Table of Contents

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 September 30, 2017

OR

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

Commission File Number 001-38135

DOVA PHARMACEUTICALS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**81-3858961**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)

240 Leigh Farm Road, Suite 245
Durham, North Carolina 27707
(Address of principal executive offices and zip code)

(919) 748-5975
(Registrant's telephone number, including area code)

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

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

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

Large Accelerated Filer	☐	Accelerated Filer	☐
Non-accelerated Filer	☒ (Do not check if 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 registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). YES ☐ NO ☒

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

Class of Common Stock	**Outstanding Shares as of November 6, 2017**
Common Stock, $0.001 par value	25,652,457

Table of Contents

Dova Pharmaceuticals, Inc.
Condensed Consolidated Balance Sheets
(in thousands, except share and per share amounts)

	September 30, 2017 (Unaudited)	December 31, 2016
ASSETS		
Current assets		
Cash and cash equivalents	$ 100,414	$ 28,709
Prepaid expenses	998	37
Total current assets	101,412	28,746
Property, plant and equipment, net	35	—
Total assets	**$ 101,447**	**$ 28,746**
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities		
Accounts payable	$ 585	$ 157
Accrued expenses	5,451	7,918
Accrued interest	631	151
Due to related party	50	85
Note payable, short-term	27,119	—
Total current liabilities	33,836	8,311
Note payable, long-term	—	13,640
Total liabilities	**33,836**	**21,951**
Commitments and contingencies		
Stockholders' equity		
Preferred stock, $0.001 par value; 10,000,000 shares authorized; 0 and 982,714 shares issued and outstanding as of September 30, 2017 and December 31, 2016, respectively	—	1
Common stock, $0.001 par value; 100,000,000 shares authorized; 25,652,457 and 17,332,257 shares issued and outstanding as of September 30, 2017 and December 31, 2016, respectively	26	17
Additional paid-in capital	115,429	33,967
Accumulated deficit	(47,844)	(27,190)
Total stockholders' equity	67,611	6,795
Total liabilities and stockholders' equity	**$ 101,447**	**$ 28,746**

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

Dova Pharmaceuticals, Inc.
Condensed Consolidated Statements of Operations
(in thousands, except share and per share amounts)
(Unaudited)

	For the three months ended		For the nine months ended	For the period from March 24, 2016 (Inception) to
	September 30, 2017	September 30, 2016	September 30, 2017	September 30, 2016
Operating expenses:				
Research and development	$ 4,426	$ 6,758	$ 11,995	$ 13,898
Research and development - licenses acquired	1,000	—	1,000	5,000
General and administrative	4,185	368	7,045	643
Total operating expenses	9,611	7,126	20,040	19,541
Loss from operations	**(9,611)**	**(7,126)**	**(20,040)**	**(19,541)**
Other income (expenses)				
Other income (expense), net	224	(10)	243	(10)
Interest expense - related party	—	(3)	—	(4)
Interest expense	(336)	(35)	(857)	(35)
Total other expenses, net	(112)	(48)	(614)	(49)
Net loss	**$ (9,723)**	**$ (7,174)**	**$ (20,654)**	**$ (19,590)**
Net loss per share, basic and diluted	$ (0.38)	$ (0.41)	$ (1.03)	$ (1.13)
Weighted average common shares outstanding, basic and diluted	25,290,709	17,332,257	20,014,226	17,297,398

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

Dova Pharmaceuticals, Inc.
Condensed Consolidated Statement of Stockholders' Equity
(in thousands, except share amounts)
(Unaudited)

	Series A preferred stock		Common stock		Additional	Accumulated	Total stockholders'
	Shares	Amount	Shares	Amount	paid-in capital	deficit	equity
Balance as of December 31, 2016	**982,714**	**$ 1**	**17,332,257**	**$ 17**	**$ 33,967**	**$ (27,190)**	**$ 6,795**
Stock-based compensation	—	—	—	—	2,761	—	2,761
Conversion of preferred stock into common stock	(982,714)	(1)	3,242,950	4	(3)	—	—
Issuance of common stock in connection with IPO, net of offering costs	—	—	5,077,250	5	78,704	—	78,709
Net loss	—	—	—	—	—	(20,654)	(20,654)
Balance as of September 30, 2017	**—**	**$ —**	**25,652,457**	**$ 26**	**$ 115,429**	**$ (47,844)**	**$ 67,611**

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

Dova Pharmaceuticals, Inc.
Condensed Consolidated Statements of Cash Flows
(in thousands)
(Unaudited)

	For the nine months ended September 30, 2017	For the period from March 24, 2016 (Inception) to September 30, 2016
Cash flows from operating activities		
Net loss	(20,654)	$ (19,590)
Adjustments to reconcile net loss to net cash used in operating activities:		
Research and development-licenses acquired, expensed	1,000	5,000
Non-cash research and development expenses	9,663	13,741
Stock-based compensation	2,761	—
Changes in operating assets and liabilities:		
Prepaid expenses	(961)	(10)
Accounts payable	428	13
Accrued expenses	1,060	93
Accrued interest	480	35
Due to related party	(35)	58
Net cash used in operating activities	(6,258)	(660)
Cash flows from investing activities		
Purchase of fixed assets	(35)	—
Net cash used in investing activities	(35)	—
Cash flows from financing activities		
Proceeds from issuance of Series A preferred stock	—	10,000
Payment of offering cost in connection with issuance of Series A preferred stock	(711)	—
Capital contribution - PBM Capital	—	696
Proceeds from the issuance of common stock in connection with IPO	86,313	—
Payment of offering cost in connection with IPO	(7,604)	—
Net cash provided by financing activities	77,998	10,696
Net increase in cash and cash equivalents	71,705	10,036
Cash and cash equivalents at the beginning of the period	28,709	—
Cash and cash equivalents at the end of the period	**100,414**	**$ 10,036**
Supplemental disclosure of cash flow information:		
Cash paid for interest	377	$ —
Supplemental disclosure of noncash investing and financing activities:		
Change in note payable	13,479	$ 6,988
Capital contribution - PBM Capital Investments, LLC - payment of AkaRx upfront purchase price	—	$ 5,000
Accrued offering costs in connection with issuance of preferred stock	—	$ 239

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

Dova Pharmaceuticals, Inc.
Notes to Condensed Consolidated Financial Statements
(Unaudited)

Note 1—Organization and description of business operations

Dova Pharmaceuticals, Inc. ("Dova") was originally formed as PBM AKX Holdings, LLC, a limited liability company formed under the laws of the State of Delaware on March 24, 2016 ("Inception"). PBM AKX Holdings, LLC changed its name to Dova Pharmaceuticals, LLC by filing a Certificate of Amendment to its Certificate of Formation with the State of Delaware on June 15, 2016. Dova converted from a limited liability company to a corporation on September 15, 2016.

Dova was founded by PBM Capital Investments, LLC and certain affiliates of PBM Capital Investments, LLC (together, "PBM Capital").

Dova is a pharmaceutical company focused on acquiring, developing and commercializing drug candidates for diseases that are treated by specialist physicians, with an initial focus on addressing thrombocytopenia, a disorder characterized by a low blood platelet count. The Company's drug candidate, avatrombopag, recently completed two identically designed pivotal Phase 3 clinical trials that evaluated avatrombopag for the treatment of thrombocytopenia in patients with chronic liver disease scheduled to undergo a procedure. On September 21, 2017, a New Drug Application ("NDA") was submitted to the U.S. Food and Drug Administration (the "FDA") for this indication. The drug has not been approved by the FDA or other regulatory authorities for any use.

Dova entered into a Stock Purchase Agreement (the "Purchase Agreement"), dated March 29, 2016, with Eisai, Inc., a Delaware corporation ("Eisai"). Under the terms of the Purchase Agreement, Dova acquired all the issued and outstanding shares of the capital stock of AkaRx, Inc., a Delaware corporation ("AkaRx"), which holds the worldwide rights relating to avatrombopag. Contemporaneous with the acquisition, AkaRx entered into a Transition Services Agreement (the "TSA") with Eisai, and Eisai agreed to finance certain costs and expenses of AkaRx related to the development of avatrombopag incurred under the TSA pursuant to the terms of a Secured Promissory Note dated March 30, 2016 (the "Note"). See Note 3 for more information on the Purchase Agreement and related transactions as well as the Note.

On August 31, 2017, Dova established a subsidiary, Dova Pharmaceuticals Ireland Limited ("Dova Ireland"), through which the Company intends to submit a Marketing Authorization Application to the European Medicines Agency. AkaRx and Dova Ireland are the Company's only subsidiaries.

The unaudited condensed consolidated financial statements of Dova and its wholly owned subsidiaries AkaRx and Dova Ireland (the "Company") include the results of operations for the three and nine months ended September 30, 2017 and for the three months ended September 30, 2016 and the period from Inception through September 30, 2016.

Forward stock split

On June 16, 2017, the Company effected a 3.3-for-one forward stock split of the Company's common stock. No fractional shares were issued in connection with the stock split. The par value and other terms of the common stock were not affected by the stock split.

All share and per share amounts, including stock options, have been retroactively adjusted in these condensed consolidated financial statements for all periods presented to reflect the 3.3-for-one forward stock split. Further, exercise prices of stock options have been retroactively adjusted in these condensed consolidated financial statements for all periods presented to reflect the 3.3-for-one forward stock split. The number of shares of the Company's preferred stock were not affected by the forward stock split; however, the conversion ratios have been adjusted to reflect the forward stock split.

Liquidity and capital resources

The Company has incurred substantial operating losses since Inception, and expects to continue to incur significant operating losses for the foreseeable future and may never become profitable. As of September 30, 2017, the Company had an accumulated deficit of $47.8 million.

Between September 19, 2016 and November 18, 2016, the Company closed on the sale of an aggregate of 982,714 shares of Series A Preferred Stock for gross proceeds of approximately $29.0 million (purchase price of $29.51 per share).

Dova Pharmaceuticals, Inc.
Notes to Condensed Consolidated Financial Statements
(Unaudited)

On July 5, 2017, the Company closed an initial public offering ("IPO") of its common stock, which resulted in the issuance and sale of 5,077,250 shares of its common stock at a public offering price of $17.00 per share, generating net proceeds of approximately $78.7 million after deducting underwriting discounts and other offering costs. Upon the closing of the IPO, all outstanding shares of the Company's Series A convertible preferred stock were automatically converted into 3,242,950 shares of the Company's common stock. The Company expects to use the net proceeds from the IPO to fund the commercialization of avatrombopag in the United States, if approved, for the clinical development of avatrombopag for additional indications, to repay the Eisai note and for general corporate purposes.

The Company believes that it has adequate cash and cash equivalents to continue to fund operations in the normal course of business for at least the next 12 months.

Amendment and restatement of certificate of incorporation and bylaws

On July 5, 2017, the Company filed an Amended and Restated Certificate of Incorporation (the "Amended Certificate") with the Secretary of State of the State of Delaware in connection with the closing of the IPO. The Company's board of directors (the "Board") and stockholders previously approved the Amended Certificate to be filed in connection with, and to be effective upon, the closing of the IPO, and the form of the Amended Certificate was filed as an exhibit to the Company's Registration Statement on Form S-1 (the "Registration Statement") filed in connection with the IPO. On July 5, 2017, the Company's Amended and Restated Bylaws (the "Amended Bylaws") became effective in connection with the closing of the IPO. The Board and stockholders previously approved the Amended Bylaws to become effective upon the closing of the IPO, and the form of the Amended Bylaws was filed as an exhibit to the Registration Statement.

The Amended Certificate amends and restates in its entirety the Company's Amended and Restated Certificate of Incorporation, as amended, and the Amended Bylaws amend and restate, in their entirety, the Company's Bylaws. Collectively, the Amended Certificate and the Amended Bylaws, among other things: (i) authorize 100,000,000 shares of common stock; (ii) eliminate all references to the previously existing series of preferred stock; and (iii) authorize 10,000,000 shares of undesignated preferred stock that may be issued from time to time by the Board in one or more series.

Note 2—Significant accounting policies

Basis of presentation and principles of consolidation

The Company's unaudited condensed consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP") and include all adjustments necessary for the fair presentation of the Company's financial position for the periods presented. The results of operations for the three and nine months ended September 30, 2017 are not necessarily indicative of the results for the full year or the results for any future periods. These financial statements should be read in conjunction with the audited consolidated financial statements and related notes for the year ended December 31, 2016 included in the Company's final prospectus for its IPO dated as of June 28, 2017 and filed with the Securities and Exchange Commission (the "SEC") on June 30, 2017 pursuant to Rule 424(b)(4) under the Securities Act of 1933, as amended.

Use of estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the condensed consolidated financial statements and the reported amounts of expenses during the reporting period. The most significant estimates in the Company's condensed consolidated financial statements relate to the valuation of preferred and common stock, the valuation of stock options and the valuation allowance of deferred tax assets resulting from net operating losses. These estimates and assumptions are based on current facts, historical experience and various other factors believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities and the recording of expenses that are not readily apparent from other sources. Actual results may differ materially and adversely from these estimates. To the extent there are material differences between the estimates and actual results, the Company's future results of operations will be affected.

Dova Pharmaceuticals, Inc.
Notes to Condensed Consolidated Financial Statements
(Unaudited)

Segments

Operating segments are identified as components of an enterprise about which separate discrete financial information is available for evaluation by the chief operating decision-maker in making decisions regarding resource allocation and assessing performance. The Company views its operations and manages its business in one operating segment.

Cash and cash equivalents

The Company considers all highly liquid investments purchased with original maturities of 90 days or less at acquisition to be cash equivalents. Cash and cash equivalents include cash held in banks and money market mutual funds.

Accrued expenses

Accrued expenses primarily consist of Eisai FTE resource fees and out-of-pocket costs due under the TSA and other professional fees. Operating costs accrued for services received but not yet invoiced or paid under the TSA were $3.2 million as of September 30, 2017. Once the expenses under the TSA are approved for application to the Note by Eisai, these accrued expenses will be converted into the Note. The Company's policy is to record these TSA accrued expenses as current liabilities until such accrued expenses are converted into the Note.

Concentrations of credit risk and off-balance sheet risk

Cash and cash equivalents are financial instruments that are potentially subject to concentrations of credit risk. The Company's cash and cash equivalents are deposited in accounts at large financial institutions, and amounts may exceed federally insured limits. The Company believes it is not exposed to significant credit risk due to the financial strength of the depository institutions in which the cash and cash equivalents are held. The Company has no financial instruments with off-balance sheet risk of loss.

Research and development costs

Research and development costs, including acquired in-process research and development expenses for which there is no alternative future use, are expensed as incurred. Advance payments for goods and services that will be used in future research and development activities are expensed when the activity has been performed or when the goods have been received rather than when the payment is made.

Research and development costs primarily consist of payments made to Eisai upon the Company's acquisition of AkaRx and for ongoing costs for activities under the TSA with Eisai for research and development services associated with clinical trials, consultants, clinical trial materials, regulatory filings, laboratory costs and other supplies. In addition, Dova research and development personnel costs and contractor or consulting resources focused on research and development activities are included in this category.

Derivatives

The Company does not use derivative instruments to hedge exposures to cash flow, market, or foreign currency risks. The Company evaluates all of its financial instruments, including note payable and equity-linked financial instruments, to determine if such instruments are derivatives or contain features that qualify as embedded derivatives.

Fair value measurement

ASC 820, *Fair Value Measurements*, provides guidance on the development and disclosure of fair value measurements. Under this accounting guidance, fair value is defined as an exit price, representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date. As such, fair value is a market-based measurement that should be determined based on assumptions that market participants would use in pricing an asset or a liability.

The accounting guidance classifies fair value measurements in one of the following three categories for disclosure purposes:

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

Dova Pharmaceuticals, Inc.
Notes to Condensed Consolidated Financial Statements
(Unaudited)

Level 2: Inputs other than Level 1 prices for similar assets or liabilities that are directly or indirectly observable in the marketplace.
Level 3: Unobservable inputs which are supported by little or no market activity and values determined using pricing models, discounted cash flow methodologies, or similar techniques, as well as instruments for which the determination of fair value requires significant judgment or estimation.

The carrying amount of the Company's financial instruments, including cash and cash equivalents and accounts payable approximate their fair values. As of September 30, 2017 and December 31, 2016, the carrying amount of the Note approximates fair value as its interest rate approximates current market rates that could be obtained by the Company with a similar guarantee by PBM Capital Investments, LLC (Level 2 inputs).

Stock-based compensation

The Company expenses stock-based compensation to employees and Board members over the requisite service period based on the estimated grant-date fair value of the awards. Stock-based awards with graded-vesting schedules are recognized on a straight-line basis over the requisite service period for each separately vesting portion of the award. The Company records the expense for stock-based compensation awards subject to performance-based milestone vesting over the remaining service period when management determines that achievement of the milestone is probable. Management evaluates when the achievement of a performance-based milestone is probable based on the expected satisfaction of the performance conditions at each reporting date. The Company estimates the fair value of stock option grants using the Black-Scholes option pricing model, and the assumptions used in calculating the fair value of stock-based awards represent management's best estimates and involve inherent uncertainties and the application of management's judgment. All stock-based compensation costs are recorded in general and administrative or research and development costs in the statements of operations based upon the underlying individual's role at the Company.

Income taxes

On September 15, 2016, Dova converted from an LLC to a C-corporation. Prior to September 15, 2016, Dova Pharmaceuticals, LLC elected to be taxed as a partnership. Therefore, Dova was not subject to income taxes until its conversion to a C-corporation on September 15, 2016. AkaRx was subject to income taxes from April 1, 2016 through September 30, 2017.

Income taxes are recorded in accordance with ASC 740, *Income Taxes* ("ASC 740"), which provides for deferred taxes using an asset and liability approach. The Company recognizes deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the financial statements or tax returns. Deferred tax assets and liabilities are determined based on the difference between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Valuation allowances are provided, if based upon the weight of available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized.

The Company accounts for uncertain tax positions in accordance with the provisions of ASC 740. When uncertain tax positions exist, the Company recognizes the tax benefit of tax positions to the extent that the benefit would more likely than not be realized assuming examination by the taxing authority. The determination as to whether the tax benefit will more likely than not be realized is based upon the technical merits of the tax position as well as consideration of the available facts and circumstances.

Net loss per share

Upon the Company's conversion to a C-corporation on September 15, 2016, 52,522 member units were converted into 17,332,257 shares of common stock. Member units of the LLC had similar rights and characteristics as the Company's common stock issued upon the conversion. In calculating net loss per share, the Company retrospectively applied the effects of the conversion to member units outstanding during the period.

Net loss per share is computed by dividing net loss by the weighted average number of common shares outstanding during the period assuming the retrospective conversion of member units described above. Since the Company had a net loss in each of the periods presented, basic and diluted net loss per common share are the same. The computations of diluted net loss per common share for the three and nine months ended September 30, 2017 did not include the stock options to purchase 1,941,141 shares of common stock, as the inclusion of these securities would have been antidilutive.

Dova Pharmaceuticals, Inc.
Notes to Condensed Consolidated Financial Statements
(Unaudited)

Recent accounting pronouncements

In February 2016, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") No. 2016-02, *Leases* (Topic 842), which supersedes FASB ASC Topic 840, *Leases* (Topic 840) and provides principles for the recognition, measurement, presentation and disclosure of leases for both lessees and lessors. The new standard requires lessees to apply a dual approach, classifying leases as either finance or operating leases based on the principle of whether or not the lease is effectively a financed purchase by the lessee. This classification will determine whether lease expense is recognized based on an effective interest method or on a straight-line basis over the term of the lease, respectively. A lessee is also required to record a right-of-use asset and a lease liability for all leases with a term of greater than twelve months regardless of classification. Leases with a term of twelve months or less will be accounted for similar to existing guidance for operating leases. The standard is effective for annual and interim periods beginning after December 15, 2018, with early adoption permitted upon issuance. The Company is currently evaluating the impact of adopting this standard on the condensed consolidated financial statements and disclosures.

In April 2016, the FASB issued ASU No. 2016-09, *Share-Based Payment: Simplifying the Accounting for Share-Based Payments*. The standard addresses 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 new standard is effective for fiscal years and interim periods within those fiscal years beginning after December 15, 2016. The Company adopted ASU 2016-09 during the first quarter of 2017 and the Company elected to account for forfeitures as they occur. Other provisions of ASU 2016-09 had no impact on the Company's condensed consolidated financial statements.

In May 2017, the FASB issued ASU 2017-09, *Compensation—Stock Compensation (Topic 718): Scope of Modification Accounting*, which clarifies when to account for a change to the terms or conditions of a share-based payment award as a modification. Under the new guidance, modification accounting is required only if the fair value, the vesting conditions, or the classification of the award (as equity or liability) changes as a result of the change in terms or conditions. It is effective prospectively for the annual period ending December 31, 2018 and interim periods within that annual period. Early adoption is permitted. The Company is currently evaluating the impact of adopting this standard on the condensed consolidated financial statements and disclosures, but does not expect it to have a significant impact.

Note 3—The purchase agreement and related transactions

Purchase agreement with Eisai

As described in Note 1, Dova entered into a Purchase Agreement dated March 29, 2016 with Eisai for all of the issued and outstanding shares of the capital stock of AkaRx. The terms of the Purchase Agreement included (i) an up-front payment of $5.0 million that was paid at closing and funded by a capital contribution by the Company's sole member, PBM Capital Investments, LLC, (ii) milestone payments up to $135.0 million in the aggregate based on annual net sales of avatrombopag, and (iii) a commitment to negotiate in good faith to secure a long-term supply agreement with Eisai to govern manufacturing support and the purchase of avatrombopag from Eisai until the later of March 30, 2021 or the third anniversary of the commercialization of avatrombopag.

The transaction was accounted for as an asset acquisition pursuant to ASU 2017-01, *Business Combinations (Topic 805), Clarifying the Definition of a Business,* as the majority of the fair value of the assets acquired was concentrated in a group of similar assets, and the acquired assets did not have outputs or employees. The assets acquired under the Purchase Agreement included a license to avatrombopag, other associated intellectual property, inventory, documentation and records, and related materials. Because avatrombopag had not yet received regulatory approval, the $5.0 million purchase price paid to date for these assets was expensed in the Company's statement of operations for the period from Inception to September 30, 2016. In addition, the potential milestone payments based on annual net sales are not yet considered probable, and no milestone payments have been accrued at September 30, 2017.

Dova Pharmaceuticals, Inc.
Notes to Condensed Consolidated Financial Statements
(Unaudited)

Long-term supply agreement with Eisai

In June 2017, the Company entered into a supply agreement with Eisai, pursuant to which the Company agreed to purchase finished drug product for avatrombopag from Eisai and Eisai agreed to supply finished drug product for avatrombopag. The initial term of the agreement will terminate on the later of March 30, 2021 and the third anniversary of the Company's first commercial sale of avatrombopag. After the initial term, the supply agreement may be renewed by mutual agreement of the parties. During the initial term, Eisai is the Company's exclusive supplier of finished drug product, except that the Company has the right to terminate the exclusivity early by payment to Eisai of a fee calculated based on the Company's forecasted purchases of avatrombopag during the remainder of the initial term. In addition, in the event that Eisai fails to deliver substantially all of the finished drug product due to the Company under the agreement, the Company may elect to seek alternative supply arrangements so long as such failure remains uncured, subject to certain exceptions. The aggregate payments to Eisai under the supply agreement for finished drug product will be the greater of a fixed payment per tablet and a payment calculated in the mid-single digit percentages of net sales of avatrombopag.

Transition services agreement

Pursuant to the terms and conditions of the TSA, Eisai agreed to manage the ongoing clinical trials for the Company through regulatory approval of avatrombopag based on an agreed upon fee schedule for services plus reimbursement of certain out of pocket expenses. Services may be provided by Eisai's full-time employees, its affiliates or third-party contractors. Payments under this agreement that exceed $51.0 million will be credited against any milestone payments due to Eisai under the Purchase Agreement. Pursuant to the TSA, payments due are being financed under the Note with Eisai as described below. The Company may terminate the services provided under the TSA on a service-by-service basis or the agreement in its entirety upon 60-days' written notice. The TSA may also be terminated (i) by mutual consent, (ii) by either party upon 60-days' written notice if the other party materially breaches the agreement and fails to cure such breach, (iii) by either party in the event of the other party's bankruptcy, insolvency or certain similar occurrences, and (iv) by either party in the event that such party is unable to perform its obligations under the agreement as a result of events outside of its reasonable control. The Company has final decision-making authority related to development of avatrombopag and the regulatory approval process.

Eisai note and security agreement

On March 30, 2016, the Company issued the Note to Eisai, which enables the Company to finance payments due to Eisai under the TSA. The principal amount of the Note will be increased by the amount of unpaid service fees and out-of-pocket expenses due and owed to Eisai under the TSA. As of September 30, 2017, the Company had outstanding borrowings of $27.1 million under the Note, additional TSA expenses of $3.2 million included in accrued expenses and the Company owed Eisai $0.6 million in accrued interest. The Note matures on March 30, 2018 and bears interest at a rate of 5% per annum. Interest is payable annually in arrears to Eisai on March 31, 2017 and 2018. The maturity of the Note may be accelerated by Eisai upon a change of control defined as any investor or group gaining more than 50% of the equity interests of AkaRx. Principal and interest under the Note can be prepaid at any time without penalty. The Note is secured by a blanket security interest on all of the assets of AkaRx, including the worldwide rights to avatrombopag. Payments due to Eisai under the Note are currently guaranteed by PBM Capital Investments, LLC.

License agreement with Astellas Pharma Inc.

The primary intellectual property related to avatrombopag is licensed from Astellas Pharma Inc. ("Astellas") on an exclusive, worldwide basis under the terms of a license agreement that the Company acquired from Eisai under the Purchase Agreement. Under the terms of the license agreement, the Company is required to make payments upon the achievement of certain milestones. On September 21, 2017, upon the filing of the NDA, the Company became obligated to make a milestone payment of $1.0 million within 30 days, which is included in accrued expenses as of September 30, 2017. The Company will be required to make additional aggregate milestone payments of up to $4.0 million to Astellas if certain other regulatory milestones are achieved. In addition, the Company will be required to pay Astellas tiered royalties ranging from the mid to high single digits on net sales of avatrombopag. No amounts have been accrued for any potential milestone payments as the payments were not deemed probable. Unless earlier terminated, this license agreement with Astellas will expire on a country-by-country and product-by-product basis upon the latest of (i) the expiration of the last-to-expire claim of the licensed patents, (ii) the expiration of any government-granted marketing exclusivity period for avatrombopag, and (iii) 10 years after the last date of launch of avatrombopag to have occurred in any country. Thereafter, the term of the license agreement may be extended for successive one-year terms if the Company notifies Astellas in writing of its desire to extend such term at least three months before it is otherwise set to expire.

Dova Pharmaceuticals, Inc.
Notes to Condensed Consolidated Financial Statements
(Unaudited)

Note 4—Related party agreements

Dova and AkaRx management services agreements

On April 1, 2016, Dova and AkaRx each entered into a Services Agreement (each, an "SA") with PBM Capital Group, LLC. Pursuant to the terms of each of the SAs, which have terms of twelve months each (and are automatically renewable for successive one-year periods), PBM Capital Group, LLC will render advisory and consulting services to Dova and AkaRx. Services provided under the SAs may include certain scientific and technical, accounting, operations and back office support services. In consideration for these services, Dova and AkaRx are each obligated to pay PBM Capital Group, LLC a monthly management fee of $25,000.

For the three and nine months ended September 30, 2017, the Company incurred expenses under the SAs of $150,000 and $450,000, respectively, which were included in general and administrative expenses.

For the three months ended September 30, 2016 the Company incurred expenses under the SAs of $150,000 and for the period from March 24, 2016 (Inception) to September 30, 2016, the Company incurred expenses under the SAs of $300,000, which were included in general and administrative expenses. No expenses under the SA were incurred prior to April 1, 2016.

As of September 30, 2017, the Company owed PBM Capital Group, LLC and its affiliates approximately $50,000.

As described more fully in Note 3, PBM Capital Investments, LLC has guaranteed payments due by the Company to Eisai.

Note 5—Stockholders' equity

Conversion to a C-Corporation and common stock

On March 29, 2016, in connection with the Purchase Agreement with Eisai for all of the issued and outstanding shares of the capital stock of AkaRx, the Company issued PBM Capital Investments, LLC an aggregate of 50,000 units in exchange for its payment to Eisai of $5.0 million on the Company's behalf in connection with the acquisition of worldwide rights to avatrombopag. On April 1, 2016, pursuant to a co-investment agreement (the "Co-Investment Agreement"), the Company issued and sold to certain affiliates of PBM Capital Investments, LLC, an aggregate of 2,522 units at a purchase price of $100.00 per unit for an aggregate purchase price of $252,200. Shortly prior to the conversion from an LLC to a C-corporation on September 15, 2016, each of the members of Dova Pharmaceuticals, LLC made a pro rata capital contribution of an aggregate $0.4 million with no increase in member units.

On September 15, 2016, the Company converted from an LLC to a C-corporation and issued 17,332,257 shares of common stock, par value $0.001, in exchange for all 52,522 outstanding membership units.

Prior to the Company's IPO, pursuant to agreements with the Company's common stockholders, Paul B. Manning, a director of the Company and the controlling person of the Company's largest stockholder, PBM Capital Investments, LLC, had sole voting and dispositive power over all outstanding shares of the Company's common stock. Mr. Manning relinquished sole voting and dispositive power over the shares of common stock held by certain affiliates of PBM Capital Investments, LLC in connection with the IPO. After the IPO, PBM Capital Investments, LLC and funds under common control with PBM Capital Investments, LLC beneficially own a majority of the Company's common stock.

Series A preferred stock

Between September 19, 2016 and November 18, 2016, the Company closed on the sale of an aggregate of 982,714 shares of Series A preferred stock for gross proceeds of $29.0 million (at a purchase price of $29.51 per share). The Series A preferred stock was entitled to non-cumulative, non-compounding dividends at 8.0% per annum (based on the original issue price), when, as and if any dividends are declared by the Board.

Each share of Series A preferred stock was convertible, at the option of the holder and at any time, into a number of fully paid and non-assessable shares of common stock determined by dividing the Series A Original Issue Price by the Series A Conversion Price in effect at the time of conversion. The Series A preferred stock was mandatorily convertible under certain conditions (i) when the Company issued shares of common stock in a public offering generating gross proceeds of at least $60.0 million to the Company, at a

Dova Pharmaceuticals, Inc.
Notes to Condensed Consolidated Financial Statements
(Unaudited)

price per share of at least $17.88, or (ii) by majority vote of the then outstanding shares of Series A preferred stock. The Series A Conversion Price was $8.94, and was subject to adjustment based on events including the issuance of additional equity securities, certain dividends and distributions, mergers and reorganizations, and stock splits and combinations.

The Series A preferred stock was not mandatorily redeemable and did not embody an unconditional obligation to settle in a variable number of equity shares. As such, the Series A preferred stock was classified as permanent equity on the consolidated balance sheet. The holders' contingent redemption right in the event of certain deemed liquidation events did not preclude permanent equity classification.

Further, the Series A preferred stock was considered an equity-like host for purposes of assessing embedded derivative features for potential bifurcation. The embedded conversion feature is considered to be clearly and closely related to the associated preferred stock host instrument and therefore was not bifurcated from the equity host. The contingent put right upon certain deemed liquidation events was not clearly and closely related to the associated preferred stock host instrument but did not meet the definition of a derivative and therefore was not bifurcated from the equity host.

Upon the closing of the IPO on July 5, 2017, all outstanding shares of the Company's Series A convertible preferred stock were automatically converted into 3,242,950 shares of the Company's common stock.

Issuance of common stock in connection with the Company's initial public offering

On July 5, 2017, the Company closed its IPO, which resulted in the issuance and sale of 5,077,250 shares of its common stock at a public offering price of $17.00 per share, generating net proceeds of approximately $78.7 million after deducting underwriting discounts and other offering costs.

Note 6—Stock-based compensation

Options

In June 2017, the Board adopted and approved the Amended and Restated 2017 Equity Incentive Plan (the "IPO Plan"), which amended and restated the Company's prior 2017 Equity Incentive Plan (the "2017 Plan") and became effective in connection with the IPO pricing on June 28, 2017. Prior to the effectiveness of the IPO Plan, the 2017 Plan provided for the grant of share-based awards to employees, directors and consultants of the Company. As a result of the effectiveness of the IPO Plan, no further grants may be made under the 2017 Plan.

The IPO Plan provides for the grant of incentive stock options to employees, and for the grant of nonstatutory options, restricted stock awards, restricted stock unit awards, stock appreciation rights, performance-based stock awards and other forms of stock awards to employees, including officers, consultants and directors. The IPO Plan also provides for the grant of performance-based cash awards to employees, including officers, consultants and directors. The Company has initially reserved 4,285,250 shares of common stock for issuance under the IPO Plan, which is the sum of (1) 2,000,000 new shares, plus (2) the number of shares reserved for issuance under the Company's 2017 Equity Incentive Plan (the "2017 Plan") at the time the IPO Plan became effective, plus (3) any shares subject to outstanding stock options or other stock awards that would have otherwise returned to the 2017 Plan (such as upon the expiration or termination of a stock award prior to exercise). The number of shares of common stock reserved for issuance under the IPO Plan will automatically increase on January 1 each year, for a period of ten years, from January 1, 2018 through January 1, 2027, by 4% of the total number of shares of the Company's common stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares as may be determined by the Board. As of September 30, 2017, 2,344,109 shares were available for grant under the IPO Plan. As of September 30, 2017, options to purchase 1,941,141 shares of the Company's common stock were outstanding at a weighted average exercise price of $6.20 per share.

Stock Option Valuation

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model. The Company historically has been a private company and lacks company-specific historical and implied volatility information. Therefore, it estimates its expected stock volatility based on the historical volatility of a publicly traded set of peer companies and expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded stock price. Due to the lack of historical exercise history, the expected term of the Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The expected term of stock options granted to nonemployees is equal to the contractual term of the option award. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of

Dova Pharmaceuticals, Inc.
Notes to Condensed Consolidated Financial Statements
(Unaudited)

the award for time periods approximately equal to the expected term of the award. Expected dividend yield is based on the fact that the Company has never paid cash dividends and does not expect to pay any cash dividends in the foreseeable future. Prior to the IPO, the fair values of the shares of common stock underlying the Company options were estimated on each grant date by the Company. In order to determine the fair value, the Company considered, among other things, contemporaneous valuations of the Company's common stock and preferred stock, the Company's business, financial condition and results of operations, including related industry trends affecting its operations; the likelihood of achieving a liquidity event, such as an initial public offering, or IPO, or sale, given prevailing market conditions; the lack of marketability of the Company's common stock; the market performance of comparable publicly traded companies; and U.S. and global economic and capital market conditions. Since the IPO, the fair value of the common stock underlying the Company's options has been based upon the closing price of the Company's common stock on the grant date.

Option awards

The fair value of the Company's option awards was estimated using the assumptions below:

	For the nine months ended September 30, 2017
Exercise price	$3.73 - $24.28
Risk-free rate of interest	1.71% - 2.16%
Term (years)	5.2 - 7.1
Expected stock price volatility	87.21% - 89.38%

The following table summarizes the Company's stock option activity under the 2017 Plan and IPO Plan for the nine months ended September 30, 2017:

	Number of shares	Weighted average exercise price	Weighted average remaining contractual life (in years)	Aggregate intrinsic value
Outstanding as of December 31, 2016	—	$ —	—	$ —
Employee options granted	1,941,141	6.20	9.6	35,102,000
Outstanding as of September 30, 2017	1,941,141	$ 6.20	9.6	$ 35,102,000
Options vested and exercisable as of September 30, 2017	—	$ —	—	$ —

The aggregate intrinsic value in the above table is calculated as the difference between fair value of the Company's closing common stock price on September 29, 2017, or $24.28 per share, and the exercise price of the stock options that had strike prices below $24.28 per share. The weighted average grant date fair value per share of options granted during the nine months ended September 30, 2017 was $7.22.

Stock-based compensation expense has been reported in the Company's condensed consolidated statements of operations for the three and nine months ended September 30, 2017 is as follows (in thousands):

	For the three months ended September 30, 2017	For the nine months ended September 30, 2017
General and administrative	$ 1,690	$ 2,179
Research and development	452	582
Total stock-based compensation	$ 2,142	$ 2,761

Dova Pharmaceuticals, Inc.
Notes to Condensed Consolidated Financial Statements
(Unaudited)

The Company's stock options generally vest as follows: 25% after 12 months of continuous services and the remaining 75% on a ratable basis over a 36-month period from 12 months after the grant date. Stock options granted during the nine months ended September 30, 2017 have a maximum contractual term of 10 years. As of September 30, 2017, there was approximately $11.2 million of total unrecognized compensation expense, related to the unvested stock options shown in the table above, which is expected to be recognized over a weighted average period of 1.6 years.

Note 7—Commitments and contingencies

Office Lease

In June 2017, the Company entered into a lease consisting of approximately 7,351 square feet of office space in Durham, North Carolina. This lease provides for current monthly payments of approximately $15,000 and expires on April 30, 2020.

On September 22, 2017, the Company entered into an amendment to its lease agreement, effective October 1, 2017, to increase the total amount of leased office space to an aggregate of approximately 14,378 square feet. The amended lease agreement requires future rental payments of $38,000 during the final three months of the year ending December 31, 2017, and payments of $0.3 million, $0.3 million and $0.1 million during the years ending December 31, 2018, 2019 and 2020, respectively.

Litigation

The Company is not a party to any material legal proceedings and is not aware of any pending or threatened claims. From time to time, the Company may be subject to various legal proceedings and claims that arise in the ordinary course of its business activities.

Item 2. Financial Information.

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

The following discussion and analysis of our financial condition, results of operations and cash flows should be read in conjunction with (1) the unaudited interim condensed consolidated financial statements and the related notes thereto included elsewhere in this Quarterly Report on Form 10-Q, and (2) the audited consolidated financial statements and notes thereto and management's discussion and analysis of financial condition and results of operations for the period from March 24, 2016 (inception) through December 31, 2016 included in our final prospectus dated June 28, 2017, filed with the Securities and Exchange Commission, or the SEC on June 30, 2017, pursuant to Rule 424(b)(4) under the Securities Act of 1933, as amended, or the Securities Act.

Forward-looking statements

This Quarterly Report on Form 10-Q contains "forward-looking statements" within the meaning of Section 27A of the Securities Act and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. These statements are often identified by the use of words such as "expect," "anticipate," "estimate," "may," "will," "should," "intend," "believe," and similar expressions, although not all forward-looking statements contain these identifying words. Although we believe the expectations reflected in these forward-looking statements are reasonable, such statements are inherently subject to significant risks and uncertainties and we can give no assurances that our expectations will prove to be correct. Actual results could differ materially from those described in this report because of numerous factors, many of which are beyond our control. These factors include, without limitation, those described in this Quarterly Report under Part II - Item 1A "Risk Factors," and in our other filings with the SEC. We undertake no obligation to update these forward-looking statements to reflect events or circumstances after the date of this report or to reflect actual outcomes.

Overview

We are a pharmaceutical company focused on acquiring, developing and commercializing drug candidates for diseases that are treated by specialist physicians, with an initial focus on addressing thrombocytopenia, a disorder characterized by a low blood platelet count. Our drug candidate, avatrombopag, which we acquired from Eisai, Inc., or Eisai, in March 2016, is an orally administered thrombopoietin receptor agonist, or TPO-RA, that we are developing for the treatment of thrombocytopenia. We have recently completed two identically designed pivotal Phase 3 clinical trials that evaluated avatrombopag for the treatment of thrombocytopenia in patients with chronic liver disease, or CLD, scheduled to undergo a procedure. Avatrombopag met the primary and secondary endpoints in each of these clinical trials with high statistical significance. Based on these results, a new drug application, or NDA, was submitted to the U.S. Food and Drug Administration, or FDA, for this initial indication on September 21, 2017.

We have global rights to avatrombopag. Our intent is to initially build a hepatology-focused sales organization in the United States. We intend to target the approximately 850 hepatologists, most of whom are working at one of the approximately 150 liver transplant centers in the United States. We may pursue collaborations with third parties to commercialize our drug candidates outside the United States, either through territorial licenses or distributor relationships.

We have a limited operating history as we were formed on March 24, 2016. Since our inception, our operations have focused on acquiring rights to avatrombopag, organizing and staffing our company, business planning, raising capital, establishing our intellectual property portfolio, conducting clinical trials and preparing for and submitting an NDA for avatrombopag. We do not have any drug candidates approved for sale and have not generated any revenue from drug sales. We have funded our operations primarily through the sale of equity and equity-linked securities. On July 5, 2017, we closed our initial public offering, or IPO, of common stock, which resulted in the issuance and sale of 5,077,250 shares of common stock at a public offering price of $17.00 per share, resulting in net proceeds of approximately $78.7 million after deducting underwriting discounts and other offering costs. Upon the closing of the IPO, all outstanding shares of our Series A convertible preferred stock were automatically converted into 3,242,950 shares of common stock. We believe that the net proceeds from the IPO, together with our existing cash and cash equivalents, will enable us to fund our operating expenses, debt service obligations and capital expenditure requirements for at least the next 12 months. The proceeds will be used to build the necessary commercial infrastructure to support the launch of avatrombopag in the United States, if approved, for clinical development of avatrombopag for follow-on indications, to repay the Eisai note and for general corporate purposes. See "—Liquidity and capital resources."

Since inception, we have incurred significant operating losses. For the nine months ended September 30, 2017 and for the period from March 24, 2016 (Inception) to December 31, 2016, our net loss was $20.7 million and $27.2 million, respectively. As of September 30, 2017, we had an accumulated deficit of $47.8 million. We expect to continue to incur significant expenses and operating losses for the foreseeable future. We anticipate that our expenses will increase significantly in connection with our ongoing activities, as we:

- continue to invest in the preclinical and clinical development of avatrombopag for the treatment of other thrombocytopenia indications;
- prepare for commercialization of avatrombopag, if approved, including the hiring of medical affairs and sales and marketing personnel;
- manufacture our drug candidate, including under our supply agreement with Eisai;
- hire additional research and development and selling, general and administrative personnel;
- maintain, expand and protect our intellectual property portfolio;
- evaluate opportunities for development of additional drug candidates; and
- incur additional costs associated with operating as a newly public company.

Stock purchase agreement with Eisai

In March 2016, we entered into the stock purchase agreement with Eisai, or the Eisai stock purchase agreement, pursuant to which we acquired the worldwide rights to avatrombopag. The terms of the Eisai stock purchase agreement included (i) an up-front payment of $5.0 million, (ii) milestone payments up to $135.0 million in the aggregate based on annual net sales of avatrombopag and (iii) a commitment to negotiate in good faith to secure a long-term supply agreement with Eisai to purchase supplies of avatrombopag from Eisai. See Note 3 "The purchase agreement and related transactions" in the accompanying notes to the condensed consolidated financial statements included in this Quarterly Report on Form 10-Q for additional information.

Transition services agreement with Eisai

In March 2016, in connection with our acquisition of the rights to avatrombopag, we entered into a transition services agreement with Eisai, or the TSA. Pursuant to the terms and conditions of the TSA, Eisai has agreed to manage the ongoing clinical trials for us through regulatory approval of avatrombopag based on an agreed upon fee schedule for services plus reimbursement of certain out-of-pocket expenses. Services may be provided by Eisai's full-time employees, its affiliates or third-party contractors. Payments due under this agreement that exceed $51.0 million will reduce any milestone payments due to Eisai under the Eisai stock purchase agreement. Pursuant to the TSA, payments due are being financed under the Eisai note described below. We will have final decision-making authority related to development of avatrombopag and the regulatory approval process.

Supply agreement with Eisai

In June 2017, we entered into a supply agreement with Eisai, pursuant to which we agreed to purchase finished drug product for avatrombopag from Eisai and Eisai agreed to supply finished drug product for avatrombopag to us. The initial term of the agreement will terminate on the later of March 30, 2021 or the third anniversary of our first commercial sale of avatrombopag. After the initial term, the supply agreement may be renewed by mutual agreement of the parties. During the initial term, Eisai is our exclusive supplier of finished drug product, except that we have the right to terminate the exclusivity early by payment to Eisai of a fee calculated based on our forecasted purchases of avatrombopag during the remainder of the initial term. In addition, in the event that Eisai fails to deliver substantially all of the finished drug product due to us under the agreement, we may elect to seek alternative supply arrangements so long as such failure remains uncured, subject to certain exceptions. The aggregate payments to Eisai under the supply agreement for finished drug product will be the greater of a fixed payment per tablet and a payment calculated in the mid-single digit percentages of net sales of avatrombopag.

Eisai note and security agreement

In March 2016, we issued a note to Eisai, or the Eisai note, which enables us to finance payments due to Eisai under the TSA. The principal amount of the Eisai note will be increased by the amount of unpaid service fees and out-of-pocket expenses due and owed to Eisai under the TSA. As of September 30, 2017, we had outstanding borrowings of $27.1 million under this Eisai note, additional TSA expenses of $3.2 million included in accrued expenses and we owed Eisai $0.6 million in accrued interest. The Eisai note matures on March 30, 2018 and bears interest at a rate of 5% per annum. Interest is payable annually in arrears to Eisai beginning on March 31, 2017 and, accordingly, we paid a single interest-only payment of $0.4 million in March 2017. Principal and interest under the Eisai note can be prepaid at any time without penalty. The Eisai note is secured by a blanket security interest on all of the assets of our wholly-owned subsidiary, AkaRx, including the worldwide rights to avatrombopag. Payments due pursuant to the Eisai note are currently guaranteed by PBM Capital Investments, LLC.

License agreement with Astellas

The primary intellectual property related to avatrombopag is licensed to us from Astellas on an exclusive, worldwide basis under the terms of a license agreement we acquired from Eisai in connection with our acquisition of the rights to avatrombopag from Eisai. Under

the terms of the license agreement, we are required to make payments upon the achievement of certain milestones. On September 21, 2017, upon the filing of the NDA, we became obligated to make a milestone payment of $1.0 million within 30 days, which is included in accrued expenses as of September 30, 2017. We will be required to make additional aggregate milestone payments of up to $4.0 million to Astellas if certain other regulatory milestones are achieved. In addition, we will be required to pay Astellas tiered royalties in the mid to high single-digit percentages on net sales of avatrombopag. No amounts have been accrued for any potential future milestone payments as such payments have not been deemed probable. See Note 3 "The purchase agreement and related transactions" in the accompanying notes to the condensed consolidated financial statements included in this Quarterly Report on Form 10-Q for additional information.

Services agreements with PBM Capital Group, LLC

In April 2016, we entered into a services agreement with PBM Capital Group, LLC, an affiliate of PBM Capital Investments, LLC, or the Dova services agreement, to engage PBM Capital Group, LLC for certain scientific and technical, accounting, operations and back office support services. We agreed to pay PBM Capital Group, LLC a flat fee of $25,000 per month for these services. The Dova services agreement had an initial term of 12 months and was extended on April 1, 2017 for an additional one-year term.

In April 2016, AkaRx entered into a services agreement with PBM Capital Group, LLC, or the AkaRx services agreement, and together with the Dova services agreement, the Services Agreements, to engage PBM Capital Group, LLC for certain scientific and technical, accounting, operations and back office support services. AkaRx agreed to pay PBM Capital Group, LLC a flat fee of $25,000 per month for these services. The AkaRx services agreement had an initial term of 12 months and was extended on April 1, 2017 for an additional one-year term.

Components of results of operations

Revenue

To date, we have not generated any revenue from drug sales. We do not expect to generate any revenue from any drug candidates that we develop unless and until we obtain regulatory approval and commercialize our drugs or enter into collaborative agreements with third parties. An NDA was submitted for the approval of avatrombopag on September 21, 2017. If avatrombopag is approved, then we may generate revenue from drug sales. We do not expect to commercialize avatrombopag before 2018, if ever.

Operating expenses

Research and development expense

Research and development expense consists of our upfront payment made to Eisai in connection with the acquisition of avatrombopag and costs incurred in connection with our research activities, most of which to-date have been incurred under the TSA and include costs associated with clinical trials, consultants, clinical trial materials, regulatory filings, facilities, laboratory expenses and other supplies.

Research and development costs are expensed as incurred. Costs for certain activities, such as manufacturing and preclinical studies and clinical trials, are generally recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and collaborators.

We expect our research and development expense will increase for the foreseeable future as we seek approval for avatrombopag and as we pursue expanded indications for avatrombopag. Drug candidates in later stages of clinical development, such as avatrombopag, generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. Additionally, we are hiring internal resources to lead and take over development work that has historically been handled by Eisai personnel under the TSA.

The duration, costs and timing of additional clinical trials for avatrombopag and any other drug candidates will depend on a variety of factors that include, but are not limited to, the following:

- number of trials required for approval;
- delays in reaching, or failing to reach, agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites or prospective CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- clinical trials of our drug candidates producing negative or inconclusive results, including failure to demonstrate statistical significance;

- per patient trial costs, including based on number of doses that patients receive;
- the number of patients that participate in the trials and then drop-out or discontinuation rates of patients;
- the number of sites included in the trials;
- the countries in which the trial is conducted;
- the length of time required to enroll eligible patients;
- potential additional safety monitoring or other studies requested by regulatory agencies;
- undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators or institutional review boards to suspend or terminate the trials;
- the duration of patient follow-up;
- timing and receipt of regulatory approvals;
- the efficacy and safety profile of the drug candidate;
- third-party contractors failing to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- regulators or institutional review boards requiring that we or our investigators suspend or terminate clinical development for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks; and
- the insufficiency or inadequacy of the supply or quality of our drug candidates or other materials necessary to conduct clinical trials of our drug candidates.

At this time, we cannot reasonably estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete the development of avatrombopag.

We are also unable to predict when, if ever, material net cash inflows will commence from sales of avatrombopag. This is due to the numerous risks and uncertainties associated with developing and commercializing avatrombopag, including the uncertainty of:

- achieving successful enrollment and completion of additional clinical trials and achieving regulatory approval of avatrombopag for the treatment of thrombocytopenia beyond its initial indication;
- establishing an appropriate safety profile;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers that provide for commercial quantities of avatrombopag manufactured at acceptable cost levels and quality standards;
- obtaining regulatory approval for the marketing of avatrombopag for the treatment of thrombocytopenia in CLD patients scheduled to undergo a procedure;
- commercializing avatrombopag, if approved, whether alone or in collaboration with others;
- whether any indication approved by regulatory authorities is narrower than we expect;
- compliance with ongoing regulatory review by the FDA, European Medicines Agency, or the EMA, or any comparable foreign regulatory authorities;
- our ability to establish sales and marketing capabilities for avatrombopag;
- the efficacy and safety of avatrombopag and potential advantages compared to alternative treatments, notwithstanding success in meeting or exceeding clinical trial endpoints;
- the size of the markets for approved indications in territories in which we receive regulatory approval, if any;
- the ability to set an acceptable price for avatrombopag and obtain coverage and adequate reimbursement from third-party payors;
- the degree of competition we face from competitive therapies;
- the ability to add operational, financial, management and information systems personnel, including personnel to support our clinical, manufacturing and planned future commercialization efforts and operations as a public company;
- retention of key research and development personnel;
- the ability to continue to build out and retain an experienced management and advisory team;
- the ability to maintain, expand and protect our intellectual property portfolio, including any licensing arrangements with respect to our intellectual property; and
- the ability to avoid and defend against third-party infringement and other intellectual property related claims.

A change in the outcome of any of these variables with respect to the development of our drug candidate would significantly change the costs, timing and viability associated with the development of that drug candidate.

General and administrative expense

General and administrative expense consists primarily of expenses under the services agreements with PBM Capital Group, LLC, salaries and other related costs, recruiting fees, professional fees for accounting and legal services and consulting fees to support the potential launch of avatrombopag. Other than payments to PBM Capital Group, LLC under the services agreements, for the period from

March 24, 2016 (Inception) to December 31, 2016, we did not pay any employee compensation or issue any stock-based compensation to any employee, director or consultant. We began paying compensation and issuing equity awards to employees during the nine months ended September 30, 2017.

We expect our general and administrative expense will increase for the foreseeable future to support our continued clinical development activities, potential commercialization of avatrombopag and increased costs of operating as a public company. These increases will likely include increased costs related to the hiring of additional personnel and fees to outside consultants, lawyers and accountants, among other expenses. Additionally, we anticipate increased costs associated with maintaining compliance with NASDAQ listing rules and SEC requirements, insurance and investor relations costs. In addition, if avatrombopag receives regulatory approval, we expect to incur expenses associated with building a sales and marketing team. However, we do not expect to receive any such regulatory approval until at least 2018, if at all.

Other expense, net

Other expense, net consists of interest expense related to the Eisai note and interest income on our cash and cash equivalents.

Results of operations for the three months ended September 30, 2017 and 2016

The following table sets forth our selected statements of operations data for the three months ended September 30, 2017 and 2016 (in thousands):

	Three months ended September 30,	
	2017	2016
Operating expenses:		
Research and development	$ 4,426	$ 6,758
Research and development - licenses acquired	1,000	—
General and administrative	4,185	368
Total operating expenses	9,611	7,126
Loss from operations	**(9,611)**	**(7,126)**
Other income (expenses)		
Other income (expense), net	224	(10)
Interest expense - related party	—	(3)
Interest expense	(336)	(35)
Total other expenses, net	(112)	(48)
Net loss	**$ (9,723)**	**$ (7,174)**

Operating expense

Research and development expense (including licenses acquired)

Research and development expenses, including licenses acquired, decreased by $1.4 million, from $6.8 million for the three months ended September 30, 2016 to $5.4 million for the three months ended September 30, 2017. For the three months ended September 30, 2017, research and development expenses included $3.2 million of expenses under the TSA, $0.3 million of consulting fees associated with the preparation and submission of the NDA and planning for additional clinical development of avatrombopag, $0.4 million of payroll-related expenses and $0.5 million of stock-based compensation expense, as well as a $1.0 million milestone payment that we became obligated to pay Astellas on September 21, 2017 upon the filing of the NDA. For the three months ended September 30, 2016, we recorded $6.8 million of research and development expenses under the TSA.

General and administrative expense

We hired our first employees and began paying compensation during the three months ended March 31, 2017. We granted our first equity awards to employees during the three months ended June 30, 2017. For the three months ended September 30, 2017, general and administrative expenses were $4.2 million, and were primarily attributable to $1.7 million of stock-based compensation expenses,

$0.6 million of payroll-related expenses, $1.0 million of consulting fees, $0.3 million of recruiting fees, $0.3 million of office operations-related expenses and $0.2 million of fees under the services agreements with PBM Capital Group, LLC. For the three months ended September 30, 2016, we recorded $0.4 million of general and administrative expenses.

Other expense, net

Other expense, net for the three months ended September 30, 2017 consisted primarily of $0.3 million of interest expense related to the Eisai note and $0.2 million of income on our money market mutual funds. For the three months ended September 30, 2016, other expense, net was insignificant.

Results of Operations for the nine months ended September 30, 2017 and the period from March 24, 2016 (Inception) to September 30, 2016

The following table sets forth our selected statements of operations data for the nine months ended September 30, 2017 and for the period from March 24, 2016 (Inception) to September 30, 2016 (in thousands):

	Nine months ended September 30, 2017	Period from March 24, 2016 (inception) to September 30, 2016
Operating expenses:		
Research and development	$ 11,995	$ 13,898
Research and development - licenses acquired	1,000	5,000
General and administrative	7,045	643
Total operating expenses	20,040	19,541
Loss from operations	**(20,040)**	**(19,541)**
Other income (expenses)		
Other income (expense), net	243	(10)
Interest expense - related party	—	(4)
Interest expense	(857)	(35)
Total other expenses, net	(614)	(49)
Net loss	**$ (20,654)**	**$ (19,590)**

Operating expense

Research and development expense (including licenses acquired)

Research and development expenses, including licenses acquired, decreased by $5.9 million, from $18.9 million for the period from March 24, 2016 to September 30, 2016 to $13.0 million for the nine months ended September 30, 2017. For the nine months ended September 30, 2017, research and development expenses included $9.8 million of expenses under the TSA, $0.9 million of consulting fees, $0.7 million of payroll-related expenses and $0.6 million of stock-based compensation expense, as well as a $1.0 million milestone payment that we became obligated to pay Astellas on September 21, 2017 upon the filing of the NDA. For the period from March 24, 2016 to September 30, 2016, we recorded $18.9 million of research and development expenses, including $13.7 million of expenses under the TSA and a $5.0 million upfront payment made to Eisai in connection with the Eisai stock purchase agreement.

General and administrative expense

We hired our first employees and began paying compensation during the nine months ended September 30, 2017. We granted our first equity awards to employees during the nine months ended September 30, 2017. For the nine months ended September 30, 2017, general and administrative expenses were $7.0 million, and were primarily attributable to $2.2 million of stock-based compensation expense, $1.2 million of payroll-related expenses, $1.8 million of consulting fees, $0.6 million of employee recruiting expenses, $0.6 million of office operations-related expenses, $0.2 million of travel expenses and $0.5 million of fees under the services agreements with PBM

Capital Group, LLC. For the period from March 24, 2016 (Inception) to September 30, 2016, we recorded $0.6 million of general and administrative expenses.

Other expense, net

Other expense, net for the nine months ended September 30, 2017 consisted primarily of $0.9 million of interest expense related to the Eisai note and $0.3 million of interest income on our money market mutual funds. For the period from March 24, 2016 to September 30, 2016, other expense, net was insignificant.

Liquidity and capital resources

Since our inception, we have not generated any revenue and have incurred net losses and negative cash flows from our operations. We have funded our operations primarily through the capital contributions from PBM Capital, sale of common stock in connection with our IPO, sale of Series A preferred stock and financing payments due to Eisai under the TSA through incurrence of debt under the Eisai note, which had a principal amount outstanding of $27.1 million and $13.6 million as of September 30, 2017 and December 31, 2016, respectively. On July 5, 2017, we closed our IPO, which resulted in the issuance and sale of 5,077,250 shares of our common stock at a public offering price of $17.00 per share, resulting in net proceeds of $78.7 million after deducting underwriting discounts and other offering costs. Upon the closing of our IPO, all outstanding shares of our Series A convertible preferred stock were automatically converted into 3,242,950 shares of common stock. As of September 30, 2017, we had $100.4 million in cash and cash equivalents.

The following table shows a summary of our cash flows for each of the periods shown below (in thousands):

	Nine months ended September 30, 2017	Period from March 24, 2016 (inception) to September 30, 2016
Cash and cash equivalents at the beginning of the period	$ 28,709	$ —
Net cash used in operating activities	(6,258)	(660)
Net cash used in investing activities	(35)	—
Net cash provided by financing activities	77,998	10,696
Cash and cash equivalents at the end of the period	**$ 100,414**	**$ 10,036**

Operating activities

Operating activities used $6.3 million of cash during the nine months ended September 30, 2017, primarily for consulting fees, payroll related expenses, office operational expenses, recruiting, professional and legal fees, travel and expenses under the services agreements with PBM Capital Group, LLC.

Operating activities use of cash in the period from March 24, 2016 (inception) to September 30, 2016 consisted primarily of $0.3 million of consulting fees and $0.3 million paid to PBM Capital Group, LLC under the SA.

Investing activities

Investing activities during the nine months ended September 30, 2017 and for the period from March 24, 2016 to September 30, 2016 were insignificant.

Financing activities

Financing activities provided $78.0 million of cash during the nine months ended September 30, 2017, consisting of the net proceeds of $78.7 million received upon the closing of our IPO on July 5, 2017 and payment of $0.7 million of offering costs for our preferred stock sold in 2016.

Financing activities provided $10.7 million of cash in the period from March 24, 2016 (Inception) to September 30, 2016, primarily from a capital contribution of $0.7 million from PBM Capital Investments, LLC and $10.0 million of net proceeds received from the issuance of Series A preferred stock.

Funding requirements

We expect our expenses to increase in connection with our ongoing activities, particularly as we seek approval of avatrombopag for its initial indication and continue the research and development of, and initiate clinical trials and seek marketing approval for, avatrombopag in other indications. In addition, if we obtain marketing approval for avatrombopag or any other drug candidates, we expect to incur significant commercialization expenses related to program sales, marketing, manufacturing and distribution to the extent that such sales, marketing and distribution are not the responsibility of potential collaborators. Furthermore, we will also incur costs as a newly public company that we have not previously incurred or have previously incurred at lower rates as a private company. Accordingly, we will likely need to obtain additional funding. If we are unable to raise capital or otherwise obtain funding when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or future commercialization efforts.

We expect our existing cash and cash equivalents, including the net proceeds from our IPO, will enable us to fund our operating expenses and capital expenditure requirements for at least the next 12 months as well as to pay off the Eisai note. We have based this estimate on assumptions that may prove to be wrong, and we could use our capital resources sooner than we currently expect.

Our future capital and operating expenditure requirements will depend on many factors, including:

- the scope, progress, results and costs of clinical trials;
- the scope, prioritization and number of our research and development programs;
- the costs, timing and outcome of regulatory review of our drug candidates;
- our ability to establish and maintain collaborations on favorable terms, if at all;
- the extent to which we are obligated to reimburse, or entitled to reimbursement of, clinical trial costs under collaboration agreements, if any;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the costs of retaining key research and development, sales and marketing personnel;
- the costs of building out internal accounting, legal, compliance and other operational and administrative functions, including after any expiration or termination of the TSA or management services agreement;
- the timing and size of any milestone payments required under our existing or future arrangements;
- the extent to which we acquire or in-license other drug candidates and technologies; and
- the costs of establishing or contracting for sales and marketing capabilities if we obtain regulatory approvals to market our drug candidates.

Identifying potential drug candidates and conducting preclinical studies and clinical trials is a time-consuming, expensive and uncertain process that takes many years to complete, and we may never generate the necessary data or results required to obtain marketing approval of and achieve sales of avatrombopag or other drug candidates. In addition, avatrombopag or any other drug candidates, if approved, may not achieve commercial success or may be limited in approved indications. Our commercial revenues, if any, will initially be derived from sales of avatrombopag, which we do not expect to be commercially available until at least 2018, if at all. In any event, we do not expect to achieve significant revenue from drug sales prior to the use of the net proceeds from our IPO. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

We will seek to obtain additional capital through the sale of debt or equity financings or other arrangements such as, collaborations, strategic alliances and licensing arrangements to fund operations; however, there can be no assurance that we will be able to raise needed capital under acceptable terms, if at all. The sale of additional equity may dilute existing stockholders and newly issued shares may contain senior rights and preferences compared to currently outstanding shares of common stock. Debt securities issued or other debt financing incurred may contain covenants and limit our ability to pay dividends or make other distributions to stockholders. If we are unable to obtain such additional financing, future operations would need to be scaled back or discontinued.

Contractual Obligations and Commitments

As of September 30, 2017, the only material change in our contractual obligations and commitments from those disclosed in the final prospectus for our IPO dated as of June 28, 2017 and filed with the SEC on June 30, 2017 pursuant to Rule 424(b)(4) was the entry into a sublease agreement in June 2017 and the amendment of this sublease agreement in September 2017, effective October 1, 2017, pursuant to which we lease office space in Durham, North Carolina. The sublease agreement, as amended, requires future rental payments of $38,000 during the final three months of the year ending December 31, 2017, and payments of $0.3 million, $0.3 million and $0.1 million during the years ending December 31, 2018, 2019 and 2020, respectively.

Off-balance sheet arrangements

We do not have any relationships with unconsolidated entities or financial partnerships, including entities sometimes referred to as structured finance or special purpose entities that were established for the purpose of facilitating off-balance sheet arrangements or other contractually narrow or limited purposes. We do not engage in off-balance sheet financing arrangements. In addition, we do not engage in trading activities involving non-exchange traded contracts. We therefore believe that we are not materially exposed to any financing, liquidity, market or credit risk that could arise if we had engaged in these relationships.

Critical accounting policies and significant judgments and estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States of America, or U.S. GAAP. The preparation of these financial statements requires us to make estimates, judgments and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent assets and liabilities as of the dates of the balance sheets and the reported amounts of expenses during the reporting periods. In accordance with U.S GAAP, we evaluate our estimates and judgments on an ongoing basis.

Significant estimates include assumptions used in the determination of share-based compensation and some of our research and development expenses. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

Other than the following critical accounting policy on stock based compensation, there were no other material changes to our critical accounting policies which are disclosed in our audited financial statements for the year ended December 31, 2016 included in our final prospectus dated June 28, 2017, and filed with the SEC on June 30, 2017 pursuant to Rule 424(b)(4).

Stock-based compensation

We expense stock-based compensation to employees and board members over the requisite service period based on the estimated grant-date fair value of the awards. Stock-based awards with graded-vesting schedules are recognized on a straight-line basis over the requisite service period for each separately vesting portion of the award. We record the expense for stock-based compensation awards subject to performance-based milestone vesting over the remaining service period when we determine that achievement of the milestone is probable. We evaluate when the achievement of a performance-based milestone is probable based on the expected satisfaction of the performance conditions at each reporting date. We estimate the fair value of stock option grants using the Black-Scholes option pricing model, and the assumptions used in calculating the fair value of stock-based awards represent management's best estimates and involve inherent uncertainties and the application of management's judgment. All stock-based compensation costs are recorded in general and administrative or research and development costs in the statements of operations based upon the underlying individual's role.

Recent accounting pronouncements

See Note 2 to our unaudited condensed consolidated financial statements in Part I, Item 1 of this Quarterly Report on Form 10-Q for a description of recent accounting pronouncements.

JOBS Act transition period

In April 2012, the JOBS Act was enacted. Section 107 of the JOBS Act provides that an "emerging growth company" can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act for complying with new or revised accounting standards. Thus, an emerging growth company can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this extended transition period and, as a result, we will adopt new or revised accounting standards on the relevant dates on which adoption of such standards is required for other public companies.

We are in the process of evaluating the benefits of relying on other exemptions and reduced reporting requirements under the JOBS Act. Subject to certain conditions, as an emerging growth company, we may rely on certain of these exemptions, including without limitation, (i) not providing an auditor's attestation report on our system of internal controls over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act and (ii) not complying with any requirement that may be adopted by the Public Company Accounting Oversight Board. We will remain an emerging growth company until the earliest to occur of (1) the last day of the fiscal year (a) ending December 31, 2022, which is the end of the fiscal year following the fifth anniversary of the completion our IPO, (b) in which we have total annual gross revenues of at least $1.07 billion or (c) in which we are deemed to be a "large accelerated filer" under the rules of the U.S. Securities and Exchange Commission, which means the market value of our common stock that is held by non-affiliates exceeds $700 million as of the prior June 30th, and (2) the date on which we have issued more than $1.0 billion in non-convertible debt during

the prior three-year period.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments, including cash equivalents, are in the form of a money market fund.

Inflation generally affects us by increasing our cost of labor and clinical trial costs. We do not believe that inflation had a material effect on our business, financial condition or results of operations for the three or nine months ended September 30, 2017.

Item 4. Controls and Procedures

Disclosure Controls and Procedures

We maintain "disclosure controls and procedures," as such term is defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, that are designed to ensure that information required to be disclosed by us in reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in Securities and Exchange Commission rules and forms, and that such information is accumulated and communicated to our management, including our Chief Executive Officer and our Chief Financial Officer, to allow timely decisions regarding required disclosure.

The design of any disclosure controls and procedures also is based in part upon certain assumptions about the likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions.

With respect to the quarter ended September 30, 2017, under the supervision and with the participation of our management, we conducted an evaluation of the effectiveness of the design and operations of our disclosure controls and procedures. Based upon this evaluation, our Chief Executive Officer and Chief Financial Officer have concluded that our disclosure controls and procedures are effective.

Management does not expect that our internal control over financial reporting will prevent or detect all errors and all fraud. A control system, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control systems are met. Further, the design of a control system must reflect the fact that there are resource constraints, and the benefits of controls must be considered relative to their costs. Because of the inherent limitations in a cost-effective control system, no evaluation of internal control over financial reporting can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud, if any, have been or will be detected.

Changes in Internal Control over Financial Reporting:

There were no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) that occurred during the fiscal quarter ended September 30, 2017 which have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Part II. Other Information

Item 1. Legal Proceedings.

We are not involved in any litigation that we believe could have a material adverse effect on our financial position or results of operations. There is no action, suit, proceeding, inquiry or investigation before or by any court, public board, government agency, self-regulatory organization or body pending or, to the knowledge of our executive officers, threatened against or affecting our company or our officers or directors in their capacities as such.

Item 1A. Risk Factors

The following information sets forth risk factors that could cause our actual results to differ materially from those contained in forward-looking statements we have made in this Quarterly Report on Form 10-Q and those we may make from time to time. You should carefully consider the risks described below, in addition to the other information contained in this Quarterly Report on Form 10-Q and our other public filings. Our business, financial condition or results of operations could be harmed by any of these risks. The risks and uncertainties described below are not the only ones we face. Additional risks not presently known to us or other factors not perceived by us to present significant risks to our business at this time also may impair our business operations.

Risks related to our business, financial position and capital needs

We have a limited operating history and have never generated any revenues.

We are a pharmaceutical company with a limited operating history. We were formed in March 2016, and our operations to date have been limited to organizing and staffing our company, acquiring worldwide rights to our drug candidate avatrombopag, raising capital and overseeing the completion of Phase 3 clinical trials of avatrombopag. We have not yet demonstrated an ability to successfully obtain marketing approval or conduct sales and marketing activities necessary for successful commercialization of avatrombopag. Consequently, we have no meaningful operations upon which to evaluate our business, and predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing drugs.

We expect our financial condition and operating results to continue to fluctuate from quarter to quarter and year to year due to a variety of factors, many of which are beyond our control. In order to succeed, we will need to transition from a company with a research and development focus to a company capable of undertaking commercial activities. We may encounter unforeseen expenses, difficulties, complications and delays, and may not be successful in such a transition.

We have incurred significant losses since our inception. We expect to incur losses over the next several years and may never achieve or maintain profitability.

Since inception, we have incurred significant net losses. We incurred net losses of $27.2 million for the period from March 24, 2016 (Inception) through December 31, 2016 and net losses of $20.7 million for the nine months ended September 30, 2017. As of September 30, 2017, we had an accumulated deficit of $47.8 million. We expect to continue to incur substantial and increasing losses for the foreseeable future, which such losses may fluctuate significantly from quarter to quarter and year to year. We have no drugs approved for commercial sale and to date we have not generated any revenue from drug sales. Because of the numerous risks and uncertainties associated with the regulatory approval process and the commercial launch of a drug, if approved for marketing, it could be years before we generate revenue from the sale of avatrombopag, if at all. Even if avatrombopag is approved for commercial sale, we anticipate incurring significant costs associated with the commercial launch of this drug, including increased sales and marketing expenses and increased personnel costs. We also expect our research and development expenses to be significant in connection with our planned clinical trials and applications for regulatory approval for avatrombopag for other indications. In addition, as a public company in the United States, we expect to incur significant expenses. As a result, we expect to continue to incur significant and increasing operating losses and negative cash flows for the foreseeable future. Accordingly, we are unable to predict when, or if, we will be able to achieve profitability and, if so, whether we will be able to sustain it.

Our ability to generate revenue and achieve and maintain profitability depends on a number of factors, including:

- our ability to obtain regulatory approval for the marketing of avatrombopag for the treatment of thrombocytopenia in CLD patients scheduled to undergo a procedure;
- our ability to comply with ongoing regulatory review by the FDA, EMA or any comparable foreign regulatory authorities;
- whether any indication approved by regulatory authorities is narrower than we expect;
- our ability to launch commercial sales of avatrombopag, if approved for marketing, whether alone or in collaboration with others;
- our ability to establish sales and marketing capabilities for avatrombopag;
- the efficacy and safety of avatrombopag and potential advantages compared to alternative treatments, notwithstanding success in meeting or exceeding clinical trial endpoints;
- the size of the markets for approved indications in territories in which we receive regulatory approval, if any;
- our ability to set an acceptable price for avatrombopag and obtain coverage and adequate reimbursement from third-party payors;
- our ability to achieve broad market acceptance of avatrombopag in the medical community and with third-party payors and consumers;
- the degree of competition we face from competitive therapies;
- our ability to maintain a supply arrangement that provides for commercial quantities of avatrombopag manufactured at acceptable cost levels and quality standards;
- our ability to successfully conduct additional clinical trials and achieve regulatory approval of avatrombopag for the treatment of thrombocytopenia beyond its initial indication;
- our ability to add operational, financial and management information systems and personnel, including personnel to support our clinical, manufacturing and planned future commercialization efforts and operations as a public company;
- our ability to continue to build out and retain an experienced management and advisory team;

- our ability to maintain, expand and protect our intellectual property portfolio, including any licensing arrangements with respect to our intellectual property; and
- our ability to avoid and defend against third-party infringement and other intellectual property related claims.

Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Failure to become and remain profitable may adversely affect the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue operations.

We are heavily dependent on the success of avatrombopag, our only drug candidate, and if avatrombopag does not receive regulatory approval or is not successfully commercialized, our business will be harmed.

We currently have no drugs that are approved for commercial sale and may never be able to develop marketable drugs. We expect that a substantial portion of our efforts and expenditures over the next few years will be devoted to avatrombopag, which is currently our only drug candidate. Accordingly, our business currently depends heavily on the successful development, regulatory approval and commercialization of avatrombopag. We cannot be certain that avatrombopag will receive regulatory approval or be successfully commercialized even if we receive regulatory approval. Moreover, we may not be successful in our efforts to expand the approval, if any, of avatrombopag for other indications. If we were required to discontinue development of avatrombopag for any indication or if avatrombopag does not receive regulatory approval or fails to achieve significant market acceptance, we would be delayed by many years in our ability to achieve profitability, if ever.

The research, testing, manufacturing, labeling, approval, sale, marketing and distribution of drugs are and will remain subject to extensive regulation by the FDA and other regulatory authorities in the United States and other countries that each have differing regulations. We are not permitted to market avatrombopag in the United States until it receives approval of an NDA from the FDA, or in any foreign countries until it receives the requisite approval from the regulatory authorities in such countries. While an NDA was submitted to the FDA in September 2017, we have not received approval of the NDA or submitted comparable applications to the EMA or other regulatory authorities. Obtaining approval of an NDA is an extensive, lengthy, expensive and inherently uncertain process. In addition, approval policies, regulations, or the type and amount of clinical data necessary to gain approval may change during the course of a drug candidate's clinical development and may vary among jurisdictions. The FDA, EMA or any comparable foreign regulatory authorities may delay, limit or deny approval of avatrombopag for many reasons, including:

- we may not be able to demonstrate that avatrombopag is safe and effective as a treatment for our targeted indications to the satisfaction of the FDA;
- the FDA, EMA or comparable foreign regulatory authorities may require additional Phase 3 clinical trials or non-clinical studies of avatrombopag, either before approval or as a post-approval commitment, which would increase our costs and prolong our development of avatrombopag;
- the results of our clinical trials may not meet the level of statistical or clinical significance required by the FDA, EMA or comparable foreign regulatory authorities for marketing approval;
- the FDA, EMA or comparable foreign regulatory authorities may disagree with the number, design, size, conduct or implementation of our clinical trials, especially in light of the fact that we deviated from the special protocol assessment, or SPA, under which the Phase 3 clinical trials were initially designed;
- the population studied in the clinical program may not be sufficiently broad or representative to assure safety in the full population for which we seek approval;
- the contract research organizations, or CROs, that we retain to conduct clinical trials may take actions outside of our control that materially adversely impact our clinical trials;
- the FDA, EMA or comparable foreign regulatory authorities may not find the data from preclinical studies and clinical trials sufficient to demonstrate that the clinical and other benefits of avatrombopag outweigh its safety risks;
- the FDA, EMA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies and clinical trials;
- the FDA, EMA or comparable foreign regulatory authorities may not accept data generated at clinical trial sites;
- if our NDA is reviewed by an advisory committee, the FDA may have difficulties scheduling an advisory committee meeting in a timely manner or the advisory committee may recommend against approval of our application or may recommend that the FDA require, as a condition of approval, additional preclinical studies or clinical trials, limitations on approved labeling or distribution and use restrictions;
- the FDA, EMA or comparable foreign regulatory authorities may require development of a risk evaluation and mitigation strategy, or REMS, as a condition of approval;
- the FDA, EMA or comparable foreign regulatory authorities may identify deficiencies in the manufacturing processes or facilities of our third-party manufacturers, including non-compliance with current Good Manufacturing Practices, or cGMPs; or
- the FDA, EMA or comparable foreign regulatory authorities may change their respective approval policies or adopt new regulations.

This lengthy approval process, as well as the unpredictability of the results of future clinical trials, may result in our failing to obtain regulatory approval to market avatrombopag, which would significantly harm our business, results of operations, and prospects.

We may require additional capital to fund our operations, and if we fail to obtain necessary financing, we may not be able to complete the development and commercialization of avatrombopag and other drug candidates.

As of September 30, 2017, we had $100.4 million in cash and cash equivalents. We expect to spend substantial amounts to complete the development of, seek regulatory approvals for and commercialize avatrombopag. Based upon our current operating plan, we believe that our existing resources, including the net proceeds from our recent initial public offering, or IPO, will enable us to fund our operating expenses and capital expenditure requirements for at least the next 12 months. This estimate is based on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we currently expect. Because the length of time and activities associated with successful development of avatrombopag is highly uncertain, we are unable to estimate the actual funds we will require for development and any approved marketing and commercialization activities.

Our future funding requirements, both near and long-term, will depend on many factors, including:

- the outcome, timing and cost of meeting regulatory requirements established by the FDA, EMA and other comparable foreign regulatory authorities;
- the initiation, progress, timing, costs and results of our planned clinical trials of avatrombopag for other indications;
- the cost of filing, prosecuting, defending, maintaining and enforcing our patent claims and other intellectual property rights;
- the cost of defending potential intellectual property disputes, including patent infringement actions brought by third parties against us for avatrombopag or any future drug candidates;
- the effect of competing technological and market developments;
- the cost and timing of establishing commercial scale manufacturing supply;
- milestone and other payments required under our agreements with Eisai, Inc., or Eisai, Astellas Pharma, Inc., or Astellas, and other collaborators and third parties;
- the cost of maintaining licensing and other arrangements with third parties, including Astellas;
- the cost of hiring additional personnel;
- the cost of operating as a public company in the United States;
- the cost of establishing sales, marketing and distribution capabilities for avatrombopag in regions where we choose to commercialize our drugs on our own; and
- the initiation, progress, timing and results of our commercialization of avatrombopag, if approved for commercial sale.

We may require additional capital to complete the potential commercialization of avatrombopag for the treatment of thrombocytopenia in CLD patients scheduled to undergo a procedure, complete the development of avatrombopag for other potential indications and execute our strategic plans by pursuing additional drug candidates for diseases treated by specialist physicians. If we were to raise additional capital through the issuance of equity or convertible securities, your ownership interest would be diluted, and the terms of these equity securities could include liquidation or other preferences that adversely affect your rights as a holder of our common stock. Debt financing, if available, could result in increased fixed payment obligations and we may be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. Our issuance of additional securities, whether equity or debt, or the possibility of such issuance, may cause the market price of our shares to decline. We could also be required to seek funds through arrangements with collaborators or others at an earlier stage than otherwise would be desirable and we may be required to relinquish rights to some of our technologies or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, operating results and prospects.

We cannot be certain that additional funding will be available on acceptable terms, or at all. If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of avatrombopag for any indication or potentially discontinue operations altogether. In addition, attempting to secure additional financing may divert the time and attention of our management from day-to-day activities, which may adversely affect our ability to develop and commercialize avatrombopag for any indication or any other future drug candidates.

We are required to make significant payments in connection with our acquisition of avatrombopag from Eisai and our failure to make these payments may adversely affect our ability to progress our development programs.

In March 2016, we acquired rights to avatrombopag from Eisai pursuant to a stock purchase agreement, or the Eisai stock purchase agreement. Under the Eisai stock purchase agreement, we are subject to significant obligations, including milestone payments of up to $135.0 million in the aggregate based on annual net sales of avatrombopag, as well as other material obligations. If we fail to make any required milestone payment when due, or if we elect to discontinue developing or commercializing the avatrombopag program, our

rights to avatrombopag, including associated intellectual property rights and regulatory rights, may revert to Eisai. In addition, in connection with our acquisition of the rights to avatrombopag, we entered into a transition services agreement with Eisai, or the TSA, pursuant to which we are obligated to pay Eisai for services provided by Eisai and for the reimbursement of certain out-of-pocket expenses. We also issued a secured promissory note to Eisai, or the Eisai note, which enables us to finance payments due to Eisai under the TSA. The Eisai note bears interest at a rate of 5% per annum and is secured by a blanket security interest on all of the assets of our wholly-owned subsidiary, AkaRx, Inc., or AkaRx, including the worldwide rights to avatrombopag. If we do not comply with our obligations under the Eisai stock purchase agreement, the TSA or the Eisai note as required, we could lose developmental and operational support from our counterparties and lose our rights to avatrombopag, which would materially and adversely affect our drug development efforts and our future financial performance.

We rely on our license agreement with Astellas to provide rights to the core intellectual property relating to avatrombopag. Any termination or loss of rights under that license agreement would have a material adverse effect on our development and commercialization of avatrombopag.

We are heavily reliant upon a license to certain core patent rights and other intellectual property necessary to the development of avatrombopag. In connection with our acquisition of the rights to avatrombopag from Eisai, we acquired an exclusive, worldwide license to the primary patents and other intellectual property related to avatrombopag from Astellas. Unless earlier terminated, our license agreement with Astellas will expire on a country-by-country and product-by-product basis upon the latest of (i) the expiration of the last-to-expire claim of the licensed patents, (ii) the expiration of any government-granted marketing exclusivity period for avatrombopag and (iii) 10 years after the last date of launch of avatrombopag to have occurred in any country. Thereafter, the term of the license agreement may be extended for successive one-year terms if we notify Astellas in writing of our desire to extend such term at least three months before it is otherwise set to expire.

Under our license agreement with Astellas, we are obligated to use commercially reasonable efforts to conduct development activities and obtain regulatory approval of avatrombopag, and pay to Astellas regulatory milestone payments and tiered royalties in the mid to high single-digit percentages in connection with the net sales of avatrombopag. If these payments become due under the terms of the license agreement, we may not have sufficient funds available to meet our obligations, which would allow Astellas to terminate the license agreement.

Additionally, if there is any conflict, dispute, disagreement or claim of non-performance between us and Astellas regarding our rights or obligations under the license agreement, including any conflict, dispute or disagreement or claim arising from our failure to satisfy our payment obligations, Astellas may have a right to terminate the license agreement. Upon termination of the license agreement by Astellas, we would be required to promptly take certain actions, including ceasing use of the licensed patents and other intellectual property, returning to Astellas or its designee or destroying proprietary information and material supplied by Astellas under the license agreement, ceasing the use and sale of avatrombopag, and granting to Astellas an exclusive license to use the trademark owned or controlled by us for avatrombopag in any countries for which Astellas has elected to terminate the license for the purpose of commercializing avatrombopag. Any termination or loss of rights under our license agreement with Astellas would materially and adversely affect our ability to develop and commercialize avatrombopag, which in turn would have a material adverse effect on our business, operating results and prospects.

We currently have a limited number of employees, and we rely on Eisai and PBM Capital Group, LLC to provide various administrative, research and development and other services.

As of September 30, 2017, we had 18 employees. We rely on the support and research and development services provided by Eisai pursuant to the TSA. We also rely on the support and administrative services provided by PBM Capital Group, LLC, which is an affiliate of our controlling stockholder, PBM Capital Investments, LLC, pursuant to our agreements with PBM Capital Group, LLC. We do not expect personnel and support staff that provide services to us under these services agreements will have as their primary responsibility the management and administration of our business or act exclusively for us. As a result, such individuals will not allocate all of their time and resources to us.

If Eisai or PBM Capital Group, LLC fail to perform their obligations in accordance with the terms of the services agreements, it could be difficult for us to operate our business, including compliance with the terms and requirements of our license agreement with Astellas. Any failure by Eisai or PBM Capital Group, LLC to effectively manage administrative, research and development or other services that they provide to us could harm our business, financial condition and results of operations. In addition, the termination of our relationships with Eisai or PBM Capital Group, LLC and any delay in appointing or finding a suitable replacement provider (if one exists) could make it difficult for us to operate our business.

Additionally, over time we will need to transition from receiving the services that Eisai and PBM Capital Group, LLC are currently providing to performing such activities internally. The TSA is scheduled to expire on March 31, 2018 and, unless the TSA is amended,

Eisai will not be obligated to perform any further services under the TSA after that date. In addition, PBM Capital Group, LLC has the right to terminate its services agreements with us and AkaRx at any time, with or without notice. If we do not have adequate financial resources or personnel and systems in place at the time that we assume responsibilities for such services, we may not be successful in effectively or efficiently transitioning these services from Eisai and PBM Capital Group, LLC, which could disrupt our business and have a material adverse effect on our financial condition and results of operations. Even if we are able to successfully transition these services, they may be more expensive or less efficient than the services we are receiving from Eisai and PBM Capital Group, LLC during the transition period.

We will need to expand our organization, and we may experience difficulties in managing this growth, which could disrupt our operations.

As of September 30, 2017, we had 18 employees and were reliant on services provided to us by PBM Capital Group, LLC and Eisai under the services agreements and TSA, respectively. We expect to hire additional employees for our clinical, scientific, medical, regulatory, operational, human resources, finance, administrative and sales and marketing teams. We may have operational difficulties in connection with identifying, hiring and integrating new personnel. Future growth would impose significant additional responsibilities on our management, including the need to identify, recruit, maintain, motivate and integrate additional employees, consultants and contractors. Also, our management may need to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, and give rise to operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development and commercialization of avatrombopag. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate or grow revenues could be reduced, and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize avatrombopag and compete effectively will depend, in part, on our ability to effectively manage any future growth.

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

We are highly dependent on the management, development, clinical, financial and business development expertise of Alex Sapir, our Chief Executive Officer, Douglas Blankenship, our Chief Financial Officer, Lee F. Allen, our Chief Medical Officer, and Kevin Laliberte, our Senior Vice President, Product Development, as well as the other members of our scientific and clinical teams. Each of these executive officers may currently terminate their employment with us at any time. We do not maintain “key person” insurance for any of our executives.

Recruiting and retaining qualified scientific and clinical personnel and manufacturing and sales and marketing personnel, will also be critical to our success. The loss of the services of our executive officers or other key employees could impede the achievement of our development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, 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 successfully develop, gain regulatory approval of and commercialize drugs. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. Many of the other pharmaceutical companies that we compete against for qualified personnel and consultants 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 and consultants than what we may offer. We may also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. This competition may be particularly intense in North Carolina, where we intend to operate our company.

We also expect to rely upon consultants for assistance in developing our clinical, regulatory and commercialization strategy. These consultants may also be engaged by third parties and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high-quality personnel and consultants, the rate at which we can successfully develop avatrombopag and grow our business will be limited.

Our employees, independent contractors, consultants, commercial collaborators, principal investigators, CROs and vendors may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements.

We are exposed to the risk that our employees, independent contractors, consultants, commercial collaborators, principal investigators, CROs, contract manufacturers and vendors may engage in fraudulent conduct or other illegal activity. Misconduct by these parties could

include intentional, reckless and/or negligent conduct or disclosure of unauthorized activities to us that violates FDA regulations, including those laws requiring the reporting of true, complete and accurate information to the FDA, manufacturing standards, federal and state healthcare laws and regulations, and laws that require the true, complete and accurate reporting of financial information or data. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Misconduct by these parties could also involve the improper use of individually identifiable information, including, without limitation, information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant civil, criminal and administrative penalties, including, without limitation, damages, fines, disgorgement, individual imprisonment, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, additional regulatory oversight and reporting requirements, and the curtailment or restructuring of our operations.

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

We face an inherent risk of product liability and personal injury exposure related to the testing of drug candidates in human clinical trials and will face an even greater risk if we commercially sell avatrombopag and any other drugs that we may develop. If we cannot successfully defend ourselves against claims that avatrombopag caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any drug candidates or drugs that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend the related litigation;
- substantial monetary awards paid to trial participants or patients;
- loss of revenue;
- reduced resources of our management to pursue our business strategy; and
- the inability to commercialize any drugs that we may develop.

We currently maintain $20 million in product liability insurance coverage in the aggregate, with a per incident limit of $20 million, which may not be adequate to cover all liabilities that we may incur. We may need to increase our insurance coverage as we expand our clinical trials or if we commence commercialization of avatrombopag. Because insurance coverage is increasingly expensive, we may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

Our business and operations would suffer in the event of computer system failures, cyber-attacks or a deficiency in our cyber-security.

Despite the implementation of security measures, our internal computer systems, and those of third parties on which we rely, are vulnerable to damage from computer viruses, malware, natural disasters, terrorism, war, telecommunication and electrical failures, cyber-attacks or cyber-intrusions over the Internet, attachments to emails, persons inside our organization, or persons with access to systems inside our organization. The risk of a security breach or disruption, particularly through cyber-attacks or cyber intrusion, including by computer hackers, foreign governments, and cyber terrorists, has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. If such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs. For example, the loss of clinical trial data from completed or 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 was to result in a loss of or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur material legal claims and liability, damage to our reputation, and the further development of avatrombopag or any future drug candidate could be delayed.

Risks related to clinical development, regulatory approval and commercialization

If we are not able to obtain required regulatory approvals, we will not be able to commercialize avatrombopag, and our ability to generate revenue will be materially impaired.

Avatrombopag and the activities associated with its development and commercialization, including its design, research, testing, manufacture, safety, efficacy, recordkeeping, labeling, packaging, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by the EMA and similar regulatory authorities outside the United States. Failure to obtain marketing approval for avatrombopag will prevent us from commercializing it.

We have not received approval from regulatory authorities to market any drug candidate in any jurisdiction, and it is possible that neither avatrombopag nor any drug candidates we may seek to develop in the future will ever obtain the appropriate regulatory approvals necessary for us to commence drug sales.

We expect to rely on Eisai and third-party consultants to assist us in filing and supporting the applications necessary to gain marketing approvals. Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish avatrombopag's safety and efficacy for that indication. Securing marketing approval also requires the submission of information about the drug manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities. If we cannot successfully obtain approval of or commercialize avatrombopag, our business may not succeed.

Clinical failure may occur at any stage of clinical development, and the results of our clinical trials may not support our proposed indications for avatrombopag.

We cannot be certain that existing clinical trial results will be sufficient to support regulatory approval of avatrombopag for the treatment of thrombocytopenia in CLD patients scheduled to undergo a procedure, or that future clinical trial results will support the effectiveness of avatrombopag in other indications. Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful, and we cannot be sure that the results of later clinical trials will replicate the results of prior clinical trials and preclinical testing. Moreover, success in clinical trials in a particular indication, such as thrombocytopenia in CLD patients scheduled to undergo a procedure, does not ensure that a drug candidate will be successful in other thrombocytopenia indications. A number of companies in the pharmaceutical industry, including biotechnology companies, have suffered significant setbacks in clinical trials, even after promising results in earlier preclinical studies or clinical trials or successful later-stage trials in other related indications. These setbacks have been caused by, among other things, preclinical findings made while clinical trials were underway and safety or efficacy observations made in clinical trials, including previously unreported adverse events. The results of preclinical and early clinical trials of our drug candidates may not be predictive of the results of later-stage clinical trials. Drug candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through preclinical and initial clinical trials. A failure of a clinical trial to meet its predetermined endpoints would likely cause us to abandon a drug candidate and may delay development of any other drug candidates. Any delay in, or termination of, our clinical trials in other indications will delay the submission of the NDA to the FDA for such indications, the marketing authorization application to the EMA or other similar applications with other relevant foreign regulatory authorities and, ultimately, our ability to commercialize avatrombopag and generate revenue.

Enrollment and retention of patients in clinical trials is an expensive and time-consuming process and could be made more difficult or rendered impossible by multiple factors outside our control.

We may encounter delays in enrolling, or be unable to enroll, a sufficient number of patients to complete any of our clinical trials, and even once enrolled, we may be unable to retain a sufficient number of patients to complete any of our trials. Patient enrollment and retention in clinical trials depends on many factors, including the size of the patient population, the nature of the trial protocol, the existing body of safety and efficacy data with respect to the study drug, the number and nature of competing treatments and ongoing clinical trials of competing drugs for the same indication, the proximity of patients to clinical sites and the eligibility criteria for the study. For example, Eisai previously discontinued a Phase 3 clinical trial evaluating avatrombopag for the treatment of immune thrombocytopenic purpura, or ITP, due to enrollment difficulties. Furthermore, any negative results we may report in clinical trials of our drug candidate may make it difficult or impossible to recruit and retain patients in other clinical trials of that same drug candidate. Other TPO-RAs, such as Promacta, have terminated clinical trials in CLD patients due to safety issues, including the incidence of portal vein thrombosis, or PVT, which is the blockage or narrowing of the vein carrying blood to the liver that can result in stroke or death. PVT can be caused by raising platelet counts above 200,000 platelets per microliter of circulating blood in CLD patients. The perception that such incidents may occur from avatrombopag due to the drug candidate having a similar mechanism of action as other TPO-RAs could adversely affect enrollment of clinical trials for avatrombopag. Delays or failures in planned patient enrollment or retention may result in increased costs, program delays or both, which could have a harmful effect on our ability to develop avatrombopag, or could render further development impossible. In addition, we expect to rely on CROs and clinical trial sites to ensure proper and timely conduct of our future clinical trials and, while we intend to enter into agreements governing their services, we will be limited in our ability to compel their actual performance.

Even if avatrombopag receives marketing approval, it may fail to achieve market acceptance by physicians, patients, third-party payors or others in the medical community necessary for commercial success.

If avatrombopag receives marketing approval, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. If it does not achieve an adequate level of acceptance, we may not generate significant revenues and become profitable. The degree of market acceptance of avatrombopag, if approved for commercial sale, will depend on a number of factors, including but not limited to:

- final labeling approved by regulatory authorities;
- the clinical efficacy and potential advantages compared to alternative treatments, notwithstanding success in meeting or exceeding clinical trial endpoints;
- the cost of treatment in relation to alternative treatments, including any similar generic treatments;
- our ability to offer our drugs for sale at competitive prices;
- the convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support;
- the availability of third-party coverage and adequate reimbursement;
- the prevalence and severity of any side effects, including PVT; and
- any restrictions on the use of avatrombopag together with other medications.

Market acceptance of avatrombopag may also be affected by the perception that TPO-RAs, because of their mechanism of action, are not safe for the acute treatment of thrombocytopenia due to the possible incidence of PVT. In addition, market acceptance may suffer if avatrombopag is perceived as having limited clinical efficacy beyond its success in meeting trial endpoints in CLD patients, including any perception by physicians that avatrombopag, although effective at increasing platelet count, may not be effective in reducing or controlling excessive bleeding in connection with a procedure.

In addition, the potential patient population for our initial indication is relatively small. This could affect the rate of adoption and as a result, market acceptance of our drug, if approved, could be much slower than anticipated.

Further, the benefits of avatrombopag compared to platelet transfusions in the acute setting may not be readily accepted by the medical community following regulatory approval, or at all, particularly if the perceived safety and efficacy risks and concerns relating to existing TPO-RAs, including incidence of PVTs, are attributed to avatrombopag. In the acute setting, platelet transfusions are the accepted standard of care to treat thrombocytopenia, and physicians may be hesitant to use a new therapy or treatment such as avatrombopag in lieu of transfusions, including due to physicians' relative familiarity with platelet transfusions and their safety and efficacy profile. For example, physicians may perceive platelet transfusions to be more effective or precise than TPO-RAs in increasing platelet counts prior to a procedure to a requisite threshold, which is subject to the discretion of the physician and thus may vary depending on the type and invasiveness of the specified procedure. Further, platelet transfusions, which are typically scheduled for the day of a procedure, may be preferred by certain physicians over TPO-RAs, including avatrombopag, to avoid the perceived inconvenience of needing to take scheduled oral doses of TPO-RA treatments in advance of the procedure. Because we expect sales of avatrombopag, if approved, to generate substantially all of our drug revenues for the foreseeable future, the failure of this drug to find market acceptance would harm our business and could require us to seek additional financing.

The market for our drug candidate may not be as large as we expect.

Our estimates of the potential market opportunity for avatrombopag include several key assumptions based on our industry knowledge, industry publications, third-party research reports and other surveys. These assumptions include the prevalence of CLD and other patients with thrombocytopenia undergoing a procedure and the number of patients with chemotherapy-induced thrombocytopenia. However, there can be no assurance that any of these assumptions are or will remain accurate. For example, physicians and surgeons exercise discretion about the requisite platelet count threshold before a procedure, notwithstanding platelet count thresholds recommended by medical professional associations, which are viewed as clinical guidelines rather than standards of care. As a result, the number of physicians that would determine that an increase in platelet count is necessary prior to a specific procedure, or that would prefer an advance treatment such as avatrombopag rather than prophylactic platelet transfusion on the same day as the procedure, may be smaller than we anticipate. Further, even if avatrombopag is approved for use in advance of highly invasive procedures, physicians may continue to prescribe platelet transfusions in advance of such procedures instead of other treatment regimens. In addition, our assumptions regarding the number of patients with thrombocytopenia that are treated in the chronic setting may be inaccurate, as physicians exercise discretion in determining when a patient with thrombocytopenia should receive chronic treatment. While we believe that our internal assumptions are reasonable, if any of these assumptions proves to be inaccurate, then the actual market for avatrombopag for any indication could be smaller than our estimates of our potential market opportunity. The degree of market acceptance by the medical community of avatrombopag following regulatory approval could also impact these assumptions and reduce

the market size for avatrombopag, including due to the factors described above in "—Even if avatrombopag receives marketing approval, it may fail to achieve market acceptance by physicians, patients, third-party payors or others in the medical community necessary for commercial success." If the actual market for avatrombopag is smaller than we expect, our drug revenue may be limited and it may be more difficult for us to achieve or maintain profitability.

In addition, the label for avatrombopag may include certain limitations on the patients and uses of avatrombopag. As a result, even if we attain market acceptance among physicians, health care payors, patients and the medical community for approved uses of avatrombopag, we may not be able to market or promote this drug candidate for all CLD patients with thrombocytopenia scheduled to undergo a procedure or for other patients with thrombocytopenia beyond the specifically approved indication.

Avatrombopag may cause adverse events or have other properties that could delay or prevent its regulatory approval or limit the scope of any approved label or market acceptance.

Adverse events caused by avatrombopag could cause us, other reviewing entities, clinical trial sites or regulatory authorities to interrupt, delay or halt clinical trials and could result in the denial of regulatory approval. For example, other TPO-RAs evaluated for the treatment of thrombocytopenia in CLD patients have had their development abandoned due to safety issues, including the incidence of PVT. In our clinical trials, adverse events related to treatment included fever, nausea and abdominal pain and one incident of PVT that was determined by the investigator to be possibly related to avatrombopag. If an unacceptable frequency or severity of adverse events are reported in our current or future clinical trials for avatrombopag, including PVTs, our ability to obtain regulatory approval for avatrombopag may be negatively impacted.

Furthermore, if any of our drugs are approved and then cause or are perceived to cause serious or unexpected side effects, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw their approval of the drug or require a REMS to impose restrictions on its distribution or other risk management measures;
- regulatory authorities may require the addition of labeling statements, such as warnings or contraindications;
- we may be required to change the way the drug is administered or to conduct additional clinical trials;
- market acceptance could be significantly hindered;
- we could be sued and held liable for harm caused to patients;
- we could elect to discontinue the sale of our drug; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the affected drug candidate, including avatrombopag, and could substantially increase the costs of commercialization.

We may expend our limited resources to pursue a particular indication and fail to capitalize on indications that may be more profitable or for which there is a greater likelihood of success.

Although our strategic plan is focused on drug candidates for diseases treated by specialist physicians, because we have limited financial and management resources, we are currently primarily focused on the development of avatrombopag for the treatment of thrombocytopenia in CLD patients scheduled to undergo a procedure. We are also planning to develop avatrombopag for patients with thrombocytopenia, regardless of etiology, prior to a procedure, regardless of the degree of invasiveness, as well as for patients with chemotherapy-induced thrombocytopenia. As a result, we may forego or delay pursuit of opportunities with other drug candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial drugs or profitable market opportunities. Our spending on current and future development programs for specific indications may not yield any commercially viable drugs. If we do not accurately evaluate the commercial potential or target market for a particular drug candidate, we may relinquish valuable rights to that drug candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such drug candidate.

If we are unable to establish sales, marketing and distribution capabilities either on our own or in collaboration with third-parties, we may not be successful in commercializing avatrombopag, if approved.

We do not have any infrastructure for the sales, marketing or distribution of our drugs, and the cost of establishing and maintaining such an organization may exceed the benefits of doing so. In order to market any drug that may be approved, we must build our sales, distribution, marketing, managerial and other non-technical capabilities or make arrangements with third parties to perform these services. To achieve commercial success for any drug for which we have obtained marketing approval, we will need a sales and

marketing organization.

We expect to build a hepatology-focused sales organization to market avatrombopag in the United States, if approved. There are significant expenses and risks involved with establishing our own sales and marketing capabilities, including our ability to hire, retain and appropriately incentivize qualified individuals, generate sufficient sales leads, provide adequate training to sales and marketing personnel, and effectively manage a geographically dispersed sales and marketing team. Any failure or delay in the development of our internal sales, marketing and distribution capabilities could delay any drug launch, which would adversely impact the commercialization of avatrombopag. For example, if the commercial launch of avatrombopag for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize our drugs on our own include:

- our inability to recruit, train and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to physicians or attain adequate numbers of physicians to prescribe any drugs; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

We may not have the resources in the foreseeable future to allocate to the sales and marketing of avatrombopag in certain markets overseas where we may seek regulatory approval. Therefore, our future success will depend, in part, on our ability to enter into and maintain collaborative relationships for such capabilities, the collaborator's strategic interest in the drug and such collaborator's ability to successfully market and sell the drug. We intend to pursue collaborative arrangements regarding the sale and marketing of avatrombopag, if approved, for certain markets overseas; however, we cannot assure you that we will be able to establish or maintain such collaborative arrangements, or if able to do so, that they will have effective sales forces. To the extent that we depend on third parties for marketing and distribution, any revenues we receive will depend upon the efforts of such third parties, and there can be no assurance that such efforts will be successful.

If we are unable to build our own sales force or negotiate a collaborative relationship for the commercialization of avatrombopag, we may be forced to delay the potential commercialization of avatrombopag or reduce the scope of our sales or marketing activities for avatrombopag. If we elect to increase our expenditures to fund commercialization activities ourselves, we may need to obtain additional capital, which may not be available to us on acceptable terms, or at all. If we are unable to establish adequate sales and marketing and distribution capabilities, either on our own or in collaboration with third parties, we will not be successful in commercializing avatrombopag and may not become profitable. We will be competing with many companies that currently have extensive and well-funded marketing and sales operations. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies, which would adversely affect our ability to commercialize avatrombopag and grow our company.

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

The development and commercialization of new drugs is highly competitive. If approved for marketing, we will face competition with respect to avatrombopag, and will face competition with respect to any drug candidates that we may seek to develop or commercialize in the future, from many different sources, including major pharmaceutical and specialty pharmaceutical companies, academic institutions and governmental agencies and public and private research institutions.

With respect to avatrombopag for the treatment of thrombocytopenia in patients with CLD scheduled to undergo a procedure, we will be primarily competing with platelet transfusions, since neither of the available TPO-RAs are approved by the FDA for this indication. However, we also anticipate some competition from TPO-RAs being used off-label. In addition, Shionogi is developing lusutrombopag for the treatment of thrombocytopenia in patients with CLD undergoing invasive surgical procedures, which has been approved in Japan and has recently completed one global Phase 3 clinical trial with approximately 200 patients.

With respect to avatrombopag for the treatment of ITP, we anticipate competing with the currently marketed TPO-RAs Promacta and Nplate. In addition, we are aware that Rigel Pharmaceuticals, Inc., argenx N.V., Bristol-Myers Squibb Company, Shire PLC, Immunomedics Inc., Protalex Inc. and others are developing drugs that may have utility for the treatment of ITP. We are also aware of several other drug candidates in earlier stages of development as potential treatments for the indications that we intend to target.

Certain of these therapies may be more competitive than avatrombopag due to their comparatively lower cost, their longer history in

clinical use and physicians' relative familiarity with their efficacy and safety profiles.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize drugs that are more effective, have fewer or less severe side effects, are more convenient or are less expensive than avatrombopag or any other drug that we may develop. Our competitors also may obtain FDA or other regulatory approval for their drugs more rapidly than we may obtain approval for our drug or with a label with fewer restrictions or a broader indication, which could result in our competitors establishing a strong market position before we are able to enter the market.

Many of the companies against which we are competing, or against which we may compete in the future, have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved drugs than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors would also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or that may be necessary for, our programs.

Risks related to our dependence on third parties

We do not have our own manufacturing capabilities and will rely on third parties to produce clinical and commercial supplies of avatrombopag and any future drug candidate.

We have limited experience in drug formulation or manufacturing and do not own or operate, and we do not expect to own or operate, facilities for drug manufacturing, storage, distribution, or testing. While avatrombopag was being developed by Eisai, it was also being manufactured by Eisai. We have also entered into a supply agreement with Eisai, pursuant to which we agreed to purchase finished drug product for avatrombopag from Eisai and Eisai agreed to supply finished drug product for avatrombopag to us. Pursuant to the supply agreement, Eisai is our exclusive supplier of finished drug product, except that we have the right to terminate the exclusivity early by payment to Eisai of a fee calculated based on our forecasted purchases of avatrombopag. In addition, in the event that Eisai fails to deliver substantially all of the finished drug product due to us under the agreement, we may elect to seek alternative supply arrangements so long as such failure remains uncured for a specified period of time, subject to certain exceptions. If Eisai is unable to supply us with sufficient commercial grade quantities of avatrombopag, and we are unable to timely establish an alternate supply from one or more third-party contract manufacturers, we could experience delays in our development efforts as we locate and qualify new manufacturers. Under such circumstances, we may be required to receive drug substance for use on a purchase order basis, and as such, there can be no assurance that we actually receive sufficient quantities.

Further, our reliance on third-party manufacturers exposes us to risks beyond our control, including the risk of:

- inability to meet our drug specifications and quality requirements consistently;
- delay or inability to procure or expand sufficient manufacturing capacity;
- manufacturing and drug quality issues, including related to scale-up of manufacturing;
- costs and validation of new equipment and facilities required for additional scale-up;
- failure to comply with cGMP and similar foreign standards;
- inability to negotiate manufacturing agreements with third parties under commercially reasonable terms;
- termination or nonrenewal of manufacturing agreements with third parties in a manner or at a time that is costly or damaging to us;
- reliance on a limited number of sources, and in some cases, single sources for drug components, such that if we are unable to secure a sufficient supply of these drug components, we will be unable to manufacture and sell avatrombopag or any future drug candidate in a timely fashion, in sufficient quantities or under acceptable terms;
- lack of qualified backup suppliers for those components that are currently purchased from a sole or single source supplier;
- operations of our third-party manufacturers or suppliers could be disrupted by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier or the issuance of a FDA Form 483 notice or warning letter;
- carrier disruptions or increased costs that are beyond our control; and
- failure to deliver our drugs under specified storage conditions and in a timely manner.

Some of these events could be the basis for FDA action, including injunction, recall, seizure, or total or partial suspension of production. In addition, our third-party manufacturers and suppliers are subject to FDA inspection from time to time. Failure by our third-party manufacturers and suppliers to pass such inspections and otherwise satisfactorily complete the FDA approval regimen with respect to

our drug candidate may result in regulatory actions such as the issuance of FDA Form 483 notices of observations, warning letters or injunctions or the loss of operating licenses. In addition, our third-party manufacturers and suppliers are subject to numerous environmental, health and safety laws and regulations, including those governing the handling, use, storage, treatment and disposal of waste products, and failure to comply with such laws and regulations could result in significant costs associated with civil or criminal fines and penalties for such third parties. Based on the severity of the regulatory action, our clinical or commercial supply of drug and packaging and other services could be interrupted or limited, which could have a material adverse effect on our business.

Any of these events could lead to clinical trial delays, failure to obtain regulatory approval or impact our ability to successfully commercialize avatrombopag or any future drug candidates.

We intend to rely on third parties to conduct, supervise and monitor our clinical trials, and if those third parties perform in an unsatisfactory manner, it may harm our business.

We intend to rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials, and we expect to have limited influence over their actual performance. Eisai is primarily responsible for managing these CROs and clinical trial sites in accordance with the terms of the TSA.

We intend to rely upon CROs to monitor and manage data for our clinical programs, as well as the execution of future nonclinical studies. We expect to control only certain aspects of our CROs' activities. Nevertheless, we will be responsible for ensuring that each of our clinical trials is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards and our reliance on the CROs does not relieve us of our regulatory responsibilities.

We and our CROs will be required to comply with the Good Laboratory Practices and Good Clinical Practices, or GCPs, which are regulations and guidelines enforced by the FDA and are also required by the Competent Authorities of the Member States of the European Economic Area and comparable foreign regulatory authorities in the form of International Conference on Harmonization guidelines for any of our drug candidates that are in clinical development. The Regulatory authorities enforce GCPs through periodic inspections of trial sponsors, principal investigators and clinical trial sites. If we or our CROs fail to comply with GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA, EMA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. Accordingly, if our CROs fail to comply with these regulations or fail to recruit a sufficient number of subjects, we may be required to repeat clinical trials, which would delay the regulatory approval process.

Our CROs will not be our employees, and we will have limited ability to influence whether or not they devote sufficient time and resources to our future clinical and nonclinical programs. These CROs may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials, or other drug development activities which could harm our competitive position. We face the risk of potential unauthorized disclosure or misappropriation of our intellectual property by CROs, which may reduce our trade secret protection and allow our potential competitors to access and exploit our proprietary technology. If our CROs do not successfully carry out their contractual duties or obligations, fail to meet expected deadlines, or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements or for any 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 any drug candidate that we develop. As a result, our financial results and the commercial prospects for any drug candidate that we develop would be harmed, our costs could increase, and our ability to generate revenues could be delayed.

If our or Eisai's relationship with these CROs terminate, we may not be able to enter into arrangements with alternative CROs or do so on commercially reasonable terms. Switching or adding additional CROs involves substantial cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines. Though we intend to carefully manage our relationships with our CROs, 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 an adverse impact on our business, financial condition and prospects.

We may seek collaborations with third parties for the development or commercialization of avatrombopag. If we are unable to enter into collaborations, or if those collaborations are not successful, we may not be able to capitalize on the market potential of avatrombopag.

We may seek third-party collaborators for the development and commercialization of avatrombopag, including if approved for marketing outside the United States. Our likely collaborators for any collaboration arrangements include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the

proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject drug candidate, the costs and complexities of manufacturing and delivering such drug candidate to patients, the potential of competing drugs, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge and industry and market conditions generally. The collaborator may also consider alternative drug candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our drug candidate. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of such drug candidate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our drug candidates or bring them to market and generate revenue.

If we do enter into any such arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our drug candidates. Our ability to generate revenue from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements.

Collaborations involving avatrombopag or any future drug candidate would pose the following risks to us:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;
- collaborators may not pursue development and commercialization of any drug candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a drug candidate, repeat or conduct new clinical trials or require a new formulation of a drug candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, drugs that compete directly or indirectly with our drug candidates if the collaborators believe that competitive drugs are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- drug candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own drug candidates or drugs, which may cause collaborators to cease to devote resources to the commercialization of drug candidates;
- a collaborator with marketing and distribution rights to one or more of our drug candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such drug candidate;
- disagreements with collaborators, including disagreements over intellectual property and other proprietary rights, contract interpretation or the preferred course of development, might cause delays or termination of the research, development or commercialization of drug candidates, might lead to additional responsibilities for us with respect to drug candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- collaborators may not properly prosecute, maintain or defend our or their intellectual property rights or may use our or their proprietary information in such a way as to invite litigation that could jeopardize or invalidate such intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe, misappropriate or otherwise violate the intellectual property rights of third parties, which may expose us to litigation and potential liability; and
- collaborations may be terminated for the convenience of the collaborator and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable drug candidates.

Collaboration agreements may not lead to development or commercialization of drug candidates in the most efficient manner or at all. If a present or future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our development or commercialization program could be delayed, diminished or terminated.

Risks related to our intellectual property

If we are unable to obtain and maintain patent protection for avatrombopag or any future drug candidate, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize technology and drugs similar or identical to ours, which could have a material adverse effect on our ability to successfully commercialize our technology and drug candidates.

Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to avatrombopag or any future drug candidate. We seek to protect our proprietary position by in-licensing intellectual property relating to avatrombopag, in particular pursuant to our licensing agreement with Astellas, and filing patent applications in the United States and abroad related to our technologies and drug candidates that are important to our business. If we or our licensors are unable to obtain or maintain patent protection with respect to avatrombopag and any future drug candidates we develop, our business, financial condition, results of operations, and prospects could be materially harmed.

The patent prosecution process is expensive and time-consuming, however, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our development output before it is too late to obtain patent protection. We may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the rights to patents licensed to third parties. Therefore, these patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States or vice versa. For example, European patent law restricts the patentability of methods of treatment of the human body more than United States law does. 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 know with certainty whether we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued that protect our technology or drugs, in whole or in part, or which effectively prevent others from commercializing competitive technologies and drugs. 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.

Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to United States patent law. These include provisions that affect the way patent applications are prosecuted and may also affect patent litigation. The United States Patent and Trademark Office, or USPTO, recently developed new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, only became effective on March 16, 2013. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation 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 and financial condition.

The issuance of a patent is not conclusive as to its inventorship, scope, validity, or enforceability, and our patents may be challenged in the courts or patent offices in the United States and abroad. We may also be subject to a third party preissuance submission of prior art to the USPTO, or become involved in opposition, derivation, revocation, reexamination, *inter partes* review, post-grant review, interference or other administrative 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, invalidate or render unenforceable our patent rights, result in our loss of exclusivity or freedom to operate, such that third parties would be able to commercialize our technology or drugs and compete directly with us, without payment to us, or we would be unable to manufacture or commercialize our drug candidates without infringing or otherwise violating third-party patent rights. Such challenges may also limit the duration of the patent protection of our technology and drug candidates. Given the amount of time required for the development, testing and regulatory review of new drug candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our patent portfolio may not provide us with sufficient rights to exclude others from commercializing drugs similar or identical to ours. In addition, such challenges may result in substantial cost and require significant time from our scientists and management, even if the eventual outcome is favorable to us. Moreover, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future drug candidates.

Even if our patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent

competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors and other third parties may be able to circumvent our patents by developing similar or alternative technologies or drugs in a non-infringing manner. Any of the foregoing events could have a material adverse effect on our business, financial condition, results of operation and prospects.

We may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time-consuming and unsuccessful.

Competitors and other third parties may infringe or otherwise violate our issued patents or other intellectual property. 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. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their patents or that our patents are invalid or unenforceable. In a patent infringement proceeding, a court may decide that a patent of ours is invalid or unenforceable, in whole or in part, construe the patent's claims narrowly or refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology. An adverse result in any litigation proceeding could put one or more of our patents at risk of being invalidated, held unenforceable or interpreted narrowly. We may find it impractical or undesirable to enforce our intellectual property against some third parties.

If we were to initiate legal proceedings against a third party to enforce a patent directed to avatrombopag, or one of our future drug candidates, the defendant could counterclaim that our patent is invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, non-enablement or insufficient written description. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO or made a misleading statement during prosecution. Third parties may also raise similar claims before the USPTO, even outside the context of litigation. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art of which we and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we would lose at least part, and perhaps all, of the patent protection on avatrombopag. Such a loss of patent protection would materially harm our business.

Interference proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be materially harmed if the prevailing party does not offer us a license on commercially reasonable terms.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation.

Most of our competitors are larger than we are and have substantially greater resources. They are, therefore, likely to be able to sustain the costs of complex patent litigation longer than we could. Accordingly, despite our efforts, we may not be able to prevent third parties from infringing upon or misappropriating our intellectual property. Litigation could result in substantial costs and diversion of management resources, which could harm our business. In addition, the uncertainties associated with litigation could compromise our ability to raise the funds necessary to continue our clinical trials, continue our internal research programs, or in-license needed technology or other drug candidates. There could also be public announcements of the results of the hearing, motions, or other interim proceedings or developments. If securities analysts or investors perceive those results to be negative, it could cause the price of shares of our common stock to decline. Any of the foregoing events could have a material adverse effect on our business, financial condition, results of operation and prospects.

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

Filing, prosecuting, maintaining and defending patents on our drug candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. For example, avatrombopag is currently covered by patents in the United States, but not in all other countries. 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 may not be able to prevent third parties from practicing our invention in such countries. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own drugs and may export otherwise infringing drugs to territories where we have patent protection, but enforcement rights are not as strong as those in the United States. These drugs may compete with our drug candidates and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of some countries do not favor the enforcement of patents and other intellectual property protection, which could make it difficult for us to stop the infringement of our patents generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate, and the damages or other remedies awarded, if any, may not be commercially meaningful.

Many countries, including European Union countries, India, Japan and China, have compulsory licensing laws under which a patent owner may be compelled under specified circumstances to grant licenses to third parties. In those countries, we may have limited remedies if patents are infringed or if we are compelled to grant a license to a third party, which could materially diminish the value of those patents. This could limit our potential revenue opportunities. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

We may be unsuccessful in licensing or acquiring intellectual property from third parties that may be required to develop and commercialize our drug candidates.

A third party may hold intellectual property, including patent rights that are important or necessary to the development and commercialization of our drug candidates. It may be necessary for us to use the patented or proprietary technology of third parties to commercialize our drug candidates, in which case we would be required to acquire or obtain a license to such intellectual property from these third parties, and we may be unable to do so on commercially reasonable terms or at all. The licensing or acquisition of third party intellectual property rights is a competitive area, and several more established companies may pursue strategies to license or acquire third party intellectual property rights that we may consider attractive or necessary. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third party intellectual property rights on terms that would allow us to make an appropriate return on our investment or at all. If we are unable to successfully obtain rights to required third party intellectual property rights or maintain the existing intellectual property rights we have, we may have to abandon development of the relevant program or drug candidate, which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Third parties may initiate legal proceedings alleging that we are infringing, misappropriating or otherwise violating their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.

Our commercial success depends upon our ability to develop, manufacture, market and sell our drug candidates and use our proprietary technologies without infringing, misappropriating or otherwise violating the intellectual property and other proprietary rights of third parties. There is considerable intellectual property litigation in the biotechnology and pharmaceutical industries. We may become party to, or threatened with, future adversarial proceedings or litigation regarding intellectual property rights with respect to our drugs and technology, including interference or derivation proceedings before the USPTO.

Numerous U.S. and foreign issued patents and pending patent applications owned by third parties exist in the fields in which we are developing our drug candidates. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future.

If we are found to infringe a third party's intellectual property rights, we could be required to obtain a license from such a third party to continue developing and marketing our drugs and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. We could be forced, including by court order, to cease commercializing the infringing technology or drug. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing our drug candidates or force us to cease some of our business operations. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, pay royalties, redesign our infringing drug or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business. Any of the foregoing events would have a material adverse effect on our business, financial condition, results of operations and prospects.

We may be subject to claims by third parties asserting that we or our employees have misappropriated their intellectual property, or claiming ownership of what we regard as our own intellectual property.

All of our employees were previously employed at other biotechnology or pharmaceutical companies. 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 these employees or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such employee's former employer. Litigation may be necessary to defend against these claims.

In addition, while it is our policy to require our employees and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact develops intellectual property that we regard as our own. Our and their assignment agreements may not be self-executing or may be breached, and we may be forced to bring claims against third parties, or defend claims they may bring against us, to determine the ownership of what we regard as our intellectual property.

If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs, delay development of our drug candidates and be a distraction to management. Any of the foregoing events would have a material adverse effect on our business, financial condition, results of operations and prospects.

Intellectual property litigation could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could compromise our ability to compete in the marketplace, including compromising our ability to raise the funds necessary to continue our clinical trials, continue our research programs, license necessary technology from third parties, or enter into development collaborations that would help us commercialize avatrombopag, if approved.

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

In addition to seeking patents for our drug candidates and technology, we also rely on trade secrets and confidentiality agreements to protect our unpatented know-how, technology, and other proprietary information and to maintain our competitive position. Trade secrets and know-how can be difficult to protect. Because we expect to rely on third parties to manufacture avatrombopag and any future drug candidates, and we expect to collaborate with third parties on the development of avatrombopag and any future drug candidates, we may be asked to, at times, share trade secrets with them. We also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development partnerships or similar agreements. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with our advisors, employees, third-party contractors and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors or other third parties, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. In addition, we may not be able to obtain adequate remedies for breaches of these agreements. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive, and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's or other third party's independent development of, or unauthorized use or disclosure of, our trade secrets, would impair our competitive position and may have a material adverse effect on our business, financial condition, results of operations and prospects.

In addition, although these agreements typically restrict the ability of our advisors, employees, third-party contractors and consultants to publish data potentially relating to our trade secrets, our agreements may contain certain limited publication rights. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of our agreements with third parties,

independent development or publication of information by any of our third-party collaborators. A competitor's or other third party's discovery of our trade secrets would impair our competitive position and have a material adverse impact on our business, financial condition, results of operations and prospects.

The validity, scope and enforceability of any patents listed in the Orange Book that cover avatrombopag can be challenged by competitors.

If avatrombopag is approved by the FDA, one or more third parties may challenge the patents covering avatrombopag, which could result in the invalidation of, or render unenforceable, some or all of the relevant patent claims or a finding of non-infringement. For example, if a third party files an Abbreviated New Drug Application, or ANDA, for a generic drug containing avatrombopag, and relies in whole or in part on studies conducted by or for us, the third party will be required to certify to the FDA that either: (1) there is no patent information listed in the FDA's Orange Book with respect to our NDA for the applicable approved drug candidate; (2) the patents listed in the Orange Book have expired; (3) the listed patents have not expired, but will expire on a particular date and approval is sought after patent expiration; or (4) the listed patents are invalid or will not be infringed by the manufacture, use or sale of the third party's generic drug. A certification that the new drug will not infringe the Orange Book-listed patents for the applicable approved drug candidate, or that such patents are invalid, is called a paragraph IV certification. If the third party submits a paragraph IV certification to the FDA, a notice of the paragraph IV certification must also be sent to us once the third party's ANDA is accepted for filing by the FDA. We may then initiate a lawsuit to defend the patents identified in the notice. The filing of a patent infringement lawsuit within 45 days of receipt of the notice automatically prevents the FDA from approving the third party's ANDA until the earliest of 30 months or the date on which the patent expires, the lawsuit is settled, or the court reaches a decision in the infringement lawsuit in favor of the third party. If we do not file a patent infringement lawsuit within the required 45-day period, the third party's ANDA will not be subject to the 30-month stay of FDA approval. Litigation or other proceedings to enforce or defend intellectual property rights are often very complex in nature, may be very expensive and time-consuming, may divert our management's attention from our core business, and may result in unfavorable results that could limit our ability to prevent third parties from competing with avatrombopag, all of which would have a material adverse effect on our business, financial condition, results of operation and prospects.

If we do not obtain protection under the Hatch-Waxman Amendments to extend the patent term and obtain data exclusivity for avatrombopag, our business may be materially harmed.

Our commercial success will largely depend on our ability to obtain and maintain patent and other intellectual property in the United States and other countries with respect to our proprietary technology, drug candidates and our target indications. Our issued patents, with claims directed to avatrombopag, are expected to expire between 2023 and 2027, excluding any extension of a patent term that may be available in a particular country. Given the amount of time required for the development, testing and regulatory review of new drug candidates, patents protecting avatrombopag might expire before or shortly after such candidates begin to be commercialized. We expect to seek extensions of patent terms in the U.S. and, if available, in other countries where we are prosecuting patents.

Depending upon the timing, duration and specifics of FDA marketing approval of avatrombopag, one or more of our U.S. patents may be eligible for limited patent term restoration under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years beyond the normal expiration of the patent as compensation for patent term lost during development and the FDA regulatory review process, which is limited to the approved indication (or any additional indications approved during the period of extension). This extension is limited to only one patent that covers the approved drug (and to only those patent claims covering the approved drug, a method for using it, or a method for manufacturing it) and cannot extend the remaining term of a patent beyond a total of 14 years from the date of drug approval. However, the applicable authorities, including the FDA and the USPTO 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. We may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request.

If we are unable to extend the expiration date of our existing patents or obtain new patents with longer expiry dates, our competitors may be able to take advantage of our investment in development and clinical trials by referencing our clinical and preclinical data to obtain approval of competing drugs following our patent expiration and launch their drug earlier than might otherwise be the case, which would have a material adverse effect on our business, financial condition, results of operation and prospects.

Changes in U.S. patent law or the patent law of other countries or jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our current or any future drug candidates.

The United States has recently enacted and implemented wide-ranging patent reform legislation. The U.S. Supreme Court has ruled on

several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on actions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce patents that we have licensed or that we might obtain in the future. Similarly, changes in patent law and regulations in other countries or jurisdictions or changes in the governmental bodies that enforce them or changes in how the relevant governmental authority enforces patent laws or regulations may weaken our ability to obtain new patents or to enforce patents that we have licensed or that we may obtain in the future.

If we fail to comply with our obligations under any license, collaboration or other agreement, we may be required to pay damages and could lose intellectual property rights that are necessary for developing and protecting our drug candidates, or we could lose certain rights to grant sublicenses.

Our technology licenses and any future licenses we enter into are likely to impose various development, commercialization, funding, milestone, royalty, diligence, sublicensing, insurance, patent prosecution and enforcement, and/or other obligations on us. The agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. If we breach any of these imposed obligations, or use the intellectual property licensed to us in an unauthorized manner, we may be required to pay damages and the licensor may have the right to terminate the license, which could result in us being unable to develop, manufacture and sell drugs that are covered by the licensed technology or enable a competitor to gain access to the licensed technology. Any resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement. Moreover, our licensors may own or control intellectual property that has not been licensed to us and, as a result, we may be subject to claims, regardless of their merit, that we are infringing or otherwise violating the licensor's rights. In addition, while we cannot currently determine the amount of the royalty obligations we would be required to pay on sales of future drugs, if any, the amounts may be significant. The amount of our future royalty obligations will depend on the technology and intellectual property we use in drugs that we successfully develop and commercialize, if any. Therefore, even if we successfully develop and commercialize drugs, we may be unable to achieve or maintain profitability. Any of the foregoing events could have a material adverse effect on our business, financial conditions, results of operations, and prospects.

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

Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and/or applications will be due to be paid to the USPTO and various government patent agencies outside of the United States over the lifetime of our owned and licensed patents and/or applications and any patent rights we may own or license in the future. We rely on our outside counsel or our licensing partners to pay these fees due to non-U.S. patent agencies. The USPTO and various non-U.S. government patent agencies require compliance with several procedural, documentary, fee payment and other similar provisions during the patent application process. In many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non-compliance 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. In such an event, potential competitors might be able to enter the market and this circumstance could have a material adverse effect on our business, financial condition, results of operations and prospects.

Any trademarks we have obtained or may obtain may be infringed or otherwise violated, or successfully challenged, resulting in harm to our business.

We expect to rely on trademarks as one means to distinguish avatrombopag, if approved for marketing, from the drugs of our competitors. Once we select new trademarks and apply to register them, our trademark applications may not be approved. Third parties may oppose or attempt to cancel our trademark applications or trademarks, or otherwise challenge our use of the trademarks. In the event that our trademarks are successfully challenged, we could be forced to rebrand our drugs, which could result in loss of brand recognition and could require us to devote resources to advertising and marketing new brands. Our competitors may infringe or otherwise violate our trademarks and we may not have adequate resources to enforce our trademarks. Any of the foregoing events may have a material adverse effect on our business, financial condition, results of operation and prospects.

Intellectual property rights do not necessarily address all potential threats to our competitive position.

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 support our competitive position. The following examples are illustrative:

- others may be able to make formulations or compositions that are the same as or similar to avatrombopag but that are not covered by the claims of the patents that we own or license;
- we or any collaborators might not have been the first to make the inventions covered by the issued patents or pending patent applications that we own or license;
- we or our licensors 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 license may not provide us with any competitive advantages, or may be held invalid or unenforceable as a result of legal challenges;
- 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 drugs for sale in our major commercial markets;
- the patents of others may harm our business; and
- we may not develop additional proprietary technologies that are patentable.

Should any of these events occur, they could have a material adverse effect on our business, financial condition, results of operations, and prospects.

Risks related to regulatory approval of our drug candidates and other legal compliance matters

Clinical development involves a lengthy and expensive process, with an uncertain outcome. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of avatrombopag or any future drug candidate we may develop.

The risk of failure for avatrombopag and any other future drug candidates we may develop is high. It is impossible to predict when or if avatrombopag will prove to be effective and safe in humans or will receive regulatory approval for the treatment of thrombocytopenia in CLD patients scheduled to undergo a procedure. Additionally, before regulatory authorities grant marketing approval for avatrombopag, for any future indications, or any future drug candidate that we seek to develop, we will be required to conduct extensive clinical trials to demonstrate safety and efficacy in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is inherently uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that believed their drug candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their drugs. In addition, we are evaluating the potential regulatory approval pathway for avatrombopag for other indications, including the treatment of adults with chronic ITP. Several clinical trials have been conducted evaluating the use of avatrombopag for the treatment of patients with chronic ITP and may utilize our clinical trial results for other indications as well. However, the FDA, EMA or any comparable foreign regulatory authority may not accept any such trial results for additional indications and may require us to conduct further clinical trials, which may require us to incur significant additional development expenses. As a result, there can be no assurance that we will continue to evaluate and pursue approval for avatrombopag in any such indications.

We may experience numerous unforeseen events during or as a result of clinical trials that could delay or prevent our ability to receive marketing approval or commercialize avatrombopag or any future drug candidate, including:

- regulators or institutional review boards may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- we may experience delays in reaching, or fail to reach, agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites or prospective CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- clinical trials of our drug candidates may produce negative or inconclusive results, including failure to demonstrate statistical significance, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon drug development programs;

- the number of patients required for clinical trials of our drug candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials or fail to return for post-treatment follow-up at a higher rate than we anticipate;
- our drug candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators or institutional review boards to suspend or terminate the trials;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- regulators or institutional review boards may require that we or our investigators suspend or terminate clinical development for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- the cost of clinical trials of our drug candidates may be greater than we anticipate; and
- the supply or quality of our drug candidates or other materials necessary to conduct clinical trials of our drug candidates may be insufficient or inadequate.

The ADAPT 1 and ADAPT 2 Phase 3 clinical trials evaluating avatrombopag were initially being conducted under an SPA with the FDA. However, after reviewing initial blinded data from the trials, protocol amendments were made. Given these deviations from the SPA, the FDA may evaluate the results from the trials with a higher level of scrutiny or may require us to perform additional clinical trials to collect more safety and efficacy data, which would delay the timing of approval of avatrombopag, if at all. We also no longer have the benefits provided by operating the Phase 3 clinical trials pursuant to the SPA.

We could also encounter delays if a clinical trial is suspended or terminated by us, by the institutional review boards of the institutions in which such trials are being conducted, by the data safety monitoring board for such trial or by the FDA or other regulatory authorities. Such authorities may impose such a suspension or termination due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. If we experience delays in the completion of, or termination of, any clinical trial of our drug candidates, the commercial prospects of our drug candidates will be harmed, and our ability to generate drug revenues from any of these drug candidates will be delayed. In addition, any delays in completing our clinical trials will increase our costs, slow down our drug candidate development and approval process and jeopardize our ability to commence drug sales and generate revenues. Any of these occurrences may harm our business, financial condition and prospects significantly. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval. If we are required to conduct additional clinical trials or other testing beyond those that we currently contemplate, if we are unable to successfully complete clinical trials of our drug candidates or other testing, if the results of these trials or tests are not favorable or if there are safety concerns, we may:

- be delayed in obtaining marketing approval for our drug candidates;
- not obtain marketing approval at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to additional post-marketing testing requirements; or
- have the drug removed from the market after obtaining marketing approval.

Our drug development costs will further increase if we experience delays in testing or marketing approvals. We do not know whether any of our future clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our drug candidates or allow our competitors to bring drugs to market before we do and impair our ability to successfully commercialize our drug candidates.

Even if we obtain FDA approval for avatrombopag in the United States, we may never obtain approval for or commercialize it in any other jurisdiction, which would limit our ability to realize its full market potential.

In order to market any drugs in any particular jurisdiction, we must establish and comply with numerous and varying regulatory requirements on a country-by-country basis regarding safety and efficacy. Approval by FDA in the United States does not ensure approval by regulatory authorities in other countries or jurisdictions. In addition, the clinical standards of care may differ significantly such that clinical trials conducted in one country may not be accepted by healthcare providers, third-party payors or regulatory authorities in other countries, and regulatory approval in one country does not guarantee regulatory approval in any other country. Approval processes vary among countries and can involve additional drug testing and validation and additional administrative review periods. Seeking foreign regulatory approval could result in difficulties and costs for us and require additional preclinical studies or clinical trials which could be costly and time consuming. Regulatory requirements can vary widely from country to country and could

delay or prevent the introduction of our drugs in those countries. We do not have any drug candidates approved for sale in any jurisdiction, including in international markets, and we do not have experience in obtaining regulatory approval in international markets. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, or if regulatory approvals in international markets are delayed, our target market will be reduced and our ability to realize the full market potential of any drug we develop will be unrealized.

A variety of risks associated with marketing avatrombopag internationally could harm our business.

We may seek regulatory approval for avatrombopag and any future drug candidates outside of the United States and, accordingly, we expect that we will be subject to additional risks related to operating in foreign countries if we obtain the necessary approvals, including:

- differing regulatory requirements in foreign countries;
- the potential for so-called parallel importing, which is what happens when a local seller, faced with high or higher local prices, opts to import goods from a foreign market (with low or lower prices) rather than buying them locally;
- unexpected changes in tariffs, trade barriers, price and exchange controls and other regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- foreign reimbursement, pricing and insurance regimes;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign taxes, including withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- difficulties staffing and managing foreign operations;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- potential liability under the Foreign Corrupt Practices Act of 1977 or comparable foreign regulations;
- challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geo-political actions, including war and terrorism.

These and other risks associated with our international operations may compromise our ability to achieve or maintain profitability.

Even if we obtain regulatory approval for avatrombopag, we will still face extensive regulatory requirements and our drugs may face future development and regulatory difficulties.

Any drug candidate for which we obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, advertising and promotional activities for such drug candidate, will be subject to continual requirements of and review by the FDA, EMA and other comparable foreign regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, cGMP requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a drug candidate is granted, the approval may not be as broad as intended or desired, may be subject to limitations on the indicated uses for which the drug candidate may be marketed or may be subject to the conditions of approval, including the requirement to implement a risk evaluation and mitigation strategy. If avatrombopag receives marketing approval, the accompanying label may limit the approved use of our drug, which could limit sales of the drug.

The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the drug. The FDA closely regulates the post-approval marketing and promotion of drugs to ensure drugs are marketed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we do not market our drugs for their approved indications, we may be subject to enforcement action for off-label marketing. Violations of the Federal Food, Drug, and Cosmetic Act relating to the promotion of prescription drugs may lead to investigations alleging violations of federal and state healthcare fraud and abuse laws, as well as state consumer protection laws.

In addition, later discovery of previously unknown adverse events or other problems with our drugs, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may have negative consequences, including:

- restrictions on such drugs, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a drug;
- restrictions on drug distribution or use;
- requirements to conduct post-marketing studies or clinical trials;

- warning letters;
- recall or withdrawal of the drugs from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- clinical holds;
- fines, restitution or disgorgement of profits or revenue;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our drugs;
- drug seizure; or
- injunctions or the imposition of civil or criminal penalties.

The FDA's policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of avatrombopag or any future drug candidate. 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.

Our current and future relationships with third-party payors, health care professionals and customers in the United States and elsewhere may be subject, directly or indirectly, to applicable anti-kickback, fraud and abuse, false claims, physician payment transparency, health information privacy and security and other healthcare laws and regulations, which could expose us to significant penalties.

Healthcare providers, physicians and third-party payors in the United States and elsewhere will play a primary role in the recommendation and prescription of any drug candidates for which we obtain marketing approval. Our current and future arrangements with health care professionals, third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations, including, without limitation, the federal Anti-Kickback Statute and the federal civil False Claims Act, that may constrain the business or financial arrangements and relationships through which we conduct clinical research, sell, market and distribute any drugs for which we obtain marketing approval. In addition, we may be subject to transparency laws and patient privacy regulation by the federal government and by the U.S. states and foreign jurisdictions in which we conduct our business. The applicable federal, state and foreign healthcare laws and regulations that may affect our ability to operate include the following:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under federal and state healthcare programs, such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it to have committed a violation. Further, several courts have interpreted the statute's intent requirement to mean that if any one purpose of an arrangement involving remuneration is to induce referrals of federal healthcare covered business, the Anti-Kickback Statute has been violated. Moreover, the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act;

- federal civil and criminal false claims laws, including, without limitation, the federal civil False Claims Act (that can be enforced through civil whistleblower or qui tam actions), and the civil monetary penalties law, which impose criminal and civil penalties against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, including the Medicare and Medicaid programs, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;

- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which imposes criminal and civil liability for, among other things, executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it to have committed a violation;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, which impose obligations on covered healthcare providers, health plans, and healthcare clearinghouses, as well as their business associates that create, receive, maintain or transmit individually identifiable health information for or on behalf of a covered entity, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;

- the Physician Payments Sunshine Act, created under Section 6002 of Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively the Affordable Care Act, and its implementing regulations, which requires specified manufacturers of drugs, devices, biologics and medical supplies for which payment is

available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services, or CMS, information related to payments or other "transfers of value" made to physicians, which is defined to include doctors, dentists, optometrists, podiatrists and chiropractors, and teaching hospitals and applicable manufacturers to report annually to CMS ownership and investment interests held by physicians and their immediate family members by the 90th day of each calendar year. All such reported information is publicly available; and

- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; state and foreign laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government or otherwise restrict payments that may be made to healthcare providers; state and foreign laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; and state and foreign laws governing the privacy and security of health information in certain 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 business arrangements with third parties will comply with applicable healthcare laws and regulations may involve substantial costs. It is possible that governmental authorities will conclude that our business practices, including our relationships with physicians and other healthcare providers, some of whom may recommend, purchase and/or prescribe avatrombopag, if approved, may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations.

If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, including, without limitation, damages, fines, disgorgement, individual imprisonment, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws and the curtailment or restructuring of our operations, which could have a material adverse effect on our business. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found not to be in compliance with applicable laws, it may be subject to criminal, civil or administrative sanctions, including exclusions from participation in government healthcare programs, which could also materially affect our business.

Recently enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize avatrombopag and affect the prices we may obtain.

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

Among policy makers and payors in the United States and elsewhere, there is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and/or expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives. In March 2010, President Obama signed into law the Affordable Care Act, a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for the healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms.

Among the provisions of the Affordable Care Act of importance to our potential drug candidates are the following:

- an annual, nondeductible fee on any entity that manufactures or imports certain branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program to 23.1% and 13.0% of the average manufacturer price for branded and generic drugs, respectively;
- expansion of healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, which include, among other things, new government investigative powers and enhanced penalties for non-compliance;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D;
- extension of manufacturers' Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations;

- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals, thereby potentially increasing manufacturers' Medicaid rebate liability;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- the new requirements under the federal open payments program and its implementing regulations;
- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

Some of the provisions of the Affordable Care Act have yet to be fully implemented, while certain provisions have been subject to judicial and Congressional challenges, as well as efforts by the Trump administration to repeal or replace certain aspects of the Affordable Care Act. For example, in Congress, the U.S. House of Representatives passed Affordable Care Act replacement legislation known as the American Health Care Act of 2017 in May 2017, which was not introduced in the Senate. More recently, the Senate Republicans have proposed multiple bills to repeal or repeal and replace portions of the Affordable Care Act. Although none of these measures have been enacted by Congress, Congress may consider other legislation to repeal or replace certain elements of the Affordable Care Act. In addition, since January 2017, President Trump has signed two Executive Orders designed to delay the implementation of certain provisions of the ACA or otherwise circumvent some of the requirements for health insurance mandated by the ACA. Further, citing legal guidance from the U.S. Department of Justice and the U.S. Department of Health and Human Services, the Trump administration has concluded that cost-sharing reduction (CSR) payments to insurance companies required under the Affordable Care Act have not received necessary appropriations from Congress and announced that it will discontinue these payments immediately until such appropriations are made. The loss of the CSR payments is expected to increase premiums on certain policies issued by qualified health plans under the Affordable Care Act. A bipartisan bill to appropriate funds for CSR payments was introduced in the Senate, but the future of that bill is uncertain. We continue to evaluate the effect that the Affordable Care Act and its possible repeal and replacement has on our business.

In addition, other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. These changes included aggregate reductions to Medicare payments to providers of 2% per fiscal year effective April 1, 2013 and, due to subsequent legislative amendments to the statute, will stay in effect through 2025, unless additional Congressional action is taken. In January 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 new laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on customers for our drugs, if approved, and, accordingly, our financial operations.

We expect that the Affordable Care Act, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved drug. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our drugs.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for drugs. In addition, 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 patient programs, reduce the cost of drugs under Medicare and reform government program reimbursement methodologies for drugs. We cannot be sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of avatrombopag, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent drug labeling and post-marketing testing and other requirements.

Coverage and adequate reimbursement may not be available for avatrombopag, which could make it difficult for us to sell our drugs profitably.

Market acceptance and sales of any drug candidates that we develop will depend in part on the extent to which reimbursement for these drugs and related treatments will be available from third-party payors, including government health administration authorities and private health insurers. Third-party payors decide which drugs they will pay for and establish reimbursement levels. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own coverage and reimbursement policies. However, decisions regarding the extent of coverage and amount of reimbursement to be provided for any drug candidates that we develop will be made on a plan-by-plan basis. One payor's determination to provide coverage for a drug does not assure that other payors will also provide coverage, and adequate reimbursement, for the drug. Additionally, a third-party payor's decision to provide coverage for a drug does not imply that an adequate reimbursement rate will be approved. Each plan determines whether or not it will provide coverage for a drug, what amount it will pay the manufacturer for the drug, and on what tier of its formulary the drug will be placed. The position of

a drug on a formulary generally determines the co-payment that a patient will need to make to obtain the drug and can strongly influence the adoption of a drug by patients and physicians. Patients who are prescribed treatments for their conditions and providers prescribing such services generally rely on third-party payors to reimburse all or part of the associated healthcare costs. Patients are unlikely to use our drugs unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our drugs.

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. In addition, we may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of our drugs in order to obtain coverage and reimbursement from third-party payors. We cannot be sure that coverage and reimbursement will be available for any drug that we commercialize and, if reimbursement is available, what the level of reimbursement will be. Inadequate coverage and reimbursement may impact the demand for, or the price of, any drug for which we obtain marketing approval. If coverage and adequate reimbursement are not available, or are available only to limited levels, we may not be able to successfully commercialize any drug candidates that we develop.

Additionally, there have been a number of legislative and regulatory proposals to change the healthcare system in the United States and in some foreign jurisdictions that could affect our ability to sell any future drugs profitably. These legislative and regulatory changes may negatively impact the reimbursement for any future drugs, following approval.

Governments outside the United States tend to impose strict price controls, which may adversely affect our revenue, if any.

In some countries, particularly the countries of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a drug. In addition, there can be considerable pressure by governments and other stakeholders on prices and reimbursement levels, including as part of cost containment measures. Political, economic and regulatory developments may further complicate pricing negotiations. To obtain coverage and reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our drug candidate to other available procedures. If reimbursement of our drugs is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be harmed, possibly materially.

We are subject to governmental economic sanctions and export and import controls that could impair our ability to compete in international markets or subject us to liability if we are not in compliance with applicable laws.

As a U.S. company, we are subject to U.S. import and export controls and economic sanctions laws and regulations, and we are required to import and export our drug candidates, technology and services in compliance with those laws and regulations, including the U.S. Export Administration Regulations, the International Traffic in Arms Regulations, and economic embargo and trade sanction programs administered by the Treasury Department's Office of Foreign Assets Control.

U.S. economic sanctions and export control laws and regulations prohibit the shipment of certain drugs and services to countries, governments and persons targeted by U.S. sanctions. While we are currently taking precautions to prevent doing any business, directly or indirectly, with countries, governments and persons targeted by U.S. sanctions and to ensure that our drug candidates, if approved, are not exported or used by countries, governments and persons targeted by U.S. sanctions, such measures may be circumvented.

Furthermore, if we export our drug candidates, if approved, the exports may require authorizations, including a license, a license exception or other appropriate government authorization. Complying with export control and sanctions regulations for a particular sale may be time-consuming and may result in the delay or loss of sales opportunities. Failure to comply with export control and sanctions regulations for a particular sale may expose us to government investigations and penalties.

If we are found to be in violation of U.S. sanctions or import or export control laws, it could result in civil and criminal, monetary and non-monetary penalties, including possible incarceration for those individuals responsible for the violations, the loss of export or import privileges and reputational harm.

We are subject to anti-corruption and anti-money laundering laws with respect to our operations and non-compliance with such laws can subject us to criminal and/or civil liability and harm our business.

We are subject to the U.S. Foreign Corrupt Practices Act of 1977, as amended, or the FCPA, the U.S. domestic bribery statute contained in 18 U.S.C. § 201, the U.S. Travel Act, the USA PATRIOT Act and possibly other anti-bribery and anti-money laundering laws in countries in which we conduct activities. Anti-corruption laws are interpreted broadly and prohibit companies and their employees and third-party intermediaries from authorizing, offering or providing, directly or indirectly, improper payments or benefits to recipients in the public or private sector. As we commercialize avatrombopag and eventually commence international sales and business, we may

engage with collaborators and third-party intermediaries to sell our drugs abroad and to obtain necessary permits, licenses and other regulatory approvals. We or our third-party intermediaries may have direct or indirect interactions with officials and employees of government agencies or state-owned or affiliated entities. We can be held liable for the corrupt or other illegal activities of these third-party intermediaries, our employees, representatives, contractors, partners and agents, even if we do not explicitly authorize such activities.

Noncompliance with anti-corruption and anti-money laundering laws could subject us to whistleblower complaints, investigations, sanctions, settlements, prosecution, other enforcement actions, disgorgement of profits, significant fines, damages, other civil and criminal penalties or injunctions, suspension and/or debarment from contracting with certain persons, the loss of export privileges, reputational harm, adverse media coverage and other collateral consequences. Responding to any action will likely result in a materially significant diversion of management's attention and resources and significant defense costs and other professional fees.

Risks related to ownership of our common stock

An active trading market for our common stock may not continue to be developed or be sustained.

Prior to June 29, 2017, there was no public market for our common stock, and we cannot assure you that an active trading market for our shares will continue to develop or be sustained. As a result, it may be difficult for you to sell shares at an attractive price or at all.

The trading price of the shares of our common stock may be volatile, and purchasers of our common stock could incur substantial losses.

Our stock price may be volatile. The stock market in general and the market for biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, investors may not be able to sell their common stock at or above the price paid for the shares. The market price for our common stock may be influenced by many factors, including:

- adverse regulatory decisions, including failure to receive regulatory approval of avatrombopag;
- any delay in our regulatory filings for avatrombopag or any future drug candidate and any adverse development or perceived adverse development with respect to the applicable regulatory authority's review of such filings, including without limitation the FDA's issuance of a "refusal to file" letter or a request for additional information;
- the commencement, enrollment or results of any future clinical trials we may conduct, or changes in the development status of avatrombopag or any other future drug candidates;
- adverse results from, delays in or termination of clinical trials;
- unanticipated serious safety concerns related to the use of avatrombopag or any other future drug candidate;
- lower than expected market acceptance of avatrombopag following approval for commercialization;
- changes in financial estimates by us or by any securities analysts who might cover our stock;
- conditions or trends in our industry;
- changes in the market valuations of similar companies;
- stock market price and volume fluctuations of comparable companies and, in particular, those that operate in the biopharmaceutical industry;
- publication of research reports about us or our industry or positive or negative recommendations or withdrawal of research coverage by securities analysts;
- announcements by us or our competitors of significant acquisitions, strategic partnerships or divestitures;
- announcements of investigations or regulatory scrutiny of our operations or lawsuits filed against us;
- investors' general perception of our company and our business;
- recruitment or departure of key personnel;
- overall performance of the equity markets;
- trading volume of our common stock;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;
- significant lawsuits, including patent or stockholder litigation;
- proposed changes to healthcare laws in the United States or foreign jurisdictions, or speculation regarding such changes;
- general political and economic conditions; and
- other events or factors, many of which are beyond our control.

In addition, in the past, stockholders have initiated class action lawsuits against pharmaceutical and biotechnology companies following periods of volatility in the market prices of these companies' stock. Such litigation, if instituted against us, could cause us to incur substantial costs and divert management's attention and resources from our business.

If equity research analysts do not publish research or reports, or publish unfavorable research or reports, about us, our business or our market, our stock price and trading volume could decline.

The trading market for our common stock will be influenced by the research and reports that equity research analysts publish about us and our business. As a newly public company, we have only limited research coverage by equity research analysts. Equity research analysts may elect not to initiate or continue to provide research coverage of our common stock, and such lack of research coverage may adversely affect the market price of our common stock. Even if we do have equity research analyst coverage, we will not have any control over the analysts or the content and opinions included in their reports. The price of our stock could decline if one or more equity research analysts downgrade our stock or issue other unfavorable commentary or research. If one or more equity research analysts ceases coverage of our company or fails to publish reports on our company regularly, demand for our stock could decrease, which in turn could cause our stock price or trading volume to decline.

A significant portion of our total outstanding shares are restricted from immediate resale but may be sold into the market in the near future. This could cause the market price of our common stock to drop significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time, subject to the restrictions and limitations described below. If our stockholders sell, or the market perceives that our stockholders intend to sell, substantial amounts of our common stock in the public market, the market price of our common stock could decline significantly.

As of November 6, 2017, we had 25,652,457 outstanding shares of common stock. Of these shares, approximately 4.6 million are freely tradable and the remaining shares of common stock will be available for sale in the public market at the end of December 2017, following the scheduled expiration of lock-up agreements between our stockholders and certain of the underwriters in connection with our recent IPO, subject, in the case of our affiliates, to the conditions of Rule 144 under the Securities Act. J.P. Morgan Securities LLC may release these stockholders from their lock-up agreements at any time and without notice, which would allow for earlier sales of shares in the public market subject to the conditions of Rule 144 under the Securities Act.

In addition, we have filed a registration statement on Form S-8 registering the issuance of approximately 6.0 million shares of common stock subject to options or other equity awards issued or reserved for future issuance under our equity incentive plans. Shares registered under this registration statement on Form S-8 are available for sale in the public market subject to vesting arrangements and exercise of options, the lock-up agreements described above and, in the case of our affiliates, the restrictions of Rule 144.

Additionally, the holders of an aggregate of approximately 21 million shares of our common stock, or their transferees, have rights, subject to some conditions, to require us to file one or more registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. If we were to register the resale of these shares, they could be freely sold in the public market without limitation. If these additional shares are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

Provisions in our corporate charter documents and under Delaware law may prevent or frustrate attempts by our stockholders to change our management and hinder efforts to acquire a controlling interest in us, and the market price of our common stock may be lower as a result.

There are provisions in our certificate of incorporation and bylaws that may make it difficult for a third party to acquire, or attempt to acquire, control of our company, even if a change of control was considered favorable by you and other stockholders. For example, our board of directors has the authority to issue up to 10,000,000 shares of preferred stock. The board of directors can fix the price, rights, preferences, privileges, and restrictions of the preferred stock without any further vote or action by our stockholders. The issuance of shares of preferred stock may delay or prevent a change of control transaction. As a result, the market price of our common stock and the voting and other rights of our stockholders may be adversely affected. An issuance of shares of preferred stock may result in the loss of voting control to other stockholders.

Our charter documents contain other provisions that could have an anti-takeover effect, including:

- only one of our three classes of directors are elected each year;
- stockholders are not entitled to remove directors other than by a 66.7% vote and only for cause;
- stockholders are not permitted to take actions by written consent;
- stockholders cannot call a special meeting of stockholders; and
- stockholders must give advance notice to nominate directors or submit proposals for consideration at stockholder meetings.

In addition, we are subject to the anti-takeover provisions of Section 203 of the Delaware General Corporation Law, which regulates

corporate acquisitions by prohibiting Delaware corporations from engaging in specified business combinations with particular stockholders of those companies. These provisions could discourage potential acquisition proposals and could delay or prevent a change of control transaction. They could also have the effect of discouraging others from making tender offers for our common stock, including transactions that may be in your best interests. These provisions may also prevent changes in our management or limit the price that investors are willing to pay for our stock.

Concentration of ownership of our common stock among our existing executive officers, directors and principal stockholders may prevent our other stockholders from influencing significant corporate decisions.

As of November 6, 2017, our executive officers, directors and current beneficial owners of 5% or more of our common stock and their respective affiliates, including funds under common control with PBM Capital Investments, LLC, in the aggregate, beneficially own a majority of our outstanding common stock. Further, PBM Capital Investments, LLC and funds under common control with PBM Capital Investments, LLC beneficially own a majority of our common stock. As a result, PBM Capital Investments, LLC can control, and these other persons, acting together, would be able to significantly influence all matters requiring stockholder approval, including the election and removal of directors, any merger, consolidation, sale of all or substantially all of our assets, or other significant corporate transactions.

Some of these persons or entities may have interests different than yours. For example, because many of these stockholders purchased their shares at prices substantially below the current market price of our common stock and have held their shares for a longer period, they may be more interested in selling our company to an acquirer than other investors, or they may want us to pursue strategies that deviate from the interests of other stockholders.

We are an "emerging growth company" and, as a result of the reduced disclosure and governance requirements applicable to emerging growth companies, our common stock may be less attractive to investors.

We are an "emerging growth company" as defined in the JOBS Act and we intend to take advantage of some of the exemptions from reporting requirements that are applicable to other public companies that are not emerging growth companies, including:

- being permitted to provide only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced "Management's discussion and analysis of financial condition and results of operations" disclosure;
- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- reduced disclosure obligations regarding executive compensation in our periodic reports, proxy statements and registration statements; and
- not being required to hold a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved.

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. We will remain an emerging growth company until the earlier of (1) the last day of the fiscal year (a) ending December 31, 2022, which is the end of the fiscal year following the fifth anniversary of the closing of our recent IPO, (b) in which we have total annual gross revenue of at least $1.07 billion, or (c) in which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeds $700 million as of the prior June 30th, and (2) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period.

Under Section 107(b) of the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies. As a result, changes in rules of U.S. generally accepted accounting principles or their interpretation, the adoption of new guidance or the application of existing guidance to changes in our business could significantly affect our financial position and results of operations.

If we fail to maintain proper and effective internal controls, our ability to produce accurate financial statements on a timely basis could be impaired.

We are subject to the reporting requirements of the Securities Exchange Act of 1934, as amended, or the Exchange Act, the

Sarbanes-Oxley Act and the rules and regulations of the NASDAQ Global Market. Section 302 of the Sarbanes-Oxley Act requires, among other things, that we report on the effectiveness of our disclosure controls and procedures in our quarterly and annual reports and, beginning with our fiscal year ending December 31, 2018, Section 404 of the Sarbanes-Oxley Act requires that we perform system and process evaluation and testing of our internal control over financial reporting to allow management to report on the effectiveness of our internal control over financial reporting in our Form 10-K filing for that year. This will require that we incur substantial additional professional fees and internal costs to expand our accounting and finance functions and that we expend significant management efforts. Prior to our IPO, we had never been required to test our internal control within a specified period, and, as a result, we may experience difficulty in meeting these reporting requirements in a timely manner.

We may identify weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our financial statements. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

If we are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act in a timely manner, or if we are unable to maintain proper and effective internal controls, we may not be able to produce timely and accurate financial statements. If that were to happen, the market price of our stock could decline and we could be subject to sanctions or investigations by the NASDAQ Global Market, the SEC or other regulatory authorities. In addition, our common stock may not be able to remain listed on the NASDAQ Global Market or any other securities exchange.

We have broad discretion in the use of proceeds from our recent IPO.

We have broad discretion over the use of proceeds from our recent IPO. We expect to use the net proceeds to us from our recent IPO, together with our existing cash and cash equivalents, to fund the commercialization of avatrombopag, if approved, to fund clinical trials of avatrombopag for additional indications beyond its initial indication, to repay a portion of our obligations under the Eisai note and for working capital and general corporate purposes. In addition, we may use a portion of the proceeds from our recent IPO to pursue our strategy to in-license or acquire additional drug candidates. Our failure to apply the net proceeds from our recent IPO effectively could compromise our ability to pursue our growth strategy and we might not be able to yield a significant return, if any, on our investment of these net proceeds.

We might not be able to utilize a significant portion of our net operating loss carryforwards.

As of December 31, 2016, we had federal and state net operating loss carryforwards of $21.9 million. The federal and state net operating loss carryforwards will begin to expire, if not utilized, by 2036. These net operating loss carryforwards could expire unused and be unavailable to offset future income tax liabilities. In addition, under Section 382 of the Internal Revenue Code of 1986, as amended, and corresponding provisions of state law, if a corporation undergoes an "ownership change," which is generally defined as a greater than 50% change, by value, in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes to offset its post-change income or taxes may be limited. In connection with our recent IPO, it is possible that we have experienced an ownership change limitation. We may experience ownership changes in the future as a result of subsequent shifts in our stock ownership, some of which may be outside of our control. If an ownership change occurs and our ability to use our net operating loss carryforwards is materially limited, it would harm our future operating results by effectively increasing our future tax obligations.

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

We have not declared or paid cash dividends on our common stock to date. We currently intend to retain our future earnings, if any, to fund the development and growth of our business. In addition, the terms of any existing or 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. Investors seeking cash dividends should not purchase our common stock.

We will incur increased costs and demands upon management as a result of being a public company.

As a public company listed in the United States, we will incur significant additional legal, accounting and other costs. These additional costs could negatively affect our financial results. In addition, changing laws, regulations and standards relating to corporate governance and public disclosure, including regulations implemented by the SEC and The NASDAQ Stock Market, may increase legal and financial compliance costs and make some activities more time-consuming. These laws, regulations and standards are subject to varying interpretations and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. We intend to invest resources to comply with evolving laws, regulations and standards, and this investment may result

in increased general and administrative expenses and a diversion of management's time and attention from revenue-generating activities to compliance activities. If notwithstanding our efforts to comply with new laws, regulations and standards, we fail to comply, regulatory authorities may initiate legal proceedings against us and our business may be harmed. Further, stockholder activism, the current political environment and the current high level of government intervention may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate.

Failure to comply with these rules might also make it more difficult for us to obtain some types of insurance, including director and officer liability insurance, and we might be forced to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. The impact of these events could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, on committees of our board of directors or as members of senior management. In addition, we expect the rules and regulations applicable to public companies to substantially increase our legal and financial compliance costs and to make some activities more time-consuming and costly. If these requirements divert the attention of our management and personnel from other business concerns, they could have a material adverse effect on our business, financial condition and results of operations. The increased costs will decrease our net income or increase our net loss, and may require us to reduce costs in other areas of our business or increase the prices of our drugs or services.

Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware is the exclusive forum for certain litigation that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware is the exclusive forum for (i) any derivative action or proceeding brought on our behalf, (ii) any action asserting a claim for breach of a fiduciary duty owed by any of our directors, officers or other employees to us or our stockholders, (iii) any action asserting a claim arising pursuant to any provision of the Delaware General Corporation Law, our amended and restated certificate of incorporation or our amended and restated bylaws or (iv) any action asserting a claim governed by the internal affairs doctrine. The choice of forum provision may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or other employees, which may discourage such lawsuits against us and our directors, officers and other employees. Stockholders who do bring a claim in the Court of Chancery could face additional litigation costs in pursuing any such claim, particularly if they do not reside in or near the State of Delaware. The Court of Chancery may also reach different judgments or results than would other courts, including courts where a stockholder considering an action may be located or would otherwise choose to bring the action, and such judgments or results may be more favorable to us than to our stockholders. Alternatively, if a court were to find the choice of forum provision contained in our amended and restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could adversely affect our business and financial condition.

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

(b) Use of IPO Proceeds

On June 28, 2017, our registration statement on Form S-1, as amended (File No 333-218479) was declared effective by the SEC in connection with our IPO, pursuant to which we sold 5,077,250 shares of common stock, $0.001 par value per share at a public offering price of $17.00 per share, including the full exercise by the underwriters of their option to purchase additional shares.

On July 5, 2017, we received net proceeds of $78.7 million, after deducting underwriting discounts and commissions and offering expenses borne by us. None of the expenses incurred by us were direct or indirect payments to any of (i) our directors or officers or their associates, (ii) persons owning 10 percent or more of our common stock, or (iii) our affiliates. The joint book-running underwriters of the IPO were J.P. Morgan Securities LLC, Jefferies LLC and Leerink Partners LLC.

There has been no material change in the planned use of proceeds from our IPO from that described in the final prospectus related to the offering, dated June 28, 2017, as filed with the SEC on June 30, 2017.

Table of Contents

Item 6. Exhibits

Exhibit No.	Description
3.1(1)	Amended and Restated Certificate of Incorporation of Dova Pharmaceuticals, Inc.
3.2(2)	Amended and Restated Bylaws of Dova Pharmaceuticals, Inc.
10.1#	Amendment to Sublease, by and between the Registrant and Paidian Research, Inc., dated as of September 22, 2017.
31.1#	Certification of Principal Executive Officer of Dova Pharmaceuticals, Inc. pursuant to Rule 13a-14(a)/15d-14(a), as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2#	Certification of Principal Financial Officer of Dova Pharmaceuticals, Inc. pursuant to Rule 13a-14(a)/15d-14(a), as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1#*	Certifications of Principal Executive Officer and Principal Financial Officer of Dova Pharmaceuticals, Inc. pursuant to 18 U.S.C. §1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101#	The following financial information from the Company's Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2017, formatted in Extensible Business Reporting Language (XBRL): (i) the Condensed Consolidated Balance Sheets, (ii) the Condensed Consolidated Statements of Operations, (iii) the Condensed Consolidated Statement of Stockholders' Equity, (iv) the Condensed Consolidated Statements of Cash Flows, and (v) Notes to the Condensed Consolidated Financial Statements (filed herewith).

(1) Previously filed as Exhibit 3.3 to Amendment No. 1 to the Company's Registration Statement on Form S-1 (File No. 333-218479), filed with the Commission on June 9, 2017, and incorporated by reference herein.
(2) Previously filed as Exhibit 3.4 to Amendment No. 1 to the Company's Registration Statement on Form S-1 (File No. 333-218479), filed with the Commission on June 9, 2017, and incorporated by reference herein.
Filed herewith.
* These certifications are being furnished solely to accompany this quarterly report pursuant to 18 U.S.C. Section 1350, and are not being filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and are not to be incorporated by reference into any filing of the registrant, whether made before or after the date hereof, regardless of any general incorporation language in such filing.

SIGNATURES

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

Dova Pharmaceuticals, Inc.

Date: November 9, 2017

By: /s/ Alex Sapir
Alex Sapir
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 10.1

FIRST AMENDMENT TO SUBLEASE AGREEMENT

THIS FIRST AMENDMENT TO SUBLEASE AGREEMENT (the "Amendment") is made effective this 22nd day of September 2017 by and between **Paidion Research, Inc.** a North Carolina corporation ("Sublessor") and **Dova Pharmaceuticals, Inc.**, a Delaware corporation ("Sublessee");

WHEREAS, Sublessor and Sublessee entered into that certain Sublease Agreement dated May, 2017 (as amended hereby, the "Sublease") under which Sublessee subleased from Sublessor a certain portion of Suites 245 and 250 on the second floor of the Palladian II building at 240 Leigh Farm Road, Durham, North Carolina, consisting of approximately 7,351 square feet of space, as more specifically described in the Sublease (the "Initial Subleased Premises") that Sublessor leases from Landlord under that certain Office Lease Agreement dated April 10, 2014 by and between Sublessor, as tenant, and Palladian Center, LLC**,** a Delaware limited liability company ("Landlord"), (as amended by First Amendment to Lease dated September 18, 2015, the "Master Lease"); and

WHEREAS, Sublessor and Sublessee desire to amend the Sublease (i) to enlarge the Initial Subleased Premises by adding the remaining portion of Suite 250 that Sublessor leases from Landlord under the Master Lease that was not subleased to Sublessee under the Sublease (the "Additional Subleased Premises") so that the total subleased premises under the Sublease shall be the entire 14,378 rentable square feet of space leased to Sublessor under the Master Lease (as enlarged and amended by such Additional Subleased Premises, and as shown on Exhibit A hereto, the "Subleased Premises") and (ii) to adjust the Minimum Rental under the Sublease to account for the increase in the size of the Subleased Premises;

NOW THEREFORE, for good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the parties hereto agree as follows:

1. Recitals; Definitions. The recitals set out above are true and accurate and are incorporated herein by this reference. All capitalized terms used, but not defined, in this Amendment shall have the meanings given to such terms in the Sublease and/or the Master Lease, as applicable.

2. Amendment to Premises. Commencing on October 1, 2017, Sublessor hereby subleases to Sublessee, and Sublessee hereby accepts and subleases from Sublessor, the Additional Subleased Premises so that the total Subleased Premises under the Sublease shall consist of the approximately 14,378 rentable square feet of space shown on Exhibit A hereto. Sublessor shall have until 11:59 P.M. on September 30, 2017 to vacate the Additional Subleased Premises. Sublessor acknowledges and agrees, with the increase in Subleased Premises pursuant to this Amendment, all parking rights leased to it through the Master Lease are now subleased to Sublessee pursuant to Section 2 of the Sublease.

3. Term of Sublease; Minimum Rental. The Sublease Term will remain unchanged and shall end with the expiration of the Master Lease on April 30, 2020. However, the monthly Minimum Rental under the Sublease is amended as follows:

October 1, 2017 to January 31, 2018	$12,582.46 per month
February 1, 2018 to May 31, 2018	$24,610.34 per month
June 1, 2018 to May 31, 2019	$25,329.24 per month
June 1, 2019 to April 30, 2020	$26,072.11 per month

4. "AS IS" Delivery; No Demising Wall; Signage. Sublessor shall deliver the Additional Subleased Premises to Sublessee on October 1, 2017 in its "AS IS" condition, with no representations or warranties and with no Landlord upfit obligations. Sublessor's obligation to construct the Demising Wall under Section 9 of the Sublease is hereby deleted, as is Sublessee's obligation to reimburse Sublessor for certain costs in connection therewith. Notwithstanding the deletion of terms regarding the Demising Wall from Section 9 of the Sublease, all rights of Sublessee set forth in such Section regarding signage shall remain. The last sentence of Section 9 of the Sublease is hereby amended by deleting the clause "after the Demising Wall has been completed," such that the rights described in such sentence shall begin immediately upon execution of this Amendment. Furthermore, to the extent that Sublessor has any rights under the Master Lease regarding signage that are not already assigned to Sublessee pursuant to the Sublease, any such rights are hereby assigned to Sublessee in their entirety. Sublessee acknowledges that Landlord may have approval rights with respect to signage under the Master Lease.

5. Landlord Consent; Condition to Amendment. It shall be a condition precedent to the effectiveness of this Amendment that Landlord shall have consented to this Amendment, which consent may be given by Landlord's signature below or in a separate consent form from Landlord, at Landlord's election.

6. Effect of Amendment. Except to the extent modified hereby, the Sublease remains in full force and effect as written. To the extent of any conflict between the Sublease and this Amendment, this Amendment shall control.

[SIGNATURE PAGE FOLLOWS]

IN WITNESS WHEREOF, the parties have caused this Amendment to be executed by their duly authorized representatives, effective as of the date set out above.

SUBLESSOR:

PAIDION RESEARCH, INC.

By:	/s/ Barry Mangum
Printed Name:	Barry Mangum
Title:	CEO

SUBLESSEE:

DOVA PHARMACEUTICALS, INC.

By:	/s/ Douglas Blankenship
Printed Name:	Douglas Blankenship
Title:	CFO

CONSENTED TO BY LANDLORD:

SUBLESSOR AND SUBLESSEE AGREE BY THEIR SIGNATURES ABOVE THAT LANDLORD IS NOT A PARTY TO THIS AMENDMENT OR TO THE SUBLEASE AND THAT LANDLORD, IN CONSENTING TO THIS AMENDMENT BY ITS SIGNATURE BELOW, PROVIDES SUCH CONSENT PURSUANT TO THE SAME TERMS AND CONDITIONS AS ARE CONTAINED IN THAT CERTAIN "CONSENT TO SUBLEASE" BY AND AMONG LANDLORD, SUBLESSOR AND SUBLESSEE DATED JUNE 9, 2017, THE TERMS AND CONDITIONS OF WHICH ARE INCORPORATED INTO THIS CONSENT PAGE BY THIS REFERENCE.

PALLADIAN CENTER, LLC

By:	/s/ James L Dean II
Printed Name:	James L Dean II
Title:	Vice President
Date:	9/22/17

EXHIBIT A

SUBLEASED PREMISES

Exhibit 31.1

CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER
PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Alex Sapir, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the period ended September 30, 2017 of Dova Pharmaceuticals, Inc. (the "registrant");

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)) 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) 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

   (c) 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(s) 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 functions):

   (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: November 9, 2017

/s/ Alex Sapir
Alex Sapir
President and Chief Executive Officer
(principal executive officer)

Exhibit 31.2

CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER
PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Douglas Blankenship, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q for the period ended September 30, 2017 of Dova Pharmaceuticals, Inc. (the "registrant");

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)) 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) 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

   (c) 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(s) 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 functions):

   (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: November 9, 2017

/s/ Douglas Blankenship
Douglas Blankenship
Chief Financial Officer
(principal financial officer)

Exhibit 32.1

CERTIFICATIONS OF
PRINCIPAL EXECUTIVE OFFICER AND PRINCIPAL FINANCIAL OFFICER
PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

Pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended, (the "Exchange Act") and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350), Alex Sapir, President and Chief Executive Officer of Dova Pharmaceuticals, Inc. (the "Company"), and Douglas Blankenship, Chief Financial Officer of the Company, each hereby certifies that, to the best of his knowledge:

1. The Company's Quarterly Report on Form 10-Q for the period ended September 30, 2017, to which this Certification is attached as Exhibit 32.1 (the "Periodic Report"), fully complies with the requirements of Section 13(a) or Section 15(d) of the Exchange Act; and

2. The information contained in the Periodic 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 9th day of November, 2017.

/s/ Alex Sapir	/s/ Douglas Blankenship
Alex Sapir	Douglas Blankenship
President and Chief Executive Officer	Chief Financial Officer
(principal executive officer)	(principal 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 the Company under the Securities Act of 1933, as amended, or the Exchange Act (whether made before or after the date of the Form 10-Q), irrespective of any general incorporation language contained in such filing.