial accounting expense and expend significant management efforts. We currently do not have an internal audit group, and we will need to hire additional accounting and financial staff with appropriate public company experience and technical accounting knowledge, and compile the system and process documentation necessary to perform the evaluation needed to comply with Section 404. We may not be able to complete our evaluation, testing and any required remediation in a timely fashion. During the evaluation and testing

process, if we fail to identify and to remediate any significant deficiencies or material weaknesses that may be identified, or encounter problems or delays in the implementation of internal control over financial reporting, we will be unable to assert that our internal control over financial reporting is effective. We cannot assure you that there will not be material weaknesses or significant deficiencies in our internal control over financial reporting in the future. Any failure to maintain internal control over financial reporting could severely inhibit our ability to accurately report our financial condition, results of operations or cash flows. If we are unable to conclude that our internal control over financial reporting is effective, or if our independent registered public accounting firm determines we have a material weakness or significant deficiency in our internal control over financial reporting, we could lose investor confidence in the accuracy and completeness of our financial reports, the market price of our common stock could decline, and we could be subject to sanctions or investigations by the NASDAQ Stock Market, the SEC or other regulatory authorities. Failure to remedy any material weakness in our internal control over financial reporting, or to implement or maintain other effective control systems required of public companies, could also restrict our future access to the capital markets.

Our disclosure controls and procedures may not prevent or detect all errors or acts of fraud.

We are subject to the periodic reporting requirements of the Securities Exchange Act of 1934, as amended, or the Exchange Act. Our disclosure controls and procedures are designed to reasonably assure that information required to be disclosed by us in reports we file or submit under the Exchange Act is accumulated and communicated to management, recorded, processed, summarized and reported within the time periods specified in the rules and forms of the SEC. We believe that any disclosure controls and procedures or internal controls and procedures, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met.

These inherent limitations include the realities that judgments in decision-making can be faulty, and that breakdowns can occur because of simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people or by an unauthorized override of the controls. Accordingly, because of the inherent limitations in our control system, misstatements or insufficient disclosures due to error or fraud may occur and not be detected.

We have incurred and will incur increased costs as a result of operating as a public company, and our management is required to devote substantial time to new compliance initiatives.

As a public company, we have incurred and will incur significant legal, accounting and other expenses that we did not incur as a private company, and these expenses may increase even more after we are no longer an “emerging growth company.” We are subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Protection Act, as well as rules adopted, and to be adopted, by the SEC and NASDAQ Stock Market. Our management and other personnel devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations have substantially increased our legal and financial compliance costs and made some activities more time-consuming and costly. The increased costs will increase our consolidated net loss. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements.

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

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

Sales of a substantial number of shares of our common stock in the public market could cause our stock price to fall.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock.

We have in the past and may in the future grant rights to some of our stockholders that require us to register the resale of our common stock or other securities on behalf of these stockholders and/or facilitate public offerings of our securities held by these stockholders, including in connection with potential future acquisition or capital-raising transactions. For example, in connection with our public offering of common stock on September 19, 2016, we entered into a registration rights

agreement with the Baker Entities that together, based on information available to us, collectively beneficially owned approximately 45.1% of our common stock as of September 19, 2016. Under the registration rights agreement, we agree that, if at any time and from time to time after December 19, 2016, the Baker Entities demand that we register their shares of our common stock for resale under the Securities Act, we would be obligated to effect such registration. On January 6, 2017, pursuant to the registration rights agreement, we registered for resale, from time to time, up to 10,536,092 shares of our common stock held by the Baker Entities. Our registration obligations under this registration rights agreement cover all shares now held or hereafter acquired by the Baker Entities, would be in effect for up to ten years, and would include our obligation to facilitate certain underwritten public offerings of our common stock by the Baker Entities in the future. If the Baker Entities or any other holders of registration rights with respect to our common stock, by exercising their registration and/or underwriting rights or otherwise, sell a large number of our shares, or the market perceives that the Baker Entities or such holders intend to sell a large number of our shares, this could adversely affect the market price of our common stock. We have registered all currently reserved shares of common stock that we may issue under our equity compensation plans and intend to register in the future any additional reserved or issued shares of common stock. These registered shares can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates. We have also filed registration statements covering the sale of up to $124.6 million (remaining following the sale of $75.4 million of common stock in September 2016) and $52.0 million (remaining following the sale of $98.0 million of common stock in September 2015) of any combination of our common stock, preferred stock, debt securities or warrants and may conduct one or more sales of securities pursuant to such registration statement, from time to time.

Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.

We expect that significant additional capital will be needed in the future to continue our planned operations. To raise capital, we may sell substantial amounts of common stock or securities convertible into or exchangeable for common stock. These future issuances of common stock or common stock-related securities, including any sale of up to $25 million worth of shares of our common stock pursuant to our Sales Agreement with Cowen and Company, LLC together with the exercise of outstanding options and any additional shares issued in connection with acquisitions, if any, may result in material dilution to our stockholders. New investors could also gain rights, preferences and privileges senior to those of holders of our common stock.

Pursuant to our 2014 Equity Incentive Plan, our compensation committee is authorized to grant equity-based incentive awards to our directors, executive officers and other employees and service providers, including officers, employees and service providers of our subsidiaries and affiliates. Future option grants and issuances of common stock under our 2014 Equity Incentive Plan may have an adverse effect on the market price of our common stock.

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.

Provisions in our amended and restated certificate of incorporation, or certificate of incorporation, and amended and restated bylaws, or bylaws, as well as provisions of Delaware law, could make it more difficult for a third party to acquire us or increase the cost of acquiring us, even if doing so would benefit our stockholders, or remove our current management. These include provisions that:

- permit our board of directors to issue up to 5,000,000 shares of preferred stock, with any rights, preferences and privileges as they may designate;
- provide that all vacancies on our board of directors, including as a result of newly created directorships, may, except as otherwise required by law, be filled by the affirmative vote of a majority of directors then in office, even if less than a quorum;
- require that any action to be taken by our stockholders must be effected at a duly called annual or special meeting of stockholders and not be taken by written consent;
- provide that stockholders seeking to present proposals before a meeting of stockholders or to nominate candidates for election as directors at a meeting of stockholders must provide advance notice in writing, and also specify requirements as to the form and content of a stockholder's notice;
- not provide for cumulative voting rights, thereby allowing the holders of a majority of the shares of common stock entitled to vote in any election of directors to elect all of the directors standing for election; and

- provide that special meetings of our stockholders may be called only by the board of directors or by such person or persons requested by a majority of the board of directors to call such 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, who are responsible for appointing the members of our management. Because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which may discourage, delay or prevent someone from acquiring us or merging with us whether or not it is desired by or beneficial to our stockholders. Under Delaware law, a corporation may not, in general, engage in a business combination with any holder of 15% or more of its capital stock unless the holder has held the stock for three years or, among other things, the board of directors has approved the transaction. Any provision of our certificate of incorporation or bylaws or Delaware law that has the effect of delaying or deterring a change in control could limit the opportunity for our stockholders to receive a premium for their shares of our common stock, and could also affect the price that some investors are willing to pay for our common stock.

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 securities or industry analysts who cover us downgrade our stock or publish inaccurate or unfavorable research about our business, 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 stock could decrease, which might cause our stock price and trading volume to decline.

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

None.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

None.

Item 6. Exhibits

Number	Description
3.1(1)	Amended and Restated Certificate of Incorporation of Aquinox Pharmaceuticals, Inc.
3.2(2)	Amended and Restated Bylaws of Aquinox Pharmaceuticals, Inc.
4.1(3)	Specimen Common Stock Certificate of the Aquinox Pharmaceuticals, Inc.
4.2(4)	Registration Rights Agreement, dated September 19, 2016, by and between Aquinox Pharmaceuticals, Inc. and the persons listed on Schedule A attached thereto.
31.1+	Certification of Chief Executive Officer pursuant to Rule 13a-14(a).
31.2+	Certification of Chief Financial Officer pursuant to Rule 13a-14(a).
32.1+	Certification of Chief Executive Officer and Chief Financial Officer pursuant to 18 U.S.C. Section 1350.
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 Labels Linkbase Document.
101.PRE+	XBRL Taxonomy Extension Presentation Linkbase Document.

\+ Filed herewith.

(1) Previously filed as an exhibit to the Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission on March 12, 2014 (File No. 001-36327) and incorporated herein by reference.

(2) Previously filed as an exhibit to the Registrant's Registration Statement on Form S-1, as amended (File No. 333-193615), filed with the Securities and Exchange Commission on January 28, 2014 and incorporated herein by reference.

(3) Previously filed as an exhibit to the Registrant's Quarterly Report on Form 10-Q for the quarter ended March 31, 2014 filed with the Securities and Exchange Commission on May 13, 2014 (File No. 001-36327) and incorporated herein by reference.

(4) Previously filed as an exhibit to the Registrant's Current Report on Form 8-K filed with the Securities and Exchange Commission on September 20, 2016 (File No. 001-36327) and incorporated herein by reference.

SIGNATURES

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

Aquinox Pharmaceuticals, Inc.
(Registrant)

Date: August 8, 2017

/s/ David J. Main
David J. Main
President and Chief Executive Officer
(Principal Executive Officer)

Date: August 8, 2017

/s/ Kamran Alam
Kamran Alam
Chief Financial Officer
(Principal Financial and Accounting Officer)

EXHIBIT INDEX

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Exhibit 31.1

CERTIFICATIONS

I, David J. Main, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Aquinox Pharmaceuticals, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   a) designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b) designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c) evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d) disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent function):

   a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 8, 2017

/s/ David J. Main

David J. Main

President and Chief Executive Officer

Exhibit 31.2

CERTIFICATIONS

I, Kamran Alam, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Aquinox Pharmaceuticals, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   a) designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b) designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c) evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d) disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent function):

   a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 8, 2017

/s/ Kamran Alam

Kamran Alam
Chief Financial Officer

Exhibit 32.1

AQUINOX PHARMACEUTICALS, INC.
CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report of Aquinox Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the quarter ended June 30, 2017, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), David J. Main, President and Chief Executive Officer of the Company, and Kamran Alam, Chief Financial Officer of the Company, each hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to the best of his knowledge:

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

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

IN WITNESS WHEREOF, the undersigned have set their hands hereto as of the 8 th day of August 2017.

/s/ David J. Main
David J. Main
President and Chief Executive Officer

/s/ Kamran Alam
Kamran Alam
Chief Financial Officer

This certification accompanies the Form 10-Q to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of Aquinox Pharmaceuticals, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Form 10-Q), irrespective of any general incorporation language contained in such filing.