

The information in this preliminary prospectus is not complete and may be changed. These securities may not be sold until the registration statement filed with the Securities and Exchange Commission is effective. This preliminary prospectus is not an offer to sell nor does it seek an offer to buy these securities in any jurisdiction where the offer or sale is not permitted.

Subject to completion, dated June 19, 2017

Prospectus

**4,062,500 shares**



## **Common Stock**

This is an initial public offering of shares of common stock by Dova Pharmaceuticals, Inc. We are selling 4,062,500 shares of our common stock. The estimated initial public offering price is between \$15.00 and \$17.00 per share.

Prior to this offering, there has been no public market for our common stock. We have applied for listing of our common stock on the NASDAQ Global Market under the symbol "DOVA."

We are an "emerging growth company" as defined under the federal securities laws and will be subject to reduced public company reporting requirements.

|   | <b>Per share</b> | <b>Total</b> |
|---|------------------|--------------|
| Initial public offering price                           | \$               | \$           |
| Underwriting discounts and commissions(1)               | \$               | \$           |
| Proceeds to Dova Pharmaceuticals, Inc., before expenses | \$               | \$           |

(1) We have also agreed to reimburse the underwriters for certain FINRA-related expenses. See "Underwriting" for a description of all compensation payable to the underwriters.

We have granted the underwriters an option for a period of 30 days to purchase up to 609,375 additional shares of common stock on the same terms and conditions set forth above.

Investing in our common stock involves a high degree of risk. See "Risk factors" beginning on page 12.

Neither the Securities and Exchange Commission nor any other regulatory body has approved or disapproved of these securities or passed upon the accuracy or adequacy of this prospectus. Any representation to the contrary is a criminal offense.

Certain of our existing stockholders and their affiliated entities and certain of our directors have indicated an interest in purchasing an aggregate of approximately \$25 million in shares of our common stock in this offering at the initial public offering price per share. However, because indications of interest are not binding agreements or commitments to purchase, the underwriters may determine to sell more, less or no shares in this offering to any of these persons or entities, or any of these persons or entities may determine to purchase more, less or no shares in this offering. The underwriters will receive the same underwriting discount on any shares purchased by these persons or entities as they will on any other shares sold to the public in this offering.

The underwriters expect to deliver the shares in New York, New York against payment to investors on or about \_\_\_\_\_, 2017.

**J.P. Morgan**

**Jefferies**

**Leerink Partners**

Prospectus dated

\_\_\_\_\_, 2017



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Neither we nor the underwriters have authorized anyone to provide you with information other than that contained in this prospectus or any free writing prospectus prepared by or on behalf of us or to which we have referred you. We and the underwriters take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you. We and the underwriters are offering to sell, and seeking offers to buy, common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus is accurate only as of the date on the front cover page of this prospectus, or other earlier date stated in this prospectus, regardless of the time of delivery of this prospectus or of any sale of our common stock.

No action is being taken in any jurisdiction outside the United States to permit a public offering of our common stock or possession or distribution of this prospectus in that jurisdiction. Persons who come into possession of this prospectus in jurisdictions outside the United States are required to inform themselves about and to observe any restrictions as to this offering and the distribution of this prospectus applicable to that jurisdiction.



## Prospectus summary

*This summary highlights information contained elsewhere in this prospectus. This summary does not contain all of the information you should consider before investing in our common stock. You should read this entire prospectus carefully, especially the “Risk factors” section beginning on page 12 and our consolidated financial statements and the related notes appearing at the end of this prospectus, before making an investment decision.*

*As used in this prospectus, unless the context otherwise requires, references to “we,” “us,” “our,” “the company” and “Dova Pharmaceuticals” refer to Dova Pharmaceuticals, Inc. and our wholly-owned subsidiary, AkaRx, Inc.*

### 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, undergoing non-emergent minimally to moderately invasive medical procedures. 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, is planned for submission to the U.S. Food and Drug Administration, or FDA, for this initial indication in the third quarter of 2017.

We believe that avatrombopag’s efficacy and safety profile in combination with its convenient oral dosing could provide advantages over other treatments for patients with thrombocytopenia. We believe avatrombopag’s pharmacokinetic, or PK, profile and pharmacodynamic, or PD, profile as well as its metabolic characteristics are the core attributes that differentiate it from the currently marketed TPO-RAs and make it a compelling treatment option for patients with thrombocytopenia. To date, avatrombopag has been evaluated in more than 20 clinical trials involving more than 1,100 subjects and has been observed to be generally well tolerated. We believe that avatrombopag may, therefore, have the potential to be used more broadly for patients with thrombocytopenia, including patients without CLD, and we are exploring regulatory and clinical development strategies that would support this expanded use.

### Thrombocytopenia and current treatments

Thrombocytopenia is characterized by a deficiency of platelets that impairs blood clot formation and increases bleeding risk. Thrombocytopenia is defined as having less than 150,000 platelets per microliter of circulating blood and is diagnosed with a routine blood test. Thrombocytopenia can result in significant bleeding risk even in cases of minor injury and increases the risk of excessive, uncontrolled bleeding during or after a medical procedure. Physicians determine how to treat thrombocytopenia, either in the acute setting prior to a medical procedure or chronically, based on a number of factors, including the patient’s platelet count, etiology of the underlying cause of thrombocytopenia, duration of required platelet count elevation and the patient’s overall health profile.

Our initial indication targets the acute treatment of thrombocytopenia in patients with CLD prior to minimally to moderately invasive medical procedures. CLD involves the progressive destruction and

regeneration of the liver over a period of more than six months. Patients with CLD have reduced platelet production when liver cell mass becomes severely damaged. In addition, these patients also have increased trapping of platelets in the spleen and thus even fewer platelets are present in circulating blood. In both instances, these patients often develop thrombocytopenia. Approximately 1.1 million CLD patients in the United States are affected by thrombocytopenia.

Patients with CLD undergo numerous non-emergent medical procedures for diagnosis and treatment of their disease, including liver biopsies, fluid removal, liver transplantation and endoscopy. Multiple medical professional associations have guidelines that recommend that patients have at least 50,000 platelets per microliter of circulating blood prior to minimally to moderately invasive medical procedures. Approximately 70,000 CLD patients in the United States have a platelet count less than 50,000 platelets per microliter of circulating blood.

Prophylactic platelet transfusion is currently the standard of care for patients who need to increase their platelet count prior to a medical procedure. Despite being the standard of care, platelet transfusions are associated with limitations that impact their use, including risk of antibody development in up to 50% of patients, short duration of effect of transfused platelets, limited supply and inconvenience of administration. There is no drug treatment approved by the FDA or the European Medicines Agency, or EMA, for thrombocytopenia in the acute setting prior to a medical procedure.

Chronic treatment of thrombocytopenia involves continuous treatment of the disorder. The substantial majority of patients who require chronic treatment suffer from immune thrombocytopenic purpura, or ITP. We estimate that chronic ITP affects approximately 60,000 adults in the United States. First-line therapy for ITP consists of corticosteroids or intravenous immunoglobulin, or IVIG. In addition to off-label rituximab and splenectomy, currently marketed TPO-RAs are used as a second-line treatment of ITP. However, we believe these available treatments have limitations that impact their use, such as limited efficacy, risk to patient safety, patient non-compliance or inconvenience.

Because of the limitations of current therapies used for thrombocytopenia in the acute and chronic setting, we believe there remains a significant unmet need for a treatment that demonstrates reliable and durable effectiveness and a favorable safety profile, that can be conveniently administered and potentially reduce the burden on patients.

## **Our drug candidate**

We believe our drug candidate, avatrombopag, has the potential to be a first-in-class drug treatment of thrombocytopenia in the acute setting and a best-in-class treatment of thrombocytopenia in the chronic setting. Avatrombopag is an orally administered, small molecule TPO-RA, which is intended to address the limitations of other existing treatments for thrombocytopenia. We recently completed two identically designed Phase 3 pivotal clinical trials, ADAPT 1 and ADAPT 2, in which all primary and secondary endpoints were met with high statistical significance. The primary endpoint for both studies was the percentage of CLD patients with thrombocytopenia undergoing a non-emergent minimally to moderately invasive medical procedure, who did not require a platelet transfusion or any rescue procedure for bleeding at each of two dose strengths of avatrombopag compared to placebo. In each trial, the percentage of subjects in each of the two avatrombopag dosing cohorts requiring a platelet transfusion or a rescue procedure for bleeding was statistically significantly lower compared to placebo (across all cohorts, p-values ranging from  $p < 0.0001$  to  $p = 0.0006$ ). We also observed a percentage of avatrombopag-treated subjects who achieved platelet counts of greater than 50,000 platelets per microliter of circulating blood on the procedure day and changes in platelet counts from baseline to procedure day, which were

statistically significant improvements over placebo. We are initially developing avatrombopag for the acute treatment of thrombocytopenia in this population of patients with CLD undergoing non-emergent minimally to moderately invasive medical procedures.

Avatrombopag is designed to mimic the effects of thrombopoietin, or TPO, *in vitro* and *in vivo*. TPO is a hormone produced in the liver and kidney that binds to its receptor, c-Mpl (myeloproliferative leukemia). Following TPO receptor binding, intracellular signaling leads to megakaryocyte growth and maturation, which results in increased platelet production. TPO-RAs, like TPO, stimulate the activation, proliferation and maturation of megakaryocytes, resulting in an increase in circulating platelet counts. Avatrombopag is a highly specific TPO-RA as it binds to the TPO receptor at a distinct site from native TPO, leaving the TPO receptor accessible to native TPO, enabling avatrombopag to have an additive effect on platelet production.

While TPO-RAs are a validated class of therapy for the chronic treatment of thrombocytopenia, they have not been approved for the acute treatment of thrombocytopenia due, in part, to the risk of side effects, including portal vein thrombosis, or PVT. In CLD patients, who often have excessive accumulation of scar tissue in the liver, portal blood flow may be significantly lower than normal putting the patient at an increased risk of developing PVT. Further, the use of some TPO-RAs may lead to an even greater risk of PVT in these patients as a sudden increase in platelets can give rise to platelet accumulation and cause further blockage of the portal vein.

We believe avatrombopag's PK/PD profile and metabolic characteristics are the attributes that differentiate it from the currently marketed TPO-RAs and make it a compelling treatment option for patients with thrombocytopenia in the acute setting. Avatrombopag has been observed to have a less variable PK/PD profile than other TPO-RAs. In addition, avatrombopag is not extensively metabolized—approximately 40% to 50% is metabolized and is mostly eliminated from the biliary route. We believe these metabolic characteristics and this PK/PD profile further reduce the risk of adverse effects, including thromboembolic events such as PVTs, in patient populations that are liver compromised, such as those with CLD.

To date, avatrombopag has been evaluated in more than 20 clinical trials involving more than 1,100 subjects and has been observed to be generally well tolerated. Based on the results of our clinical trials, we also believe avatrombopag has the potential for use in a broader population of thrombocytopenia patients regardless of disease etiology undergoing a broader set of medical procedures, including, for example, joint replacements. It also has the potential to treat patients who develop thrombocytopenia after receiving chemotherapy. In addition, we are evaluating the potential regulatory approval pathway for avatrombopag for the treatment of adults with chronic ITP based on results from a completed Phase 3 trial in this patient population.

We hold the worldwide rights to avatrombopag for all current and future indications. Our owned and in-licensed patents provide us with composition of matter and method of use exclusivity with respect to avatrombopag in the United States, including a composition of matter patent that expires in 2025, with possible patent term extension, if approved, up to 2030.

The following table summarizes our lead development programs:

| Avatrombopag: Multiple Indications for Various Types of Thrombocytopenia  |  |         |         |         |   |
|---|--|---------|---------|---------|---|
|   | Preclinical  | Phase 1 | Phase 2 | Phase 3 | Next expected milestone                                   |
| <b>Acute treatment of thrombocytopenia</b>  |  |         |         |         |   |
| Thrombocytopenia in CLD patients scheduled to undergo a medical procedure <sup>(1)</sup>                              | [Progress bar spanning Preclinical, Phase 1, Phase 2, and Phase 3] |         |         |         | File NDA in Q3 2017                                       |
| Thrombocytopenia in broader population of patients scheduled to undergo a medical procedure or surgery <sup>(2)</sup> | [Progress bar spanning Preclinical, Phase 1, and Phase 2]          |         |         |         | Initiate later stage clinical development program in 2018 |
| Chemotherapy-Induced Thrombocytopenia (CIT)   | [Progress bar spanning Preclinical, Phase 1, and Phase 2]          |         |         |         | Initiate later stage clinical development program in 2018 |
| <b>Chronic treatment of thrombocytopenia</b>  |  |         |         |         |   |
| Immune Thrombocytopenic Purpura (ITP)   | [Progress bar spanning Preclinical, Phase 1, Phase 2, and Phase 3] |         |         |         | Meet with FDA to discuss prior clinical trial results     |

<sup>1</sup> Medical procedure refers to a non-emergent, minimally to moderately invasive medical procedure.  
<sup>2</sup> Surgery includes spectrum of minimally invasive to highly invasive medical procedures. Highly invasive surgeries are those for which multiple medical professional association guidelines recommend that patients have greater than 100,000 platelets per microliter of circulating blood.

Our management team has extensive experience ranging from identifying and acquiring drug candidates, drug development and global registrations through global commercial launches. We are also being supported by a leading group of biotech investors including PBM Capital, Perceptive Advisors and Paulson & Company, Inc.

## Our strategy

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. To achieve our goals, we are pursuing the following strategies:

- **Advance the development of our late-stage drug candidate, avatrombopag, for regulatory approval in the United States and Europe.** In the first quarter of 2017, we completed two identically designed pivotal Phase 3 clinical trials for avatrombopag in patients with CLD undergoing a non-emergent minimally to moderately invasive medical procedure. Based on the results of these trials, an NDA for avatrombopag is planned for submission to the FDA in the third quarter of 2017. In addition, as our Phase 3 trials were also designed to be pivotal trials in Europe, we intend to submit a marketing authorization application to the EMA in the first half of 2018.
- **Maximize the commercial potential of avatrombopag.** Our intent is to initially build a hepatology-focused sales organization in the United States. We have begun to execute this strategy by hiring key executives with global commercial launch experience. In the future, we also may selectively partner with leading companies that we believe can contribute additional resources and know-how for the development and commercialization of avatrombopag for additional indications and geographic regions, further enhancing the value of our drug candidate.
- **Expand the breadth of indications for avatrombopag in other patient populations with thrombocytopenia.** Based on the results from our Phase 2 and Phase 3 clinical trials, we also believe avatrombopag has the potential for use in a broader population of thrombocytopenia patients regardless



of disease etiology undergoing a broader set of medical procedures, including, for example, joint replacements. We believe it may also have the potential to treat patients who develop thrombocytopenia after receiving chemotherapy. In addition, we are evaluating the potential regulatory approval pathway for avatrombopag for the treatment of adults with chronic ITP based on results from a completed Phase 3 trial in this patient population.

- **Employ a value-driven approach to build a pipeline of drug candidates.** Using a similar approach to our identification and acquisition of avatrombopag, we intend to employ a value-driven strategy to identify, acquire, develop and commercialize drug candidates for diseases that are treated by specialist physicians.
- **Maintain and strengthen our intellectual property portfolio.** Our intellectual property strategy aims to protect and control the development and commercialization of our drug candidates. Our owned and in-licensed patents for avatrombopag provide us with composition of matter and method of use exclusivity with respect to avatrombopag in the United States, including a composition of matter patent that expires in 2025, with possible patent term extension up to 2030. We also hold patents and applications in major world markets with respect to avatrombopag, which are projected to expire between 2023 and 2027, excluding any extension of patent term that may be available in a particular country. We will seek to broaden the scope of and increase the geographic reach of our patent protection throughout the world.

### **Risks associated with our business**

Our ability to implement our business strategy is subject to numerous risks that you should be aware of before making an investment decision. These risks are described more fully in the section entitled “Risk factors,” immediately following this prospectus summary. These risks include the following, among others:

- We have a limited operating history and have never generated any product revenues. We expect to incur losses over the next several years and may never achieve or maintain profitability.
- 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 our only current drug candidate, avatrombopag and any other potential drug candidates in the future.
- We may be 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.
- Our consolidated financial statements have been prepared assuming that we will continue as a going concern.
- We are heavily dependent on the success of avatrombopag and if avatrombopag does not receive regulatory approval or is not successfully commercialized, our business will be harmed.
- 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.
- 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.

- 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.
- 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 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 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.
- 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 products similar or identical to ours, which could have a material adverse effect on our ability to successfully commercialize our technology and drug candidates.
- Concentration of ownership of our common stock among our existing executive officers, directors and principal stockholders, including PBM Capital Investments, LLC, may prevent new investors from influencing significant corporate decisions.

### **Implications of being an emerging growth company**

We qualify as an “emerging growth company” as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. An emerging growth company may take advantage of relief from certain reporting requirements and other burdens that are otherwise applicable generally to public companies. These provisions include:

- an exception from compliance with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, as amended, or the Sarbanes-Oxley Act;
- reduced disclosure about our executive compensation arrangements in our periodic reports, proxy statements and registration statements; and
- exemptions from the requirements of holding non-binding advisory votes on executive compensation or golden parachute arrangements.

We may take advantage of these provisions for up to five years or such earlier time that we no longer qualify as an emerging growth company. We would cease to qualify as an emerging growth company if we have more than \$1.07 billion in annual revenue, we are deemed to be a “large accelerated filer” under the rules of the U.S. Securities and Exchange Commission, or SEC, which means the market value of our common stock that is held by non-affiliates exceeds \$700 million as of the prior June 30th or we issue more than \$1.0 billion of non-convertible debt over a three-year period. We may choose to take advantage of some but not all of these reduced reporting burdens. For example, we may take advantage of the exemption from auditor attestation on the effectiveness of our internal control over financial reporting. To the extent that we take advantage of these reduced reporting burdens, the information that we provide stockholders may be different than you might obtain from other public companies in which you hold equity interests.

In addition, under 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.

### **Our corporate information**

We were originally formed as a limited liability company under the laws of the state of Delaware in March 2016 under the name PBM AKX Holdings, LLC. In June 2016, we amended our certificate of formation to change our name to Dova Pharmaceuticals, LLC. In September 2016, we converted from a limited liability company to a corporation, Dova Pharmaceuticals, Inc. Our principal executive offices are located at 240 Leigh Farm Road, Suite 245, Durham, NC 27707, and our telephone number is (844) 506-3682. Our website address is [www.dova.com](http://www.dova.com). The information contained in, or accessible through, our website is not incorporated by reference into this prospectus, and you should not consider any information contained in, or that can be accessed through, our website as part of this prospectus or in deciding whether to purchase our common stock.

We own various U.S. federal trademark applications and unregistered trademarks, including our company name. All other trademarks or trade names referred to in this prospectus are the property of their respective owners. Solely for convenience, the trademarks and trade names in this prospectus are referred to without the symbols ® and ™, but such references should not be construed as any indication that their respective owners will not assert, to the fullest extent under applicable law, their rights thereto.

## The offering

|  |  |
|--|--|
| Common stock offered by us . . . . .                         | 4,062,500 shares   |
| Common stock to be outstanding after this offering . . . . . | 24,637,707 shares  |
| Option to purchase additional shares . .                     | The underwriters have a 30-day option to purchase a maximum of 609,375 additional shares of common stock from us at the public offering price, less underwriting discounts and commissions, on the same terms as set forth in this prospectus.   |
| Use of proceeds . . . . .                                    | We estimate that the net proceeds from the sale of the shares of common stock in this offering will be approximately \$58.6 million, or approximately \$67.6 million if the underwriters exercise their option to purchase additional shares in full, assuming an initial public offering price of \$16.00 per share (which is the midpoint of the price range set forth on the cover page of this prospectus), after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. We intend to use the net proceeds from this offering, together with our existing cash and cash equivalents, as follows: (i) approximately \$19.0 million to fund the commercialization of avatrombopag, if approved, (ii) approximately \$21.0 million to fund clinical trials of avatrombopag for additional indications beyond its initial indication, (iii) approximately \$30.0 million as repayment to Eisai of a portion of our obligations under the Eisai note and (iv) the balance for other general corporate purposes, including general and administrative expenses and working capital. See “Use of proceeds” beginning on page 62. |
| Directed share program . . . . .                             | At our request, the underwriters have reserved for sale at the initial public offering price per share up to 406,250 shares of our common stock, or 10% of the shares of common stock offered pursuant to this prospectus, to certain individuals through a directed share program, including employees, directors and other persons associated with us. If purchased by these persons, these shares will not be subject to a lock-up restriction, except in the case of shares purchased by any director or officer, which will be subject to a 180-day lock-up restriction described under “Underwriting” elsewhere in this prospectus. The number of shares of common stock available for sale to the general public will be reduced by the number of reserved shares sold to these individuals. Any reserved shares not purchased by these individuals will be offered by the underwriters to the general public on the same basis as the other shares of common stock offered pursuant to this prospectus. See “Underwriting” beginning on page 166.  |
| Risk factors . . . . .                                       | See “Risk factors” beginning on page 12 and the other information included in this prospectus for a discussion of factors you should consider carefully before deciding to invest in our common stock.   |

Proposed NASDAQ Global Market  
symbol . . . . . “DOVA”

The number of shares of our common stock to be outstanding after this offering is based on 20,575,207 shares of our common stock (including preferred stock on an as-converted basis) outstanding as of March 31, 2017 and excludes:

- 1,332,375 shares of common stock issuable upon exercise of stock options awarded as of March 31, 2017 pursuant to our 2017 Equity Incentive Plan, or the 2017 Plan, at a weighted average exercise price of \$3.73 per share;
- 393,366 shares of our common stock issuable under exercise of stock options awarded after March 31, 2017 pursuant to our 2017 Plan at a weighted average exercise price of \$4.72 per share; and
- a maximum of 4,285,250 shares of common stock reserved for future issuance under the Amended and Restated 2017 Equity Incentive Plan, or the IPO Plan, to be adopted in connection with this offering. The maximum number of shares reserved for future issuance under the IPO Plan includes 2,000,000 new shares of common stock reserved for issuance under the IPO Plan plus up to 2,285,250 shares of common stock reserved for issuance or issuable upon the exercise of the stock options awarded under the 2017 Plan that could become available under the IPO Plan upon cancellation, forfeiture or non-issuance of such shares after effectiveness of the IPO Plan.

Unless otherwise indicated, this prospectus reflects and assumes the following:

- the automatic conversion of all outstanding shares of our preferred stock on a 3.3-for-one basis into 3,242,950 shares of our common stock, which will occur immediately prior to the closing of this offering;
- no exercise of outstanding options after March 31, 2017;
- the filing of our amended and restated certificate of incorporation and the adoption of our amended and restated bylaws, which will occur upon the closing of this offering;
- a 3.3-for-one forward stock split for our common stock effected on June 16, 2017; and
- no exercise by the underwriters of their option to purchase additional shares of our common stock.

Certain of our existing stockholders and their affiliated entities and certain of our directors have indicated an interest in purchasing an aggregate of approximately \$25 million in shares of our common stock in this offering at the initial public offering price per share. However, because indications of interest are not binding agreements or commitments to purchase, the underwriters may determine to sell more, less or no shares in this offering to any of these persons or entities, or any of these persons or entities may determine to purchase more, less or no shares in this offering. The underwriters will receive the same underwriting discount on any shares purchased by these persons or entities as they will on any other shares sold to the public in this offering.

## Summary consolidated financial data

The following tables set forth, for the periods and as of the dates indicated, our summary financial data. The consolidated statement of operations data for the period from March 24, 2016 (inception) through December 31, 2016 is derived from our audited consolidated financial statements appearing elsewhere in this prospectus. The consolidated statements of operations data for the period from March 24, 2016 through March 31, 2016 and for the three months ended March 31, 2017 and the consolidated balance sheet as of March 31, 2017 are derived from our unaudited consolidated financial statements appearing elsewhere in this prospectus. We have prepared the unaudited condensed consolidated financial statements on the same basis as the audited consolidated financial statements, and the unaudited financial data include, in our opinion, all adjustments consisting only of normal recurring adjustments that we consider necessary for a fair presentation of our consolidated financial position and results of operations for these periods. You should read this data together with our consolidated financial statements and related notes included elsewhere in this prospectus and the information under the captions "Selected consolidated financial data" and "Management's discussion and analysis of financial condition and results of operations." Our historical results are not necessarily indicative of our future results and our operating results for the three months ended March 31, 2017 are not necessarily indicative of the results that may be expected for the entire year ending December 31, 2017.

|   | Period from<br>March 24,<br>2016 (inception) to<br>December 31, 2016 | Period from<br>March 24,<br>2016 (inception) to<br>March 31, 2016 | Three Months<br>Ended<br>March 31, 2017 |
|---|--|---|---|
| (in thousands, except share and per share data)   |  |   |   |
| <b>Statement of Operations Data:</b>  |  |   |   |
| Expenses:   |  |   |   |
| Research and development expenses . . . . .   | \$ 20,842  | \$ 150  | \$ 4,276                                |
| Research and development expenses—licenses<br>acquired . . . . .                            | 5,000  | 5,000   | —                                       |
| General and administrative expenses . . . . .   | 1,201  | 12  | 955                                     |
| Total operating expenses . . . . .  | <u>27,043</u>  | <u>5,162</u>  | <u>5,231</u>                            |
| Loss from operations . . . . .  | (27,043)   | (5,162)   | (5,231)                                 |
| Other expense, net . . . . .  | (147)  | —   | (193)                                   |
| Net loss . . . . .  | <u>\$ (27,190)</u>   | <u>\$ (5,162)</u>   | <u>\$ (5,424)</u>                       |
| Basic and diluted net loss per common share . . .   | <u>\$ (1.57)</u>   | <u>\$ (0.31)</u>  | <u>\$ (0.31)</u>                        |
| Weighted-average basic and diluted common<br>shares . . . . .                               | <u>17,332,257</u>  | <u>16,500,000</u>   | <u>17,332,257</u>                       |
| Pro forma basic and diluted net loss per<br>common share (unaudited)(1) . . . . .           | <u>\$ (1.32)</u>   |   | <u>\$ (0.26)</u>                        |
| Pro forma basic and diluted weighted-average<br>shares outstanding (unaudited)(1) . . . . . | <u>20,575,207</u>  |   | <u>20,575,207</u>                       |

(1) See Note 2 to our consolidated financial statements included elsewhere in this prospectus for an explanation of the method used to calculate the historical and pro forma basic and diluted net loss per common share.

The following table presents our summary balance sheet data as of March 31, 2017:

- on an actual basis;
- on a pro forma basis to give effect to the automatic conversion of all outstanding shares of our preferred stock on a 3.3-for-one basis into an aggregate of 3,242,950 shares of common stock, which will occur immediately prior to the closing of this offering; and
- on a pro forma as adjusted basis to give further effect to our issuance and sale of 4,062,500 shares of common stock in this offering at an assumed initial public offering price of \$16.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

| (in thousands)                          | As of March 31, 2017 |           |                                      |
|---|----------------------|-----------|--------------------------------------|
|   | Actual               | Pro forma | Pro forma as adjusted<br>(unaudited) |
| <b>Consolidated Balance Sheet Data:</b> |                      |           |                                      |
| Cash and cash equivalents . . . . .     | \$26,645             | \$26,645  | \$ 85,195                            |
| Working capital . . . . .               | \$ 1,202             | \$ 1,202  | \$ 59,752                            |
| Total assets . . . . .                  | \$26,840             | \$26,840  | \$85,390                             |
| Note payable, short-term . . . . .      | \$ 20,537            | \$ 20,537 | \$ 20,537                            |
| Total liabilities . . . . .             | \$25,469             | \$25,469  | \$25,469                             |
| Total stockholders' equity . . . . .    | \$ 1,371             | \$ 1,371  | \$ 59,921                            |

The pro forma and pro forma as adjusted information discussed above is illustrative only and will be revised based on the actual initial public offering price and other terms of our initial public offering determined at pricing. Each \$1.00 increase (decrease) in the assumed initial public offering price of \$16.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted amount of each of cash and cash equivalents, working capital, total assets and total stockholders' equity by \$3.8 million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. Similarly, each increase (decrease) of 1.0 million shares in the number of shares offered by us at the assumed initial public offering price would increase (decrease) each of cash and cash equivalents, working capital, total assets and total stockholders' equity by \$14.9 million.

## Risk factors

*Investing in our common stock involves a high degree of risk. Before you invest in our common stock, you should carefully consider the following risks, as well as general economic and business risks, and all of the other information contained in this prospectus. Any of the following risks could have a material adverse effect on our business, operating results and financial condition and cause the trading price of our common stock to decline, which would cause you to lose all or part of your investment. When determining whether to invest, you should also refer to the other information contained in this prospectus, including our financial statements and the related notes thereto. The risks and uncertainties described below are not the only ones we face. Additional risks and uncertainties not presently known to us may also adversely affect our business.*

### **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 \$5.4 million for the three months ended March 31, 2017. As of March 31, 2017, we had an accumulated deficit of \$32.6 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, we expect to incur significant expenses as a public company in the United States following the consummation of this offering. 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 undergoing a non-emergent, minimally to moderately invasive medical 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. A decline in the value of our company also could cause you to lose all or part of your investment.

***Our consolidated financial statements have been prepared assuming that we will continue as a going concern.***

We have incurred recurring losses from operations since inception which raises substantial doubt about our ability to continue as a going concern. If we are unable to obtain sufficient funding, our business, prospects, financial condition and results of operations will be materially and adversely affected and we may be unable to continue as a going concern. If we are unable to continue as a going concern, we may have to liquidate our assets and may receive less than the value at which those assets are carried on our audited financial statements, and it is likely that investors will lose all or a part of their investment. After this offering, if there remains substantial doubt about our ability to continue as a going concern, investors or other financing sources may be unwilling to provide additional funding to us on commercially reasonable terms or at all.

***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. We have not submitted an NDA to the FDA or comparable applications to 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 March 31, 2017, we had \$26.6 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 the net proceeds from this offering, together with our existing resources, will enable us to fund our operating expenses and capital expenditure requirements for at least the next 12 months, including the submission of the NDA to the FDA for the approval of avatrombopag for the treatment of thrombocytopenia in CLD patients undergoing a non-emergent,

minimally to moderately invasive medical procedure. 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.

Even with the net proceeds of this offering, we may require additional capital to complete the potential commercialization of avatrombopag for the treatment of thrombocytopenia in CLD patients undergoing a non-emergent, minimally to moderately invasive medical 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 March 31, 2017, we had only four 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. For a description of the terms of the services agreement and these arrangements, see the section titled “Business–Intellectual property–Services agreements with PBM Capital Group, LLC.”

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 the Company 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 March 31, 2017, we had four 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, engineering, 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 and will continue to be able to do so after the closing of this offering. 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. Effective upon the closing of this offering, we will adopt a code of business conduct and ethics, but 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 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 and your investment will be adversely affected.

***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 undergoing a non-emergent, minimally to moderately invasive medical 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 undergoing a non-emergent, minimally to moderately invasive medical 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 will delay the submission of the NDA to the FDA, 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 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 medical 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 medical 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 medical 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 non-emergent medical 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 medical 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 medical procedure, or that would prefer an advance treatment such as avatrombopag rather than prophylactic platelet transfusion on the same day as the medical 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 undergoing a non-emergent

minimally to moderately invasive medical 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 undergoing a non-emergent, minimally to moderately invasive medical procedure. We are also planning to develop avatrombopag for patients with thrombocytopenia, regardless of etiology, prior to a medical 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 undergoing a non-emergent minimally to moderately invasive medical 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 undergoing a non-emergent, minimally to moderately invasive medical 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 90<sup>th</sup> 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.

Since its enactment there have been judicial and Congressional challenges to certain aspects of the Affordable Care Act. As a result, there have been delays in the implementation of, and action taken to repeal or replace, certain aspects of the Affordable Care Act. In January 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities under the Affordable Care Act to waive, defer, grant exemptions from, or delay the implementation of any provision of the Affordable

Care Act that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. Further, in May 2017, following the passage of the budget resolution for fiscal year 2017, the U.S. House of Representatives passed legislation known as the American Health Care Act, which, if enacted, would amend or repeal significant portions of the Affordable Care Act. However, the U.S. Senate is unlikely to adopt the American Health Care Act as passed by the House of Representatives. The U.S. Senate could adopt additional legislation to amend or replace elements of the Affordable Care Act. Thus, it is uncertain if or when the American Health Care Act will become law. 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 this offering, ownership of our common stock and our status as a public company**

***An active trading market for our common stock may not develop and you may not be able to resell your shares of our common stock at or above the initial offering price, if at all.***

Prior to this offering, there has been no public market for our common stock. The initial public offering price for our common stock will be determined through negotiations with the underwriters and may not be indicative of the price at which our common stock will trade after the closing of this offering. Although we have applied to list our common stock on the NASDAQ Global Market, an active trading market for our

shares may never develop or be sustained following this offering. If an active market for our common stock does not develop or is not sustained, it may be difficult for you to sell shares you purchased in this offering 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. We do not currently have and may never obtain research coverage by equity research analysts. Equity research analysts may elect not to provide research coverage of our common stock after this offering, and such lack of research coverage may adversely affect the market price of our common stock. In the event 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 us regularly, demand for our stock could decrease, which in turn could cause our stock price or trading volume to decline.

***If you purchase shares of our common stock in this offering, you will suffer immediate dilution of your investment.***

We expect the initial public offering price of our common stock to be substantially higher than the net tangible book value per share of our common stock. Therefore, if you purchase shares of our common stock in this offering, you will pay a price per share that substantially exceeds our pro forma as adjusted net tangible book value per share after this offering. Based on an assumed initial public offering price of \$16.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, you will experience immediate dilution of \$13.57 per share, representing the difference between our pro forma as adjusted net tangible book value per share after this offering and the assumed initial public offering price. In addition, to the extent outstanding stock options are exercised, there will be further dilution to investors in this offering. See "Dilution" for a more detailed description of the dilution to investors in the offering.

***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 following this offering, the market price of our common stock could decline significantly.

Upon the closing of this offering, we will have 24,637,707 outstanding shares of common stock, after giving effect to the conversion of our convertible preferred stock outstanding as of March 31, 2017 into 3,242,950 shares of our common stock. Of these shares, the shares sold in this offering will be freely tradable and the remaining shares of common stock will be available for sale in the public market beginning 180 days after the date of this prospectus following the expiration of lock-up agreements between our stockholders and certain of the underwriters for this offering, 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, promptly following the closing of this offering, we intend to file one or more registration statements 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 these registration statements on Form S-8 will be 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, after this offering, the holders of an aggregate of 20,575,207 shares of our common stock, or their transferees, will 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 as they will be in effect following this offering 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 will have 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 will also contain other provisions that could have an anti-takeover effect, including:

- only one of our three classes of directors will be elected each year;
- stockholders will not be entitled to remove directors other than by a 66 $\frac{2}{3}$ % vote and only for cause;
- stockholders will not be 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 new investors from influencing significant corporate decisions.***

Upon the closing of this offering, 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, will, in the aggregate, beneficially own approximately 64% of our outstanding common stock. Further, PBM Capital Investments, LLC and funds under common control with PBM Capital Investments, LLC will beneficially own approximately 57% of our common stock. As a result, PBM Capital Investments, LLC will be able to 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. In addition, based on an assumed initial public offering price of \$16.00 per share, if our principal stockholders and their affiliated entities and certain of our directors purchase all of the shares they have indicated an interest in purchasing in this offering, the number of shares of our common stock beneficially owned by our executive officers, directors and current beneficial owners of 5% or more of our common stock and their respective affiliates will, in the aggregate, increase to 70% of our common stock.

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 price at which shares are being sold in this offering 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.

See “Principal stockholders” elsewhere in this prospectus for more information regarding the ownership of our outstanding common stock by our executive officers, directors and principal stockholders and their affiliates.

***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 in this prospectus;
- 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) following the fifth anniversary of the closing of this offering, (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.***

After the closing of this offering, we will be 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 this offering, we have 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 will have broad discretion in the use of proceeds from this offering and may invest or spend the proceeds in ways with which you do not agree and in ways that may not increase the value of your investment.***

We will have broad discretion over the use of proceeds from this offering. You may not agree with our decisions, and our use of the proceeds may not yield any return on your investment. We expect to use the net proceeds to us from this offering, 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 this offering to pursue our strategy to in-license or acquire additional drug candidates. Our failure to apply the net proceeds from this offering 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. You will not have the opportunity to influence our decisions on how to use our net proceeds from this offering.

***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. We may experience ownership changes in the future as a result of subsequent shifts in our stock ownership, including as a result of this offering, 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 and you may never receive a return on your investment.***

You should not rely on an investment in our common stock to provide dividend income. 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.

## Special note regarding forward-looking statements

This prospectus contains forward-looking statements that involve substantial risks and uncertainties. The forward-looking statements are contained principally in the sections entitled “Prospectus summary,” “Risk factors,” “Management’s discussion and analysis of financial condition and results of operations,” “Business” and elsewhere in this prospectus. In some cases, you can identify forward-looking statements by terms such as “may,” “will,” “should,” “expect,” “plan,” “anticipate,” “could,” “intend,” “target,” “project,” “estimate,” “believe,” “estimate,” “predict,” “potential” or “continue” or the negative of these terms or other similar expressions intended to identify statements about the future. These statements speak only as of the date of this prospectus and involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. We have based these forward-looking statements largely on our current expectations and projections about future events and financial trends that we believe may affect our business, financial condition and results of operations. These forward-looking statements include, without limitation, statements about the following:

- the timing, progress and results of clinical trials of avatrombopag and any other drug candidates, including statements regarding the timing of initiation and completion of studies or trials and related preparatory work, the period during which the results of the trials will become available and our research and development programs;
- the timing of any submission of filings for regulatory approval of avatrombopag, and the timing of and our ability to obtain and maintain regulatory approval of avatrombopag for any indication;
- our expectations regarding the scope of any approved indication for avatrombopag;
- our ability to expand the indications for which avatrombopag may be approved;
- our expectations regarding the size of the patient populations for, market acceptance and opportunity for and clinical utility of avatrombopag or any other drug candidates, if approved for commercial use;
- our ability to rely on Eisai for transition services under the TSA, including with respect to the development of avatrombopag;
- our manufacturing capabilities and strategy, including the scalability and commercial viability of our manufacturing methods and processes, including our ability to maintain our supply agreement with Eisai;
- our ability to successfully commercialize avatrombopag;
- our estimates of our expenses, ongoing losses, future revenue, capital requirements and our needs for or ability to obtain additional financing;
- our strategic plans and expectations for, and our ability to identify, develop and obtain regulatory approval for, new drug candidates;
- the implementation of our strategic plan to identify and develop treatments for diseases treated by specialist physicians;
- our ability to establish or maintain collaborations or strategic relationships;
- our ability to identify, recruit and retain key personnel;

- our ability to protect and enforce our intellectual property protection for avatrombopag, and the scope of such protection;
- our expected use of proceeds from this offering;
- our financial performance;
- our competitive position and the development of and projections relating to our competitors or our industry;
- the impact of laws and regulations;
- our expectations regarding the time during which we will be an emerging growth company under the JOBS Act; and
- the potential purchases of common stock by certain of our existing stockholders and their affiliated entities and certain of our directors in this offering.

Because forward-looking statements are inherently subject to risks and uncertainties, some of which cannot be predicted or quantified and some of which are beyond our control, you should not rely on these forward-looking statements as predictions of future events. The events and circumstances reflected in our forward-looking statements may not be achieved or occur and actual results could differ materially from those projected in the forward-looking statements. You should refer to the “Risk factors” section of this prospectus for a discussion of important factors that may cause our actual results to differ materially from those expressed or implied by our forward-looking statements. Moreover, we operate in an evolving environment. New risk factors and uncertainties may emerge from time to time, and it is not possible for management to predict all risk factors and uncertainties. As a result of these factors, we cannot assure you that the forward-looking statements in this prospectus will prove to be accurate. Except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise. You should, however, review the factors and risks and other information we describe in the reports we will file from time to time with the SEC after the date of this prospectus.

You should read this prospectus and the documents that we reference in this prospectus and have filed as exhibits to the registration statement, of which this prospectus is a part, completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of our forward-looking statements by these cautionary statements.



## Industry and other data

We obtained the industry, statistical and market data in this prospectus from our own internal estimates and research as well as from industry and general publications and research, surveys and studies conducted by third parties. All of the market data used in this prospectus involves a number of assumptions and limitations. While we believe that the information from these industry publications, surveys and studies is reliable, the industry in which we operate is subject to a high degree of uncertainty and risk due to a variety of important factors, including those described in the section titled “Risk factors.” These and other factors could cause results to differ materially from those expressed in the estimates made by third parties and by us.

## Use of proceeds

We estimate that the net proceeds from our issuance and sale of 4,062,500 shares of our common stock in this offering will be approximately \$58.6 million (or \$67.6 million if the underwriters exercise in full their option to purchase additional shares), assuming an initial public offering price of \$16.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

Each \$1.00 increase (decrease) in the assumed initial public offering price of \$16.00 per share would increase (decrease) the net proceeds to us from this offering by approximately \$3.8 million, assuming the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. Each increase (decrease) of 1.0 million in the number of shares we are offering would increase (decrease) the net proceeds to us from this offering, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us, by approximately \$14.9 million, assuming the assumed initial public offering price stays the same.

As of March 31, 2017, we had cash and cash equivalents of \$26.6 million. We intend to use the net proceeds from this offering, together with our existing cash and cash equivalents, as follows:

- approximately \$19.0 million to fund the commercialization of avatrombopag, if approved;
- approximately \$21.0 million to fund clinical trials of avatrombopag for additional indications beyond its initial indication;
- approximately \$30.0 million as repayment to Eisai of a portion of our obligations under the Eisai note; and
- the balance for other general corporate purposes, including general and administrative expenses and working capital.

For a description of the terms of the Eisai note, see the section titled “Management’s discussion and analysis of financial condition and results of operations—Eisai note and security agreement.”

Our expected use of net proceeds from this offering represents our current intentions based upon our present plans and business condition. As of the date of this prospectus, we cannot predict with complete certainty all of the particular uses for the net proceeds to be received upon the completion of this offering or the actual amounts that we will spend on the uses set forth above. We believe opportunities may exist from time to time to expand our current business through the acquisition or in-license of complementary drug candidates. While we have no current agreements for any specific acquisitions or in-licenses at this time, we may use a portion of the net proceeds for these purposes.

The amounts and timing of our actual expenditures will depend on numerous factors, including the progress of our clinical trials and other development and commercialization efforts for avatrombopag, as well as the amount of cash used in our operations. Based on our current operational plans and assumptions, we expect our cash and cash equivalents, together with the net proceeds from this offering, will be sufficient to enable us to commence the commercialization of avatrombopag, if approved. With respect to conducting clinical trials of avatrombopag for additional indications beyond its initial indication, we expect that we may require additional funds as these programs progress, the amounts of which will depend on the ultimate clinical development paths we pursue. However, we cannot estimate with certainty the amount of net proceeds to be used for the purposes described above. We may find it necessary or advisable to use the net proceeds for other purposes, and we will have broad discretion in the application of the net proceeds. Pending the uses described above, we plan to invest the net proceeds from this offering in short- and intermediate-term, interest-bearing obligations, investment-grade instruments, certificates of deposit or direct or guaranteed obligations of the U.S. government.

## **Dividend policy**

We have never declared or paid, and do not anticipate declaring or paying, in the foreseeable future, any cash dividends on our capital stock. We currently intend to retain all available funds and any future earnings to support our operations and finance the growth and development of our business. Any future determination related to our dividend policy will be made at the discretion of our board of directors and will depend upon, among other factors, our results of operations, financial condition, capital requirements, contractual restrictions, business prospects and other factors our board of directors may deem relevant.

## Capitalization

The following table sets forth our cash and cash equivalents, and our capitalization as of March 31, 2017:

- on an actual basis;
- on a pro forma basis to give effect to (i) the automatic conversion of all outstanding shares of our preferred stock on a 3.3-for-one basis into an aggregate of 3,242,950 shares of common stock, which will occur immediately prior to the closing of this offering; and (ii) the filing and effectiveness of our amended and restated certificate of incorporation upon the closing of this offering; and
- on a pro forma as adjusted basis to give further effect to our issuance and sale of 4,062,500 shares of common stock in this offering at an assumed initial public offering price of \$16.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting the estimated underwriting discounts and estimated offering expenses payable by us.

You should read this information in conjunction with our consolidated financial statements and the related notes appearing at the end of this prospectus, the “Management’s discussion and analysis of financial condition and results of operations” section and other financial information contained in this prospectus.

|  | As of March 31, 2017                               |           |                          |
|--|--|-----------|--------------------------|
|  | Actual   | Pro forma | Pro forma<br>as adjusted |
|  | (in thousands, except share<br>and per share data) |           |                          |
| Cash and cash equivalents . . . . .  | \$ 26,645  | \$ 26,645 | \$ 85,195                |
| Note payable, short-term . . . . .   | \$ 20,537  | \$ 20,537 | \$ 20,537                |
| Stockholders’ equity:  |  |           |                          |
| Preferred stock:   |  |           |                          |
| Series A preferred stock; par value \$0.001 per share,<br>1,400,000 shares authorized, 982,714 shares issued and<br>outstanding, actual; and no shares authorized, issued and<br>outstanding pro forma and pro forma as adjusted . . . . .   | 1  | —         | —                        |
| Common stock; par value \$0.001 per share, 23,100,000 shares<br>authorized, 17,332,257 shares issued and outstanding, actual;<br>100,000,000 shares authorized and 20,575,207 shares issued<br>and outstanding pro forma; 100,000,000 shares authorized<br>and 24,637,707 shares issued and outstanding pro forma as<br>adjusted . . . . . | 17   | 21        | 25                       |
| Additional paid-in capital . . . . .   | 33,967   | 33,963    | 92,509                   |
| Accumulated deficit . . . . .  | (32,614)   | (32,614)  | (32,614)                 |
| Total stockholders’ equity . . . . .   | 1,371  | 1,371     | 59,921                   |
| Total capitalization . . . . .   | \$ 21,908  | \$ 21,908 | \$ 80,458                |

Each \$1.00 increase (decrease) in the assumed initial public offering price of \$16.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted amount of each of cash and cash equivalents, additional paid-in capital, total stockholders’ equity and total capitalization by approximately \$3.8 million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after

deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. Similarly, each increase (decrease) of 1.0 million shares in the number of shares offered by us at the assumed initial public offering price of \$16.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted amount of each of cash and cash equivalents, additional paid in capital, total stockholders' equity and total capitalization by approximately \$14.9 million.

The number of shares of our common stock outstanding in the table above excludes:

- 1,332,375 shares of common stock issuable upon exercise of stock options awarded as of March 31, 2017 pursuant to our 2017 Plan at a weighted average exercise price of \$3.73 per share;
- 393,366 shares of our common stock issuable under exercise of stock options awarded after March 31, 2017 pursuant to our 2017 Plan at a weighted average exercise price of \$4.72 per share; and
- a maximum of 4,285,250 shares of common stock reserved for future issuance under the IPO Plan to be adopted in connection with this offering. The maximum number of shares reserved for future issuance under the IPO Plan includes 2,000,000 new shares of common stock reserved for issuance under the IPO Plan plus up to 2,285,250 shares of common stock reserved for issuance or issuable upon the exercise of the stock options awarded under the 2017 Plan that could become available under the IPO Plan upon cancellation, forfeiture or non-issuance of such shares after the IPO Plan effectiveness.

## Dilution

If you invest in our common stock in this offering, your ownership interest will be immediately diluted to the extent of the difference between the initial public offering price per share and the pro forma as adjusted net tangible book value per share of our common stock after this offering.

As of March 31, 2017, we had a historical net tangible book value of \$1.4 million, or \$0.08 per share of common stock. Our historical net tangible book value per share represents total tangible assets less total liabilities, divided by the number of shares of our common stock outstanding as of March 31, 2017.

Our pro forma net tangible book value as of March 31, 2017 was \$1.4 million, or \$0.07 per share of our common stock. Pro forma net tangible book value per share represents total tangible assets less total liabilities, divided by the number of shares of our common stock outstanding as of March 31, 2017, after giving effect to the automatic conversion of all outstanding shares of our preferred stock into common stock immediately prior to the closing of this offering.

After giving further effect to the sale of 4,062,500 shares of common stock in this offering at an assumed initial public offering price of \$16.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma as adjusted net tangible book value as of March 31, 2017 would have been approximately \$59.9 million, or approximately \$2.43 per share. This amount represents an immediate increase in pro forma net tangible book value of \$2.36 per share to our existing stockholders and immediate dilution of approximately \$13.57 per share to new investors in this offering. We determine dilution by subtracting the pro forma as adjusted net tangible book value per share after this offering from the amount of cash that a new investor paid for a share of common stock in this offering.

The following table illustrates this dilution:

|   |             |                        |
|---|-------------|------------------------|
| Assumed initial public offering price per share . . . . .   |             | \$16.00                |
| Historical net tangible book value per share as of March 31, 2017 . . . . .   | \$ 0.08     |                        |
| Decrease per share attributable to the conversion of our preferred stock into common stock on a 3.3-for-one basis . . . . . | (0.01)      |                        |
| Pro forma net tangible book value per share as of March 31, 2017 . . . . .  | 0.07        |                        |
| Increase per share attributable to this offering . . . . .  | <u>2.36</u> |                        |
| Pro forma as adjusted net tangible book value per share after this offering . . . . .                                       |             | <u>\$ 2.43</u>         |
| Dilution per share to new investors in this offering . . . . .  |             | <u><u>\$ 13.57</u></u> |

Each \$1.00 increase (decrease) in the assumed initial public offering price of \$16.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted net tangible book value per share after this offering by approximately \$0.15, and dilution in pro forma net tangible book value per share to new investors by approximately \$0.85, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. An increase of 1.0 million shares in the number of shares we are offering would increase the pro forma as adjusted net tangible book value per share after this offering by \$0.49 and decrease the dilution per share to new investors participating in this offering by \$0.49, assuming no change in the assumed initial public offering price per share and after deducting the estimated

underwriting discounts and commissions. A decrease of 1.0 million shares in the number of shares we are offering would decrease the pro forma as adjusted net tangible book value per share after this offering by \$0.53 and increase the dilution per share to new investors participating in this offering by \$0.53, assuming no change in the assumed initial public offering price per share and after deducting the estimated underwriting discounts and commissions.

If the underwriters exercise their option to purchase additional shares of our common stock in full, the pro forma as adjusted net tangible book value after this offering would be \$2.73 per share, the increase in pro forma net tangible book value per share would be \$2.66 and the dilution per share to new investors would be \$13.27 per share, in each case assuming an initial public offering price of \$16.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus.

The following table summarizes, as of March 31, 2017 on the pro forma as adjusted basis described above, the differences between the number of shares purchased from us, the total consideration paid to us in cash and the average price per share that existing stockholders and investors in this offering paid for such shares. The calculation below is based on an assumed initial public offering price of \$16.00 per share, which is the midpoint of the price range set forth on the cover page of this prospectus, before deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

|                                      | Shares purchased |         | Total consideration |         | Average price per share |
|--------------------------------------|------------------|---------|---------------------|---------|-------------------------|
|                                      | Number           | Percent | Amount              | Percent |                         |
| Existing stockholders . . . . .      | 20,575,207       | 84%     | \$ 34,251,890       | 35%     | \$ 1.66                 |
| Investors in this offering . . . . . | 4,062,500        | 16      | 65,000,000          | 65      | \$16.00                 |
| Total . . . . .                      | 24,637,707       | 100%    | \$ 99,251,890       | 100%    |                         |

The foregoing tables and calculations are based on the number of shares of our common stock outstanding as of March 31, 2017, after giving effect to the automatic conversion of all outstanding shares of our preferred stock into common stock immediately prior to the closing of this offering, and excludes:

- 1,332,375 shares of common stock issuable upon exercise of stock options awarded as of March 31, 2017 pursuant to our 2017 Plan at a weighted average exercise price of \$3.73 per share;
- 393,366 shares of our common stock issuable under exercise of stock options awarded after March 31, 2017 pursuant to our 2017 Plan at a weighted average exercise price of \$4.72 per share; and
- a maximum of 4,285,250 shares of common stock reserved for future issuance under the IPO Plan to be adopted in connection with this offering. The maximum number of shares reserved for future issuance under the IPO Plan includes 2,000,000 new shares of common stock reserved for issuance under the IPO Plan plus up to 2,285,250 shares of common stock reserved for issuance or issuable upon the exercise of the stock options awarded under the 2017 Plan that could become available under the IPO Plan upon cancellation, forfeiture or non-issuance of such shares after the IPO Plan effectiveness.

To the extent any of these outstanding options is exercised, there will be further dilution to new investors.

If the underwriters exercise their option to purchase additional shares of our common stock in full:

- the percentage of shares of common stock held by existing stockholders before this offering will decrease to approximately 81% of the total number of shares of our common stock outstanding after this offering; and

- the number of shares purchased by investors in this offering will increase to 4,671,875 shares, or approximately 19% of the total number of shares of our common stock outstanding after this offering.

We may choose to raise additional capital through the sale of equity or equity-linked securities due to market conditions or strategic considerations for our current or future development and commercialization plans. To the extent that we issue additional shares of common stock or other equity or equity-linked securities in the future, there will be further dilution to investors participating in this offering.

Certain of our existing stockholders and their affiliated entities and certain of our directors have indicated an interest in purchasing an aggregate of approximately \$25 million in shares of our common stock in this offering at the initial public offering price per share. Based on an assumed initial public offering price of \$16.00 per share, these persons and entities would purchase an aggregate of approximately 1,562,500 of the 4,062,500 shares in this offering based on these indications of interest. However, because indications of interest are not binding agreements or commitments to purchase, the underwriters may determine to sell more, less or no shares in this offering to any of these persons or entities, or any of these persons or entities may determine to purchase more, less or no shares in this offering. The foregoing discussion and tables do not reflect any potential purchases by these persons or entities or their affiliated entities.



## Selected consolidated financial data

The following tables set forth, for the periods and as of the dates indicated, our selected consolidated financial data. The balance sheet data as of December 31, 2016 and the statement of operations data for the period from March 24, 2016 (inception) through December 31, 2016 are derived from our audited consolidated financial statements appearing elsewhere in this prospectus. The consolidated statements of operations data for the period from March 24, 2016 through March 31, 2016 and for the three months ended March 31, 2017 and the consolidated balance sheet as of March 31, 2017 are derived from our unaudited consolidated financial statements appearing elsewhere in this prospectus. We have prepared the unaudited condensed consolidated financial statements on the same basis as the audited consolidated financial statements, and the unaudited financial data include, in our opinion, all adjustments consisting only of normal recurring adjustments that we consider necessary for a fair presentation of our consolidated financial position and results of operations for these periods. You should read this data together with our consolidated financial statements and related notes included elsewhere in this prospectus and the information under the captions "Management's discussion and analysis of financial condition and results of operations." Our historical results are not necessarily indicative of our future results and our operating results for the three months ended March 31, 2017 are not necessarily indicative of the results that may be expected for the entire year ending December 31, 2017.

|   | Period from<br>March 24, 2016<br>(inception)<br>to December 31,<br>2016 | Period from<br>March 24, 2016<br>(inception)<br>to March 31,<br>2016 | Three Months<br>Ended<br>March 31,<br>2017 |
|---|---|--|--|
|   | (in thousands,<br>except share and<br>per share data)                   |  |  |
| <b>Statement of Operations Data:</b>  |   |  |  |
| Expenses:   |   |  |  |
| Research and development expenses . . . . .   | \$ 20,842   | \$ 150   | \$ 4,276                                   |
| Research and development expenses—licenses<br>acquired . . . . .                            | 5,000   | 5,000  | —  |
| General and administrative expenses . . . . .   | 1,201   | 12   | 955  |
| Total operating expenses . . . . .  | 27,043  | 5,162  | 5,231                                      |
| Loss from operations . . . . .  | (27,043)  | (5,162)  | \$ (5,231)                                 |
| Other expense, net . . . . .  | (147)   | —  | (193)                                      |
| Net loss . . . . .  | \$ (27,190)   | \$ (5,162)   | \$ (5,424)                                 |
| Basic and diluted net loss per common share . . . . .                                       | \$ (1.57)   | \$ (0.31)  | \$ (0.31)                                  |
| Weighted-average basic and diluted common<br>shares . . . . .                               | 17,332,257  | 16,500,000   | 17,332,257                                 |
| Pro forma basic and diluted net loss per<br>common share (unaudited)(1) . . . . .           | \$ (1.32)   |  | \$ (0.26)                                  |
| Pro forma basic and diluted weighted-average<br>shares outstanding (unaudited)(1) . . . . . | 20,575,207  |  | 20,575,207                                 |

(1) See Note 2 to our consolidated financial statements included elsewhere in this prospectus for an explanation of the method used to calculate the historical and pro forma basic and diluted net loss per common share.

|  | As of<br>December 31, 2016 | As of<br>March 31, 2017 |
|--|----------------------------|-------------------------|
| <hr/>  |                            |                         |
| <b>(in thousands)</b>                            |                            |                         |
| <b>Balance Sheet Data:</b>                       |                            |                         |
| Cash and cash equivalents . . . . .              | \$ 28,709                  | \$26,645                |
| Working capital . . . . .                        | \$ 20,435                  | \$ 1,202                |
| Total assets . . . . .                           | \$ 28,746                  | \$26,840                |
| Note payable, long-term and short-term . . . . . | \$ 13,640                  | \$ 20,537               |
| Total liabilities . . . . .                      | \$ 21,951                  | \$25,469                |
| Total stockholders' equity . . . . .             | \$ 6,795                   | \$ 1,371                |

## Management's discussion and analysis of financial condition and results of operations

*You should read the following discussion and analysis of our financial condition and results of operations together with our consolidated financial statements and the related notes and other financial information included elsewhere in this prospectus. Some of the information contained in this discussion and analysis or set forth elsewhere in this prospectus, including information with respect to our plans and strategy for our business, includes forward-looking statements that involve risks and uncertainties. You should review the "Risk Factors" section of this prospectus for a discussion of important factors that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.*

### 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 in March 2016, is an orally administered 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 CLD undergoing non-emergent minimally to moderately invasive medical procedures. Avatrombopag met the primary and secondary endpoints in each of these clinical trials with high statistical significance. Based on these results, an NDA is planned for submission to the FDA for this initial indication in the third quarter of 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 the submission of 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. From inception through March 31, 2017, we have raised an aggregate of \$29.7 million from capital contributions from PBM Capital Investments, LLC and certain affiliates of PBM Capital Investments, LLC, to which we refer collectively as PBM Capital, and from the sale of shares of our Series A preferred stock. In addition, PBM Capital Investments, LLC paid Eisai \$5.0 million on our behalf in connection with our acquisition of worldwide rights to avatrombopag, which we accounted for as a capital contribution by PBM Capital Investments, LLC.

Since inception, we have incurred significant operating losses. For the period from March 24, 2016 to December 31, 2016 and for the three months ended March 31, 2017, our net loss was \$27.2 million and \$5.4 million, respectively. As of March 31, 2017, we had an accumulated deficit of \$32.6 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:

- file for regulatory approval in the United States for avatrombopag for thrombocytopenia in patients with CLD undergoing non-emergent minimally to moderately invasive medical procedures;
- 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 public company following the completion of this offering.

#### ***Stock purchase agreement with Eisai***

In March 2016, we entered into 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 the section titled “Business–Intellectual property–Agreements with Eisai” for additional information.

#### ***Transition services agreement with Eisai***

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 and 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 the Eisai note to Eisai, 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 March 31, 2017, we had outstanding borrowings of \$20.5 million under this Eisai note and we do not owe Eisai any 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. See the section titled “Business–Intellectual property–Agreements with Eisai” for additional information.

#### ***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 will be required to make aggregate milestone payments of up to \$5.0 million to Astellas if certain 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 the section titled “Business–Intellectual property–License agreement with Astellas” 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, our wholly-owned subsidiary, 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. We plan to submit an NDA for the approval of

avatrombopag in the third quarter of 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 undergoing a non-emergent, minimally to moderately invasive medical 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, 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 and professional fees for accounting and legal services. Other than payments to PBM Capital Group, LLC under the services agreements, for the period from March 24, 2016 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 quarter ended March 31, 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 being a public company including expenses related to services 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.

*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 Period from March 24, 2016 (inception) to March 31, 2016 and the Three Months Ended March 31, 2017

The following table sets forth our selected statements of operations data for the period from March 24, 2016 to March 31, 2016 and the three months ended March 31, 2017:

|  | Period from<br>March 24, 2016<br>(inception)<br>to March 31, 2016 | For the<br>Three Months Ended<br>March 31, 2017 |
|--|---|---|
| (in thousands)                                   |   |   |
| Operating expenses:                              |   |   |
| Research and development .....                   | \$ 150  | \$ 4,276  |
| Research and development—licenses acquired ..... | 5,000   | —   |
| General and administrative .....                 | 12  | 955   |
| Total operating expenses .....                   | 5,162   | 5,231   |
| Other expense, net .....                         | —   | (193)   |
| Net loss .....                                   | \$(5,162)   | \$(5,424)                                       |

### *Operating expense*

#### *Research and development expense*

For the period from March 24, 2016 to March 31, 2016, we recorded \$5.0 million of research and development expenses related to the upfront payment made to Eisai in connection with the stock purchase agreement. For the three months ended March 31, 2017, we recorded \$4.3 million of costs under the TSA.

#### *General and administrative expense*

We did not have any material general and administrative expenses for the period from March 24, 2016 to March 31, 2016. We hired our first employees and began paying compensation and issuing equity awards to employees during the three months ended March 31, 2017. For the three months ended March 31, 2017, general and administrative expenses were \$1.0 million, and were primarily attributable to \$0.2 million of payroll-related expenses, \$0.3 million of consulting fees and \$0.2 million of fees under the services agreements with PBM Capital Group, LLC.

#### *Other expense, net*

There was no other expense, net for the period from March 24, 2016 to March 31, 2016. Other expense, net for the three months ended March 31, 2017 consisted primarily of \$0.2 million of interest expense related to the Eisai note.

## Results of operations from March 24, 2016 (inception) to December 31, 2016

The following table sets forth our selected statements of operations data for the period from March 24, 2016 to December 31, 2016:

|                                      | Period from March 24, 2016<br>(inception) to December 31,<br>2016 |
|--------------------------------------|---|
| <hr/>                                |   |
| (in thousands)                       |   |
| Operating expenses:                  |   |
| Research and development . . . . .   | \$ 25,842   |
| General and administrative . . . . . | 1,201   |
| Total operating expenses . . . . .   | 27,043  |
| Other expense, net . . . . .         | (147)   |
| Net loss . . . . .                   | \$(27,190)  |

### *Operating expense*

#### *Research and development expense*

Research and development expenses were \$25.8 million for the period from March 24, 2016 to December 31, 2016, and were primarily attributable to the \$5.0 million upfront payment made to Eisai in connection with the Eisai stock purchase agreement, and \$20.8 million of costs under the TSA.

#### *General and administrative expense*

General and administrative expenses were \$1.2 million for the period from March 24, 2016 to December 31, 2016, and were primarily attributable to expenses under the services agreements with PBM Capital Group, LLC, recruiting fees and professional fees for accounting and legal services. Other than the services fees to PBM Capital Group, LLC, for the period from March 24, 2016 to December 31, 2016, we did not pay any employee compensation or issue any stock-based compensation to any employee, director or consultant.

#### *Other expense, net*

Other expense, net for the period from March 24, 2016 to December 31, 2016 was \$0.1 million and primarily consisted of interest expense related to the Eisai note, offset by interest income on our cash and cash equivalents.

## 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 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 \$13.6 million and \$20.5 million as of December 31, 2016 and March 31, 2017, respectively. From inception through March 31, 2017, we have raised an aggregate of \$29.7 million from capital contributions from PBM Capital and from the sale of shares of our Series A preferred stock. In addition, PBM Capital Investments, LLC paid Eisai \$5.0 million on our behalf in connection with our acquisition of worldwide rights to avatrombopag, which we accounted for as a capital contribution by PBM Capital Investments, LLC. As of March 31, 2017, we had \$26.6 million in cash and cash equivalents.

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

|   | Period from<br>March 24, 2016<br>(inception) to<br>March 31, 2016 | For the<br>Three Months<br>Ended<br>March 31, 2017 | Period from<br>March 24, 2016<br>(inception)<br>to December 31, 2016 |
|---|---|--|--|
| <i>(in thousands)</i>   |   |  |  |
| Cash and cash equivalents at beginning of period . . . . .    | \$ —  | \$ 28,709  | \$ 0   |
| Net cash used in operating activities . . . . .               | —   | (1,353)  | (987)  |
| Net cash provided by (used in) financing activities . . . . . | —   | (711)  | 29,696   |
| Cash and cash equivalents at end of period                    | <u>\$ —</u>   | <u>\$ 26,645</u>                                   | <u>\$ 28,709</u>   |

*Operating activities*

Operating activities used \$1.4 million of cash during the three months ended March 31, 2017, primarily for expenses under the services agreements with PBM Capital Group, LLC, consulting fees and professional fees.

Operating activities used \$1.0 million of cash in 2016, primarily for expenses under the services agreements with PBM Capital Group, LLC, recruiting fees and professional fees.

*Financing activities*

Financing activities used \$0.7 million of cash during the three months ended March 31, 2017 for costs associated with the sale of Series A preferred stock.

Financing activities provided \$29.7 million of cash in 2016, primarily from the sale of 982,714 shares of Series A preferred stock to various investors.

*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, following the completion of this offering, we expect to incur additional costs as a public 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, together with the net proceeds from this offering, 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. 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 this offering. 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.

Our consolidated financial statements appearing elsewhere in this prospectus have been prepared on a going concern basis, which contemplates the realization of assets and satisfaction of liabilities in the normal course of business. The consolidated financial statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might result from the outcome of this uncertainty. We anticipate incurring additional losses until such time, if ever, that we can obtain marketing approval to sell, and then generate significant sales of, avatrombopag. We will need substantial additional financing to fund our operations and to develop and commercialize our drug candidate. These factors raise substantial doubt about our ability to continue as a going concern.

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. See “Risk factors—Risks related to our business, financial position and capital needs—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.”

## Contractual obligations and commitments

The commitment amounts in the table below are associated with contracts that are enforceable and legally binding and that specify all significant terms, including fixed or minimum services to be used, fixed, minimum or variable price provisions, and the approximate timing of the actions under the contracts. The table does not include obligations under agreements that we can cancel without a significant penalty.

The following table summarizes our commitments to settle contractual obligations at December 31, 2016:

| Contractual obligations<br>(in thousands)      | Total            | Less than     |                  |              | After<br>5 years |
|--|------------------|---------------|------------------|--------------|------------------|
|  |                  | 1 year        | 1 to 3 years     | 4 to 5 years |                  |
| Long-term obligations(1) . . . . .             | \$ 13,791        | \$ 151        | \$ 13,640        | \$—          | \$—              |
| Interest on debt obligations(1)(2) . . . . .   | 853              | 171           | 682              | —            | —                |
| Purchase obligations(2)(3) . . . . .           | 224              | 224           | —                | —            | —                |
| <b>Total contractual obligations . . . . .</b> | <b>\$ 14,868</b> | <b>\$ 546</b> | <b>\$ 14,322</b> | <b>\$—</b>   | <b>\$—</b>       |

(1) Long-term obligations arise from the Eisai note which enables us to finance payments due to Eisai under the TSA. The TSA primarily includes activities and costs for clinical trials, consultants, clinical trial materials, regulatory filings, laboratory expenses and other supplies. 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. 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. Principal and interest under the Eisai note can be prepaid at any time without penalty. The Eisai note is secured by a security interest on all of the assets of AkaRx, including the worldwide rights to avatrombopag. Payments due under the Eisai note are currently guaranteed by PBM Capital Investments, LLC. See Note 3 to our consolidated financial statements beginning on page F-1 of this prospectus for a description of the Eisai stock purchase agreement and related transactions.

(2) These obligations are not reflected in the accompanying balance sheets.

(3) We have open purchase orders for \$224,000, which include \$74,000 for research and development consulting and \$150,000 for the services agreements with PBM Capital Group, LLC. Substantially all of our purchase orders may be canceled without significant penalty to us.

## 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 consolidated financial statements, which have been prepared in accordance with U.S. GAAP. The preparation of these consolidated 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 date of the balance sheet and the reported amounts of expenses during the reporting period. In accordance with U.S. GAAP, we evaluate our estimates and judgments on an ongoing basis. The most significant estimates 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. We base our estimates and assumptions on current facts, our limited historical experience from operating for one year and 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.

We define our critical accounting policies as those accounting principles that require us to make subjective estimates and judgments about matters that are uncertain and are likely to have a material impact on our financial condition and results of operations, as well as the specific manner in which we apply those principles. While our significant accounting policies are more fully described in Note 2 to our consolidated financial statements appearing elsewhere in this prospectus, we believe the following are the critical accounting policies used in the preparation of our consolidated financial statements that require significant estimates and judgments:

#### *Fair value of common stock*

Beginning in March 2017 with the approval by our board of directors of the 2017 Plan, the fair values of the shares of common stock underlying our options were estimated on each grant date by our board of directors. In order to determine the fair value, our board of directors considered, among other things, contemporaneous valuations of our common stock and preferred stock prepared by unrelated third-party valuation firms in accordance with the guidance provided by the American Institute of Certified Public Accountants 2013 Practice Aid, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation*, or the Practice Aid. Given the absence of a public trading market of our capital stock, our board of directors will exercise reasonable judgment and consider a number of objective and subjective factors to determine the best estimate of the fair value of our common and preferred stock, including:

- contemporaneous third-party valuations of our common stock;
- the prices, rights, preferences and privileges of our preferred stock relative to our common stock;
- our business, financial condition and results of operations, including related industry trends affecting our operations;
- the likelihood of achieving a liquidity event, such as an initial public offering, or IPO, or sale of our company, given prevailing market conditions;
- the lack of marketability of our common stock;
- the market performance of comparable publicly traded companies; and
- U.S. and global economic and capital market conditions and outlook.

#### *Common stock valuation methodology*

In estimating the fair market value of our common stock, our board of directors first determined the equity value of our business using accepted valuation methods.

We conducted a valuation as of December 31, 2016 which used our recent Series A preferred stock financing as a starting point and determined the equity value of our company based on a “back solve” methodology that utilized the option pricing method, a value allocation methodology prescribed in the AICPA’s guide “Valuation of Privately-Held-Company Equity Securities Issued as Compensation”. The

allocation methodology also allocated that equity value across the securities in our capital structure—our Series A preferred stock and common stock. A discount for lack of marketability was then applied to conclude a fair market value for each share of common stock as of December 31, 2016.

For purposes of options awarded on March 28, 2017 and April 3, 2017, we estimated the value of our common stock as of March 27, 2017 to determine if there was any change in value since the December 31 valuation. We started with the implied equity value derived from the December 31, 2016 valuation and considered various quantitative factors, including the percent change in the market capitalization of the identified comparable public companies, and two biotechnology indices, from December 31, 2016 through March 27, 2017. We also considered qualitative factors based on changes to our pipeline and events that have implications for the overall value of our company, including the receipt of positive clinical trial results for avatrombopag in March 2017 and our efforts to date to pursue the sale of our common stock via an IPO, including selecting the underwriters for this offering and initiating the preparation of the registration statement for this offering. We used a hybrid equity valuation and allocation model to determine our total equity value and resulting common stock per share value as of the valuation date. Specifically, we considered the possibility of a near term IPO and a scenario where no IPO takes place. As part of our qualitative analysis to estimate the impact of the positive clinical trial results on our underlying value, we reviewed relevant academic studies that examined the impact of a positive announcement before and after the actual announcement on a biotechnology company's stock price.

Based on our analysis of relevant quantitative and qualitative factors, we estimated an increase to our company's value from the December value conclusion. After allocating the increased value across our equity securities, a discount for lack of marketability was then applied to conclude a fair market value for each share of common stock as of March 27, 2017.

For purposes of options awarded on April 14, 2017, we determined that the estimated fair market value of our common stock had not changed from the value as of March 27, 2017.

For purposes of options awarded in May 2017, we estimated the value of our common stock as of May 25, 2017 to determine if there was any change in value since the March 27, 2017 valuation. We started with the implied equity value derived from the March 27, 2017 valuation and considered various quantitative factors, including the percent change in the market capitalization of the identified comparable public companies, and two biotechnology indices, from March 27, 2017 through May 25, 2017. We also considered qualitative factors based on changes to our pipeline and events that have implications for the overall value of our company, including the progress made toward the sale of our common stock via an IPO including the initial confidential submission to the SEC of a registration statement on Form S-1 on April 21, 2017.

Based on our analysis of relevant quantitative and qualitative factors, we estimated an increase to our value from the March valuation. After allocating the increased value across our equity securities, a discount for lack of marketability was then applied to conclude a fair market value for each share of common stock as of May 25, 2017.

The following table presents the award dates, accounting grant dates and related exercise prices of stock options that we awarded from March 28, 2017 through May 25, 2017, along with the fair values per share as of the accounting grant date utilized to calculate stock-based compensation expense.

| Award date     | Grant date <sup>(1)</sup> | Number of shares underlying options | Exercise price per option | Common stock fair value per share on grant date |
|----------------|---------------------------|-------------------------------------|---------------------------|---|
| March 28, 2017 | June 2, 2017              | 1,332,375                           | \$3.73                    | \$7.32  |
| April 3, 2017  | June 2, 2017              | 33,000                              | \$3.73                    | \$7.32  |
| April 14, 2017 | June 2, 2017              | 251,466                             | \$3.73                    | \$7.32  |
| May 25, 2017   | June 2, 2017              | 108,900                             | \$7.32                    | \$7.32  |

(1) Represents the accounting grant dates at which all of the accounting prerequisites had been met in order to issue the stock options and all terms had been communicated to stock option recipients.

Following the closing of this offering, the fair value of our common stock will be determined based on the closing price of our common stock on the NASDAQ Global Market.

### ***Income taxes***

On September 15, 2016, we converted from an LLC to a C-corporation. Prior to September 15, 2016, we elected to be taxed as a partnership. Therefore, we were not subject to income taxes until our conversion to a C-corporation on September 15, 2016. AkaRx was subject to income taxes from March 29, 2016 through March 31, 2017.

Income taxes are recorded in accordance with ASC 740, *Income Taxes*, or ASC 740, which provides for deferred taxes using an asset and liability approach. We recognize deferred tax assets and liabilities for the expected future tax consequences of events that have been included in our consolidated financial statements or tax returns. Deferred tax assets and liabilities are determined based on the difference between our consolidated 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.

We account for uncertain tax positions in accordance with the provisions of ASC 740. When uncertain tax positions exist, we recognize 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.

### **Recent accounting pronouncements**

See Note 2 to our consolidated financial statements beginning on page F-1 of this prospectus for a description of recent accounting pronouncements applicable to our consolidated financial statements.

### **Qualitative and quantitative 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 period from March 24, 2016 to December 31, 2016 or for the three months ended March 31, 2017.

### **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) following the fifth anniversary of the completion of this offering, (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.

# Business

## 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, undergoing non-emergent minimally to moderately invasive medical procedures. 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, is planned for submission to the U.S. Food and Drug Administration, or FDA, for this initial indication in the third quarter of 2017.

We believe that avatrombopag's efficacy and safety profile in combination with its convenient oral dosing could provide advantages over other treatments for thrombocytopenia. To date, avatrombopag has been evaluated in more than 20 clinical trials involving more than 1,100 subjects and has been observed to be generally well tolerated. We believe that avatrombopag may, therefore, have the potential to be used more broadly for patients with thrombocytopenia, including patients without CLD, and we are exploring regulatory and clinical development strategies that would support this expanded use.

### *Overview of thrombocytopenia and current treatments*

Thrombocytopenia is characterized by a deficiency of platelets that impairs blood clot formation and increases bleeding risk. Thrombocytopenia is defined as having less than 150,000 platelets per microliter of circulating blood and is diagnosed with a routine blood test. Thrombocytopenia can result in significant bleeding risk even in cases of minor injury. Platelet deficiency can also increase the risk of excessive, uncontrolled bleeding during or after a medical procedure.

Physicians determine how to treat thrombocytopenia, either in the acute setting or chronically, based on a number of factors, including the patient's platelet count, etiology of the underlying cause of thrombocytopenia, duration of required platelet count elevation and the patient's overall health profile. Acute prophylactic treatment of thrombocytopenia currently involves platelet transfusion in advance of medical procedures or in connection with other medical treatments that reduce platelet counts, such as chemotherapy. Despite being the standard of care, platelet transfusions are associated with limitations that impact their use including risk of antibody development in up to 50% of patients, short duration of effect of transfused platelets, limited supply and inconvenience of administration. There is no drug treatment approved by the FDA or the European Medicines Agency, or EMA, for thrombocytopenia in the acute setting prior to a medical procedure.

Chronic treatment of thrombocytopenia involves continuous treatment of the disorder. The substantial majority of patients who require chronic treatment suffer from immune thrombocytopenic purpura, or ITP. Treatments for chronic ITP target one of the following mechanisms: reduction of autoimmune activity that causes abnormal platelet destruction, surgical removal of the spleen to prevent trapping of platelets in the spleen or stimulation of platelet production. First-line therapy for ITP is typically focused on reducing autoimmune activity and consists of corticosteroids or intravenous immunoglobulin, or IVIG. Second line alternatives include rituximab, which is used off-label to reduce autoimmune activity, and splenectomy to

address the trapping of the platelets in the spleen. Currently marketed TPO-RAs, which aim to stimulate platelet production, are also used in the second line treatment of chronic ITP. However, we believe these available treatments have limitations that impact their use, such as limited efficacy, risk to patient safety, patient non-compliance or inconvenience.

Because of the limitations of current therapies used for thrombocytopenia in the acute and chronic setting, we believe there remains a significant unmet need for a treatment that demonstrates reliable and durable effectiveness and a favorable safety profile, that can be conveniently administered and potentially reduce the burden on patients.

### ***Our drug candidate***

Our drug candidate, avatrombopag, is designed to mimic the effects of thrombopoietin, or TPO, *in vitro* and *in vivo*. TPO is a hormone produced in the liver and kidney that binds to its receptor, c-Mpl (myeloproliferative leukemia). Following TPO receptor binding, intracellular signaling leads to megakaryocyte growth and maturation, which results in increased platelet production. TPO-RAs, like TPO, stimulate the activation, proliferation and maturation of megakaryocytes, resulting in an increase in circulating platelet counts. Avatrombopag is a highly specific TPO-RA as it binds to the TPO receptor at a distinct site from native TPO, leaving the TPO receptor accessible to native TPO, enabling avatrombopag to have an additive effect on platelet production.

We believe avatrombopag has the potential to be a first-in-class treatment of thrombocytopenia in the acute setting and a best-in-class treatment of thrombocytopenia in the chronic setting. We believe avatrombopag addresses the shortcomings associated with standard of care platelet transfusions, including the risk of antibody development in up to 50% of patients, short duration of effect of transfused platelets, limited supply and inconvenience of administration. In addition, we believe avatrombopag's efficacy and safety profiles and convenient once-daily oral dosing differentiate it from the TPO-RAs currently marketed for the chronic treatment of thrombocytopenia. We believe attributes specific to avatrombopag make it a compelling treatment option for patients with thrombocytopenia in both the acute and chronic settings.

We recently completed two identically designed Phase 3 pivotal clinical trials, ADAPT 1 and ADAPT 2, involving an aggregate of 435 patients with CLD, in which all primary and secondary endpoints were met with high statistical significance. The primary endpoint for both studies was the percentage of CLD patients with thrombocytopenia undergoing a non-emergent minimally to moderately invasive medical procedure who did not require a platelet transfusion or any rescue procedure for bleeding at each of two dose strengths of avatrombopag compared to placebo. We refer to minimally to moderately invasive medical procedures as procedures for which medical guidelines generally recommend patients have at least 50,000 platelets per microliter of circulating blood before the procedure. In each trial, the percentage of subjects in each of the two avatrombopag dosing cohorts requiring a platelet transfusion or a rescue procedure for bleeding was statistically significantly lower compared to placebo (across all cohorts, p-values ranged from  $p < 0.0001$  to  $p = 0.0006$ ). We also observed a statistically significant improvement in the percentage of avatrombopag-treated subjects who achieved platelet counts of greater than 50,000 platelets per microliter of circulating blood on the procedure day and in the change in platelet counts from baseline to procedure day. We are initially developing avatrombopag for the acute treatment of thrombocytopenia in the population of patients with CLD undergoing non-emergent minimally to moderately invasive medical procedures.

There are an estimated 7.4 million CLD patients in the United States. Thrombocytopenia affects approximately 1.1 million of those patients and approximately 70,000 of those patients have a platelet count of less than 50,000 platelets per microliter of circulating blood. Based on a third-party survey of 155

community and academic physicians that we commissioned in 2017, we estimate that approximately 25% of these patients are treated with currently available TPO-RAs off-label and approximately 60% of these patients are treated with platelet transfusions in order to raise their platelet counts prior to a procedure. These patients generally undergo one to three medical procedures per year. Most of the CLD patients affected by thrombocytopenia are treated by one of the approximately 850 hepatologists, most of whom are working at one of the approximately 150 liver transplant centers in the United States.

In addition to ADAPT 1 and ADAPT 2, avatrombopag has also been evaluated in one Phase 3 trial in adults with chronic ITP, five Phase 2 trials in various thrombocytopenia patient populations, 15 Phase 1 trials and numerous preclinical studies, and has been observed to be generally well tolerated in over 1,100 patients. Based on the safety and efficacy profile established by these trials and studies, we also believe avatrombopag has the potential for use in a broader population of thrombocytopenia patients regardless of disease etiology undergoing a broader set of medical procedures, including, for example, joint replacements. We believe it may also have the potential to treat patients who develop thrombocytopenia after receiving chemotherapy. In addition, we are evaluating the potential regulatory approval pathway for avatrombopag for the treatment of adults with chronic ITP based on results from a completed Phase 3 trial in this patient population.

We hold the worldwide rights to avatrombopag for all current and future indications, which we acquired from Eisai in March 2016. Our intellectual property strategy aims to protect and control the development and commercialization of avatrombopag. Our owned and in-licensed patents provide us with composition of matter and method of use exclusivity with respect to avatrombopag in the United States, including a composition of matter patent that expires in 2025, with possible patent term extension up to 2030. We also hold patents and applications in major world markets with respect to avatrombopag, which are projected to expire between 2023 and 2027, excluding any extension of patent term that may be available in a particular country.

### ***Management***

Our management team has extensive experience ranging from identifying and acquiring drug candidates, drug development and global registrations through global commercial launches. The members of our management team have held senior leadership positions at a number of pharmaceutical and biotechnology companies, including Amgen, Genentech, GlaxoSmithKline, Novartis, Pfizer, United Therapeutics and Wyeth. We believe that the breadth of experience and successful track record of our management team, combined with our broad network of established relationships with leaders in the industry and medical community provide us with unique insights into drug development and commercialization. Further, we are being supported by a leading group of biotech investors including PBM Capital Investments, LLC, Perceptive Advisors and Paulson & Company, Inc.

## Pipeline

The following table summarizes our lead development programs. We hold the worldwide rights to avatrombopag for these indications.

| Avatrombopag: Multiple Indications for Various Types of Thrombocytopenia   |  |         |         |         |   |
|--|--|---------|---------|---------|---|
|  | Preclinical  | Phase 1 | Phase 2 | Phase 3 | Next expected milestone                                   |
| <b>Acute treatment of thrombocytopenia</b>   |  |         |         |         |   |
| Thrombocytopenia in CLD patients scheduled to undergo a medical procedure <sup>(1)</sup>   | [Progress bar spanning Preclinical, Phase 1, Phase 2, and Phase 3] |         |         |         | File NDA in Q3 2017                                       |
| Thrombocytopenia in broader population of patients scheduled to undergo a medical procedure or surgery <sup>(2)</sup>  | [Progress bar spanning Preclinical, Phase 1, and Phase 2]          |         |         |         | Initiate later stage clinical development program in 2018 |
| Chemotherapy-Induced Thrombocytopenia (CIT)  | [Progress bar spanning Preclinical, Phase 1, and Phase 2]          |         |         |         | Initiate later stage clinical development program in 2018 |
| <b>Chronic treatment of thrombocytopenia</b>   |  |         |         |         |   |
| Immune Thrombocytopenic Purpura (ITP)  | [Progress bar spanning Preclinical, Phase 1, Phase 2, and Phase 3] |         |         |         | Meet with FDA to discuss prior clinical trial results     |
| <small><sup>1</sup> Medical procedure refers to a non-emergent, minimally to moderately invasive medical procedure.<br/> <sup>2</sup> Surgery includes spectrum of minimally invasive to highly invasive medical procedures. Highly invasive surgeries are those for which multiple medical professional association guidelines recommend that patients have greater than 100,000 platelets per microliter of circulating blood.</small> |  |         |         |         |   |

## Our strategy

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. To achieve our goals, we are pursuing the following strategies:

- **Advance the development of our late-stage drug candidate, avatrombopag, for regulatory approval in the United States and Europe.** In the first quarter of 2017, we completed two identically designed pivotal Phase 3 clinical trials for avatrombopag in patients with CLD undergoing a non-emergent minimally to moderately invasive medical procedure. Based on the results of these trials, an NDA for avatrombopag is planned for submission to the FDA in the third quarter of 2017. In addition, as our Phase 3 trials were also designed to be pivotal trials in Europe, we intend to submit a marketing authorization application, or MAA, to the EMA in the first half of 2018.
- **Maximize the commercial potential of avatrombopag.** Our intent is to initially build a hepatology-focused sales organization in the United States. We have begun to execute this strategy by hiring key executives with global commercial launch experience. In the future, we also may selectively partner with leading companies that we believe can contribute additional resources and know-how for the development and commercialization of avatrombopag for additional indications and geographic regions, further enhancing the value of our drug candidate.
- **Expand the breadth of indications for avatrombopag in other patient populations with thrombocytopenia.** Based on the results from our Phase 2 and Phase 3 clinical trials, we also believe avatrombopag has the potential for use in a broader population of thrombocytopenia patients regardless of disease etiology undergoing a broader set of medical procedures, including, for example, joint

replacements. We believe it may also have the potential to treat patients who develop thrombocytopenia after receiving chemotherapy. In addition, we are evaluating the potential regulatory approval pathway for avatrombopag for the treatment of adults with chronic ITP based on results from a completed Phase 3 trial in this patient population.

- **Employ a value-driven approach to build a pipeline of drug candidates.** Using a similar approach to our identification and acquisition of avatrombopag, we intend to employ a value-driven strategy to identify, acquire, develop and commercialize drug candidates for diseases that are treated by specialist physicians.
- **Maintain and strengthen our intellectual property portfolio.** Our intellectual property strategy aims to protect and control the development and commercialization of our drug candidates. Our owned and in-licensed patents for avatrombopag provide us with composition of matter and method of use exclusivity with respect to avatrombopag in the United States, including a composition of matter patent that expires in 2025, with possible patent term extension up to 2030. We also hold patents and applications in major world markets with respect to avatrombopag, which are projected to expire between 2023 and 2027, excluding any extension of patent term that may be available in a particular country. We will seek to broaden the scope of and increase the geographic reach of our patent protection throughout the world.

## **Background of thrombocytopenia**

Thrombocytopenia is a disorder characterized by a low blood platelet count. Platelets, also known as thrombocytes, are cells in the blood that help control bleeding both by clumping and forming a hemostatic plug and by activating other clotting mechanisms. Megakaryocytes, which are large cells found in the bone marrow, are the source of platelets that are released into the bloodstream. Platelet production is regulated by TPO, a hormone produced in the liver and kidney that binds to the c-Mpl receptor. Following TPO receptor binding, intracellular signaling leads to megakaryocyte growth and maturation, which results in increased platelet production. Platelets are continuously produced and their life span averages 8 to 10 days.

Thrombocytopenia results from decreased production of platelets, increased destruction of platelets or trapping of platelets in the spleen, the causes of which can be inherited or acquired. Thrombocytopenia is characterized by a deficiency of platelets that impairs blood clot formation and increases bleeding risk. Thrombocytopenia is defined as having less than 150,000 platelets per microliter of circulating blood and is diagnosed with a routine blood test. An individual with normal platelet counts has platelets measured in the range of 150,000 to 450,000 platelets per microliter of circulating blood. Thrombocytopenia can result in significant bleeding risk even in cases of minor injury and can also increase the risk of excessive, uncontrolled bleeding during or after a medical procedure.

Physicians determine how best to treat thrombocytopenia, either acutely or chronically, based on a number of factors, including the patient's platelet count, etiology of the underlying cause of thrombocytopenia, duration of required platelet count elevation and the patient's overall health profile. Acute treatment of thrombocytopenia currently involves prophylactic platelet transfusion in advance of a medical procedure or in connection with other treatments that reduce platelet counts, such as chemotherapy. Chronic treatment of thrombocytopenia involves continuous treatment of the disorder.

## Current treatments for thrombocytopenia and their limitations

### *Acute treatments for thrombocytopenia*

There is no drug treatment approved by the FDA or the EMA for thrombocytopenia in the acute setting prior to a medical procedure. Prophylactic platelet transfusion is currently the standard of care for patients who need to increase their platelet count prior to a medical procedure.

Multiple medical professional associations have guidelines that recommend that patients have at least 50,000 platelets per microliter of circulating blood prior to minimally to moderately invasive medical procedures, such as epidural anesthesia, liver biopsy and endoscopy with biopsy. The need to increase platelet counts becomes even more critical with more invasive medical procedures, because of the higher risk of uncontrolled bleeding during and after the procedure. For highly invasive procedures, such as vascular, cardiac, brain or spine surgeries, many medical professional association guidelines recommend that patients have at least 100,000 platelets per microliter of circulating blood. However, depending on the etiology of the underlying cause of the thrombocytopenia and the patient's overall health profile, physicians and surgeons exercise discretion about the requisite platelet count threshold before a medical procedure.

In the United States, every year, there are an estimated 1.9 million units of platelets transfused in connection with a total of 1.2 million platelet transfusions. Approximately 125,000 of those platelet transfusions are for more invasive planned surgical procedures, and approximately 125,000 are administered for patients with chemotherapy-induced thrombocytopenia, or CIT. We estimate that the total costs associated with a platelet transfusion are approximately \$9,000 per transfusion.

Despite being the standard of care for treating thrombocytopenia prior to a non-emergent medical procedure, platelet transfusions are associated with limitations that we believe can be addressed by avatrombopag. These limitations may include:

*Risk of refractoriness, or antibody development.* Patients with thrombocytopenia associated with chronic diseases often require multiple non-emergent procedures and, as a result, need multiple platelet transfusions. Antibody development renders subsequent transfusions less effective in increasing platelet count. Refractoriness, or antibody development, occurs in up to 50% of patients.

*Short duration of effect of platelets.* The life span of platelets normally averages 8 to 10 days. As time passes and older platelets begin to die, the efficacy of the transfused platelets becomes significantly reduced. Accordingly, physicians typically require the platelet transfusion to be scheduled on the same day as the medical procedure. Even if the platelet transfusion occurs on the same day as the medical procedure, the transfused platelets vary in age based on their time of collection and limited life span.

*Convenience.* Coordinating the scheduling of the platelet transfusion and medical procedure can result in increased complexity and inconvenience to the patient. Platelet transfusions typically require a scheduled visit with a transfusion time of 30 minutes to four hours, and commonly take place at a hospital or infusion center separate from the location of the medical procedure.

*Limited supply.* Even with proper storage, a unit of platelets has a shelf life of only 5 days, resulting in an ongoing need for platelet donations. The process for donating platelets is much more cumbersome and time consuming compared to whole blood donations. Donors are required to remain still for up to three hours as platelets are removed from the blood thus making it more difficult to

maintain a steady stream of platelet donors. Unpredictable platelet availability can lead to scheduling difficulties, procedure delays and cancellations. In addition, platelet shortages are more common outside of major medical centers.

*Risk of adverse immune reaction or blood-to-blood disease transmission.* While rare, an immune reaction to transfused platelets can be very serious, including anaphylactic reactions. In rare situations, a platelet transfusion can also result in disease transmission similar to other blood component transfusions.

Despite the shortcomings of platelet transfusions, they remain the standard of care for treating thrombocytopenia prior to a non-emergent medical procedure.

### ***Chronic treatments for thrombocytopenia (ITP)***

Patients with less than 30,000 platelets per microliter of circulating blood may require active, chronic management of their thrombocytopenia, depending on the etiology of the underlying cause of thrombocytopenia, duration of required platelet count elevation and the patient's overall health profile. The substantial majority of patients who require chronic treatment suffer from immune thrombocytopenic purpura, or ITP, which is a disorder that occurs when certain immune system cells mistakenly produce antibodies against platelets. These antibodies attach to platelets resulting in platelet destruction. ITP can also result in damage to megakaryocytes, leading to impaired platelet production.

ITP is considered chronic when the disorder has persisted for more than 12 months. We estimate that chronic ITP affects approximately 60,000 adults in the United States. Treatments for chronic ITP target one of the following mechanisms: reduction of autoimmune activity that causes abnormal platelet destruction, surgical removal of the spleen to prevent trapping of platelets in the spleen or stimulation of platelet production. First-line therapy is typically focused on reducing autoimmune activity and consists of corticosteroids or IVIG. Second line alternatives include rituximab, which is used off-label to reduce autoimmune activity, and splenectomy to address the trapping of the platelets in the spleen. TPO-RAs, which aim to stimulate platelet production, are also used in the second line treatment of chronic ITP.

### ***Reducing autoimmune activity***

Corticosteroids are predominantly used as the first-line treatment for ITP and raise platelet counts by suppressing the immune system so as to reduce the abnormal destruction of platelets. However, chronic use of corticosteroids is associated with a number of significant side effects, including osteoporosis, hypertension, acute increases in blood glucose, diabetes, depression and weight gain. Moreover, corticosteroids do not lead to durable remissions in the majority of patients with ITP, ultimately necessitating a second-line treatment.

If patients have active bleeding or there are contraindications for the use of corticosteroids, IVIG is the other first-line chronic treatment option. IVIG contains pooled immunoglobulin G, or IgG, from the plasma of approximately 1,000 or more blood donors and introduces these high levels of exogenously added IgG antibodies to the bloodstream. These IgG antibodies then compete with the patient's auto-antibodies in signaling various pathways, thereby lowering the impact of the patient's auto-antibodies. IVIG therapy can raise platelet counts within days in most patients, but its effect is usually transient, similar to a platelet transfusion. Additionally, IVIG is expensive and difficult to administer, which limits its use as a therapy. Side effects of IVIG, like other immunomodulating agents, include weakness, sweating, acute deep venous blood clots, nausea and vomiting.



For second-line treatment of ITP, rituximab, a monoclonal antibody, is used off-label to reduce autoimmune activity. Rituximab acts by reducing the number of cells that produce antibodies, including antibodies that can attach to platelets. However, rituximab has been shown to have limited efficacy as well as serious side effects, including reactivation of hepatitis B or hepatitis C infections.

For more severe ITP, a splenectomy is sometimes used as second-line treatment due to the spleen's role in antibody-mediated platelet destruction and trapping of platelets. Splenectomies involve surgical risk, are irreversible and are becoming less common as a treatment because of their limited success in raising platelet counts. Splenectomies also result in the subsequent impairment of the immune system due to the loss of multiple hematologic and immunologic functions. In addition, splenectomies lead to increased probability of complications and fatalities among patients over 65 given their highly invasive nature.

### *Stimulating platelet production*

Of the approximately 60,000 adults with chronic ITP in the United States, we estimate up to 27,000 require continuous treatment beyond corticosteroids and IVIG. In addition to off-label rituximab, TPO-RAs are used as a second-line treatment of ITP. TPO-RAs are a validated class of therapy for the treatment of ITP and other thrombocytopenic conditions. TPO-RAs, like TPO, stimulate the activation, proliferation and maturation of megakaryocytes, resulting in an increase in circulating platelet counts. There are two TPO-RAs approved for chronic ITP and other thrombocytopenic conditions:

- Eltrombopag, marketed as Promacta, is an oral small molecule that binds to a site on the TPO receptor that is distinct from native TPO. Eltrombopag was approved for use in patients with ITP in the United States in 2008 and its indications have subsequently been expanded to include aplastic anemia and cirrhosis due to chronic hepatitis C during interferon therapy. While effective, Promacta has a black box warning for hepatotoxicity in patients with chronic hepatitis C and use of the drug requires monthly tests to determine the degree of liver disease and liver function. Promacta also includes dietary restrictions such as the requirement that the drug be taken on an empty stomach, at least one hour before or two hours after a meal, and may not be administered within two hours before and four hours after taking antacids, calcium-rich foods or certain kinds of vitamin supplements. Sales of Promacta were \$635 million in 2016.
- Romiplostim, marketed as Nplate, is a recombinant polypeptide that binds to and activates the TPO receptor. It was approved for use in patients with ITP in the United States in 2008. Nplate is administered subcutaneously on a weekly basis, which is not as convenient as orally administered drugs. Sales of Nplate were \$584 million in 2016.

Because of the limitations of current therapies used for thrombocytopenia in the acute and chronic setting, we believe there remains a significant unmet need for a treatment that demonstrates reliable and durable effectiveness and a favorable safety profile, that can be conveniently administered orally and potentially reduce the burden on patients.

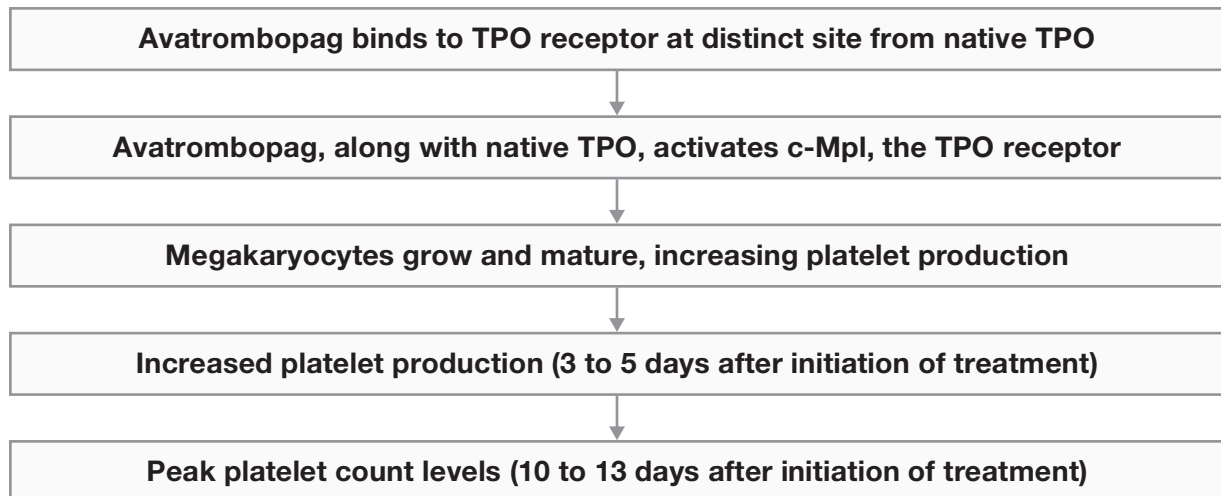
### **Our solution: avatrombopag**

We believe our drug candidate, avatrombopag, has the potential to be a first-in-class drug treatment of thrombocytopenia in the acute setting and a best-in-class treatment of thrombocytopenia in the chronic setting. Avatrombopag is an orally administered, small molecule TPO-RA, which is intended to address the limitations of other existing treatments for thrombocytopenia. We recently completed two identically designed Phase 3 pivotal clinical trials, ADAPT 1 and ADAPT 2, in which all primary and secondary endpoints were met with high statistical significance. The primary endpoint for both studies was the

percentage of CLD patients with thrombocytopenia undergoing a non-emergent minimally to moderately invasive medical procedure, who did not require a platelet transfusion or any rescue procedure for bleeding at each of two dose strengths of avatrombopag compared to placebo. We refer to minimally to moderately invasive medical procedures as procedures for which medical guidelines generally recommend patients to have at least 50,000 platelets per microliter of circulating blood before the procedure. In each trial, the percentage of subjects in each of the two avatrombopag dosing cohorts requiring a platelet transfusion or a rescue procedure for bleeding was statistically significantly lower compared to placebo (across all cohorts, p-values ranging from  $p < 0.0001$  to  $p = 0.0006$ ). We also observed a percentage of avatrombopag-treated subjects who achieved platelet counts of greater than 50,000 platelets per microliter of circulating blood on the procedure day and changes in platelet counts from baseline to procedure day, which were statistically significant improvements over placebo. We are initially developing avatrombopag for the acute treatment of thrombocytopenia in this population of patients with CLD undergoing non-emergent minimally to moderately invasive medical procedures.

Like native TPO and the currently marketed TPO-RAs, avatrombopag stimulates megakaryocyte growth and maturation leading to increased platelet production. Avatrombopag works in conjunction with the body's native TPO by being active at the same TPO receptor. Importantly, however, avatrombopag binds to the TPO receptor at a distinct site from native TPO, leaving the TPO receptor accessible to native TPO, enabling avatrombopag to have an additive effect on the proliferation of megakaryocytes. Typically, the time required for megakaryocytes to complete platelet production is approximately five days. Avatrombopag's onset of effect is seen in three to five days on average, with peak levels of platelet production observed 10 to 13 days after initiation of treatment. In both *in vitro* and *in vivo* studies on platelet function, avatrombopag was observed to lead to the production of healthy, functioning platelets.

The figure below describes the mechanism of action for avatrombopag.



In CLD patients, who often have excessive accumulation of scar tissue in the liver, portal blood flow may be significantly lower than normal which puts these patients at an increased risk of developing portal vein thrombosis, or PVT. Further, the use of some TPO-RAs may lead to an even greater risk of PVT in these patients as a sudden increase in platelets can give rise to platelet accumulation and cause further blockage of the portal vein. While an individual with normal platelet counts has between 150,000 to 450,000 platelets per microliter of circulating blood, platelet counts greater than 200,000 platelets per microliter of circulating blood can heighten the risk of PVT in a CLD patient. Therefore, physicians must carefully

manage platelet counts in these patients so that platelet counts do not exceed 200,000 platelets per microliter of circulating blood.

We believe having a more predictable pharmacokinetic, or PK, profile and pharmacodynamic, or PD, profile would allow physicians to prescribe treatments to patients based on their initial platelet counts without increased risk of adverse effects, such as PVTs, or lack of efficacy across the potential patient population. Avatrombopag has been observed to have a less variable PK/PD profile than other TPO-RAs. We believe the enzymes that are involved in the metabolism of avatrombopag, which differ from the enzymes involved in the metabolism of other TPO-RAs, lead to this lower PK/PD variability. In addition, avatrombopag is not extensively metabolized—approximately 40% to 50% is metabolized and is mostly eliminated away from the biliary route. We believe these metabolic characteristics further reduce the risk of adverse effects, including thromboembolic events such as PVTs, in patient populations that are liver compromised, such as those with CLD.

We believe avatrombopag's PK/PD profile and metabolic characteristics are the core attributes that differentiate it from the currently marketed TPO-RAs and make it a compelling treatment option for patients with thrombocytopenia in the acute setting.

- Efficacy—Based on avatrombopag's relatively predictable PK/PD profile and high potency, we believe we can establish a consistent dosing regimen over five days to provide a therapeutically meaningful increase in platelet counts, reducing the need for platelet transfusion prior to a non-emergent medical procedure or the need for a rescue transfusion following the procedure.
- Safety—Avatrombopag has been administered to over 1,100 patients and has been observed to be generally well tolerated in clinical trials. We believe avatrombopag's metabolic characteristics may reduce the risk of PVT as compared to other TPO-RAs. In the ADAPT 1 and ADAPT 2 clinical trials, in which 277 patients with CLD were treated with avatrombopag, there was only one treatment emergent thromboembolic event observed.
- Convenience—Avatrombopag allows for a patient-friendly once-daily oral medication taken with food at home for five days, starting approximately 10 to 13 days prior to a non-emergent medical procedure. The predictable four-day window of maximum platelet counts provides additional flexibility for scheduling in the elective surgery setting. This is distinct from platelet transfusions, which must occur the day of the procedure and limit scheduling flexibility.

Additionally, we believe avatrombopag specifically addresses the shortcomings associated with platelet transfusions in several ways, including:

- reduced risk of antibody development and the associated development of refractoriness;
- extended duration of effect by maintaining platelet counts for a window of four days versus only hours for platelet transfusions, potentially allowing for a greater window of time for procedure scheduling;
- increased ease of use and patient convenience with oral dosing, which avoids the need to schedule a visit to an infusion center;
- reduced demand on a scarce resource, as platelet shortages continue to exist across parts of the United States and Europe; and
- reduced risks of adverse reactions and infection and none of the risks associated with blood-to-blood disease transmission.

In addition, based on a third-party survey of 155 physicians that we commissioned in 2017, the collective responses from the physicians indicated a preference for a drug with attributes we believe are demonstrated by avatrombopag over either platelet transfusions or currently available TPO-RAs across nine factors, including overall efficacy, overall safety, ease of use and risk of thrombotic events. Based on the results of our clinical trials, we also believe avatrombopag has the potential for use in a broader population of thrombocytopenia patients regardless of disease etiology undergoing a broader set of medical procedures, including, for example, joint replacements. We believe it may also have the potential for the treatment of patients who develop thrombocytopenia after receiving chemotherapy. In addition, we are evaluating the potential regulatory approval pathway for avatrombopag for the treatment of adults with chronic ITP based on results from a completed Phase 3 trial in this patient population.

## **Avatrombopag clinical development program**

In March 2016, we acquired the worldwide rights to avatrombopag from Eisai. Prior to our acquisition, avatrombopag had completed one Phase 3 trial, five Phase 2 trials and 15 Phase 1 trials, including single- and multiple-dose, dose-ranging and tolerability trials, in which it was observed to be generally well tolerated. Additionally, in the Phase 2 and Phase 3 clinical trials, all primary endpoints were met with high statistical significance. Avatrombopag has also been evaluated in multiple preclinical, PK, safety and toxicology studies that we believe will provide support for an NDA filing. Following our acquisition of avatrombopag, we completed two identically designed Phase 3 pivotal clinical trials, ADAPT 1 and ADAPT 2, which were ongoing at the time of the acquisition, to evaluate its safety and efficacy compared to placebo for the treatment of thrombocytopenia in CLD patients undergoing a non-emergent minimally to moderately invasive medical procedure. In these trials, avatrombopag was observed to achieve the primary efficacy endpoint with high statistical significance and was observed to be generally well tolerated.

Eisai originally submitted an investigational new drug application, or IND, for avatrombopag in 2011 for CLD patients with thrombocytopenia undergoing an elective, or non-emergent, procedure. Eisai also submitted INDs for avatrombopag in 2009 for CIT, and in 2005 for ITP. Eisai continues to hold these INDs for avatrombopag, although we have the right to have these INDs transferred to us at any time. We expect Eisai to submit the NDA for avatrombopag, and we intend to request transfer of these INDs and the NDA prior to our commercialization of avatrombopag.

### ***Avatrombopag for the treatment of thrombocytopenia associated with CLD prior to a non-emergent minimally to moderately invasive medical procedure***

#### *Overview of chronic liver disease*

We are initially focused on developing avatrombopag for the treatment of thrombocytopenia in CLD patients undergoing non-emergent minimally to moderately invasive medical procedures. CLD involves the progressive destruction and regeneration of the liver over a period of more than six months. CLD consists of a wide range of liver etiologies, including hepatitis, such as hepatitis B and hepatitis C viruses, and non-alcoholic steatohepatitis (NASH); liver cirrhosis; and hepatocellular carcinoma. There are an estimated 7.4 million CLD patients in the United States.

Patients with CLD often present with advanced liver fibrosis, or excessive accumulation of scar tissue resulting from ongoing inflammation and liver cell death. Patients with CLD can experience substantial fluid build-up in the abdomen, swelling, weakness and easy bruising. The buildup of scar tissue can disrupt the metabolic functions of the liver. Because the production of TPO is dependent on functional liver cell mass, TPO production is reduced when liver cell mass becomes severely damaged. The resulting decrease in TPO production leads to a reduction in megakaryocytes and platelet production. Patients with CLD also have

increased trapping of platelets in the spleen and thus even fewer platelets are present in circulating blood. In both instances, these patients often develop thrombocytopenia. Thrombocytopenia affects approximately 1.1 million CLD patients in the United States and approximately 70,000 of those patients have a platelet count of less than 50,000 platelets per microliter of circulating blood.

Patients with CLD undergo numerous non-emergent medical procedures for diagnosis and treatment of their disease, including liver biopsies, fluid removal, liver transplantation and endoscopy. These medically necessary procedures are scheduled in advance at a time selected by the patient and treating physician. Thrombocytopenia can complicate the treatment of patients with CLD who require a procedure as part of their routine clinical care because it reduces the ability of blood to clot efficiently, thus putting patients at risk of excessive bleeding either during or after a medical or surgical procedure. As a result, if a patient has thrombocytopenia, doctors may delay or even cancel these procedures until the patient is able to achieve a sufficient platelet count to adequately control bleeding during and following the procedure. For CLD patients with thrombocytopenia and platelet counts less than 50,000 platelets per microliter of circulating blood who are undergoing a non-emergent medical procedure, platelet transfusions are used as the standard of care to increase platelet counts prior to the procedure. Despite being the standard of care, platelet transfusions are associated with limitations that impact their use including risk of antibody development in up to 50% of patients, short duration of effect of transfused platelets, limited supply and inconvenience of administration.

Further, the use of some TPO-RAs may lead to an even greater risk of PVT in these patients as a sudden increase in platelets can give rise to platelet accumulation and cause further blockage of the portal vein. While an individual with normal platelet counts has between 150,000 to 450,000 platelets per microliter of circulating blood, platelet counts greater than 200,000 platelets per microliter of circulating blood can heighten the risk of PVT in a CLD patient. Therefore, physicians must carefully manage platelet counts in these patients so that platelet counts do not exceed 200,000 platelets per microliter of circulating blood.

Neither Promacta nor Nplate has been approved by the FDA or EMA for the acute treatment of thrombocytopenia due, in part, to the risk of side effects, including PVT (in the case of Promacta). We believe avatrombopag has the potential to provide an effective, durable, convenient and safe alternative to platelet transfusions for treating patients with CLD undergoing minimally to moderately invasive non-emergent medical procedures.

#### *Clinical development*

To date, avatrombopag has completed two Phase 2 clinical trials and two Phase 3 clinical trials in patients with thrombocytopenia associated with CLD undergoing a non-emergent minimally to moderately invasive medical procedure. Based on the trial results for avatrombopag to date, an NDA is planned for submission to the FDA in the third quarter of 2017. We also intend to submit a MAA to the EMA in the first half

of 2018. We plan to include data from the clinical trials summarized in the table below in our NDA submission.

| Trial   | Once-Daily Oral Dosage         | Trial Completion Date | Subjects |                           | Achieved Primary Endpoint | Key Efficacy Results   |
|---|--------------------------------|-----------------------|----------|---------------------------|---------------------------|--|
|   |                                |                       | Total    | Treated with Avatrombopag |                           |  |
| <p><b>Phase 3 Study 310 (ADAPT 1):</b></p> <ul style="list-style-type: none"> <li>Global, multicenter, randomized, double-blind, placebo-controlled, parallel group trial</li> <li>Evaluated efficacy and safety</li> <li>Subjects with thrombocytopenia associated with CLD prior to non-emergent minimally to moderately invasive medical procedures</li> </ul> | 40 mg/60 mg for 5 days         | March 2017            | 231      | 149                       | ✓                         | <p>Subjects who did not require a platelet transfusion or any rescue procedure for bleeding:</p> <ul style="list-style-type: none"> <li>Lower Platelet Cohort: 65.6% (subjects who received avatrombopag) vs. 22.9% (subjects who received placebo) (<math>p &lt; 0.0001</math>).</li> <li>Higher Platelet Cohort: 88.1% (subjects who received avatrombopag) vs. 33.3% (subjects who received placebo) (<math>p &lt; 0.0001</math>).</li> </ul> |
| <p><b>Phase 3 Study 311 (ADAPT 2):</b></p> <ul style="list-style-type: none"> <li>Global, multicenter, randomized, double-blind, placebo-controlled, parallel group trial</li> <li>Evaluated efficacy and safety</li> <li>Subjects with thrombocytopenia associated with CLD prior to non-emergent minimally to moderately invasive medical procedures</li> </ul> | 40 mg/60 mg for 5 days         | March 2017            | 204      | 128                       | ✓                         | <p>Subjects who did not require a platelet transfusion or any rescue procedure for bleeding:</p> <ul style="list-style-type: none"> <li>Lower Platelet Cohort: 68.6% (subjects who received avatrombopag) vs. 34.9% (subjects who received placebo) (<math>p = 0.0006</math>).</li> <li>Higher Platelet Cohort: 87.9% (subjects who received avatrombopag) vs. 38.2% (subjects who received placebo) (<math>p &lt; 0.0001</math>).</li> </ul>    |
| <p><b>Phase 2 Study 202:</b></p> <ul style="list-style-type: none"> <li>Multicenter, randomized, double-blind, placebo-controlled, parallel group trial</li> <li>Evaluated efficacy, safety and PK</li> <li>Subjects with thrombocytopenia associated with CLD prior to non-emergent minimally to moderately invasive medical procedures</li> </ul>               | 10 mg/20 mg/40 mg/80 mg/100 mg | December 2011         | 130      | 93                        | ✓                         | Percentage of subjects with a platelet increase from baseline of at least 20,000 platelets per microliter of circulating blood and an overall platelet count of at least 50,000 platelets per microliter of circulating blood at least once from day 4 to day 8, increased statistically significantly ( $p < 0.01$ ) for subjects receiving avatrombopag vs. subjects receiving placebo   |
| <p><b>Phase 2 Study 204:</b></p> <ul style="list-style-type: none"> <li>Randomized, double-blind, placebo-controlled, parallel-group trial</li> <li>Evaluated efficacy, safety, and PK</li> <li>Japanese subjects with thrombocytopenia associated with CLD</li> </ul>  | 20 mg/40 mg/60 mg for 5 days   | April 2015            | 33       | 28                        | ✓                         | Percentage of subjects with a platelet increase from baseline of at least 20,000 platelets per microliter of circulating blood and an overall platelet count of at least 50,000 platelets per microliter of circulating blood at visit 4 increased statistically significantly for subjects receiving 40 mg per day and 60 mg per day doses of avatrombopag vs. subjects receiving placebo   |

### Phase 3 trials (ADAPT 1 and ADAPT 2)

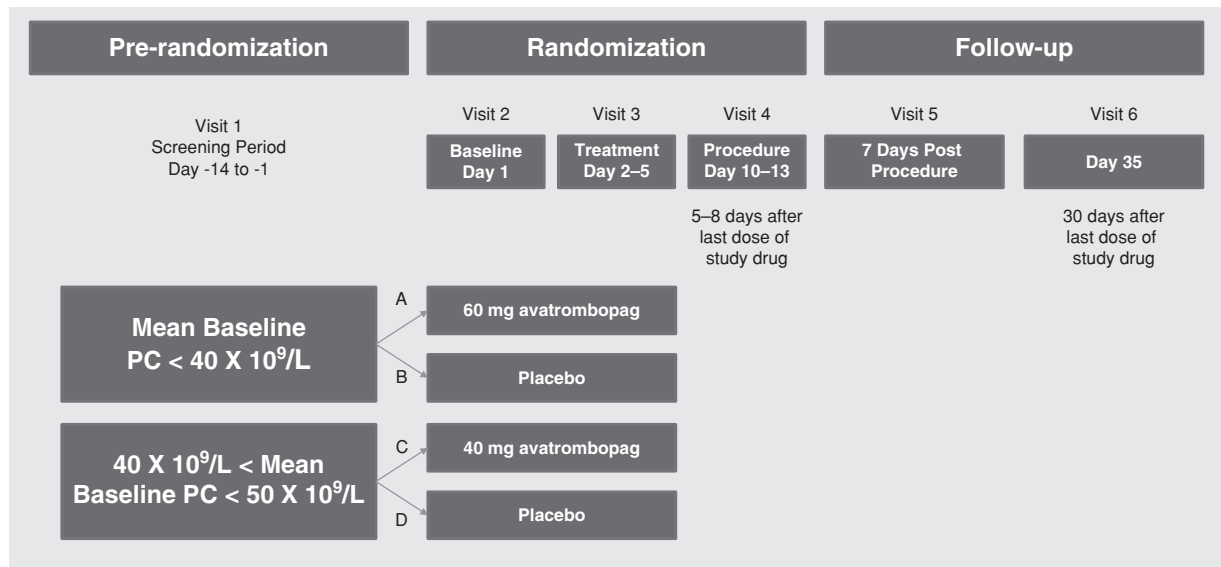
#### Trial design

We recently completed two identically designed Phase 3 pivotal clinical trials to evaluate the efficacy of avatrombopag compared to placebo for the treatment of thrombocytopenia in CLD patients undergoing a non-emergent minimally to moderately invasive medical procedure. These randomized, double-blind, placebo-controlled, parallel-group trials were conducted in geographically overlapping regions in the United States and internationally.

Each trial consisted of three phases: pre-randomization, randomization and follow-up. The pre-randomization phase involved an initial screening visit that took place within two weeks prior to randomization. During the randomization phase, subjects were divided into two cohorts according to their mean baseline platelet count: 1) a lower platelet count cohort, which included those subjects with platelet count below 40,000 platelets per microliter of circulating blood and 2) a higher platelet count cohort, which included those subjects with platelet counts between 40,000 and 50,000 platelets per microliter of circulating blood. In both studies, subjects in both dosing cohorts were randomized on a 2-to-1 basis between avatrombopag and placebo. Subjects in the lower platelet count cohort received either a 60 mg per day dose of avatrombopag or placebo for five days, while subjects in the higher platelet count cohort received either a 40 mg per day dose of avatrombopag or placebo for five days.

The non-emergent minimally to moderately invasive medical procedure was performed between five and eight days following the last dose of study drug. Permitted surgical procedures included procedures with a low risk of bleeding, such as fluid removal or a gastrointestinal endoscopy, moderate risk of bleeding, such as a liver biopsy or a bronchoscopy, and high risk of bleeding, such as dental procedures.

During the follow-up phase, subjects were evaluated seven days after the procedure and 30 days following the last dose. The trial design of our two Phase 3 trials is shown in the figure below.



### Endpoints

The primary efficacy endpoint for both trials was the percentage of subjects who did not require a platelet transfusion or any rescue procedure for bleeding after randomization and up to seven days following their procedure.

Secondary efficacy endpoints included:

- Percentage of subjects who achieve platelet counts of greater than 50,000 platelets per microliter of circulating blood on the day of the procedure (i.e., prior to receiving a platelet transfusion or undergoing the procedure).
- Change from baseline in platelet count on the day of the procedure (i.e., prior to receiving a platelet transfusion or undergoing the procedure).

The primary and secondary efficacy endpoints were powered to show statistical significance and superiority compared to placebo. We also evaluated the safety of avatrombopag in the trials.

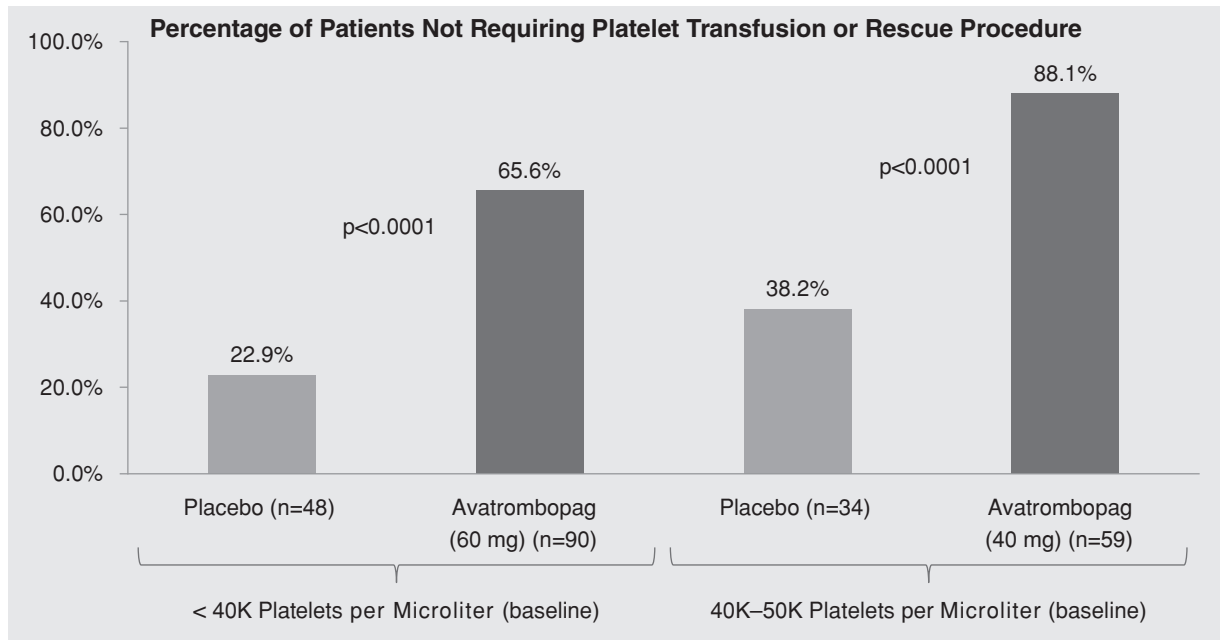
#### *ADAPT 1–enrollment*

A total of 231 subjects were enrolled in ADAPT 1 at 75 sites across 19 different countries. 158 subjects were male and 73 subjects were female. 128 subjects were Caucasian, 43 subjects were Korean, 24 subjects were Chinese, 22 subjects were from other Asian countries and 14 subjects were other ethnicities. Of the 231 subjects enrolled, 138 subjects had a platelet count below 40,000 platelets per microliter of circulating blood and 93 subjects had a platelet count between 40,000 and 50,000 platelets per microliter of circulating blood. In the lower platelet count cohort, 48 subjects received placebo, of whom 46 completed the trial, and 90 subjects received avatrombopag, of whom 85 subjects completed the trial. In the higher platelet count cohort, 34 subjects received placebo, all of whom completed the trial, and 59 subjects received avatrombopag, of whom 55 subjects completed the trial. Of the nine subjects who did not complete the trial, most did not complete the follow-up period or withdrew their consent, though one subject receiving a 60 mg per day dose of avatrombopag withdrew due to muscle pain and anemia.

#### *ADAPT 1–efficacy results*

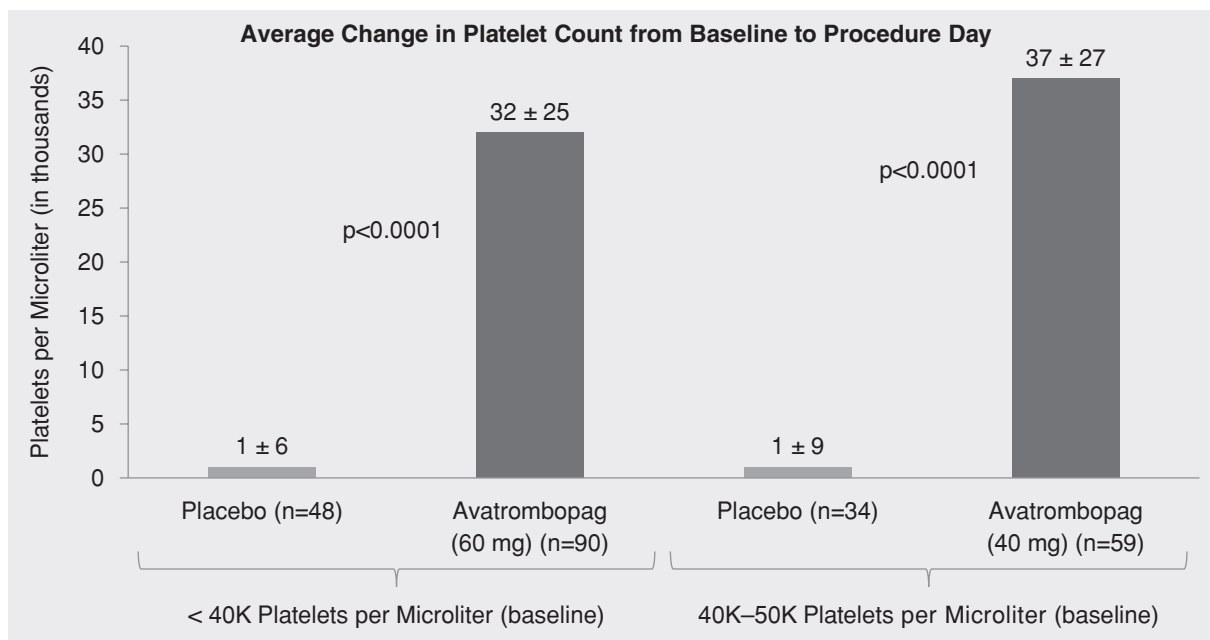
In ADAPT 1, as shown in the figure below, for the primary endpoint, we observed a statistically significant improvement in the percentage of subjects who did not require a platelet transfusion or any rescue procedure for bleeding after randomization for both the 60 mg dose/day of avatrombopag and the 40 mg dose/day of avatrombopag compared to placebo in the lower and higher platelet count cohorts, respectively. Of the subjects who completed the trial in the lower platelet count cohort, 65.6% of subjects who received avatrombopag did not require a platelet transfusion or any rescue procedure for bleeding, compared to 22.9% of subjects who received placebo ( $p < 0.0001$ ). Of the subjects who completed the trial in the higher platelet count cohort, 88.1% of subjects who received avatrombopag did not require a platelet transfusion or any rescue procedure for bleeding, compared to 38.2% of subjects who received placebo ( $p < 0.0001$ ). P-value is a conventional statistical method for measuring the statistical significance of clinical results. A p-value of 0.05 or less represents the standard statistical significance threshold in clinical trials, meaning that there is a less than 1-in-20 (i.e., 5%) likelihood that the observed results occurred by chance.





We also observed a statistically significant improvement in the percentage of subjects who achieved platelet counts of greater than 50,000 platelets per microliter of circulating blood on the day of the procedure. Of the subjects who completed the trial in the lower platelet count cohort, 68.9% of subjects who received avatrombopag achieved platelet counts of greater than 50,000 platelets per microliter of circulating blood on the day of the procedure, compared to 4.2% of subjects who received placebo ( $p < 0.0001$ ). Of the subjects who completed the trial in the higher platelet count cohort, 88.1% of subjects who received avatrombopag achieved platelet counts of greater than 50,000 platelets per microliter of circulating blood on the day of the procedure, compared to 20.6% of subjects who received placebo ( $p < 0.0001$ ).

Finally, we observed a statistically significant improvement in the change from baseline in platelet count on the day of the procedure, as shown in the figure below. Of the subjects who completed the trial in the lower platelet count cohort, subjects who received avatrombopag increased their platelet count by an average of 31,900 platelets per microliter of circulating blood from baseline, compared to an average increase of 1,000 platelets per microliter of circulating blood for subjects who received placebo ( $p < 0.0001$ ). Of the subjects who completed the trial in the higher platelet count cohort, subjects who received avatrombopag increased their platelet count by an average of 37,100 platelets per microliter of circulating blood from baseline, compared to an average increase of 1,000 platelets per microliter of circulating blood for subjects who received placebo ( $p < 0.0001$ ). No subjects who received avatrombopag were observed to have a platelet count exceeding 200,000 platelets per microliter of circulating blood on the procedure day.

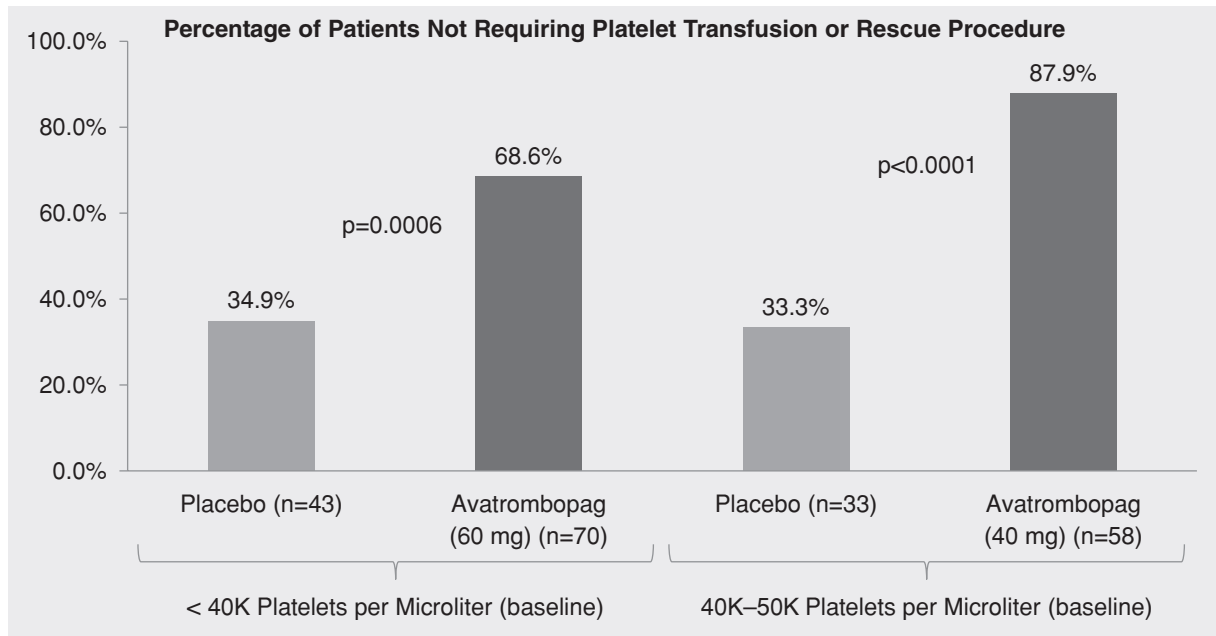


#### *ADAPT 2—enrollment*

A total of 204 subjects were enrolled in ADAPT 2 at 74 sites across 16 different countries. 127 subjects were male and 77 subjects were female. 131 subjects were Caucasian, 50 subjects were Japanese and 23 subjects were other ethnicities. Of the 204 subjects enrolled, 113 subjects had a platelet count below 40,000 platelets per microliter of circulating blood and 91 subjects had a platelet count between 40,000 and 50,000 platelets per microliter of circulating blood. In the lower platelet count cohort, 43 subjects received placebo, of whom 37 completed the trial, and 70 subjects received avatrombopag, of whom 68 subjects completed the trial. In the higher platelet count cohort, 33 subjects received placebo, of whom 31 completed the trial, and 58 subjects received avatrombopag, of whom 55 subjects completed the trial. Of the 13 subjects who did not complete the trial, most did not complete the follow-up period or withdrew their consent, though one subject receiving placebo died during the trial.

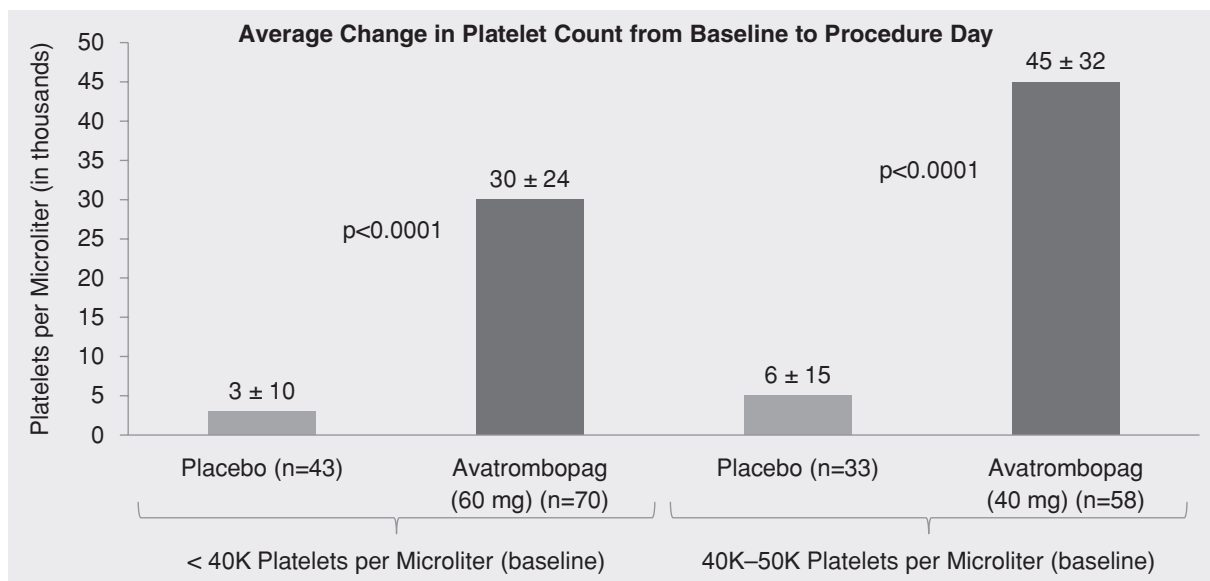
#### *ADAPT 2—efficacy results*

Consistent with ADAPT 1, in ADAPT 2, for the primary endpoint, we also observed a statistically significant improvement in the percentage of subjects who did not require a platelet transfusion or any rescue procedure for bleeding after randomization for both the 60 mg dose/day of avatrombopag and the 40 mg dose/day of avatrombopag compared to placebo in both the lower and higher platelet count cohorts, respectively. Of the subjects who completed the trial in the lower platelet count cohort, 68.6% of subjects who received avatrombopag did not require a platelet transfusion or any rescue procedure for bleeding, compared to 34.9% of subjects who received placebo ( $p=0.0006$ ). Of the subjects who completed the trial in the higher platelet count cohort, 87.9% of subjects who received avatrombopag did not require a platelet transfusion or any rescue procedure for bleeding, compared to 33.3% of subjects who received placebo ( $p<0.0001$ ). These results are shown in the figure below.



We also observed a statistically significant improvement in the percentage of subjects who achieved platelet counts of greater than 50,000 platelets per microliter of circulating blood on the day of the procedure. Of the subjects who completed the trial in the lower platelet count cohort, 65.7% of subjects who received avatrombopag achieved platelet counts of greater than 50,000 platelets per microliter of circulating blood on the day of the procedure, compared to 7.0% of subjects who received placebo ( $p<0.0001$ ). Of the subjects who completed the trial in the higher platelet count cohort, 93.1% of subjects who received avatrombopag achieved platelet counts of greater than 50,000 platelets per microliter of circulating blood on the day of the procedure, compared to 39.4% of subjects who received placebo ( $p<0.0001$ ).

Finally, we observed a statistically significant improvement in the change from baseline in platelet count on the day of the procedure, as shown in the figure below. Of the subjects who completed the trial in the lower platelet count cohort, subjects who received avatrombopag increased their platelet count by an average of 30,400 platelets per microliter of circulating blood from baseline, compared to an average increase of 3,000 platelets per microliter of circulating blood for subjects who received placebo ( $p<0.0001$ ). Of the subjects who completed the trial in the higher platelet count cohort, subjects who received avatrombopag increased their platelet count by an average of 44,900 platelets per microliter of circulating blood from baseline, compared to an average increase of 5,700 platelets per microliter of circulating blood for subjects who received placebo ( $p<0.0001$ ). Of the subjects who received avatrombopag, one subject was observed to have a platelet count exceeding 200,000 platelets per microliter of circulating blood prior to the procedure day, one subject was observed to have a platelet count exceeding 200,000 platelets per microliter of circulating blood on the procedure day, and one subject was observed to have a platelet count exceeding 200,000 platelets per microliter of circulating blood after the procedure day. Despite these elevated platelet counts, none of these patients experienced a PVT.



#### Safety results for ADAPT 1 and ADAPT 2

In ADAPT 1 and ADAPT 2, avatrombopag was observed to be generally well tolerated at both the 40 mg and 60 mg dose. Treatment emergent adverse events, or TEAEs, including fever, nausea and abdominal pain, were similar in frequency in the avatrombopag and placebo groups. The incidence and severity of TEAEs, which were graded on the Common Terminology Criteria for Adverse Events, or CTCAE, scale, observed in avatrombopag-treated patients and in the placebo groups in the ADAPT 1 and ADAPT 2 clinical trials are summarized in the chart below.

| ADAPT 1 and ADAPT 2 Treatment Emergent Safety Results                          |            |              |
|--|------------|--------------|
| Category   | Placebo    | Avatrombopag |
|  | (n=156)    | (n=274)      |
| TEAEs  | 86 (55.1%) | 148 (54.0%)  |
| Treatment-related TEAEs  | 20 (12.8%) | 26 (9.5%)    |
| TEAE with CTCAE grade 3 or above   | 16 (10.3%) | 30 (10.9%)   |
| Serious TEAEs  | 14 (9.0%)  | 20 (7.3%)    |
| Deaths   | 1 (0.6%)   | 2 (0.7%)     |
| Other serious adverse events   | 13 (8.3%)  | 18 (6.6%)    |
| Life threatening   | 0 (0.0%)   | 1 (0.4%)     |
| Required inpatient hospitalization or prolongation of existing hospitalization | 11 (7.1%)  | 18 (6.6%)    |
| Important medical events   | 3 (1.9%)   | 0 (0.0%)     |
| TEAEs leading to study drug withdrawal   | 0 (0.0%)   | 2 (0.7%)     |

In the higher platelet count cohort of the ADAPT 1 trial, two subjects died more than 28 days after conclusion of treatment with avatrombopag and the deaths were considered not related to treatment. The

half life of avatrombopag is approximately 19 hours. These patients also suffered from numerous co-morbidities.

In the higher platelet count cohort in the ADAPT 2 trial, one subject experienced a partial thrombosis of the right branch of the portal vein. The highest platelet count recorded for this subject after treatment was 77,000 platelets per microliter of circulating blood and, at the time of identification of the partial thrombosis, the subject was observed to have a platelet count of 61,000 platelets per microliter of circulating blood. The partial thrombosis was judged by the investigator to be non-serious and possibly related to treatment.

### *Phase 2 clinical trials*

From 2009 to 2011, a Phase 2, randomized, multicenter, placebo-controlled, double-blind, parallel-group trial (Study 202) was conducted to evaluate the efficacy, safety, and population PK of once-daily oral avatrombopag tablets used up to seven days in subjects with CLD and thrombocytopenia prior to non-emergent minimally to moderately invasive medical procedures. Subjects either received a 100 mg single dose of avatrombopag, followed by a 20, 40 or 80 mg dose of avatrombopag once a day for six days, or a 80 mg single dose of avatrombopag, followed by a 10 mg of avatrombopag once a day for six days or a 20 mg dose of avatrombopag once a day for three days. The results of this trial were published in the Journal of Hepatology in 2014. A total of 130 subjects were enrolled in the trial, which was conducted at 29 sites in the United States.

In Study 202 the primary endpoint was the percentage of responders, defined as subjects with a platelet increase from baseline of at least 20,000 platelets per microliter of circulating blood and an overall platelet count of at least 50,000 platelets per microliter of circulating blood at least once from day 4 to day 8. In the study, a statistically significant increase ( $p < 0.01$ ) was observed for the primary endpoint for subjects receiving avatrombopag compared to subjects receiving placebo. In addition, 5% of subjects in the avatrombopag treatment groups required a platelet transfusion prior to the procedure, compared to 35% of subjects who received placebo requiring a platelet transfusion prior to the procedure ( $p < 0.05$ ).

Avatrombopag was also observed to be generally well tolerated in Study 202, even in subjects initially treated with 80 mg and 100 mg doses of avatrombopag. There was an overall higher incidence of treatment emergent adverse events in the avatrombopag group (83.9%) compared to the placebo group (75.7%). The overall incidence of serious treatment emergent adverse events was higher in the combined avatrombopag group (17.2%) compared to the combined placebo group (10.8%). One subject in the avatrombopag group died due to serious adverse events, though the cause of death was not confirmed via autopsy. In addition, one subject who received an initial 100 mg dose of avatrombopag, followed by 80 mg doses of avatrombopag once a day for six days, experienced PVT, which was asymptomatic and judged by the investigator to be not serious and possibly related to the trial drug, and one subject in the avatrombopag group discontinued from the trial due to nausea and vomiting. The commonly reported treatment emergent adverse events and serious treatment emergent adverse events were consistent with adverse events typically experienced by CLD patients with thrombocytopenia.

In addition, from 2014 to 2015, a second Phase 2, randomized, double-blind, placebo-controlled, parallel-group trial (Study 204) was conducted to evaluate the efficacy, safety, and PK of once-daily oral avatrombopag in Japanese subjects with CLD and thrombocytopenia. A total of 39 subjects were enrolled in the trial, which was conducted at 21 sites in Japan.

In Study 204, the primary endpoint was the percentage of responders, defined as subjects with a platelet increase from baseline of at least 20,000 platelets per microliter of circulating blood and an overall

platelet count of at least 50,000 platelets per microliter of circulating blood at visit 4, which occurred approximately 10 days after the initiation of treatment. In the study, a statistically significant increase was observed for the primary endpoint in subjects receiving 40 mg per day and 60 mg per day doses of avatrombopag compared to subjects receiving placebo. Avatrombopag was also observed to be generally well tolerated in Study 204 for each of the 20 mg, 40 mg and 60 mg avatrombopag dose groups. No deaths, serious adverse events or treatment emergent adverse events were reported.

#### *Regulatory approval plan*

Based on the results from these trials, an NDA for avatrombopag is planned for submission to the FDA in the third quarter of 2017. In addition, as our Phase 3 trials were also designed to be pivotal trials in Europe, we intend to submit a MAA to the EMA in the first half of 2018.

#### ***Avatrombopag for the acute treatment of thrombocytopenia patients, including patients without CLD***

In addition to pursuing marketing approval for avatrombopag for the treatment of thrombocytopenia in CLD patients undergoing a non-emergent minimally to moderately invasive medical procedure, we believe avatrombopag has the potential for use in a broader population of thrombocytopenia patients regardless of disease etiology undergoing a broader set of medical procedures, including, for example, joint replacements. We believe it may also have the potential to treat patients who develop thrombocytopenia after receiving chemotherapy.

- *Patients with thrombocytopenia, regardless of etiology, prior to a medical procedure, regardless of the degree of invasiveness.* In addition to our initial focus on the treatment of thrombocytopenia in CLD patients undergoing a non-emergent minimally to moderately invasive medical procedure, we intend to explore avatrombopag for the acute treatment of patients with thrombocytopenia in broader patient populations. This includes patients with thrombocytopenia associated with other etiologies besides CLD, and patients with thrombocytopenia, regardless of etiology, undergoing highly invasive medical procedures that require patients to have more than 100,000 platelets per microliter of circulating blood. There are approximately 100,000 platelet transfusions performed prior to surgery in the United States each year in patients with thrombocytopenia not associated with CLD. For patients with thrombocytopenia undergoing highly invasive medical procedures, in particular, we believe avatrombopag has the potential to increase platelet counts above 100,000 platelets per microliter of circulating blood, thereby enabling the surgeon to conduct the planned procedure. We intend to initiate a late-stage clinical development program in 2018 to evaluate avatrombopag for the treatment of a broader population of patients with thrombocytopenia undergoing invasive surgical procedures.
- *Patients with chemotherapy-induced thrombocytopenia (CIT).* CIT is a common complication in cancer patients undergoing chemotherapy treatment. CIT increases the risk of bleeding and also can require oncologists to reduce the dose or delay chemotherapy treatment. Such changes in treatment dosing and timing can compromise chemotherapy treatment and lead to sub-optimal outcomes in cancer patients. The current standard of care for CIT is prophylactic platelet transfusions. Approximately 400,000 patients with solid tumors in the United States are treated with chemotherapy each year, of which approximately 175,000 patients develop CIT. We intend to initiate a late-stage clinical development program to evaluate avatrombopag for the potential treatment of patients with CIT being treated for solid tumors in 2018.

#### ***Avatrombopag for the chronic treatment of thrombocytopenia in adults with chronic ITP***

We are also evaluating the potential regulatory approval pathway for avatrombopag for the treatment of adults with chronic ITP based on results from a completed Phase 3 trial in this patient population. We

estimate that chronic ITP affects approximately 60,000 adults in the United States, of which up to 27,000 may require continuous treatment beyond corticosteroids and IVIG.

To date, avatrombopag has completed one Phase 3 clinical trial and two Phase 2 clinical trials evaluating the use of avatrombopag for the treatment of adults with chronic ITP, in which an aggregate of 128 subjects received avatrombopag. In these trials, avatrombopag was observed to achieve the primary efficacy endpoints with statistical significance and was observed to be generally well tolerated. The results of these trials have previously been submitted to the FDA, but not formally discussed. Success in these previous trials does not ensure that we will be able to adequately demonstrate the efficacy and safety of avatrombopag to the FDA. We intend to request a meeting with the FDA to discuss the results from these trials, and based upon the feedback we receive from the FDA, we intend to evaluate the appropriate approval path of avatrombopag for the treatment of adults with chronic ITP.

## **Manufacturing**

We do not have any manufacturing facilities. We rely on Eisai, acting as our contract manufacturer, for the production of avatrombopag for clinical trials and commercial supply, and expect to continue to rely on Eisai for the commercial manufacture of avatrombopag if it receives marketing approval. We have entered into a long-term supply agreement with Eisai to govern this manufacturing support. We believe commercial manufacture will occur at the same facilities at which pre-commercial manufacturing and analytical testing was performed.

Eisai's manufacturing facilities have produced avatrombopag tablets and executed analytical testing on multiple development batches and six batches at commercial scale since 2010, employing routine methods and equipment that are commonly used in the pharmaceutical industry for oral solid dosage processing. The facility that synthesizes the API has successfully completed process validation. Process validation is currently ongoing at the drug manufacturing facility and is expected to be completed in the second half of 2017. All analytical test methods for both the API as well as the drug have been successfully validated at these facilities.

We believe Eisai's manufacturing processes are in compliance with guidance from global regulatory authorities such that the drug should meet the manufacturing requirements for world-wide distribution pending appropriate regulatory clearance in the applicable jurisdiction. There is an active stability program designed to ensure the safety and efficacy of the drug while in distribution. We anticipate that avatrombopag finished product will have a shelf life of at least 36 months at the time of launch.

## **Commercialization**

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. As we expand the indications for avatrombopag, we also intend to broaden our sales force to target hematologists. We have begun to execute on this strategy by hiring key executives with global commercial launch experience.

We may pursue collaborations with third parties to commercialize our drug candidates outside the United States, either through territorial licenses or distributor relationships. In the future, we also may selectively partner with leading companies that we believe can contribute additional resources and know-how for the

development and commercialization of avatrombopag for additional indications and geographic regions, further enhancing the value of our drug candidate.

We believe that it is imperative to keep the patient at the center of our focus, and we intend to work with and listen closely to key stakeholders, including patient advocacy groups, healthcare professionals, key opinion leaders and academic institutions, to ensure that we clearly understand their issues, insights and recommendations. The feedback from and collaboration with these groups will inform our key strategies.

## Competition

The pharmaceutical industry is characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary drugs. We face potential competition from many different sources, including major pharmaceutical, specialty pharmaceutical and biotechnology companies, academic institutions, governmental agencies and public and private research institutions. Any drug candidates that we successfully develop and commercialize will compete with existing treatments and new treatments that may become available in the future.

The key competitive factors affecting the success of avatrombopag, if approved, are likely to be its efficacy, safety, convenience, pricing and durability.

With respect to avatrombopag for the treatment of thrombocytopenia in patients with CLD undergoing a non-emergent minimally to moderately invasive medical procedure, we will be primarily competing with platelet transfusions, since neither of the available TPO-RAs are FDA-approved 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 thrombocytopenia in patients with ITP, we anticipate competing directly 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.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize drugs that are safer, more effective, more convenient, less expensive or with a more favorable label than avatrombopag or any other drug that we may develop. Our competitors also may obtain FDA or other regulatory approvals for their drugs more rapidly than we may obtain approval for our drug, 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 will also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and subject registration for clinical trials, as well as in acquiring technologies complementary to, or that may be necessary for, our programs.



## Intellectual property

Our commercial success depends in part on our ability to obtain and maintain proprietary protection for avatrombopag and any of our future drug candidates, novel discoveries, drug development technologies and know-how; to operate without infringing on or otherwise violating the proprietary rights of others; and to prevent others from infringing or otherwise violating our proprietary rights. Our policy is to seek to protect our proprietary position by, among other methods, filing or in-licensing U.S. and foreign patents and patent applications related to our drug candidate and other proprietary technology, inventions and improvements that are important to the development and implementation of our business. We also rely on trademarks, trade secrets, know-how, continuing technological innovation and potential in-licensing opportunities to develop and maintain our proprietary position.

While we seek broad coverage under our pending patent applications, there is always a risk that an modification of the manufacturing process may allow a competitor to avoid infringement claims. In addition, patents, if granted, expire and we cannot provide any assurance that any patents will be issued from our pending or any future applications or that any issued patents will adequately protect our drugs or drug candidates.

As of March 31, 2017, we own 24 foreign patents and two foreign patent applications related to avatrombopag, and we license from Astellas three granted U.S. patents, 40 foreign patents and two foreign patent applications related to avatrombopag. Our patent portfolio for avatrombopag includes a patent family directed to the avatrombopag composition of matter and methods of using avatrombopag, which is expected to expire between 2023 and 2027, excluding any extension of patent term that may be available. Our patent portfolio for avatrombopag also includes a European patent application directed to the crystalline avatrombopag compound, which, if issued, would expire in 2032, excluding any extension of patent term that may be available.

Individual patents extend for varying periods depending on the date of filing of the patent application or the date of patent issuance and the legal term of patents in the countries in which they are obtained. Generally, patents issued from regularly filed applications in the United States are granted a term of 20 years from the earliest effective filing date. In addition, in certain instances, a patent term can be adjusted to recapture a portion of the United States Patent and Trademark Office, or the USPTO, delay in issuing the patent, and extended to recapture a portion of the patent term effectively lost as a result of the FDA regulatory review period of the drug covered by the patent. However, as to the FDA component, the restoration period cannot be longer than five years, the total patent term including the restoration period must not exceed 14 years following FDA approval of the drug, and the extension may only apply to 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). There can be no assurance that any such patent term adjustment or extension will be obtained. The duration of foreign patents varies in accordance with provisions of applicable local law, but typically is also 20 years from the earliest effective filing date. However, the actual protection afforded by a patent varies on a product-by-product basis, from country to country and depends upon many factors, including the type of patent, the scope of its coverage, the availability of regulatory-related extensions, the availability of legal remedies in a particular country and the validity and enforceability of the patent.

Furthermore, we rely upon trade secrets, know-how and continuing technological innovation to develop and maintain our competitive position. We seek to protect our proprietary information, in part, using confidentiality agreements with our commercial partners, collaborators, employees and consultants and invention assignment agreements with our employees. We also have confidentiality agreements or

invention assignment agreements with our commercial partners and selected consultants. These agreements are designed to protect our proprietary information and, in the case of the invention assignment agreements, to grant us ownership of technologies that are developed through a relationship with a third party. These agreements may be breached, and we may not have adequate remedies for any such breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors. To the extent that our commercial partners, collaborators, employees and consultants use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

Our commercial success will also depend in part on not infringing upon or otherwise violating the intellectual property and proprietary rights of third parties. If we are found to infringe a third party's intellectual property rights, we could be required to obtain a license from such 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 also 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. For more information regarding these risks, please see "Risk Factors—Risks related to our intellectual property."

#### *Agreements with Eisai*

We acquired worldwide rights to avatrombopag from Eisai in March 2016 pursuant to a stock purchase agreement, or the Eisai stock purchase agreement. Under the Eisai stock purchase agreement, we acquired all of the shares of our wholly-owned subsidiary AkaRx, Inc., or AkaRx. Pursuant to the Eisai stock purchase agreement, Eisai received an upfront payment of \$5.0 million and we are obligated to pay Eisai aggregate milestone payments up to \$135.0 million based on annual net sales of avatrombopag, which would be calculated on an annual basis after commercialization. The rights to avatrombopag, including any associated intellectual property and regulatory rights, are subject to a right of reversion in the event we fail to make any required milestone payment or in the event we elect to discontinue the avatrombopag program. Until we pay Eisai all of the milestone payments contemplated under the Eisai stock purchase agreement, we must use commercially reasonable efforts to commercialize and sell avatrombopag. Eisai has a right of first negotiation if we should seek to grant a third party the right to market avatrombopag in Japan, China, South Korea or Taiwan.

In March 2016, in connection with the acquisition of the global rights to avatrombopag from Eisai, AkaRx entered into a transition services agreement with Eisai, or the TSA. Pursuant to the TSA, Eisai has agreed to continue as sponsor of the ongoing clinical trials for avatrombopag and to provide certain clinical, project management, pharmacovigilance, medical writing, regulatory and CMC services for us related to the avatrombopag program.

Under the terms of the TSA and the Eisai stock purchase agreement, Eisai agreed to oversee and manage the ongoing clinical trials and continue to hold and maintain the INDs for avatrombopag. Pursuant to the Eisai stock purchase agreement, we have the right to have the INDs transferred to us at any time. We are obligated to pay Eisai for services provided by Eisai personnel based on a fixed payment schedule. To the extent that service fees and out-of-pocket costs payable by us to Eisai under the TSA exceed \$51.0 million, our obligation to pay milestone payments under the Eisai stock purchase agreement will be reduced. We 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, (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.

At the time that AkaRx entered into the TSA, it also issued the Eisai note to Eisai. Under the terms of the Eisai note, the principal consists solely of the unpaid out-of-pocket costs and service fees due under the TSA. The Eisai note matures on March 30, 2018 and bears interest at a rate of 5% per annum. As of March 31, 2017, we had outstanding borrowings of \$20.5 million under this Eisai note, and we did not owe Eisai any accrued interest. A single interest-only payment of \$0.4 million was paid in March 2017. The Eisai note is secured by a blanket security interest on all of the assets of AkaRx, including the worldwide rights to avatrombopag. Payments due pursuant to the Eisai note are currently guaranteed by PBM Capital Investments, LLC.

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 and 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. Under the terms of the agreement, Eisai is required to assist us with transferring the technology associated with manufacturing avatrombopag to a new manufacturer at our request and to transfer or license to us any rights necessary to manufacture finished drug product, subject to the satisfaction of certain conditions set forth in the supply agreement. In addition, at our request, Eisai is required to supply us with starting materials, active pharmaceutical ingredients or bulk product for avatrombopag, subject to the satisfaction of certain conditions set forth in the supply agreement.

#### *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 will be required to make aggregate milestone payments of up to \$5.0 million to Astellas if certain 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. Under the terms of the license agreement, we must use commercially reasonable efforts to conduct development activities and obtain regulatory approval of avatrombopag. 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.

### *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. The Dova services agreement is terminable at will by either party with or without notice.

In April 2016, AkaRx entered into a services agreement with PBM Capital Group, LLC, or the AkaRx services agreement, 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. The AkaRx services agreement is terminable at will by either party with or without notice.

## **Government regulation**

### *FDA drug approval process*

In the United States, drugs are subject to extensive regulation by the FDA. The Federal Food, Drug, and Cosmetic Act, and other federal and state statutes and regulations, govern, among other things, the research, development, clinical and preclinical testing, manufacture, storage, recordkeeping, approval, labeling, promotion and marketing, distribution, post-approval monitoring and reporting, sampling and import and export of drugs. The processes for obtaining regulatory approvals in the United States and in foreign countries and jurisdictions, along with subsequent compliance with applicable statutes and regulations and other regulatory authorities, require the expenditure of substantial time and financial resources. Failure to comply with applicable U.S. requirements at any time during the drug development process may subject a company to a variety of administrative or judicial sanctions, such as FDA refusal to approve pending NDAs warning or untitled letters, imposition of a clinical hold, withdrawal of approval, drug recalls, drug seizures, total or partial suspension of production or distribution, injunctions, fines, civil penalties and criminal prosecution.

We cannot market a drug candidate in the United States until the drug has received FDA approval.

The steps required before a drug may be marketed in the United States generally include the following:

- completion of extensive pre-clinical laboratory tests, animal studies, and formulation studies in accordance with the FDA's GLP regulations;
- submission to the FDA of an IND for human clinical testing, which must become effective before human clinical trials may begin;
- approval by an independent institutional review board, or IRB, at each clinical site or a central IRB serving multiple client sites before each trial may be initiated;
- performance of adequate and well-controlled human clinical trials in accordance with GCP requirements to establish the safety and efficacy of the drug for each proposed indication, conducted in accordance with GCP;
- submission to the FDA of an NDA after completion of all pivotal clinical trials;
- satisfactory completion of an FDA advisory committee review, if applicable
- satisfactory completion of an FDA study site and/or sponsor inspections;

- satisfactory completion of an FDA pre-approval inspection of the manufacturing facility or facilities at which the active pharmaceutical ingredient, or API, and finished drug are produced and tested to assess compliance with cGMP requirements; and
- FDA review and approval of the NDA prior to any commercial marketing or sale of the drug in the United States.

Satisfaction of FDA pre-market approval requirements typically takes many years and the actual time required may vary substantially based upon the type, complexity and novelty of the drug or disease.

Preclinical tests include laboratory evaluation of drug chemistry, formulation and toxicity, as well as animal trials to assess the characteristics and potential safety and efficacy of the drug. The conduct of the preclinical tests must comply with federal regulations and requirements, including good laboratory practices. An IND sponsor must submit the results of preclinical testing to the FDA as part of an IND along with other information, including information about drug chemistry, manufacturing and controls and a proposed clinical trial protocol. Long-term preclinical tests, such as animal tests of reproductive toxicity and carcinogenicity, may continue after the IND is submitted.

A 30-day waiting period after the submission of each IND is required prior to the commencement of clinical testing in humans. If the FDA has neither commented on nor questioned the IND within this 30-day period, the clinical trial proposed in the IND may begin. If the FDA raises concerns or questions about the conduct of the trial, such as whether human research subjects will be exposed to an unreasonable health risk, the IND sponsor and the FDA must resolve any outstanding FDA concerns or questions before clinical trials can proceed.

Clinical trials involve the administration of the investigational new drug to healthy volunteers or patients under the supervision of a qualified investigator. Clinical trials must be conducted in compliance with federal regulations, including GCP requirements, which include the requirement that all research subjects provide their informed consent in writing for their participation in any clinical trial. Clinical trials are conducted under protocols detailing the objectives of the trial, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. Each protocol and subsequent protocol amendment must be submitted to the FDA as part of the IND.

The FDA may order the temporary, or permanent, discontinuation of a clinical trial at any time, or impose other sanctions, if it believes that the clinical trial either is not being conducted in accordance with FDA requirements or presents an unacceptable risk to the clinical trial patients. The study protocol and informed consent information for patients in clinical trials must also be submitted to an IRB, for approval for each site at which the clinical trial will be conducted. An IRB may also require the clinical trial at the site to be halted, either temporarily or permanently, for failure to comply with the IRB's requirements, or may impose other conditions. Information about certain clinical trials must be submitted within specific timeframes to the National Institutes of Health, or NIH, for public dissemination on their [www.clinicaltrials.gov](http://www.clinicaltrials.gov) website.

Clinical trials to support NDAs for marketing approval are typically conducted in three sequential phases, but the phases may overlap. In Phase 1, the initial introduction of the drug into a small number (between 20 and 100) of healthy human subjects or patients, the drug is tested to assess pharmacological actions, side effects associated with increasing doses and, if possible, early evidence on effectiveness. Phase 2 usually involves trials in a larger patient population (up to several hundred) to determine metabolism, PK, the effectiveness of the drug for a particular indication, dosage tolerance and optimum dosage, and to identify common adverse effects and safety risks. If a compound demonstrates evidence of effectiveness and an acceptable safety profile in Phase 2 evaluations, Phase 3 clinical trials are undertaken to obtain the

additional information about clinical efficacy and safety in a larger number of patients (between 300 and 3,000), typically at geographically dispersed clinical trial sites, to permit the FDA to evaluate the overall benefit-risk relationship of the drug and to provide adequate information for the labeling of the drug. In most cases, the FDA requires two adequate and well controlled Phase 3 clinical trials to demonstrate the efficacy of the drug. A single Phase 3 clinical trial with other confirmatory evidence may be sufficient in some instances where the study is a large multicenter trial demonstrating internal consistency and a statistically very persuasive finding of a clinically meaningful effect on mortality, reversible morbidity or prevention of a disease with a potentially serious outcome, when confirmation of the result in a second trial would be practically or ethically impossible.

The FDA and an IND sponsor may agree in writing on the design and size of clinical studies intended to form the primary basis of a claim of effectiveness in an NDA. This process is known as a Special Protocol Assessment, or SPA. An SPA agreement is not a guarantee of drug approval by the FDA or approval of any permissible claims about the drug, but does establish some agreements with the FDA regarding key features of the study design and analysis, including, for example, the primary endpoint, number of subjects and statistical methodology. The FDA retains significant latitude and discretion in interpreting the terms of the SPA agreement and the data and results from any study that is the subject of an SPA agreement. In particular, an SPA agreement is not binding on the FDA if previously unrecognized public health concerns later come to light, other new scientific concerns regarding drug safety or efficacy arise, clinical treatment standards change with the approval of other new drugs the IND sponsor fails to comply with the protocol agreed upon, or the relevant data, assumptions, or information provided by the IND sponsor when requesting an SPA agreement change, are found to be false statements or misstatements, or are found to omit relevant facts. An SPA agreement may not be changed by the sponsor or the FDA after the trial begins except with the written agreement of the sponsor and the FDA, or if the FDA determines that a substantial scientific issue essential to determining the safety or effectiveness of the drug was identified after the testing began.

Clinical studies at each phase of development may not be completed successfully within any specified period, or at all. Furthermore, the FDA, an IRB, the sponsor or the data monitoring committee, if applicable, may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research subjects are being exposed to an unacceptable health risk. The FDA will typically inspect one or more clinical sites to assure compliance with GCP and the integrity of the clinical data submitted, as well as the sponsor's offices, if appropriate.

After completion of the required clinical testing, an NDA is prepared and submitted to the FDA. FDA approval of the NDA is required before marketing of the drug may begin in the United States. The NDA must include the results of all preclinical, clinical and other testing and a compilation of data relating to the drug's pharmacology, chemistry, manufacture and controls. In addition, under the Pediatric Research Equity Act, or PREA, an NDA must contain data to assess the safety and effectiveness of the drug for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the drug is safe and effective. The cost of preparing and submitting an NDA is substantial. The submission of most NDAs is additionally subject to a substantial application user fee, and the manufacturer and sponsor under an approved NDA are also subject to annual drug and establishment user fees. These fees are typically increased annually. Under the Prescription Drug User Fee Act, or PDUFA, guidelines that are currently in effect, the FDA has a goal of ten months from the date of "filing" of a standard NDA for a new molecular entity to review and act on the submission. This review typically takes 12 months from the date the NDA is submitted to FDA because the FDA has approximately two months to make a "filing" decision.

The FDA has 60 days from its receipt of an NDA to determine whether the application will be accepted for filing based on the agency's threshold determination that it is sufficiently complete to permit substantive review.

Once the submission is accepted for filing, the FDA begins an in-depth review. The FDA may request additional information rather than accept an NDA for filing. In this event, the application must be resubmitted with the additional information. The resubmitted application is also subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review. The FDA reviews an NDA to determine, among other things, whether the drug is safe and effective, whether the data are consistent and reliable and whether the facility in which it is manufactured, processed, packaged or held meets industry and regulatory standards designed to assure the drug's continued safety, quality and purity.

The FDA may also refer applications for a novel drug, or drug that presents difficult questions of safety or efficacy, to an advisory committee—typically a panel that includes clinicians and other experts—for review, evaluation and a recommendation as to whether the application should be approved. The FDA is not bound by the recommendation of an advisory committee, but it generally follows such recommendations. Before approving an NDA, the FDA will typically inspect one or more clinical sites to assure compliance with GCPs. Additionally, the FDA will inspect the facility or the facilities at which the drug is manufactured. The FDA will not approve the drug unless compliance with cGMPs is satisfactory and the NDA contains data that provide substantial evidence that the drug is safe and effective in the indication studied.

After the FDA evaluates the NDA and the manufacturing facilities, it issues either an approval letter or a complete response letter. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing, or information, in order for the FDA to reconsider the application. If, or when, those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the NDA and the FDA has determined that the drug is safe and effective and provides clinical benefit, the FDA will issue an approval letter. The FDA has committed to reviewing such resubmissions in two or six months depending on the type and complexity of information included.

An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications, i.e., the label. Even if the FDA approves a drug, it may limit the approved indications for use of the drug, require that contraindications, warnings or precautions be included in the drug labeling, require that post-approval studies, including Phase 4 clinical trials, be conducted to further assess a drug's safety after approval, require testing and surveillance programs to monitor the drug after commercialization, or impose other conditions, including distribution and use restrictions or other risk management mechanisms under a risk evaluation and mitigation strategy, or REMS, to ensure that the benefits of the drug outweigh the potential risks. A REMS can include a medication guide, a communication plan for healthcare professionals and elements to assure safe use, such as special training and certification requirements for individuals who prescribe or dispense the drug, requirements that patients enroll in a registry and other measures that the FDA deems necessary to assure the safe use of the drug. The requirement for a REMS can materially affect the potential market and profitability of the drug. The FDA may prevent or limit further marketing of a drug based on the results of post-marketing studies or surveillance programs. Once granted, drug approvals may be withdrawn if compliance with regulatory standards is not maintained or problems are identified following initial marketing.

Changes to some of the conditions established in an approved application, including changes in indications, labeling, or manufacturing processes or facilities, require submission and FDA approval of a new NDA or NDA supplement before the change can be implemented. An NDA supplement for a new indication typically requires clinical data similar to that in the original application, and the FDA uses the same procedures and actions in reviewing NDA supplements as it does in reviewing NDAs. Such supplements are typically reviewed within 10 months of receipt.

#### *Post-approval requirements*

At the time an NDA is approved, a drug may be subject to certain post-approval requirements, which may include additional clinical trials. In addition, the FDA closely regulates the post-approval marketing and promotion of drugs, including standards and regulations for direct-to-consumer advertising, off-label promotion, industry-sponsored scientific and educational activities and promotional activities involving the internet and social media. Drugs may be marketed and promoted only for the approved indications and in accordance with the provisions of the approved labeling.

Post-marketing pharmacovigilance activities, adverse event reporting and submission of periodic reports are required following FDA approval of an NDA. The FDA also routinely conducts inspections of the sponsor or delegated vendors to ensure compliance with quality systems, appropriate oversight, collection and review of safety data and product complaints. The FDA also may require post-marketing testing, known as Phase 4 testing, REMS, surveillance to monitor the effects of an approved drug, or restrictions on the distribution or use of the drug. In addition, quality-control, drug manufacture, packaging and labeling procedures must continue to conform to cGMPs after approval. Drug manufacturers and certain of their subcontractors are required to register their establishments with the FDA and certain state agencies. Registration with the FDA subjects entities to periodic unannounced inspections by the FDA, during which the agency inspects manufacturing facilities to assess compliance with cGMPs. Accordingly, manufacturers must continue to expend time, money and effort in the areas of production and quality-control to maintain compliance with cGMPs. Later discovery of previously unknown problems with a drug, including adverse events of unanticipated severity or frequency, or failure to comply with regulatory requirements, may result in mandatory revisions to the approved labeling to add new safety information, imposition of post-market studies or clinical trials to assess new safety risks or imposition of distribution or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the drug, complete withdrawal of the drug from the market or drug recalls;
- fines, warning letters or holds on post-approval clinical trials;
- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of drug approvals;
- barring individuals from continued involvement in the pharmaceutical industry;
- drug seizure or detention, or refusal to permit the import or export of drugs; or
- injunctions or the imposition of civil or criminal penalties.

The FDA strictly regulates marketing, labeling, advertising and promotion of drugs that are placed on the market. Drugs may be promoted only for the approved indications and in accordance with the provisions of the approved label. The FDA and other agencies actively monitor compliance and enforce the laws and



regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability.

#### *Foreign regulation*

In order to market any drug outside of the United States, we need to comply with numerous and varying regulatory requirements of other countries and jurisdictions regarding quality, safety and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of our drugs. Whether or not we obtain FDA approval for a drug, we would need to also obtain the necessary approvals by the comparable foreign regulatory authorities before we can commence clinical trials or marketing of the drug in foreign countries and jurisdictions. Although many of the issues discussed above with respect to the United States apply similarly in the context of the European Union, the approval process varies between countries and jurisdictions and can involve additional drug testing, clinical trials and additional administrative review periods. The time required to obtain approval in other countries and jurisdictions might differ from and be longer than that required to obtain FDA approval. Regulatory approval in one country or jurisdiction does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country or jurisdiction may negatively impact the regulatory process in others.

#### *Other healthcare laws*

Although we currently do not have any drugs on the market, our current and future business operations may be subject to additional healthcare regulation and enforcement by the federal government and by authorities in the states and foreign jurisdictions in which we conduct our business. Such laws include, without limitation, state and federal anti-kickback, fraud and abuse, false claims, privacy and security, price reporting and physician sunshine laws. Some of our pre-commercial activities are subject to some of these laws.

The federal Anti-Kickback Statute makes it illegal for any person or entity, including a prescription drug manufacturer or a party acting on its behalf to knowingly and willfully, directly or indirectly, solicit, receive, offer, or pay any remuneration that is intended to induce the referral of business, including the purchase, order, lease of any good, facility, item or service for which payment may be made under a federal healthcare program, such as Medicare or Medicaid. The term “remuneration” has been broadly interpreted to include anything of value. The Anti-Kickback Statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on one hand and prescribers, purchasers, formulary managers, and beneficiaries on the other.

Although there are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution, the exceptions and safe harbors are drawn narrowly. Practices that involve remuneration that may be alleged to be intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exception or safe harbor. Failure to meet all of the requirements of a particular applicable statutory exception or regulatory safe harbor does not make the conduct per se illegal under the Anti-Kickback Statute. Instead, the legality of the arrangement will be evaluated on a case-by-case basis based on a cumulative review of all its facts and circumstances. 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. In addition, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. Violations of this law are punishable by up to five years in prison, and can also result in criminal fines, civil money penalties and exclusion from participation in federal healthcare programs.

Moreover, 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 federal civil False Claims Act.

The federal civil False Claims Act prohibits, among other things, any person or entity from knowingly presenting, or causing to be presented, for payment to, or approval by, federal programs, including Medicare and Medicaid, claims for items or services, including drugs, that are false or fraudulent or not provided as claimed. Persons and entities can be held liable under these laws if they are deemed to “cause” the submission of false or fraudulent claims by, for example, providing inaccurate billing or coding information to customers or promoting a drug off-label. In addition, our future activities relating to the reporting of wholesaler or estimated retail prices for our drugs, the reporting of prices used to calculate Medicaid rebate information and other information affecting federal, state and third-party reimbursement for our drugs, and the sale and marketing of our drugs, are subject to scrutiny under this law. Penalties for federal civil False Claims Act violations may include up to three times the actual damages sustained by the government, plus mandatory civil penalties of between \$10,781.40 and \$21,562.80 for each separate false claim, the potential for exclusion from participation in federal healthcare programs, and, although the federal False Claims Act is a civil statute, False Claims Act violations may also implicate various federal criminal statutes.

HIPAA created new federal criminal statutes that prohibit among other actions, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, including private third-party payors, knowingly and willfully embezzling or stealing from a healthcare benefit program, willfully obstructing a criminal investigation of a healthcare offense, and knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services. Like 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 in order to have committed a violation.

The civil monetary penalties statute imposes penalties against any person or entity that, among other things, is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent.

Also, many states have similar fraud and abuse statutes or regulations that may be broader in scope and may apply regardless of payor, in addition to items and services reimbursed under Medicaid and other state programs.

Additionally, to the extent that any of our drugs are sold in a foreign country, we may be subject to similar foreign laws.

HIPAA, as amended by HITECH, and their implementing regulations, including the final omnibus rule published on January 25, 2013, mandate, among other things, the adoption of uniform standards for the electronic exchange of information in common healthcare transactions, as well as standards relating to the privacy and security of individually identifiable health information, which require the adoption of administrative, physical and technical safeguards to protect such information. Among other things, HITECH makes HIPAA’s security standards directly applicable to business associates, defined as independent contractors or agents of covered entities that create, receive or obtain protected health information in connection with providing a service for or on behalf of a covered entity. HITECH also increased the civil and criminal penalties that may be imposed against covered entities and business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to

enforce the federal HIPAA laws and seek attorney's fees and costs associated with pursuing federal civil actions. In addition, certain state laws govern the privacy and security of health information in certain circumstances, some of which are more stringent than HIPAA and many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts. Failure to comply with these laws, where applicable, can result in the imposition of significant civil and criminal penalties.

The Physician Payments Sunshine Act, which was created under the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively the Affordable Care Act, imposes, among other things, annual reporting requirements for covered manufacturers for certain payments and other transfers of value provided to physicians and teaching hospitals, as well as certain ownership and investment interests held by physicians and their immediate family members. Failure to submit timely, accurately and completely the required information for all payments, transfers of value and ownership or investment interests may result in civil monetary penalties of up to an aggregate of \$150,000 per year and up to an aggregate of \$1 million per year for "knowing failures." Certain states also mandate implementation of compliance programs, impose restrictions on drug manufacturer marketing practices or require the tracking and reporting of gifts, compensation or other remuneration to physicians.

Because we intend to commercialize drugs that could be reimbursed under a federal healthcare program and other governmental healthcare programs, we intend to develop a comprehensive compliance program that establishes internal control to facilitate adherence to the rules and program requirements to which we will or may become subject. Although the development and implementation of compliance programs designed to establish internal control and facilitate compliance can mitigate the risk of investigation, prosecution, and penalties assessed for violations of these laws, the risks cannot be entirely eliminated.

If our operations are found to be in violation of any of such laws or any other governmental regulations that apply to us, we may be subject to penalties, including, without limitation, administrative, civil and criminal penalties, damages, fines, disgorgement, contractual damages, reputational harm, diminished profits and future earnings, 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, the curtailment or restructuring of our operations, exclusion from participation in federal and state healthcare programs and individual imprisonment, any of which could adversely affect our ability to operate our business and our financial results.

#### *Health reform*

In the United States and foreign jurisdictions, there have been a number of legislative and regulatory changes to the healthcare system that could affect our future results of operations. There have been and continue to be a number of initiatives at the United States federal and state levels that seek to reduce healthcare costs.

In particular, the Affordable Care Act has had a significant impact on the healthcare industry. The Affordable Care Act was designed to expand coverage for the uninsured while at the same time containing overall healthcare costs. With regard to drugs, among other things, the Affordable Care Act revised the definition of "average manufacturer price" for calculating and reporting Medicaid drug rebates on outpatient prescription drug prices and imposed a significant annual fee on companies that manufacture or import certain branded prescription drugs. Substantial new provisions affecting compliance have also been enacted, which may require us to modify our business practices with healthcare providers and entities, and certain provisions are not yet, or have only recently become, effective.

In addition, there have been judicial and Congressional challenges to certain aspects of the Affordable Care Act. As a result, there have been delays in the implementation of, and action taken to repeal or replace, certain aspects of the Affordable Care Act. Most recently, in May 2017, following the passage of the budget resolution for fiscal year 2017, the U.S. House of Representatives passed legislation known as the American Health Care Act, which, if enacted, would amend or repeal significant portions of the Affordable Care Act. However, the U.S. Senate is unlikely to adopt the American Health Care Act as passed by the House of Representatives. The U.S. Senate could adopt additional legislation to amend or replace elements of the Affordable Care Act. Thus, it is uncertain if or when the American Health Care Act will become law. Although we cannot predict the ultimate content, timing or effect of any changes to the Affordable Care Act or other federal and state reform efforts, we continue to evaluate the effect that the Affordable Care Act, as amended or replaced, will have on our business. In the coming years, additional legislative and regulatory changes could be made to governmental health programs that could significantly impact pharmaceutical companies and the success of our drug candidate.

In addition, other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. In August 2011, the President signed into law the Budget Control Act of 2011, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend to Congress proposals in spending reductions. The Joint Select Committee did not achieve a targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, triggering the legislation's automatic reduction to several government programs. These included reductions to Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013 and, due to subsequent legislative amendments to the statute, will stay in effect through 2025 unless additional Congressional action is taken. Additionally, in January 2013, the American Taxpayer Relief Act of 2012 was signed into law, 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. In addition, there has been heightened governmental scrutiny recently over the manner in which drug manufacturers set prices for their marketed drugs, which have resulted in several 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.

Moreover, the Drug Supply Chain Security Act, imposes new obligations on manufacturers of drugs, among others, related to drug tracking and tracing, which will be phased in over several years beginning in 2016. Among the requirements of this legislation, manufacturers will be required to provide certain information regarding the drug to individuals and entities to which drug ownership is transferred, label drug with a drug identifier, and keep certain records regarding the drug. The transfer of information to subsequent drug owners by manufacturers will eventually be required to be done electronically. Manufacturers will also be required to verify that purchasers of the manufacturers' drugs are appropriately licensed. Further, under this new legislation, manufacturers will have drug investigation, quarantine, disposition, and notification responsibilities related to counterfeit, diverted, stolen, and intentionally adulterated drugs, as well as drugs that are the subject of fraudulent transactions or which are otherwise unfit for distribution such that they would be reasonably likely to result in serious health consequences or death.

#### *Coverage and reimbursement*

Sales of our drug candidates, once approved, will depend, in part, on the extent to which the costs of our drugs will be covered by third-party payors, such as government health programs, private health insurers and managed care organizations. Third-party payors generally decide which drugs they will cover and establish certain reimbursement levels for such drugs. In particular, in the U.S., private health insurers and

other third-party payors often provide reimbursement for drugs and services based on the level at which the government (through the Medicare or Medicaid programs) provides reimbursement for such treatments. Patients who are prescribed treatments for their conditions and providers performing the prescribed 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. Sales of our drug candidate, and any future drug candidates, will therefore depend substantially on the extent to which the costs of our drug candidate, and any future drug candidates, will be paid by third-party payors. Additionally, the market for our drug candidate, and any future drug candidates, will depend significantly on access to third-party payors' formularies without prior authorization, step therapy, or other limitations such as approved lists of treatments for which third-party payors provide coverage and reimbursement. Additionally, coverage and reimbursement for therapeutic drugs can differ significantly from payor to payor. One third-party payor's decision to cover a particular drug or service does not ensure that other payors will also provide coverage for the drug or service, or will provide coverage at an adequate reimbursement rate. As a result, the coverage determination process will require us to provide scientific and clinical support for the use of our drugs to each payor separately and will be a time-consuming process.

Third-party payors are developing increasingly sophisticated methods of controlling healthcare costs and increasingly challenging the prices charged for drugs and services. If these third-party payors do not consider our drugs to be cost-effective compared to other therapies, they may not cover our drugs once approved as a benefit under their plans or, if they do, the level of reimbursement may not be sufficient to allow us to sell our drugs on a profitable basis. Decreases in third-party reimbursement for our drugs once approved or a decision by a third-party payor to not cover our drugs could reduce or eliminate utilization of our drugs and have an adverse effect on our sales, results of operations and financial condition. Additionally, the containment of healthcare costs has become a priority of federal and state governments and the prices of drugs have been a focus in this effort. The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost-containment programs, including price controls and transparency requirements, restrictions on reimbursement and requirements for substitution of generic drugs. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could limit our net revenue and results. In addition, state and federal healthcare reform measures have been and will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for drugs and services, which could result in reduced demand for our drugs once approved or additional pricing pressures.

#### *Additional regulation*

In addition to the foregoing, state and federal laws regarding environmental protection and hazardous substances, including the Occupational Safety and Health Act, the Resource Conservation and Recovery Act and the Toxic Substances Control Act, affect our business. These and other laws govern the use, handling and disposal of various biologic, chemical and radioactive substances used in, and wastes generated by, operations. If our operations result in contamination of the environment or expose individuals to hazardous substances, we could be liable for damages and governmental fines. Equivalent laws have been adopted in third countries that impose similar obligations.

#### *U.S. Foreign Corrupt Practices Act*

The U.S. Foreign Corrupt Practices Act, or FCPA, prohibits U.S. corporations and individuals from engaging in certain activities to obtain or retain business abroad or to influence a person working in an official

capacity. It is illegal to pay, offer to pay or authorize the payment of anything of value to any foreign government official, government staff member, political party or political candidate in an attempt to obtain or retain business or to otherwise influence a person working in an official capacity. The scope of the FCPA includes interactions with certain healthcare professionals in many countries. Equivalent laws have been adopted in other foreign countries that impose similar obligations.

## **Employees**

As of May 31, 2017, we had seven full-time employees. None of our employees is party to a collective bargaining agreement. We consider our relationship with our employees to be good.

## **Facilities**

We operate in a 7,351 square foot facility in Durham, North Carolina pursuant to a sublease agreement that expires in April 2020. We believe that our existing facilities are adequate to meet our current needs, and that suitable additional alternative spaces will be available in the future on commercially reasonable terms.

## **Legal proceedings**

From time to time, we may be involved in various claims and legal proceedings relating to claims arising out of our operations. We are not currently a party to any legal proceedings that, in the opinion of our management, are likely to have a material adverse effect on our business. Regardless of outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

# Management

## Executive officers and directors

The following table provides information regarding our current executive officers and directors, including their ages as of March 31, 2017:

| Name                              | Age | Position(s)                                     |
|-----------------------------------|-----|---|
| <b>Executive Officers</b>         |     |   |
| Alex Sapir . . . . .              | 50  | President, Chief Executive Officer and Director |
| Douglas Blankenship . . . . .     | 55  | Chief Financial Officer                         |
| Lee F. Allen, M.D., Ph.D. . . . . | 65  | Chief Medical Officer                           |
| Kevin Laliberte . . . . .         | 39  | Senior Vice President, Product Development      |
| <b>Non-Employee Directors</b>     |     |   |
| Steven M. Goldman . . . . .       | 65  | Director  |
| Roger A. Jeffs . . . . .          | 55  | Director  |
| Paul B. Manning . . . . .         | 61  | Chairman of the Board of Directors              |
| Alfred J. Novak . . . . .         | 69  | Director  |
| Sean Stalfort . . . . .           | 47  | Director  |

## Executive officers

*Alex Sapir* has served as our President and Chief Executive Officer since January 2017. From January 2006 to May 2016, Mr. Sapir served as Executive Vice President, Marketing and Sales for United Therapeutics Corporation, a biotechnology company focused on the development and commercialization of unique products to address the unmet medical needs of patients with chronic and life-threatening conditions. Prior to his time at United Therapeutics Corporation, from January 2003 to January 2005 Mr. Sapir served as Senior Director, Marketing for Guilford Pharmaceuticals. He began his career at GlaxoSmithKline serving in various commercial roles in the United States and Europe. Mr. Sapir is a routine guest lecturer on the topic of pharmaceutical marketing strategy at Duke University's Fuqua School of Business. Mr. Sapir holds a B.A. in Economics from Franklin and Marshall College and an M.B.A. from Harvard Business School.

*Douglas Blankenship* has served as our Chief Financial Officer since March 2017. From November 2015 to February 2017, Mr. Blankenship took a sabbatical to focus on family and relocated to the Research Triangle, North Carolina. From October 2008 to October 2015, Mr. Blankenship served as Director, Finance—Global Quality & Compliance and Technical Regulatory, of Genentech and Roche Pharmaceuticals, a biotechnology company focused on discovering, developing, manufacturing and commercializing medicines to treat patients with serious or life-threatening medical conditions. Prior to his time at Genentech, from June 2006 to January 2008, Mr. Blankenship served as Finance Director for Amgen Technology Ireland. Mr. Blankenship holds a B.S. in Accounting from California Polytechnic State University, San Luis Obispo and an M.B.A. from The Wharton School, University of Pennsylvania.

*Lee F. Allen, M.D., Ph.D.* has served as our Chief Medical Officer since April 2017. From January 2016 to April 2017, Dr. Allen served as Chief Medical Officer managing Clinical Development, Medical Affairs and Regulatory Affairs at Argos Therapeutics. Dr. Allen served as Chief Medical Officer at Spectrum Pharmaceuticals from March 2013 to January 2016. From August 2007 to March 2013, Dr. Allen served as Chief Medical Officer at AMAG Pharmaceuticals. From 2003 through 2007, Dr. Allen served as the Clinical

Site Head and Global Oncology Therapeutic Area Director for Wyeth Pharmaceuticals' research and development site in Cambridge, MA. From 1999 to 2003, Dr. Allen helped establish Oncology as a new therapeutic area at Pfizer and served as Executive Director and Site Therapeutic Area Leader for the Ann Arbor Oncology portfolio. Dr. Allen holds a Ph.D. and M.D. from the Rutgers Biomedical and Health Sciences (formerly the University of Medicine and Dentistry of New Jersey), and completed Residency training in Internal Medicine and Fellowship training in Hematology and Oncology at the Duke University Medical Center. As a postdoc, Dr. Allen investigated signal transduction pathways at Duke in the Howard Hughes Laboratory of Nobel Laureate, Dr. Robert Lefkowitz, and has authored or co-authored more than 50 publications.

*Kevin Laliberte* has served as our Senior Vice President, Product Development since March 2017. From 2003 to March 2017, Dr. Laliberte held various positions at United Therapeutics Corporation, including as the Senior Vice President, Product Development and Clinical Operations from March 2015 to March 2017, as the Associate Vice President Product Development from March 2013 to February 2015 and as the Senior Director Product Development from 2010 to March 2013. Dr. Laliberte holds a Pharm.D. from the University of Michigan and completed a Drug Development and Clinical Research Fellowship at the University of North Carolina School of Pharmacy and GlaxoSmithKline.

### **Non-employee directors**

*Steven M. Goldman* has served as a member of our board of directors since May 2017. Mr. Goldman has been a Partner of Kramer Levin Naftalis & Frankel LLP since 2009. Mr. Goldman specializes in mergers and acquisitions, financings, joint ventures, private placements, leveraged buyouts and general corporate counseling. He has represented numerous banks in connection with financing of acquisitions and recapitalizations, and insurance brokerages in connection with regulatory enforcement issues and in purchase and sale transactions. From 2006 to 2009, before joining Kramer Levin, Mr. Goldman was the State of New Jersey's Department of Banking and Insurance Commissioner, appointed by Governor Jon S. Corzine. In that role, Mr. Goldman chaired the Reinsurance Task Force at the National Association of Insurance Commissioners which completed a framework for modernizing the regulation of reinsurance in the United States and between the United States and other countries. Mr. Goldman has served as a director of Bank Leumi USA since May 2015. Mr. Goldman also served as a Director of ConnectOne Bank and ConnectOne Bancorp, Inc from 2011 to February 2014. He also Chaired the International Insurance Relations Committee of the NAIC, and the Reinsurance and other Forms of Risk Transfer Subcommittee on behalf of the United States at the International Association of Insurance Supervisors from 2007 to 2009. Mr. Goldman has testified before Congress on multiple occasions regarding insurance regulation and health care reform. Mr. Goldman is on the Dean's Board of Advisors for The George Washington University Law School, is a Member of the Bar in New York and New Jersey and is currently the Chair of the Operations and Finance Committee, a member of the Executive Committee, and Assistant Treasurer of the New Jersey Performing Arts Center and a member of the Board of Managers of Theatre Square Development Company. Mr. Goldman holds an A.B. in Political Science from Boston University, a J.D. from the George Washington University and an L.L.M. in taxation from New York University. Our board of directors believes that Mr. Goldman should serve as a director based on his experience both as a practicing attorney and as the Commissioner of the New Jersey Department of Banking and Insurance, which allows him to provide the Board with valuable insight on matters of corporate governance, regulatory compliance and relations and structuring of transactions.

*Roger A. Jeffs* has served as a member of our board of directors since May 2017. Dr. Jeffs has served as a Senior Advisor of United Therapeutics Corporation since June 2016. Dr. Jeffs served as the Co-Chief Executive Officer of United Therapeutics Corporation from January 2015 to June 2016 and President of



United Therapeutics Corporation from April 2001 to June 2016. Dr. Jeffs joined United Therapeutics Corporation in September 1998 as Director of Research, Development and Medical and also served as its Chief Operating Officer from April 2001 to December 2014. Prior to 1998, Dr. Jeffs worked at Amgen, Inc. as Manager of Clinical Affairs and Associate Director of Clinical Research from 1995 to 1998. Dr. Jeffs served as a Director of United Therapeutics Corporation from 2002 until June 2016. Dr. Jeffs has been a Director of Axsome Therapeutics, Inc. since December 2014 and serves as its Lead Director. Dr. Jeffs holds a B.S. in Chemistry from Duke University and Ph.D. in Pharmacology from the University of North Carolina. Our board of directors believes that Dr. Jeffs should serve as a director based on his scientific background and business experience, coupled with his experience as a Chief Executive Officer of a publicly-traded biotechnology company.

*Paul B. Manning* has served as the Chairman of our board of directors since June 2017 and as a member of our board of directors since September 2016. Mr. Manning is the President and Chief Executive Officer of PBM Capital Group, LLC, a private equity investment firm in the business of investing in healthcare and life-science related companies, which he founded in 2010. Prior to that, Mr. Manning founded PBM Products in 1997, a producer of infant formula and baby food, which was sold to Perrigo Corporation in 2010. Mr. Manning is a director of various private companies and AveXis, Inc., a publicly traded clinical-stage gene therapy company, and was previously on the board of directors of Perrigo Corporation and Concordia Healthcare Corp. Mr. Manning received a B.S. in microbiology from the University of Massachusetts. Our board of directors believes that Mr. Manning should serve as a director based upon on his over 30 years of managerial and operational experience in the healthcare industry and as an investor in healthcare related companies.

*Alfred J. Novak* has served as a member of our board of directors since May 2017. Mr. Novak has served on the board of LivaNova, a global medical device company since October 2015. From 2007 until October 2015, Mr. Novak served on the board of Cyberonics, until its merger with Sorin S.p.a. to form LivaNova. From April 2014 until March 2015, Mr. Novak served as President and Chief Executive Officer of Syntheon Cardiology, LLC, an early-stage company developing a percutaneous prosthetic aortic heart valve. From September 1999 until January 2014, he served on the board of directors of OrbusNeich Medical Technology Company, Ltd., a privately held interventional cardiology company, where he was Chairman and Chief Executive Officer from 2010 until October 2013. He previously served as Chairman of the board of directors of ProRhythm, Inc., a privately held company dedicated to the treatment of atrial fibrillation through the use of ultrasound technologies. In 1998, he was a founder of Syntheon, LLC, a privately held company that focused on minimally invasive medical devices for the gastroenterology and vascular markets. From 2002 until 2006, Mr. Novak was the President, Chief Executive Officer and a director of Novoste Corporation, a publicly held interventional cardiology company. From 1998 until 2002, Mr. Novak was a member of the board of directors of Sutura, Inc., a vascular closure company. Mr. Novak was President, Chief Executive Officer and a director of Biosense, Inc., an electrophysiology company, from 1996 until 1998, when it was acquired by Johnson & Johnson. He was employed by Cordis Corporation, then a publicly held cardiology company, from 1984 until 1996, when it was acquired by Johnson & Johnson. Mr. Novak currently serves on the board of directors of Restoring Heroes Foundation, an organization devoted to assisting veterans with obtaining access to new and progressive therapies. Formerly, he was on the board of Goodwill Industries of South Florida. Mr. Novak received an M.B.A. from the Wharton School, University of Pennsylvania, and a B.S. in Marine Transportation from the United States Merchant Marine Academy. Our board of directors believes that Mr. Novak should serve as a director based on his broad operating executive experience as Chief Executive Officer and Chief Financial Officer at medical device companies, his board of director experience at medical device companies, his expertise concerning new product

development, regulatory approval and commercialization of medical devices and his finance and accounting expertise.

*Sean Stalfort* has served as a member of our board of directors since September 2016. Mr. Stalfort has been a partner at PBM Capital Group, LLC, a private equity investment firm in the business of investing in healthcare and life-science related companies, since May 2010. Prior to joining PBM Capital Group, LLC, Mr. Stalfort was the Executive Vice President for New Business Development/M&A for PBM Products. Mr. Stalfort is also a founding Partner of Octagon Partners and Octagon Finance, historic tax credit real estate companies. Mr. Stalfort is a director of several private healthcare companies. Mr. Stalfort also served as our President from April 2016 until December 2016. Mr. Stalfort received a B.A. in Business Economics and Political Science from Brown University. Our board of directors believes that Mr. Stalfort should serve as a director based upon on his years as an investor in healthcare related companies.

## **Board composition and election of directors**

### ***Board composition***

Our board of directors currently consists of six members, each of whom serves as directors pursuant to the board composition provisions of our amended and restated certificate of incorporation and our amended and restated investors' rights agreement, or IRA, that we entered into with certain of our investors, which is further described under "Certain relationships and related party transactions" in this prospectus. The IRA provides that one director will be a representative of the holders of our Series A preferred stock and will be designated by Perceptive Life Sciences Master Fund, Ltd., which seat is currently vacant. The foregoing provisions of the IRA will terminate immediately prior to the completion of this offering. Upon the termination of these provisions, there will be no further contractual rights or obligations regarding the nomination or election of our directors. Thereafter, each of our current directors will continue to serve until the election and qualification of his or her successor, or his earlier death, resignation or removal.

The primary responsibilities of our board of directors are to provide oversight, strategic guidance, counseling and direction to our management. Our board of directors meets on a regular basis and additionally as required.

### ***Classified board of directors***

In accordance with the terms of our amended and restated certificate of incorporation and amended and restated bylaws, which will become effective upon the closing of this offering, our board of directors will be divided into three classes, each of which will consist, as nearly as possible, of one-third of the total number of directors constituting our entire board of directors and directors in each class will serve staggered three-year terms. At each annual meeting of stockholders, the successors to directors whose terms then expire will be elected to serve from the time of election and qualification until the third annual meeting following such election. Our directors will be divided among the three classes as follows:

- Class I, which will consist of Paul B. Manning and Alfred J. Novak, whose terms will expire at the first annual meeting of stockholders to be held following the completion of this offering;
- Class II, which will consist of Alex Sapir and Roger A. Jeffs, whose terms will expire at the second annual meeting of stockholders to be held following the completion of this offering; and
- Class III, which will consist of Sean Stalfort and Steven M. Goldman, whose terms will expire at the third annual meeting of stockholders to be held following the completion of this offering.

Our amended and restated bylaws, which will become effective upon completion of this offering, will provide that the authorized number of directors may be changed only by resolution approved by a majority of our board of directors. Any additional directorships resulting from an increase in the number of directors will be distributed among the three classes so that, as nearly as possible, each class will consist of one-third of the directors. In accordance with the terms of our amended and restated certificate of incorporation and amended and restated bylaws that will each become effective upon the closing of this offering, our directors may be removed only for cause by the affirmative vote of the holders of at least 75% of the votes that all our stockholders would be entitled to cast in an annual election of directors.

The division of our board of directors into three classes with staggered three-year terms may delay or prevent stockholder efforts to effect a change of our management or a change in control.

#### ***Director independence***

Applicable NASDAQ rules require a majority of a listed company's board of directors to be comprised of independent directors within one year of listing. In addition, NASDAQ rules require that, subject to specified exceptions, each member of a listed company's audit, compensation and nominating and corporate governance committees be independent and that audit committee members also satisfy independence criteria set forth in Rule 10A-3 under the Exchange Act of 1934, as amended, or the Exchange Act. The NASDAQ independence definition includes a series of objective tests, such as that the director is not, and has not been for at least three years, one of our employees, that neither the director nor any of his family members has engaged in various types of business dealings with us and that the director is not associated with the holders of more than 5% of our common stock. In addition, under applicable NASDAQ rules, a director will only qualify as an "independent director" if, in the opinion of the listed company's board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director.

Our board of directors has determined that all of our directors, except Mr. Sapir, Mr. Manning and Mr. Stalfort, are independent directors, as defined under applicable NASDAQ rules. In making such determination, our board of directors considered the relationships that each such non-employee director has with our company and all other facts and circumstances that our board of directors deemed relevant in determining his or her independence, including the beneficial ownership of our capital stock by each non-employee director. We intend to rely on phase-in periods under the NASDAQ rules with respect to director independence, which allow us to have less than a majority of independent directors upon the date of listing of our common stock, so long as our board has a majority of independent directors within one year of the date of listing. Accordingly, we plan to have a board of directors comprised of a majority of independent directors within one year of the date of listing.

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

#### ***Role of the board in risk oversight***

One of the key functions of our board of directors is informed oversight of our risk management process. Our board of directors does not have a standing risk management committee, but rather administers this oversight function directly through the board of directors as a whole, as well as through various standing committees of our board of directors that address risks inherent in their respective areas of oversight. In particular, our board of directors is responsible for monitoring and assessing strategic risk exposure and our audit committee has the responsibility to consider and discuss our major financial risk exposures and the steps our management has taken to monitor and control these exposures, including guidelines and policies to govern the process by which risk assessment and management is undertaken. The audit committee also monitors compliance with legal and regulatory requirements.

## Board committees

Our board of directors has established an audit committee, compensation committee and a nominating and corporate governance committee, each of which operate pursuant to a committee charter. Our board of directors may establish other committees to facilitate the management of our business. The composition and functions of each committee are described below.

### *Audit committee*

Upon completion of this offering, our audit committee will consist of Alfred J. Novak, Steven M. Goldman and Roger A. Jeffs, with Mr. Novak serving as chair of the audit committee. Our board of directors has determined that each of these individuals meets the independence requirements of the Sarbanes-Oxley Act of 2002, as amended, or the Sarbanes-Oxley Act, Rule 10A-3 under the Exchange Act and the applicable listing standards of NASDAQ. Each member of our audit committee can read and understand fundamental financial statements in accordance with NASDAQ audit committee requirements. In arriving at this determination, the board has examined each audit committee member's scope of experience and the nature of their prior and/or current employment.

Our board of directors has determined that Mr. Novak qualifies as an audit committee financial expert within the meaning of SEC regulations and meets the financial sophistication requirements of the NASDAQ Listing Rules. In making this determination, our board has considered Mr. Novak's formal education and previous and current experience in financial and accounting roles. Both our independent registered public accounting firm and management periodically meet privately with our audit committee.

The functions of this committee include, among other things:

- evaluating the performance, independence and qualifications of our independent auditors and determining whether to retain our existing independent auditors or engage new independent auditors;
- reviewing and approving the engagement of our independent auditors to perform audit services and any permissible non-audit services;
- monitoring the rotation of partners of our independent auditors on our engagement team as required by law;
- prior to engagement of any independent auditor, and at least annually thereafter, reviewing relationships that may reasonably be thought to bear on their independence, and assessing and otherwise taking the appropriate action to oversee the independence of our independent auditor;
- reviewing our annual and quarterly consolidated financial statements and reports, including the disclosures contained under the caption "Management's discussion and analysis of financial condition and results of operations," and discussing the statements and reports with our independent auditors and management;
- reviewing with our independent auditors and management significant issues that arise regarding accounting principles and financial statement presentation and matters concerning the scope, adequacy and effectiveness of our financial controls;
- reviewing with management and our auditors any earnings announcements and other public announcements regarding material developments;
- establishing procedures for the receipt, retention and treatment of complaints received by us regarding financial controls, accounting or auditing matters and other matters;

- preparing the report that the SEC requires in our annual proxy statement;
- reviewing and providing oversight of any related-person transactions in accordance with our related person transaction policy and reviewing and monitoring compliance with legal and regulatory responsibilities, including our code of business conduct and ethics;
- reviewing our major financial risk exposures, including the guidelines and policies to govern the process by which risk assessment and risk management is implemented;
- reviewing on a periodic basis our investment policy; and
- reviewing and evaluating on an annual basis the performance of the audit committee and the audit committee charter.

We believe that the composition and functioning of our audit committee complies with all applicable requirements of the Sarbanes-Oxley Act, and all applicable SEC and NASDAQ rules and regulations. We intend to comply with future requirements to the extent they become applicable to us.

#### ***Compensation committee***

Upon completion of this offering, our compensation committee will consist of Roger A. Jeffs, Steven M. Goldman and Paul B. Manning, with Dr. Jeffs serving as chair of the compensation committee. Dr. Jeffs and Mr. Goldman are non-employee directors, as defined in Rule 16b-3 promulgated under the Exchange Act and are “outside directors,” as defined pursuant to Section 162(m) of the Internal Revenue Code of 1986, as amended, or the Code. Our board of directors has determined that Dr. Jeffs and Mr. Goldman are “independent” as defined under the applicable listing standards of NASDAQ, including the standards specific to members of a compensation committee. We are permitted to phase in our compliance with the independent compensation committee requirements set forth by the NASDAQ listing standards as follows: (1) one independent member at the time of listing, (2) a majority of independent members within 90 days of listing and (3) all independent members within one year of listing. Within one year of our listing on the NASDAQ Global Market, we expect that Mr. Manning will have resigned from our compensation committee and that any new directors added to the compensation committee will be independent under NASDAQ listing rules, a non-employee director, as defined in Rule 16b-3 promulgated under the Exchange Act, and an “outside director,” as defined pursuant to Section 162(m) of the Code. The functions of this committee include, among other things:

- reviewing, modifying and approving (or if it deems appropriate, making recommendations to the full board of directors regarding) our overall compensation strategy and policies;
- making recommendations to the full board of directors regarding the compensation and other terms of employment of our executive officers;
- reviewing and making recommendations to the full board of directors regarding performance goals and objectives relevant to the compensation of our executive officers and assessing their performance against these goals and objectives;
- reviewing and approving (or if it deems it appropriate, making recommendations to the full board of directors regarding) the equity incentive plans, compensation plans and similar programs advisable for us, as well as modifying, amending or terminating existing plans and programs;

- evaluating risks associated with our compensation policies and practices and assessing whether risks arising from our compensation policies and practices for our employees are reasonably likely to have a material adverse effect on us;
- reviewing and making recommendations to the full board of directors regarding the type and amount of compensation to be paid or awarded to our non-employee board members;
- establishing policies with respect to votes by our stockholders to approve executive compensation to the extent required by Section 14A of the Exchange Act and, if applicable, determining our recommendations regarding the frequency of advisory votes on executive compensation;
- reviewing and assessing the independence of compensation consultants, legal counsel and other advisors as required by Section 10C of the Exchange Act;
- administering our equity incentive plans;
- establishing policies with respect to equity compensation arrangements;
- reviewing the competitiveness of our executive compensation programs and evaluating the effectiveness of our compensation policy and strategy in achieving expected benefits to us;
- reviewing and making recommendations to the full board of directors regarding the terms of any employment agreements, severance arrangements, change in control protections and any other compensatory arrangements for our executive officers;
- reviewing with management and approving our disclosures under the caption “Compensation discussion and analysis” in our periodic reports or proxy statements to be filed with the SEC, to the extent such caption is included in any such report or proxy statement;
- preparing the report that the SEC requires in our annual proxy statement; and
- reviewing and evaluating on an annual basis the performance of the compensation committee and the compensation committee charter.

We believe that the composition and functioning of our compensation committee complies with all applicable requirements of the Sarbanes-Oxley Act, and all applicable SEC and NASDAQ rules and regulations. We intend to comply with future requirements to the extent they become applicable to us.

***Nominating and corporate governance committee***

Upon completion of this offering, our nominating and corporate governance committee will consist of Steven M. Goldman, Alfred J. Novak and Sean Stalfort, with Mr. Goldman serving as chair of the nominating and corporate governance committee. Our board of directors has determined that Mr. Goldman and Mr. Novak are “independent” as defined under the applicable listing standards of NASDAQ and SEC rules and regulations. We are permitted to phase in our compliance with the independent nominating and corporate governance committee requirements set forth by the NASDAQ listing standards as follows: (1) one independent member at the time of listing, (2) a majority of independent members within 90 days of listing and (3) all independent members within one year of listing. Within one year of our listing on the NASDAQ Global Market, we expect that Mr. Stalfort will have resigned from our nominating and corporate governance committee and that any new directors added to the nominating and corporate governance

committee will be independent under NASDAQ listing rules. The functions of this committee include, among other things:

- identifying, reviewing and evaluating candidates to serve on our board of directors;
- determining the minimum qualifications for service on our board of directors;
- evaluating director performance on the board and applicable committees of the board and determining whether continued service on our board is appropriate;
- evaluating, nominating and recommending individuals for membership on our board of directors;
- evaluating nominations by stockholders of candidates for election to our board of directors;
- considering and assessing the independence of members of our board of directors;
- developing a set of corporate governance policies and principles and recommending to our board of directors any changes to such policies and principles;
- reviewing and making recommendations to the board of directors with respect to management succession planning;
- considering questions of possible conflicts of interest of directors as such questions arise; and
- reviewing and evaluating on an annual basis the performance of the nominating and corporate governance committee and the nominating and corporate governance committee charter.

We believe that the composition and functioning of our nominating and corporate governance committee complies with all applicable requirements of the Sarbanes-Oxley Act, and all applicable SEC and NASDAQ rules and regulations. We intend to comply with future requirements to the extent they become applicable to us.

Our board of directors may from time to time establish other committees.

### **Compensation committee interlocks and insider participation**

None of our directors who serve as a member of our compensation committee is, or has at any time during the past year been, one of our officers or employees. None of our executive officers currently serves, or in the past year has served, as a member of the board of directors or compensation committee of any other entity that has one or more executive officers serving on our board of directors or compensation committee.

### **Code of business conduct and ethics**

Effective upon the closing of this offering, our board of directors has adopted a Code of Business Conduct and Ethics, or the Code of Conduct, applicable to all of our employees, executive officers and directors. Following the closing of this offering, the Code of Conduct will be available on our website at [www.dova.com](http://www.dova.com). The nominating and corporate governance committee of our board of directors will be responsible for overseeing the Code of Conduct and must approve any waivers of the Code of Conduct for employees, executive officers and directors. In addition, we intend to post on our website all disclosures that are required by law or the listing standards of the applicable stock exchange concerning any amendments to, or waivers from, any provision of the Code of Conduct.

## **Non-employee director compensation**

In the year ended December 31, 2016, we did not pay any fees to, make any equity awards or non-equity awards to, or pay any other compensation to the non-employee members of our board of directors for their services as directors. Our non-employee directors only received reimbursement of their actual out-of-pocket costs and expenses incurred in connection with attending board meetings.

During 2017 and prior to this offering, we granted options to purchase 33,000 shares of our common stock under the 2017 Plan to each of Steven M. Goldman, Alfred Novak and Roger A. Jeffs, in connection with the appointment of each to our board of directors. One-third of the shares subject to each option vest on May 25, 2018 (the first anniversary of the vesting commencement date) and the remaining shares vest in 24 equal monthly installments thereafter, subject to the holder's continuous service as of each such date and subject to full acceleration in the event of a change in control, as defined in the 2017 Plan, during such continuous service. The options have a post-termination exercise period of twelve months upon termination of the holder's continuous service with us for any reason other than cause, disability or death.

### ***Non-employee director compensation policy***

In anticipation of this offering and the increased responsibilities of our directors as directors of a public company, our board of directors has adopted a non-employee director compensation policy, which will go into effect upon the pricing of this offering, pursuant to which each of our directors who is not an employee of our company or affiliated with an entity that beneficially owns 5% or more of our outstanding shares of common stock, which as of the pricing of this offering will be all directors other than Mr. Sapir, Mr. Manning and Mr. Stalfort, will be eligible to receive compensation for service on our board of directors and committees of our board of directors.

Each eligible director will receive an annual cash retainer of \$30,000 for serving on our board of directors. The chairperson of each of the audit, compensation and nominating and corporate governance committees of our board of directors will be entitled an additional annual cash retainer of \$5,000. All annual cash compensation amounts will be payable in equal quarterly installments in advance within the first 30 days of each quarter in which the service will occur.

In addition, each new eligible director who joins our board of directors after the pricing of this offering will be granted a non-statutory stock option to purchase 33,000 shares of common stock under our IPO Plan, with one-third of the shares vesting on the first anniversary of the date of grant and the remaining shares vesting in 24 equal monthly installments thereafter, subject to continued service as a director through the applicable vesting date.

On the date of each annual meeting of our stockholders, each eligible director who continues to serve as a director of our company following the meeting will be granted a non-statutory stock option to purchase 16,500 shares of our common stock under our IPO Plan, vesting monthly over one year from the grant date and in any event will be fully vested on the date of the next annual meeting of our stockholders, subject to continued service as a director through the applicable vesting date.

The exercise price per share of each stock option granted under the non-employee director compensation policy will be equal to the closing price of our common stock on the NASDAQ Global Market on the date of grant. Each stock option will have a term of ten years from the date of grant, subject to earlier termination in connection with a termination of the eligible director's continuous service with us (provided that upon a termination of service other than for death, disability or cause, the post-termination exercise period will be 12 months from the date of termination), and will vest in full upon a change in control transaction.



## Executive compensation

### Overview

Due to our limited operating history as described in the section titled “Management’s discussion and analysis of financial condition and results of operations,” until 2017, we did not have an executive compensation program and we did not pay any employee compensation or issue any stock-based compensation to any employee, director or consultant. During our limited operating history in 2016, Paul B. Manning acted as our Chief Executive Officer and Sean Stalfort acted as our President; we refer to Mr. Manning and Mr. Stalfort as our named executive officers for 2016. Both Mr. Manning and Mr. Stalfort are employees of PBM Capital Group, LLC. In January 2017, we hired Mr. Sapir as our President and Chief Executive Officer. In March 2017, we hired Mr. Blankenship as our Chief Financial Officer and in April 2017, we hired Dr. Allen as our Chief Medical Officer. Mr. Manning and Mr. Stalfort ceased serving as executive officers in January 2017.

The following tables and accompanying narrative disclosure set forth information about the limited compensation paid to PBM Capital Group, LLC that may be attributed to Mr. Manning’s and Mr. Stalfort’s services to us during 2016. Although Mr. Sapir, Mr. Blankenship and Dr. Allen commenced services with us in 2017, we have included information in the following narrative regarding each of such officers’ compensation where it may be material to an understanding of our executive compensation program.

### 2016 Summary compensation table

Although we did not pay Mr. Manning or Mr. Stalfort any base salary, bonus or stock-based or other compensation during 2016, we have services agreements with PBM Capital Group, LLC, which provide for certain scientific and technical, accounting, operations and back office support services as well as legal and professional fees and consulting services associated with the formation of our company and other corporate matters, as further described in the section titled “Certain relationships and related party transactions,” pursuant to which we pay PBM Capital Group, LLC a flat fee of \$25,000 per month. We paid \$0.4 million to PBM Capital Group, LLC pursuant to our services agreements with PBM Capital Group, LLC during the period from March 24, 2016 through December 31, 2016. Other than the portion of the management fees paid to PBM Capital Group, LLC that may be attributable to Mr. Manning’s and Mr. Stalfort’s services to us, we did not pay any other compensation, benefits or perquisites for Mr. Manning’s or Mr. Stalfort’s services to us during 2016.

### Outstanding equity awards at December 31, 2016

As of December 31, 2016, Mr. Manning and Mr. Stalfort did not hold any outstanding equity awards, nor did we grant, cancel or modify any equity awards during 2016. We granted equity awards to our current executive officers in 2017 pursuant to the terms of their employment agreements, described directly below under the section titled “–Employment agreements.”

### Employment agreements

We have entered into employment agreements with each of our current executive officers. The key terms of the agreements with our current Chief Executive Officer, Chief Financial Officer and Chief Medical Officer are described below. For a discussion of the severance pay and other benefits provided in connection with a termination of employment of our executive officers, please see “–Payments upon termination or change in control” below.

*Mr. Sapir*

We entered into an employment agreement with Mr. Sapir, our President and Chief Executive Officer, in January 2017. Pursuant to the terms of his employment agreement, Mr. Sapir's employment is at will and may be terminated at any time by us or Mr. Sapir. Under the terms of the agreement, Mr. Sapir is entitled to receive an annual base salary of \$400,000 and an annual bonus of up to 45% of his annual base salary based upon our board of directors' assessment of Mr. Sapir's performance and our attainment of targeted goals as set by the board of directors in their sole discretion. In accordance with the agreement, Mr. Sapir was also awarded an option to purchase 914,100 shares of our common stock on March 28, 2017 under our 2017 Plan. 25% of the shares subject to the option vest on January 3, 2018 (the first anniversary of Mr. Sapir's commencement of employment) and the remaining shares vest in 36 equal monthly installments thereafter, subject to Mr. Sapir's continued service and subject to full acceleration in the event of a sale event, as defined in Mr. Sapir's agreement, during such continued service. Pursuant to his agreement, Mr. Sapir also entered into a confidentiality, inventions assignment, non-competition and non-solicitation agreement with us.

*Mr. Blankenship*

We entered into an employment agreement with Mr. Blankenship, our Chief Financial Officer, in March 2017. Pursuant to the terms of his employment agreement, Mr. Blankenship's employment is at will and may be terminated at any time by us or Mr. Blankenship. Under the terms of the agreement, Mr. Blankenship is entitled to receive an annual base salary of \$250,000 and an annual bonus of up to 40% of his annual base salary based upon our board of directors' assessment of Mr. Blankenship's performance and our attainment of targeted goals as set by the board of directors in their sole discretion. In accordance with the agreement, Mr. Blankenship was also awarded an option to purchase 228,525 shares of our common stock on March 28, 2017 under our 2017 Plan. 25% of the shares subject to the option vest on March 1, 2018 (the first anniversary of Mr. Blankenship's commencement of employment) and the remaining shares vest in 36 equal monthly installments thereafter, subject to Mr. Blankenship's continued service and subject to full acceleration in the event of a sale event, as defined in Mr. Blankenship's agreement, during such continued service. Pursuant to his agreement, Mr. Blankenship also entered into a confidentiality, inventions assignment, non-competition and non-solicitation agreement with us.

*Dr. Allen*

We entered into an employment agreement with Dr. Allen, our Chief Medical Officer, in April 2017. Pursuant to the terms of his employment agreement, Dr. Allen's employment is at will and may be terminated at any time by us or Dr. Allen. Under the terms of the agreement, Dr. Allen is entitled to receive an annual base salary of \$400,000 and an annual bonus of up to 40% of his annual base salary based upon our board of directors' assessment of Dr. Allen's performance and our attainment of targeted goals as set by the board of directors in their sole discretion. In accordance with the agreement, Dr. Allen was also awarded an option to purchase 251,466 shares of our common stock on April 14, 2017 under our 2017 Plan. 25% of the shares subject to the option vest on April 14, 2018 (the first anniversary of Dr. Allen's commencement of employment) and the remaining shares vest in 36 equal monthly installments thereafter, subject to Dr. Allen's continued service and subject to full acceleration in the event of a sale event, as defined in Dr. Allen's agreement, during such continued service. Pursuant to his agreement, Dr. Allen also entered into a confidentiality, inventions assignment, non-competition and non-solicitation agreement with us.

#### *Dr. Laliberte*

We entered into an employment agreement with Dr. Laliberte, our Senior Vice President, Product Development, in March 2017. Pursuant to the terms of his employment agreement, Dr. Laliberte's employment is at will and may be terminated at any time by us or Dr. Laliberte. Under the terms of the agreement, Dr. Laliberte is entitled to receive an annual base salary of \$275,000 and an annual bonus of up to 40% of his annual base salary based upon our board of directors' assessment of Dr. Laliberte's performance and our attainment of targeted goals as set by the board of directors in its sole discretion. In accordance with the agreement, Dr. Laliberte was also awarded an option to purchase 115,500 shares of our common stock on March 28, 2017 under our 2017 Plan. 25% of the shares subject to this option vest on March 23, 2018 (the first anniversary of Dr. Laliberte's commencement of employment) and the remaining shares vest in 36 equal monthly installments thereafter. Dr. Laliberte was also awarded a second option to purchase 24,750 shares of our common stock on March 28, 2017 under our 2017 Plan. The shares subject to this option will commence vesting at the time we successfully complete two Phase 3/4 clinical trial protocols, as determined by our board of directors in its sole discretion. After commencement of vesting, 25% of the shares subject to this option vest on the first anniversary of the vesting commencement date and the remaining shares vest in 36 equal monthly installments thereafter. If the shares subject to the second option have not commenced vesting by September 30, 2017, the option shall immediately terminate. The vesting of shares subject to each option is subject to Dr. Laliberte's continued service and subject to full acceleration in the event of a sale event, as defined in Dr. Laliberte's agreement, during such continued service. Pursuant to his agreement, Dr. Laliberte also entered into a confidentiality, inventions assignment, non-competition and non-solicitation agreement with us.

#### **Payments upon termination or change in control**

The definitions of "cause," "good reason" and "sale event" referenced below are defined in the individual employment agreements with each of the named executive officers.

#### *Mr. Sapir*

Pursuant to his employment agreement, Mr. Sapir is entitled to severance benefits if, after January 3, 2018, his employment is terminated without cause or if he resigns for good reason, subject to his execution of a release. If after January 3, 2018 but on or before January 3, 2019, Mr. Sapir is terminated without cause or resigns for good reason, he is eligible to receive six months of continued base salary and premiums for continued health coverage. If after January 3, 2019, Mr. Sapir is terminated without cause or resigns for good reason, he is eligible to receive 12 months of continued base salary and premiums for continued health coverage. If Mr. Sapir is employed by us as of the closing of a sale event, then all remaining shares of common stock underlying his outstanding options will vest.

#### *Mr. Blankenship*

Pursuant to his employment agreement, Mr. Blankenship is entitled to severance benefits if, after March 1, 2018, his employment is terminated without cause or if he resigns for good reason, subject to his execution of a release. If after March 1, 2018 but on or before March 1, 2019, Mr. Blankenship is terminated without cause or resigns for good reason, he is eligible to receive six months of continued base salary and premiums for continued health coverage. If after March 1, 2019, Mr. Blankenship is terminated without cause or resigns for good reason, he is eligible to receive 12 months of continued base salary and premiums for continued health coverage. If Mr. Blankenship is employed by us as of the closing of a sale event, then all remaining shares of common stock underlying his outstanding options will vest.

#### *Dr. Allen*

Pursuant to his employment agreement, Dr. Allen is entitled to severance benefits if, after April 14, 2018, his employment is terminated without cause or if he resigns for good reason, subject to his execution of a release. If after April 14, 2018 but on or before April 14, 2019, Dr. Allen is terminated without cause or resigns for good reason, he is eligible to receive six months of continued base salary and premiums for continued health coverage. If after April 14, 2019, Dr. Allen is terminated without cause or resigns for good reason, he is eligible to receive 12 months of continued base salary and premiums for continued health coverage. If Dr. Allen is employed by us as of the closing of a sale event, then all remaining shares of common stock underlying his outstanding options will vest.

#### *Dr. Laliberte*

Pursuant to his employment agreement, Dr. Laliberte is entitled to severance benefits if, after March 23, 2018, his employment is terminated without cause or if he resigns for good reason, subject to his execution of a release. If after March 23, 2018 but on or before March 23, 2019, Dr. Laliberte is terminated without cause or resigns for good reason, he is eligible to receive six months of continued base salary and premiums for continued health coverage. If after March 23, 2019, Dr. Laliberte is terminated without cause or resigns for good reason, he is eligible to receive 12 months of continued base salary and premiums for continued health coverage. If Dr. Laliberte is employed by us as of the closing of a sale event, then all remaining shares of common stock underlying his outstanding options will vest.

### **Equity benefit plans**

#### *Amended and restated 2017 equity incentive plan*

In June 2017, our board of directors adopted the IPO Plan, which was also approved by our stockholders in June 2017. The IPO Plan will become effective in connection with the pricing of this offering and will amend and restate the 2017 Plan. After the IPO Plan becomes effective, no further awards will be granted under the 2017 Plan.

#### *Awards*

Our IPO Plan provides for the grant of incentive stock options, or ISOs, within the meaning of Section 422 of the Code, to our employees and our affiliates' employees, and for the grant of nonstatutory options, or NSOs, restricted stock awards, restricted stock unit awards, stock appreciation rights, performance-based stock awards and other forms of stock awards, which we refer to collectively as stock awards, to our employees, including officers, consultants and directors. Our IPO Plan also provides for the grant of performance-based cash awards to our employees, including officers, consultants and directors.

#### *Share reserve*

Initially, the aggregate number of shares of our common stock that may be issued pursuant to stock awards under the IPO Plan after the IPO Plan becomes effective is 4,285,250 shares, which is the sum of (1) 2,000,000 new shares, plus (2) the number of shares reserved for issuance under our 2017 Plan at the time our IPO Plan becomes effective, plus (3) any shares subject to outstanding stock options or other stock awards that would have otherwise returned to our 2017 Plan (such as upon the expiration or termination of a stock award prior to exercise). Additionally, the number of shares of our common stock reserved for issuance under our IPO Plan will automatically increase on January 1 of each year, beginning on January 1, 2018 (assuming the IPO Plan becomes effective before such date) and continuing through and including January 1, 2027, by 4% of the total number of shares of our capital stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares determined by our board of

directors. The maximum number of shares that may be issued upon the exercise of ISOs under our IPO Plan is 8,570,500 shares.

Shares subject to stock awards granted under the IPO Plan will not reduce the number of shares of common stock available for issuance under the IPO Plan if they (i) expire or otherwise terminate without all of the shares covered by such stock award having been issued or (ii) are settled in cash. Additionally, if any shares of common stock issued pursuant to stock awards under the IPO Plan are repurchased or reacquired by us or forfeited back to us for any reason, including a failure to vest, then the repurchased, reacquired or forfeited shares will revert to and again become available for issuance under the IPO Plan. Any shares that we reacquire as consideration for the exercise or purchase price of a stock award or to satisfy tax withholding obligations related to a stock award will again become available for issuance under the IPO Plan.

No person may be granted stock awards covering more than 2,000,000 shares of our common stock under our IPO Plan during any calendar year pursuant to stock options, stock appreciation rights and other stock awards whose value is determined by reference to an increase over an exercise or strike price of at least 100% of the fair market value on the date the stock award is granted. Additionally, no person may be granted in a calendar year a performance stock award covering more than 2,000,000 shares or a performance cash award having a maximum value in excess of \$2,000,000. Such limitations are designed to help assure that any deductions to which we would otherwise be entitled with respect to such awards will not be subject to the \$1,000,000 limitation on the income tax deductibility of compensation paid to any covered executive officer imposed by Section 162(m) of the Code. In addition, the maximum number of shares of our common stock subject to stock awards granted under the IPO Plan or otherwise during any one calendar year to any non-employee director, taken together with any cash fees paid by us to such non-employee director during such calendar year for service on our board of directors, will not exceed \$600,000 in total value, or, with respect to the calendar year in which a non-employee director is first appointed or elected to our board of directors, \$1,100,000.

As of the date hereof, no awards have been granted and no shares of our common stock have been issued under the IPO Plan.

#### *Administration*

Our board of directors, or a duly authorized committee thereof, will have the authority to administer the IPO Plan. Our board of directors will initially delegate its authority to administer the IPO Plan to our compensation committee under the terms of the compensation committee's charter. Our board of directors may also delegate to one or more of our officers the authority to (1) designate employees other than officers to receive specified stock awards and (2) determine the number of our shares of common stock to be subject to such stock awards. Subject to the terms of the IPO Plan, the administrator has the authority to determine the terms of awards, including recipients, the exercise price or strike price of stock awards, if any, the number of shares of common stock subject to each stock award, the fair market value of a share of common stock, the vesting schedule applicable to the awards, together with any vesting acceleration, the form of consideration, if any, payable upon exercise or settlement of the stock award and the terms and conditions of the award agreements for use under the IPO Plan.

The administrator has the power to modify outstanding awards under our IPO Plan. Subject to the terms of the IPO Plan, the administrator has the authority to reprice any outstanding stock award, cancel any outstanding stock award and grant in substitution a new stock award, cash or other consideration, or take any other action that is treated as a repricing under generally accepted accounting principles, with the consent of any adversely affected participant.

### *Stock options*

ISOs and NSOs are granted pursuant to stock option agreements adopted by the plan administrator. The plan administrator determines the exercise price for a stock option, within the terms and conditions of the IPO Plan, provided that the exercise price of a stock option generally cannot be less than 100% of the fair market value of our common stock on the date of grant. Options granted under the IPO Plan vest at the rate specified by the plan administrator.

The plan administrator determines the term of stock options granted under the IPO Plan, up to a maximum of 10 years. Unless the terms of an optionholder's stock option agreement provide otherwise, if an optionholder's service relationship with us, or any of our affiliates, ceases for any reason other than disability, death or cause, the optionholder may generally exercise any vested options for a period of three months following the cessation of service. The option term may be extended in the event that exercise of the option following such a termination of service is prohibited by applicable securities laws or our insider trading policy. If an optionholder's service relationship with us or any of our affiliates ceases due to disability or death, or an optionholder dies within a certain period following cessation of service, the optionholder or a beneficiary may generally exercise any vested options for a period of 12 months in the event of disability and 18 months in the event of death. In the event of a termination for cause, options generally terminate immediately upon the termination of the individual for cause. In no event may an option be exercised beyond the expiration of its term.

Acceptable consideration for the purchase of common stock issued upon the exercise of a stock option will be determined by the plan administrator and may include (1) cash, check, bank draft or money order, (2) a broker-assisted cashless exercise, (3) the tender of shares of our common stock previously owned by the optionholder, (4) a net exercise of the option if it is an NSO, and (5) other legal consideration approved by the plan administrator.

Unless the plan administrator provides otherwise, options generally are not transferable except by will, the laws of descent and distribution, or (subject to the approval of our board of directors or an authorized officer) pursuant to a domestic relations order. Subject to the approval of our board of directors or an authorized officer, the optionholder may designate a beneficiary who may exercise the option following the optionholder's death.

### *Tax limitations on incentive stock options*

The aggregate fair market value, determined at the time of grant, of our common stock with respect to ISOs that are exercisable for the first time by an optionholder during any calendar year under all of our stock plans may not exceed \$100,000. Options or portions thereof that exceed such limit will generally be treated as NSOs. No ISO may be granted to any person who, at the time of the grant, owns or is deemed to own stock possessing more than 10% of our total combined voting power or that of any of our affiliates unless (1) the option exercise price is at least 110% of the fair market value of the stock subject to the option on the date of grant, and (2) the option is not exercisable after the expiration of five years from the date of grant.

### *Restricted stock awards*

Restricted stock awards are granted pursuant to restricted stock award agreements adopted by the plan administrator. Restricted stock awards may be granted in consideration for (1) cash, check, bank draft or money order, (2) services rendered to us or our affiliates, or (3) any other form of legal consideration acceptable to the plan administrator. Common stock acquired under a restricted stock award may, but need not, be subject to a share repurchase option in our favor in accordance with a vesting schedule to be

determined by the plan administrator. A restricted stock award may be transferred only upon such terms and conditions as set by the plan administrator. Except as otherwise provided in the applicable award agreement, restricted stock that has not vested will be forfeited or repurchased by us upon the participant's cessation of continuous service for any reason.

#### *Restricted stock unit awards*

Restricted stock unit awards are granted pursuant to restricted stock unit award agreements adopted by the plan administrator. Restricted stock unit awards may be granted in consideration for any form of legal consideration acceptable to the plan administrator. A restricted stock unit award may be settled by cash, delivery of stock, a combination of cash and stock as deemed appropriate by the plan administrator, or in any other form of consideration set forth in the restricted stock unit award agreement. Additionally, dividend equivalents may be credited in respect of shares covered by a restricted stock unit award. Except as otherwise provided in the applicable award agreement, restricted stock units that have not vested will be forfeited upon the participant's cessation of continuous service for any reason.

#### *Stock appreciation rights*

Stock appreciation rights are granted pursuant to stock appreciation grant agreements adopted by the plan administrator. The plan administrator determines the strike price for a stock appreciation right, which generally cannot be less than 100% of the fair market value of our common stock on the date of grant. Upon the exercise of a stock appreciation right, we will pay the participant an amount equal to the product of (1) the excess of the per share fair market value of our common stock on the date of exercise over the strike price, multiplied by (2) the number of shares of common stock with respect to which the stock appreciation right is exercised. A stock appreciation right granted under the IPO Plan vests at the rate specified in the stock appreciation right agreement as determined by the plan administrator.

The plan administrator determines the term of stock appreciation rights granted under the IPO Plan, up to a maximum of 10 years. Unless the terms of a participant's stock appreciation right agreement provides otherwise, if a participant's service relationship with us or any of our affiliates ceases for any reason other than cause, disability or death, the participant may generally exercise any vested stock appreciation right for a period of three months following the cessation of service. The stock appreciation right term may be further extended in the event that exercise of the stock appreciation right following such a termination of service is prohibited by applicable securities laws. If a participant's service relationship with us, or any of our affiliates, ceases due to disability or death, or a participant dies within a certain period following cessation of service, the participant or a beneficiary may generally exercise any vested stock appreciation right for a period of 12 months in the event of disability and 18 months in the event of death. In the event of a termination for cause, stock appreciation rights generally terminate immediately upon the occurrence of the event giving rise to the termination of the individual for cause. In no event may a stock appreciation right be exercised beyond the expiration of its term.

#### *Performance awards*

The IPO Plan permits the grant of performance-based stock and cash awards that may qualify as performance-based compensation that is not subject to the \$1,000,000 limitation on the income tax deductibility of compensation paid to a covered executive officer imposed by Section 162(m) of the Code. To help assure that the compensation attributable to performance-based awards will so qualify, our compensation committee can structure such awards so that stock or cash will be issued or paid pursuant to such award only after the achievement of certain pre-established performance goals during a designated performance period.

The performance goals that may be selected include one or more of the following: (i) earnings (including earnings per share and net earnings); (ii) earnings before interest, taxes and depreciation; (iii) earnings before interest, taxes, depreciation and amortization; (iv) earnings before interest, taxes, depreciation, amortization and legal settlements; (v) earnings before interest, taxes, depreciation, amortization, legal settlements and other income (expense); (vi) earnings before interest, taxes, depreciation, amortization, legal settlements, other income (expense) and stock-based compensation; (vii) earnings before interest, taxes, depreciation, amortization, legal settlements, other income (expense), stock-based compensation and changes in deferred revenue; (viii) earnings before interest, taxes, depreciation, amortization, legal settlements, other income (expense), stock-based compensation, other non-cash expenses and changes in deferred revenue; (ix) total stockholder return; (x) return on equity or average stockholder's equity; (xi) return on assets, investment, or capital employed; (xii) stock price; (xiii) margin (including gross margin); (xiv) income (before or after taxes); (xv) operating income; (xvi) operating income after taxes; (xvii) pre-tax profit; (xviii) operating cash flow; (xix) sales or revenue targets; (xx) increases in revenue or product revenue; (xxi) expenses and cost reduction goals; (xxii) improvement in or attainment of working capital levels; (xxiii) economic value added (or an equivalent metric); (xxiv) market share; (xxv) cash flow; (xxvi) cash flow per share; (xxvii) cash balance; (xxviii) cash burn; (xxix) cash collections; (xxx) share price performance; (xxxi) debt reduction; (xxxii) implementation or completion of projects or processes (including, without limitation, discovery of a pre-clinical drug candidate, recommendation of a drug candidate to enter a clinical trial, clinical trial initiation, clinical trial enrollment and dates, clinical trial results, regulatory filing submissions (such as IND, BLA and NDA), regulatory filing acceptances, regulatory or advisory committee interactions, regulatory approvals, and product supply); (xxxiii) stockholders' equity; (xxxiv) capital expenditures; (xxxv) financings; (xxxvi) operating profit or net operating profit; (xxxvii) workforce diversity; (xxxviii) growth of net income or operating income; (xxxix) employee retention; (xl) initiation of studies by specific dates; (xli) budget management; (xlii) submission to, or approval by, a regulatory body (including, but not limited to the FDA) of an applicable filing or a product; (xliii) regulatory milestones; (xliv) progress of internal research or development programs; (xlv) progress of partnered programs; (xlvi) partner satisfaction; (xlvii) timely completion of clinical trials; (xlviii) milestones related to research development (including, but not limited to, preclinical and clinical studies), product development and manufacturing; (xlix) expansion of sales in additional geographies or markets; (l) research progress, including the development of programs; (li) strategic partnerships or transactions (including in-licensing and out-licensing of intellectual property; (lii) filing of patent applications and granting of patents; and (liii) to the extent that an award is not intended to comply with Section 162(m) of the Code, other measures of performance selected by our board of directors.

The performance goals may be based on a company-wide basis, with respect to one or more business units, divisions, affiliates, or business segments, and in either absolute terms or relative to the performance of one or more comparable companies or the performance of one or more relevant indices. Unless specified otherwise (i) in the award agreement at the time the award is granted or (ii) in such other document setting forth the performance goals at the time the goals are established, we will appropriately make adjustments in the method of calculating the attainment of performance goals as follows: (1) to exclude restructuring and/or other nonrecurring charges; (2) to exclude exchange rate effects; (3) to exclude the effects of changes to generally accepted accounting principles; (4) to exclude the effects of any statutory adjustments to corporate tax rates; (5) to exclude the effects of items that are "unusual" in nature or occur "infrequently" as determined under generally accepted accounting principles; (6) to exclude the dilutive effects of acquisitions or joint ventures; (7) to assume that any business divested by us achieved performance objectives at targeted levels during the balance of a performance period following such divestiture; (8) to exclude the effect of any change in the outstanding shares of our common stock by



reason of any stock dividend or split, stock repurchase, reorganization, recapitalization, merger, consolidation, spin-off, combination or exchange of shares or other similar corporate change, or any distributions to common stockholders other than regular cash dividends; (9) to exclude the effects of stock based compensation and the award of bonuses under our bonus plans; (10) to exclude costs incurred in connection with potential acquisitions or divestitures that are required to be expensed under generally accepted accounting principles; (11) to exclude the goodwill and intangible asset impairment charges that are required to be recorded under generally accepted accounting principles; and (12) to exclude the effects of the timing of acceptance for review and/or approval of submissions to the FDA or any other regulatory body. In addition, we retain the discretion to reduce or eliminate the compensation or economic benefit due upon attainment of the goals. The performance goals may differ from participant to participant and from award to award.

#### *Other stock awards*

The plan administrator may grant other awards based in whole or in part by reference to our common stock. The plan administrator will set the number of shares under the stock award and all other terms and conditions of such awards.

#### *Changes to capital structure*

In the event there is a specified type of change in our capital structure, such as a split, reverse split or recapitalization, appropriate adjustments will be made to (1) the class and maximum number of shares reserved for issuance under our IPO Plan, (2) the class and maximum number of shares by which the share reserve may increase automatically each year, (3) the class and maximum number of shares that may be issued upon the exercise of ISOs, (4) the class and maximum number of shares subject to stock awards that can be granted to any person in a calendar year (as established under the IPO Plan pursuant to Section 162(m) of the Code) and (5) the class and number of shares and exercise price, strike price or purchase price, if applicable, subject to outstanding stock awards.

#### *Corporate transactions*

The IPO Plan provides that in the event of a specified corporate transaction (or a change in control, as described below), the administrator has discretion to take any of the following actions with respect to stock awards:

- arrange for the assumption, continuation or substitution of a stock award by the surviving or acquiring corporation;
- arrange for the assignment of any reacquisition or repurchase rights held by us to the surviving or acquiring corporation;
- accelerate the vesting, in whole or in part, of the stock award and, if applicable, the period during which the stock award may be exercised, to a date prior to the effective time of the transaction and provide for its termination if not exercised (if applicable) at or prior to the effective time of the transaction;
- arrange for the lapse, in whole or in part, of any reacquisition or repurchase right held by us with respect to the stock award;
- cancel the stock award, to the extent not vested or not exercised prior to the effective time of the transaction, in exchange for such cash consideration, if any, as our board of directors, in its sole discretion, may consider appropriate; or

- make a payment, in such form as determined by the administrator, equal to the excess, if any, of the value of the property that would have been received if such award was exercised immediately prior to the effective time of the transaction over any exercise price payable by the holder in connection with such exercise.

The administrator is not obligated to treat all stock awards or portions of stock awards, even those that are of the same type, in the same manner. The administrator may take different actions with respect to the vested and unvested portions of a stock award.

Under the IPO Plan, a corporate transaction is generally defined as the consummation of (1) a sale or other disposition of all or substantially all of our assets; (2) a sale or other disposition of more than 50% of our outstanding securities; (3) a merger, consolidation or similar transaction following which we are not the surviving entity; or (4) a merger, consolidation or similar transaction following which we are the surviving entity but the shares of our common stock outstanding immediately prior to such transaction are converted or exchanged into other property by virtue of the transaction.

#### *Change in control*

In addition to the above, the administrator may provide, in an individual award agreement or in any other written agreement between us and the participant, that the stock award will be subject to additional acceleration of vesting and exercisability in the event of a change in control. In the absence of such a provision, no such acceleration will automatically occur.

Under the IPO Plan, a change in control is generally defined as (1) the acquisition by a person or entity of more than 50% of our combined voting power other than by merger, consolidation or similar transaction; (2) a consummated merger, consolidation or similar transaction immediately after which our stockholders cease to own more than 50% of the combined voting power of the surviving entity (or its parent) in substantially the same proportions as their ownership of the outstanding voting securities of the Company immediately prior to such transaction; (3) our stockholders or board of directors approves a plan of complete dissolution or liquidation or a complete dissolution or liquidation of the Company will otherwise occur, except for a liquidation into a parent entity; (4) a consummated sale, lease, exclusive license or other disposition of all or substantially all of our assets or (5) when a majority of our board of directors becomes comprised of individuals who were not serving on our board of directors on the date of pricing for this offering, or the incumbent board, or whose nomination, appointment, or election was not approved by a majority of the incumbent board then still in office.

#### *Plan amendment or termination*

Our board of directors has the authority to amend, suspend, or terminate the IPO Plan, provided that such action does not impair the existing rights of any participant without such participant's written consent. No ISOs may be granted after the tenth anniversary of the earlier of (i) the date our board of directors adopted the IPO Plan or (ii) the date our stockholders approved the IPO Plan.

#### *2017 Equity incentive plan*

In March 2017, our board of directors adopted our 2017 Plan, which was approved by our stockholders in April 2017. As described above, our board of directors has adopted, and our stockholders have approved, the IPO Plan that will become effective in connection with the execution and delivery of the underwriting agreement related to this offering, and, upon its effectiveness, the IPO Plan will supersede and replace the 2017 Plan. The description set forth below reflects the 2017 Plan, as currently in effect. After the IPO Plan becomes effective, no further stock awards will be granted under the 2017 Plan.

### *Stock awards*

Our 2017 Plan provides for the grant of incentive stock options, or ISOs, within the meaning of Section 422 of the Internal Revenue Code, or the Code, to our employees and our affiliates' employees, and for the grant of nonstatutory options, or NSOs, restricted stock awards, restricted stock unit awards, stock appreciation rights and other forms of stock awards to our employees, including officers, consultants and directors.

### *Share reserve*

The maximum number of shares of common stock that may be issued under the 2017 Plan is 2,285,250 shares. The maximum number of shares of common stock that may be issued pursuant to the exercise of ISOs under the 2017 Plan is 4,570,500 shares. Shares issued under the 2017 Plan may be authorized but unissued or reacquired shares of common stock.

Shares subject to stock awards granted under the 2017 Plan will not reduce the number of shares of common stock available for issuance under the 2017 Plan if they (i) expire or otherwise terminate without all of the shares covered by such stock award having been issued or (ii) are settled in cash. Additionally, if any shares of common stock issued pursuant to stock awards under the 2017 Plan are repurchased or reacquired by us or forfeited back to us for any reason, including a failure to vest, then the repurchased, reacquired or forfeited shares will revert to and again become available for issuance under the 2017 Plan. Any shares that we reacquire as consideration for the exercise or purchase price of a stock award or to satisfy tax withholding obligations related to a stock award will again become available for issuance under the 2017 Plan.

As of June 2, 2017, there were 559,509 shares available for the grant of stock awards under our 2017 Plan, and there were outstanding stock options covering a total of 1,725,741 shares that were granted under our 2017 Plan.

### *Administration*

Our board of directors, or a duly authorized committee thereof, will have the authority to administer the 2017 Plan. Our board of directors will initially delegate its authority to administer the 2017 Plan to our compensation committee under the terms of the compensation committee's charter. Our board of directors may also delegate to one or more of our officers the authority to (1) designate employees other than officers to receive specified stock awards and (2) determine the number of our shares of common stock to be subject to such stock awards. Subject to the terms of the 2017 Plan, the administrator has the authority to determine the terms of awards, including recipients, the exercise price or strike price of stock awards, if any, the number of shares of common stock subject to each stock award, the fair market value of a share of common stock, the vesting schedule applicable to the awards, together with any vesting acceleration, the form of consideration, if any, payable upon exercise or settlement of the stock award and the terms and conditions of the award agreements for use under the 2017 Plan.

The administrator has the power to modify outstanding awards under our 2017 Plan. Subject to the terms of the 2017 Plan, the administrator has the authority to reprice any outstanding stock award, cancel any outstanding stock award and grant in substitution a new stock award, cash or other consideration, or take any other action that is treated as a repricing under generally accepted accounting principles, with the consent of any adversely affected participant.

### *Stock options*

ISOs and NSOs are granted pursuant to stock option agreements adopted by the plan administrator. The plan administrator determines the exercise price for a stock option, within the terms and conditions of the 2017 Plan, provided that the exercise price of a stock option generally cannot be less than 100% of the fair market value of our common stock on the date of grant. Options granted under the 2017 Plan vest at the rate specified by the plan administrator.

The plan administrator determines the term of stock options granted under the 2017 Plan, up to a maximum of 10 years. Unless the terms of an optionholder's stock option agreement provide otherwise, if an optionholder's service relationship with us, or any of our affiliates, ceases for any reason other than disability, death or cause, the optionholder may generally exercise any vested options for a period of three months following the cessation of service. The option term may be extended in the event that exercise of the option following such a termination of service is prohibited by applicable securities laws or our insider trading policy. If an optionholder's service relationship with us or any of our affiliates ceases due to disability or death, or an optionholder dies within a certain period following cessation of service, the optionholder or a beneficiary may generally exercise any vested options for a period of 12 months in the event of disability and 18 months in the event of death. In the event of a termination for cause, options generally terminate immediately upon the termination of the individual for cause. In no event may an option be exercised beyond the expiration of its term.

Acceptable consideration for the purchase of common stock issued upon the exercise of a stock option will be determined by the plan administrator and may include (1) cash, check, bank draft or money order, (2) a broker-assisted cashless exercise, (3) the tender of shares of our common stock previously owned by the optionholder, (4) a net exercise of the option if it is an NSO, and (5) other legal consideration approved by the plan administrator.

Unless the plan administrator provides otherwise, options generally are not transferable except by will, the laws of descent and distribution, or (subject to the approval of our board of directors or an authorized officer) pursuant to a domestic relations order. Subject to the approval of our board of directors or an authorized officer, the optionholder may designate a beneficiary who may exercise the option following the optionholder's death.

### *Tax limitations on incentive stock options*

The aggregate fair market value, determined at the time of grant, of our common stock with respect to ISOs that are exercisable for the first time by an optionholder during any calendar year under all of our stock plans may not exceed \$100,000. Options or portions thereof that exceed such limit will generally be treated as NSOs. No ISO may be granted to any person who, at the time of the grant, owns or is deemed to own stock possessing more than 10% of our total combined voting power or that of any of our affiliates unless (1) the option exercise price is at least 110% of the fair market value of the stock subject to the option on the date of grant, and (2) the option is not exercisable after the expiration of five years from the date of grant.

### *Changes to capital structure*

In the event there is a specified type of change in our capital structure, such as a split, reverse split or recapitalization, appropriate adjustments will be made to (1) the class and maximum number of shares reserved for issuance under our 2017 Plan, (2) the class and maximum number of shares that may be issued upon the exercise of ISOs and (3) the class and number of shares and exercise price, strike price or purchase price, if applicable, subject to outstanding stock awards.

### *Corporate transactions*

The 2017 Plan provides that in the event of a specified corporate transaction or a change in control (as described below), the administrator has discretion to take any of the following actions with respect to stock awards:

- arrange for the assumption, continuation or substitution of a stock award by the surviving or acquiring corporation;
- arrange for the assignment of any reacquisition or repurchase rights held by us to the surviving or acquiring corporation;
- accelerate the vesting, in whole or in part, of the stock award and, if applicable, the period during which the stock award may be exercised, to a date prior to the effective time of the transaction and provide for its termination if not exercised (if applicable) at or prior to the effective time of the transaction;
- arrange for the lapse, in whole or in part, of any reacquisition or repurchase right held by us with respect to the stock award;
- cancel the stock award, to the extent not vested or not exercised prior to the effective time of the corporate transaction, in exchange for such cash consideration, if any, as our board of directors, in its sole discretion, may consider appropriate; or
- make a payment, in such form as determined by the administrator, equal to the excess, if any, of the value of the property that would have been received if such award was exercised immediately prior to the effective time of the corporate transaction over any exercise price payable by the holder in connection with such exercise.

The administrator is not obligated to treat all stock awards or portions of stock awards, even those that are of the same type, in the same manner. The administrator may take different actions with respect to the vested and unvested portions of a stock award.

Under the 2017 Plan, a corporate transaction is generally defined as the consummation of (1) a sale or other disposition of all or substantially all of our assets; (2) a sale or other disposition of more than 50% of our outstanding securities; (3) a merger, consolidation or similar transaction following which we are not the surviving entity; or (4) a merger, consolidation or similar transaction following which we are the surviving entity but the shares of our common stock outstanding immediately prior to such transaction are converted or exchanged into other property by virtue of the transaction.

### *Change in control*

In addition to the above, the administrator may provide, in an individual award agreement or in any other written agreement between us and the participant, that the stock award will be subject to additional acceleration of vesting and exercisability in the event of a change in control. In the absence of such a provision, no such acceleration will automatically occur.

Under the 2017 Plan, a change in control is generally defined as (1) the acquisition by a person or entity of more than 50% of our combined voting power other than by merger, consolidation or similar transaction; (2) a consummated merger, consolidation or similar transaction immediately after which our stockholders cease to own more than 50% of the combined voting power of the surviving entity (or its parent) in substantially the same proportions as their ownership of the outstanding voting securities of the Company immediately prior to such transaction; (3) our stockholders or board of directors approves a plan of

complete dissolution or liquidation or a complete dissolution or liquidation of the Company will otherwise occur, except for a liquidation into a parent entity; or (4) a consummated sale, lease, exclusive license or other disposition of all or substantially all of our assets.

#### *Plan amendment or termination*

Our board of directors has the authority to amend, suspend, or terminate the 2017 Plan, provided that such action does not impair the existing rights of any participant without such participant's written consent. Unless terminated sooner by our board of directors, the 2017 Plan will automatically terminate on the day before the tenth (10th) anniversary of the earlier of (1) the date the 2017 Plan was adopted by our board, or (2) the date the 2017 Plan was approved by our stockholders. No ISOs may be granted on or after the tenth anniversary of the date our board of directors adopted the 2017 Plan.

#### **Limitation on liability and indemnification of directors and officers**

Our amended and restated certificate of incorporation, which will be effective immediately prior to the completion of this offering, limits our directors' liability to the fullest extent permitted under Delaware corporate law. Delaware corporate law provides that directors of a corporation will not be personally liable for monetary damages for breach of their fiduciary duties as directors, except for liability:

- for any transaction from which the director derives an improper personal benefit;
- for any act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;
- under Section 174 of the Delaware General Corporation Law (unlawful payment of dividends or redemption of shares); or
- for any breach of a director's duty of loyalty to the corporation or its stockholders.

If the Delaware General Corporation Law is amended to authorize corporate action further eliminating or limiting the personal liability of directors, then the liability of our directors shall be eliminated or limited to the fullest extent permitted by the Delaware General Corporation Law, as so amended.

Delaware law and our amended and restated bylaws, which will be effective immediately prior to consummation of this offering, provide that we will, in certain situations, indemnify our directors and officers and may indemnify other employees and other agents, to the fullest extent permitted by law. Any indemnified person is also entitled, subject to certain limitations, to payment or reimbursement of reasonable expenses (including attorneys' fees and disbursements) in advance of the final disposition of the proceeding.

We maintain a directors' and officers' insurance policy pursuant to which our directors and officers are insured against liability for certain actions taken in their capacities as directors and officers. We believe that these provisions in our amended and restated certificate of incorporation and amended and restated bylaws and this insurance policy are necessary to attract and retain qualified persons as directors and officers.

Insofar as indemnification for liabilities arising under the Securities Act, may be permitted to directors, officers or control persons, in the opinion of the SEC, such indemnification is against public policy as expressed in the Securities Act and is therefore unenforceable.

## **Certain relationships and related party transactions**

The following includes a summary of transactions since March 24, 2016 to which we have been a party, in which the amount involved in the transaction exceeded \$120,000, and in which any of our directors, executive officers or, to our knowledge, beneficial owners of more than 5% of our voting securities or any member of the immediate family of any of the foregoing persons had or will have a direct or indirect material interest. Other than described below, there have not been, nor are there currently any proposed, transactions or series of similar transactions to which we have been or will be a party other than compensation arrangements, which include equity and other compensation, termination, change in control and other arrangements, which are described under “Executive compensation.”

### **Participation in this offering**

Certain of our existing beneficial owners of more than 5% of our voting securities and their affiliated entities and certain of our directors have indicated an interest in purchasing an aggregate of approximately \$23 million in shares of our common stock in this offering at the initial public offering price per share. Based on an assumed initial public offering price of \$16.00 per share, these persons and entities would purchase an aggregate of approximately 1,437,500 of the 4,062,500 shares in this offering based on these indications of interest. However, because indications of interest are not binding agreements or commitments to purchase, the underwriters may determine to sell more, less or no shares in this offering to any of these persons or entities, or any of these persons or entities may determine to purchase more, less or no shares in this offering.

### **Conversion from limited liability company to corporation**

In September 2016, we converted from a Delaware limited liability company named Dova Pharmaceuticals, LLC (formerly known as PBM AKX Holdings, LLC), or the LLC, to Dova Pharmaceuticals, Inc., a Delaware corporation. We refer to this activity as the Conversion. The Conversion was effected pursuant to a plan of conversion whereby each unit of membership of the LLC was converted into 330 shares of our common stock. Additionally, we terminated the LLC’s operating agreement in connection with the Conversion. As part of the Conversion, the members of the LLC became our stockholders in the same ownership proportions as immediately prior to the Conversion. Effective upon the Conversion, our stockholders entered into a stockholders agreement which contained provisions similar to those set forth in the LLC’s operating agreement immediately prior to the Conversion.

### **Services agreements with PBM Capital Group, LLC**

In April 2016, we entered into the Dova services agreement with PBM Capital Group, LLC, an affiliate of PBM Capital Investments, LLC, a beneficial owner of more than 5% of our common stock and an entity controlled by Paul B. Manning, one of our directors, 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. We paid \$0.2 million to PBM Capital Group, LLC pursuant to the Dova services agreement during the period from March 24, 2016 through December 31, 2016, and these amounts are included within general and administrative expense in our consolidated statements of operations for such period.

In April 2016, our wholly-owned subsidiary, AkaRx, Inc., or AkaRx, entered into the AkaRx services agreement with PBM Capital Group, LLC 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. We paid \$0.2 million to PBM Capital Group, LLC pursuant to the AkaRx services agreement during the period from March 24, 2016 through December 31, 2016, and these amounts are included within general and administrative expense in our consolidated statements of operations for such period.

### **Guarantee by PBM Capital Investments, LLC**

In March 2016, we entered into a transition services agreement with Eisai, or the TSA. In connection with the TSA, AkaRx issued 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. Principal and interest under the Eisai note can be prepaid at any time without penalty. Payments due pursuant to the Eisai note are currently guaranteed by PBM Capital Investments, LLC.

### **Private placements of our securities**

In March 2016, we issued PBM Capital Investments, LLC an aggregate of 50,000 units in exchange for its payment to Eisai of \$5.0 million on our behalf in connection with our acquisition of worldwide rights to avatrombopag. In April 2016, we entered into a co-investment agreement, or the co-investment agreement, with PBM Capital Investments, LLC, and certain affiliates of PBM Capital Investments, LLC, which we refer to as the Co-Investors. Pursuant to the co-investment agreement, we issued and sold to the Co-Investors an aggregate of 2,522 units at a purchase price of \$100.00 per unit for an aggregate purchase price of \$252,200. Each unit was converted into 330 shares of our common stock in connection with the Conversion. Paul B. Manning, one of our directors, has sole voting and dispositive power over the shares held by PBM Capital Investments, LLC. Mr. Manning has sole voting and shared dispositive power over the shares held by the Co-Investors.

In June 2017, PBM Capital Investments, LLC distributed 1,757,700 shares of common stock to certain of its members, for no additional consideration, in accordance with the terms of its operating agreement. Under the terms of the distribution, Mr. Manning retains sole voting and shared dispositive power over the distributed shares through the completion of this offering, at which time Mr. Manning's voting and dispositive power over the distributed shares, as well as the shares held by the Co-Investors, will terminate. The transferees in the distribution are subject to the lock-up restrictions described under "Underwriting" elsewhere in this prospectus.

### **Series A preferred stock financing**

From September to November 2016, we sold an aggregate of 982,714 shares of our Series A preferred stock at a price of \$29.51 per share for aggregate gross proceeds of \$29.0 million. 338,868 shares were sold to Perceptive Life Sciences Master Fund, Ltd., a beneficial owner of more than 5% of our capital stock, for a purchase price of \$10.0 million. In addition, 33,886 shares were sold to one of the Co-Investors for a purchase price of \$1.0 million. Each share of Series A preferred stock is convertible into 3.3 shares of our common stock and such shares are expected to automatically convert immediately prior to the completion of this offering.



## **Investors' rights agreement**

In connection with our Series A preferred stock financing, we entered into an investors' rights agreement, or the IRA. The IRA contains voting rights, information rights, board observer rights, pro rata participation rights and registration rights, among other things, with certain holders of our capital stock. In addition, as described in "Management—Board composition and election of directors—Board composition," the IRA entitles certain holders of our capital stock to designate a director to our board. Pursuant to the terms of the agreement, each of these rights will terminate immediately prior to the closing of this offering, except for the registration rights, as more fully described in "Description of capital stock—Registration rights."

## **Employment agreements**

We have entered into employment-related agreements with our current executive officers, including our named executive officers. For more information regarding these agreements, see "Executive compensation—Employment agreements" and "Executive compensation—Payments upon termination or change in control."

## **Indemnification agreements**

In connection with this offering, we will enter into indemnification agreements with each of our directors and executive officers. These agreements, among other things, will require us to indemnify each director (and in certain cases their related venture capital funds) and executive officer to the fullest extent permitted by Delaware law, including indemnification of expenses such as attorneys' fees, judgments, fines and settlement amounts incurred by the director or executive officer in any action or proceeding, including any action or proceeding by or in right of us, arising out of the person's services as a director or executive officer.

## **Stock option grants to executive officers and directors**

We have granted stock options to our named executive officer as more fully described in the section titled "Executive compensation."

## **Policies and procedures for transactions with related persons**

Prior to this offering, we have not had a formal policy regarding approval of transactions with related parties. Prior to the pricing of this offering, we expect to adopt a related person transaction policy that sets forth our procedures for the identification, review, consideration and approval or ratification of related person transactions. The policy will become effective immediately upon the effectiveness of the registration statement on Form S-1 of which this prospectus is a part. For purposes of our policy only, a "related person transaction" is a transaction, arrangement or relationship, or any series of similar transactions, arrangements or relationships, in which we and any related person are, were or will be participants in which the amount involved exceeds \$120,000. Transactions involving compensation for services provided to us as an employee or director are not covered by this policy. A "related person" is any executive officer, director or beneficial owner of more than 5% of any class of our voting securities, including any of their immediate family members and any entity owned or controlled by such persons.

Under the policy, if a transaction has been identified as a related person transaction, including any transaction that was not a related person transaction when originally consummated or any transaction that was not initially identified as a related person transaction prior to consummation, our management must

present information regarding the related person transaction to our audit committee, or, if audit committee approval would be inappropriate, to another independent body of our board of directors, for review, consideration and approval or ratification. The presentation must include a description of, among other things, the material facts, the interests, direct and indirect, of the related persons, the benefits to us of the transaction and whether the transaction is on terms that are comparable to the terms available to or from, as the case may be, an unrelated third party or to or from employees generally. Under the policy, we will collect information that we deem reasonably necessary from each director, executive officer and, to the extent feasible, significant stockholder to enable us to identify any existing or potential related-person transactions and to effectuate the terms of the policy. In addition, under our Code of Conduct that we expect to adopt prior to the completion of this offering, our employees and directors will have an affirmative responsibility to disclose any transaction or relationship that reasonably could be expected to give rise to a conflict of interest. In considering related person transactions, our audit committee, or other independent body of our board of directors, will take into account the relevant available facts and circumstances including, but not limited to:

- the risks, costs and benefits to us;
- the impact on a director's independence in the event that the related person is a director, immediate family member of a director or an entity with which a director is affiliated;
- the availability of other sources for comparable services or products; and
- the terms available to or from, as the case may be, unrelated third parties or to or from employees generally.

The policy requires that, in determining whether to approve, ratify or reject a related person transaction, our audit committee, or other independent body of our board of directors, must consider, in light of known circumstances, whether the transaction is in, or is not inconsistent with, our best interests and those of our stockholders, as our audit committee, or other independent body of our board of directors, determines in the good faith exercise of its discretion.

## Principal stockholders

The following table sets forth information regarding beneficial ownership of our capital stock as of June 15, 2017 by:

- each person, or group of affiliated persons, known by us to beneficially own more than 5% of our common stock;
- each of our directors;
- each of our named executive officers; and
- all of our current executive officers and directors as a group.

We have determined beneficial ownership in accordance with the rules of the SEC. Under these rules, beneficial ownership includes any shares of common stock as to which the individual or entity has sole or shared voting power or investment power. Applicable percentage ownership is based on 20,575,207 shares of common stock outstanding as of June 15, 2017, after giving effect to the conversion of our outstanding Series A preferred stock. In computing the number of shares beneficially owned by an individual or entity and the percentage ownership of that person, shares of common stock subject to options held by such person as of June 15, 2017 that are currently exercisable or will become exercisable within 60 days of June 15, 2017 are considered outstanding, although these shares are not considered outstanding for purposes of computing the percentage ownership of any other person.

Certain of our existing stockholders and their affiliated entities and certain of our directors have indicated an interest in purchasing an aggregate of approximately \$25 million in shares of our common stock in this offering at the initial public offering price per share. Based on an assumed initial public offering price of \$16.00 per share, these persons and entities would purchase an aggregate of approximately 1,562,500 of the 4,062,500 shares in this offering based on these indications of interest. However, because indications of interest are not binding agreements or commitments to purchase, the underwriters may determine to sell more, less or no shares in this offering to any of these persons or entities, or any of these persons or entities may determine to purchase more, less or no shares in this offering. The following table does not reflect any potential purchases by these persons or entities or their affiliated entities, nor does it give effect to any shares that may be acquired by our stockholders, directors or executive officers pursuant to the directed share program.

Unless noted otherwise, the address of all listed stockholders is c/o Dova Pharmaceuticals, Inc. 240 Leigh Farm Road, Suite 245, Durham, NC 27707.

Each of the stockholders listed has sole voting and investment power with respect to the shares beneficially owned by the stockholder unless noted otherwise, subject to community property laws where applicable.

| Name and address of beneficial owner   | Number of shares beneficially owned | Percentage of shares beneficially owned |                |
|--|-------------------------------------|---|----------------|
|  |                                     | Before offering                         | After offering |
| <b>Greater than 5% stockholders</b>  |                                     |   |                |
| PBM Capital Investments, LLC(1) . . . . .  | 17,444,080                          | 84.8%                                   | 70.8%          |
| Perceptive Life Sciences Master Fund, Ltd.(2) . . . . .                              | 1,118,264                           | 5.4                                     | 4.5            |
| <b>Directors and named executive officers</b>  |                                     |   |                |
| Alex Sapir(3) . . . . .  | 914,100                             | 4.3                                     | 3.7            |
| Doug Blankenship(4) . . . . .  | 228,525                             | 1.1                                     | *              |
| Lee F. Allen, M.D., Ph.D.(5) . . . . .   | 251,466                             | 1.2                                     | 1.0            |
| Kevin Laliberte(6) . . . . .   | 140,250                             | *                                       | *              |
| Steven M. Goldman(7) . . . . .   | 33,000                              | —                                       | *              |
| Roger A. Jeffs(7) . . . . .  | 33,000                              | —                                       | *              |
| Paul B. Manning(1) . . . . .   | 17,444,080                          | 84.8                                    | 70.8           |
| Alfred J. Novak(7) . . . . .   | 33,000                              | —                                       | *              |
| Sean Stalfort(8) . . . . .   | 604,665                             | 2.9                                     | 2.5            |
| <b>All current executive officers and directors as a group (9 persons) . . . . .</b> | <b>19,077,421</b>                   | <b>85.9</b>                             | <b>77.4</b>    |

\* Represents beneficial ownership of less than 1%.

(1) Consists of (a) 12,214,953 shares of common stock held by PBM Capital Investments, LLC, (b) 1,733,226 shares of common stock held by BKB Growth Investments, LLC, or BKB, and (c) an aggregate of 3,384,078 shares of common stock and 111,823 shares of common stock issuable upon conversion of shares of Series A preferred stock held by other Co-Investors and certain members of PBM Capital Investments, LLC, or the PBM Members. Paul B. Manning, one of our directors, has sole voting and investment power with respect to the shares held by PBM Capital Investments, LLC and BKB. Mr. Manning also has sole voting power and shared investment power with respect to shares held by the PBM Members and the Co-Investors, including the 604,665 shares of common stock held by Mr. Stalfort. Mr. Manning's voting and investment power with respect to the shares held by the PBM Members and the Co-Investors will terminate upon the completion of this offering. The business address for PBM Capital Investments, LLC and Mr. Manning is 200 Garrett Street, Suite S, Charlottesville, VA 22902.

(2) Consists of 1,118,264 shares of common stock issuable upon conversion of shares of Series A preferred stock held by Perceptive Life Sciences Master Fund, Ltd. The business address for Perceptive Life Sciences Master Fund Ltd. is 51 Astor Place, 10<sup>th</sup> Floor, New York, NY 10003. Joseph Edelman holds voting and/or dispositive power over the shares held by Perceptive Life Sciences Master Fund Ltd.

(3) Consists of 914,100 shares that may be acquired pursuant to early exercise features of options that vest in accordance with their terms. Any shares issued upon the exercise of unvested options are subject to a repurchase right in favor of us if Mr. Sapir does not satisfy the option's vesting requirements. In any event, shares acquired upon an early exercise may not be disposed of until the vesting period has been satisfied.

(4) Consists of 228,525 shares that may be acquired pursuant to early exercise features of options that vest in accordance with their terms. Any shares issued upon the exercise of unvested options are subject to a repurchase right in favor of us if Mr. Blankenship does not satisfy the option's vesting requirements. In any event, shares acquired upon an early exercise may not be disposed of until the vesting period has been satisfied.

(5) Consists of 251,466 shares that may be acquired pursuant to early exercise features of options that vest in accordance with their terms. Any shares issued upon the exercise of unvested options are subject to a repurchase right in favor of us if Dr. Allen does not satisfy the option's vesting requirements. In any event, shares acquired upon an early exercise may not be disposed of until the vesting period has been satisfied.

(6) Consists of 140,250 shares that may be acquired pursuant to early exercise features of options that vest in accordance with their terms. Any shares issued upon the exercise of unvested options are subject to a repurchase right in favor of us if Dr. Laliberte does not satisfy the option's vesting requirements. In any event, shares acquired upon an early exercise may not be disposed of until the vesting period has been satisfied.

(7) Consists of 33,000 shares that may be acquired pursuant to early exercise features of options that vest in accordance with their terms. Any shares issued upon the exercise of unvested options are subject to a repurchase right in favor of us if such holder does not satisfy the option's vesting requirements. In any event, shares acquired upon an early exercise may not be disposed of until the vesting period has been satisfied.

(8) Mr. Stalfort has entered into an agreement with us and PBM Capital Investments, LLC to assign the voting power of his shares to PBM Capital Investments, LLC. Mr. Manning has sole voting power and Mr. Stalfort and Mr. Manning share investment power with respect to these shares. The business address for Mr. Stalfort is 200 Garrett Street, Suite S, Charlottesville, VA 22902.

## Description of capital stock

*The following description of our capital stock and provisions of our amended and restated certificate of incorporation and amended and restated bylaws to be effective following the completion of this offering are summaries. You should also refer to the amended and restated certificate of incorporation and the amended and restated bylaws, which are filed as exhibits to the registration statement of which this prospectus is part.*

### General

Upon the completion of this offering, our amended and restated certificate of incorporation will authorize us to issue up to 100,000,000 shares of common stock, \$0.001 par value per share, and 10,000,000 shares of preferred stock, \$0.001 par value per share, all of which shares of preferred stock will be undesignated. Our board of directors may establish the rights and preferences of the preferred stock from time to time. As of March 31, 2017, we had outstanding 17,332,257 shares of common stock, held by 15 stockholders of record. As of March 31, 2017, after giving effect to the conversion of all of the outstanding shares of our Series A preferred stock into 3,242,950 shares of common stock, there would have been 20,575,207 shares of common stock issued and outstanding, held by 27 stockholders of record.

### Common stock

#### *Voting rights*

Each holder of our common stock is entitled to one vote for each share on all matters submitted to a vote of the stockholders, including the election of directors. Under our amended and restated certificate of incorporation and amended and restated bylaws, which will become effective upon consummation of this offering, our stockholders will not have cumulative voting rights. Because of this, the holders of a majority of the shares of common stock entitled to vote in any election of directors can elect all of the directors standing for election, if they should so choose.

#### *Dividends*

Subject to preferences that may be applicable to any then-outstanding preferred stock, holders of common stock are entitled to receive ratably those dividends, if any, as may be declared from time to time by the board of directors out of legally available funds.

#### *Liquidation*

In the event of our liquidation, dissolution or winding up, holders of common stock will be entitled to share ratably in the net assets legally available for distribution to stockholders after the payment of all of our debts and other liabilities and the satisfaction of any liquidation preference granted to the holders of any then-outstanding shares of preferred stock.

#### *Rights and preferences*

Holders of common stock have no preemptive, conversion or subscription rights and there are no redemption or sinking fund provisions applicable to the common stock. The rights, preferences and privileges of the holders of common stock are subject to, and may be adversely affected by, the right of the holders of shares of any series of preferred stock that we may designate in the future.

## **Preferred stock**

As of March 31, 2017, there were 982,714 shares of preferred stock outstanding, consisting of 982,714 shares of Series A preferred stock. All currently outstanding shares of preferred stock will convert automatically into 3,242,950 shares of common stock immediately prior to the closing of this offering.

Following the closing of this offering, our board of directors will have the authority under our amended and restated certificate of incorporation, without further action by our stockholders, to issue up to 10,000,000 shares of preferred stock in one or more series, to establish from time to time the number of shares to be included in each such series, to fix the rights, preferences and privileges of the shares of each wholly unissued series and any qualifications, limitations or restrictions thereon, and to increase or decrease the number of shares of any such series, but not below the number of shares of such series then outstanding.

Our board of directors may authorize the issuance of preferred stock with voting or conversion rights that could adversely affect the voting power or other rights of the holders of our common stock. The purpose of authorizing our board of directors to issue preferred stock and determine its rights and preferences is to eliminate delays associated with a stockholder vote on specific issuances. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions and other corporate purposes, could, among other things, have the effect of delaying, deferring or preventing a change in control of us and may adversely affect the market price of our common stock and the voting and other rights of the holders of our common stock. It is not possible to state the actual effect of the issuance of any shares of preferred stock on the rights of holders of common stock until the board of directors determines the specific rights attached to that preferred stock.

We have no present plans to issue any shares of preferred stock following completion of this offering.

## **Registration rights**

We and the beneficial owners of our common stock and Series A preferred stock have entered into the IRA. The registration rights provisions of this agreement provide those holders with demand and piggyback registration rights with respect to their shares of our common stock, including common stock issuable upon conversion of our Series A preferred stock in connection with this offering, which we refer to herein as registrable shares. After registration pursuant to these rights, such shares of common stock will become freely tradable without restriction under the Securities Act. The IRA restricts us from granting additional registration rights to any other party without the consent of a majority of the holders of registrable securities unless such additional registration rights are no more favorable than those in the IRA.

### ***Demand registration rights***

At any time beginning 180 days following the effective date of the registration statement of which this prospectus is a part, the holders of at least a majority of the registrable shares, voting as a single class, who are party to the IRA have the right to demand that we file a Form S-1 registration statement for the registration of their shares of common stock. These registration rights are subject to specified conditions and limitations, including the right of a managing underwriter to limit the number of shares included in any such registration under specified circumstances. Upon such a request, we are required to effect the registration as expeditiously as possible. An aggregate of 20,575,207 shares of common stock will be entitled to these demand registration rights upon the consummation of this offering. We are not obligated to file a registration statement pursuant to this provision on more than one occasion (unless such registration statement was not declared effective by the SEC).

### ***Piggyback registration rights***

If we propose to register any of our common stock under the Securities Act of 1933, as amended, or the Securities Act, either for our own account or for the account of other stockholders, other than pursuant to certain specified registrations (including relating to company stock option plans), the holders of registrable shares will each be entitled to notice of the registration and will be entitled to include their registrable shares in the related registration statement. These piggyback registration rights are subject to specified conditions and limitations, including the right of a managing underwriter to limit the number of shares included in any such registration under specified circumstances. An aggregate of 20,575,207 shares of common stock will be entitled to these piggyback registration rights upon the consummation of this offering.

### ***Registration on Form S-3***

At any time after we become eligible to file a registration statement on Form S-3, the holders of at least a majority of our shares of common stock have the right to demand that we file a registration statement on Form S-3, and holders of such shares will be entitled, upon their written request, to have such shares registered by us on a Form S-3 registration statement at our expense, provided that such requested registration has an anticipated aggregate offering size to the public of at least \$5.0 million, net of offering expenses, and subject to other specified conditions and limitations. We are not obligated to file a registration statement pursuant to this provision on more than one occasion in any 12-month period (unless such registration statement was not declared effective by the SEC).

In the event that any registration in which the holders of registrable shares participate pursuant to our IRA is an underwritten public offering, we agree to enter into an underwriting agreement containing customary terms for such offering.

### ***Expenses of registration***

We are required to pay all expenses, including fees and expenses of one counsel to represent the selling stockholders (up to \$75,000 total), relating to any demand, piggyback or Form S-3 registration, other than underwriting discounts and commissions, stock transfer taxes and any additional fees of counsel for the selling stockholders, subject to specified conditions and limitations. We are not required to pay registration expenses if a demand registration request is withdrawn at the request of a majority of holders of registrable securities to be registered, unless holders of a majority of the registrable securities agree to forfeit their right to one demand registration.

The IRA contains customary cross-indemnification provisions, pursuant to which we are obligated to indemnify the selling stockholders in the event of material misstatements or omissions in the applicable registration statement attributable to us, and the selling stockholders are obligated to indemnify us for material misstatements or omissions in the registration statement attributable to them, subject to certain limitations.

### ***Termination of registration rights***

The registration rights granted under the IRA will terminate upon the earlier of the fifth anniversary of the completion of this offering and a liquidation event for the Company.

## Anti-takeover provisions

### *Section 203 of the Delaware General Corporation Law*

We are subject to Section 203 of the Delaware General Corporation Law, which prohibits a Delaware corporation from engaging in any business combination with any interested stockholder for a period of three years after the date that such stockholder became an interested stockholder, with the following exceptions:

- before such date, the board of directors of the corporation approved either the business combination or the transaction that resulted in the stockholder becoming an interested stockholder;
- upon completion of the transaction that resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction began, excluding for purposes of determining the voting stock outstanding, but not the outstanding voting stock owned by the interested stockholder, those shares owned (i) by persons who are directors and also officers and (ii) employee stock plans in which employee participants do not have the right to determine confidentially whether shares held subject to the plan will be tendered in a tender or exchange offer; or
- on or after such date, the business combination is approved by the board of directors and authorized at an annual or special meeting of the stockholders, and not by written consent, by the affirmative vote of at least 66 $\frac{2}{3}$ % of the outstanding voting stock that is not owned by the interested stockholder.

In general, Section 203 defines a “business combination” to include the following:

- any merger or consolidation involving the corporation or any direct or indirect majority-owned subsidiary of the corporation and the interested stockholder;
- any sale, transfer, pledge or other disposition of 10% or more of the assets of the corporation involving the interested stockholder (in one transaction or a series of transactions);
- subject to certain exceptions, any transaction that results in the issuance or transfer by the corporation or by any direct or indirect majority-owned subsidiary of the corporation of any stock of the corporation or of such subsidiary to the interested stockholder;
- any transaction involving the corporation or any direct or indirect majority-owned subsidiary of the corporation that has the effect of increasing the proportionate share of the stock or any class or series of the corporation beneficially owned by the interested stockholder; or
- the receipt by the interested stockholder of the benefit of any loans, advances, guarantees, pledges or other financial benefits by or through the corporation.

In general, Section 203 defines an “interested stockholder” as an entity or person who, together with the person’s affiliates and associates, beneficially owns, or within three years prior to the time of determination of interested stockholder status did own, 15% or more of the outstanding voting stock of the corporation.

### *Certificate of incorporation and bylaws to be in effect upon the completion of this offering*

Our amended and restated certificate of incorporation to be in effect upon the completion of this offering, or our restated certificate, will provide for our board of directors to be divided into three classes with staggered three-year terms. Only one class of directors will be elected at each annual meeting of our



stockholders, with the other classes continuing for the remainder of their respective three-year terms. Because our stockholders do not have cumulative voting rights, stockholders holding a majority of the shares of common stock outstanding will be able to elect all of our directors. Our restated certificate and our amended and restated bylaws to be effective upon the completion of this offering, or our restated bylaws, will also provide that directors may be removed by the stockholders only for cause upon the vote of 66<sup>2</sup>/<sub>3</sub>% or more of our outstanding common stock. Furthermore, the authorized number of directors may be changed only by resolution of the board of directors, and vacancies and newly created directorships on the board of directors may, except as otherwise required by law or determined by the board, only be filled by a majority vote of the directors then serving on the board, even though less than a quorum.

Our restated certificate and restated bylaws will also provide that all stockholder actions must be effected at a duly called meeting of stockholders and will eliminate the right of stockholders to act by written consent without a meeting. Our restated bylaws will also provide that only our Chairman of the board, Chief Executive Officer or the board of directors pursuant to a resolution adopted by a majority of the total number of authorized directors may call a special meeting of stockholders.

Our restated bylaws will also provide that stockholders seeking to present proposals before a meeting of stockholders to nominate candidates for election as directors at a meeting of stockholders must provide timely advance notice in writing, and will specify requirements as to the form and content of a stockholder's notice.

Our restated certificate and restated bylaws will provide that the stockholders cannot amend many of the provisions described above except by a vote of 66<sup>2</sup>/<sub>3</sub>% or more of our outstanding common stock. As described in “—Preferred stock” above, our restated certificate will give our board of directors the authority, without further action by our stockholders, to issue up to 10,000,000 shares of preferred stock in one or more series.

The combination of these provisions will make it more difficult for our existing stockholders to replace our board of directors as well as for another party to obtain control of us by replacing our board of directors. Since our board of directors has the power to retain and discharge our officers, these provisions could also make it more difficult for existing stockholders or another party to effect a change in management. In addition, the authorization of undesignated preferred stock makes it possible for our board of directors to issue preferred stock with voting or other rights or preferences that could impede the success of any attempt to change our control.

These provisions are intended to enhance the likelihood of continued stability in the composition of our board of directors and its policies and to discourage coercive takeover practices and inadequate takeover bids. These provisions are also designed to reduce our vulnerability to hostile takeovers and to discourage certain tactics that may be used in proxy fights. However, such provisions could have the effect of discouraging others from making tender offers for our shares and may have the effect of delaying changes in our control or management. As a consequence, these provisions may also inhibit fluctuations in the market price of our stock that could result from actual or rumored takeover attempts. We believe that the benefits of these provisions, including increased protection of our potential ability to negotiate with the proponent of an unfriendly or unsolicited proposal to acquire or restructure our company, outweigh the disadvantages of discouraging takeover proposals, because negotiation of takeover proposals could result in an improvement of their terms.

## **Choice of forum**

Our restated certificate will provide that the Court of Chancery of the state of Delaware will be the exclusive forum for:

- any derivative action or proceeding brought on our behalf;
- any action asserting a breach of fiduciary duty;
- any action asserting a claim against us arising pursuant to the Delaware General Corporation Law, our restated certificate, or our amended and restated bylaws; or
- any action asserting a claim against us that is governed by the internal affairs doctrine.

The enforceability of similar choice of forum provisions in other companies' certificates of incorporation has been challenged in legal proceedings, and it is possible that, in connection with any action, a court could find the choice of forum provisions contained in our restated certificate to be inapplicable or unenforceable in such action.

## **Transfer agent and registrar**

The transfer agent and registrar for our common stock is American Stock Transfer & Trust Company. The transfer agent's address is 6201 15<sup>th</sup> Avenue, Brooklyn, NY 11219.

## **Stock exchange listing**

We have applied for listing of our common stock on the NASDAQ Global Market under the trading symbol "DOVA."

## Shares eligible for future sale

Prior to this offering, no public market existed for our common stock. Future sales of our common stock in the public market, or the availability of such shares for sale in the public market, could adversely affect market prices prevailing from time to time. As described below, only a limited number of shares will be available for sale shortly after this offering due to contractual and legal restrictions on resale. Nevertheless, sales of our common stock in the public market after such restrictions lapse, or the perception that those sales may occur, could adversely affect the prevailing market price at such time and our ability to raise equity capital in the future.

Based on the number of shares outstanding as of March 31, 2017, upon the closing of this offering and assuming no exercise of the underwriters' option to purchase additional shares, 24,637,707 shares of common stock will be outstanding, assuming no outstanding options are exercised. All of the shares of common stock sold in this offering will be freely tradable without restrictions or further registration under the Securities Act, except for any shares sold to our "affiliates," as that term is defined under Rule 144 under the Securities Act. The remaining 20,575,207 shares of common stock held by existing stockholders are "restricted securities," as that term is defined in Rule 144 under the Securities Act. Restricted securities may be sold in the public market only if registered or if their resale qualifies for exemption from registration described below under Rule 144 promulgated under the Securities Act or another available exemption.

As a result of the lock-up agreements described below and the provisions of Rules 144 and 701 under the Securities Act, the shares of common stock that will be deemed restricted securities after this offering will be available for sale in the public market as follows:

- none of the existing restricted shares will be eligible for immediate sale upon the completion of this offering; and
- 20,575,207 restricted shares will be eligible for sale in the public market upon expiration of lock-up agreements 180 days after the date of this prospectus, subject in certain circumstances to the volume, manner of sale and other limitations under Rule 144 and Rule 701 under the Securities Act, which are summarized below.

### **Rule 144**

In general, non-affiliate persons who have beneficially owned restricted shares of our common stock for at least six months, and any affiliate of the company who owns either restricted or unrestricted shares of our common stock, are entitled to sell their securities without registration with the SEC under an exemption from registration provided by Rule 144 under the Securities Act.

#### ***Non-affiliates***

Any person who is not deemed to have been one of our affiliates at the time of, or at any time during the three months preceding, a sale may sell an unlimited number of restricted securities under Rule 144 if:

- the restricted securities have been held for at least six months, including the holding period of any prior owner other than one of our affiliates (subject to certain exceptions);
- we have been subject to the Exchange Act periodic reporting requirements for at least 90 days before the sale; and

- we are current in our Exchange Act reporting at the time of sale.

Any person who is not deemed to have been an affiliate of ours at the time of, or at any time during the three months preceding, a sale and has held the restricted securities for at least one year, including the holding period of any prior owner other than one of our affiliates, will be entitled to sell an unlimited number of restricted securities without regard to the length of time we have been subject to Exchange Act periodic reporting or whether we are current in our Exchange Act reporting. Non-affiliate resales are not subject to the manner of sale, volume limitation or notice filing provisions of Rule 144.

### ***Affiliates***

Persons seeking to sell restricted securities who are our affiliates at the time of, or any time during the three months preceding, a sale, would be subject to the restrictions described above. They are also subject to additional restrictions, by which such person would be required to comply with the manner of sale and notice provisions of Rule 144 and would be entitled to sell within any three-month period only that number of securities that does not exceed the greater of either of the following:

- 1% of the number of shares of our common stock then outstanding, which will equal approximately 250,000 shares immediately after the completion of this offering based on the number of shares outstanding as of March 31, 2017; or
- the average weekly trading volume of our common stock on the stock exchange on which our shares are listed during the four calendar weeks preceding the filing of a notice on Form 144 with respect to the sale.

Additionally, persons who are our affiliates at the time of, or any time during the three months preceding, a sale may sell unrestricted securities under the requirements of Rule 144 described above, without regard to the six month holding period of Rule 144, which does not apply to sales of unrestricted securities.

### **Rule 701**

Rule 701 under the Securities Act, as in effect on the date of this prospectus, permits resales of shares in reliance upon Rule 144 but without compliance with certain restrictions of Rule 144, including the holding period requirement. Most of our employees, executive officers or directors who purchased shares under a written compensatory plan or contract may be entitled to rely on the resale provisions of Rule 701, but all holders of Rule 701 shares are required to wait until 90 days after the date of this prospectus before selling their shares. However, substantially all Rule 701 shares are subject to lock-up agreements as described below and in the section titled “Underwriting” and will become eligible for sale upon the expiration of the restrictions set forth in those agreements.

### **Form S-8 registration statements**

We intend to file one or more registration statements on Form S-8 under the Securities Act to register all shares of common stock subject to outstanding stock options and common stock issued or issuable under our 2017 Plan and IPO Plan. We expect to file the registration statement covering shares offered pursuant to our stock plans as soon as practicable after the closing of this offering, permitting the resale of such shares by non-affiliates in the public market without restriction under the Securities Act and the sale by affiliates in the public market, subject to compliance with the resale provisions of Rule 144 and expiration or release from the terms of the lock-up agreements described above.

## **Lock-up agreements**

We, our executive officers and directors and the holders of our common stock outstanding on the date of this prospectus have entered into lock-up agreements or otherwise agreed that we and they will not, subject to limited exceptions, (i) offer, pledge, announce the intention to sell, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase or otherwise dispose of, directly or indirectly, or file with the SEC a registration statement under the Securities Act relating to, any shares of our common stock or securities convertible into or exchangeable or exercisable for any shares of our common stock, or publicly disclose the intention to make any offer, sale, pledge, disposition or filing, or (ii) enter into any swap or other arrangement that transfers all or a portion of the economic consequences associated with the ownership of any shares of common stock or any such other securities (regardless of whether any of these transactions are to be settled by the delivery of shares of common stock or such other securities, in cash or otherwise), in each case without the prior written consent of J.P. Morgan Securities LLC for a period of 180 days after the date of this prospectus.

## **Registration rights**

Upon the closing of this offering, the holders of 20,575,207 shares of our common stock, including common stock issuable upon the conversion of our preferred stock, or their transferees, will be entitled to specified rights with respect to the registration of their registrable shares under the Securities Act, subject to certain limitations and the expiration, waiver or termination of the lock-up agreements. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act immediately upon effectiveness of the registration. See “Description of capital stock—Registration rights” for additional information.

## Material U.S. federal income tax consequences to non-U.S. holders

The following is a discussion of the material U.S. federal income tax considerations applicable to non-U.S. holders (as defined below) with respect to their ownership and disposition of shares of our common stock issued pursuant to this offering, but does not purport to be a complete analysis of all potential tax effects. All prospective non-U.S. holders of our common stock should consult their own tax advisors with respect to the U.S. federal income tax consequences of the purchase, ownership and disposition of our common stock, as well as any consequences arising under the U.S. estate tax or under the laws of any other taxing jurisdiction, including any state, local and non-U.S. tax consequences and any U.S. federal non-income tax consequences. In general, a non-U.S. holder means a beneficial owner of our common stock (other than a partnership or an entity or arrangement treated as a partnership for U.S. federal income tax purposes) that is not, for U.S. federal income tax purposes:

- an individual who is a citizen or resident of the United States;
- a corporation, or an entity treated as a corporation for U.S. federal income tax purposes, created or organized in the United States or under the laws of the United States or of any state thereof or the District of Columbia;
- an estate, the income of which is subject to U.S. federal income tax regardless of its source; or
- a trust if (1) a U.S. court can exercise primary supervision over the trust's administration and one or more U.S. persons have the authority to control all of the trust's substantial decisions or (2) the trust has a valid election in effect under applicable U.S. Treasury Regulations to be treated as a U.S. person.

This discussion is based on current provisions of the U.S. Internal Revenue Code of 1986, as amended, which we refer to as the Code, existing U.S. Treasury Regulations promulgated thereunder, published administrative rulings and judicial decisions, all as in effect as of the date of this prospectus supplement. These laws are subject to change and to differing interpretation, possibly with retroactive effect. Any change or differing interpretation could alter the tax consequences to non-U.S. holders described in this prospectus supplement.

This discussion is limited to non-U.S. holders that hold shares of our common stock as a capital asset within the meaning of Section 1221 of the Code (generally, for investment). This discussion does not address all aspects of U.S. federal income taxation that may be relevant to a particular non-U.S. holder in light of that non-U.S. holder's individual circumstances, nor does it address any aspects of U.S. estate or gift tax, or any state, local or non-U.S. taxes. This discussion also does not consider any specific facts or circumstances that may apply to a non-U.S. holder and does not address the special tax rules applicable to particular non-U.S. holders, such as holders that own, or are deemed to own, more than 5% of our capital stock (except to the extent specifically set forth below), corporations that accumulate earnings to avoid U.S. federal income tax, tax-exempt organizations, banks, financial institutions, insurance companies, brokers, dealers or traders in securities, commodities or currencies, tax-qualified retirement plans, holders subject to the alternative minimum tax or Medicare contribution tax, holders holding our common stock as part of a hedge, straddle or other risk reduction strategy, conversion transaction or other integrated investment, holders deemed to sell our common stock under the constructive sale provisions of the Code, controlled foreign corporations, passive foreign investment companies and U.S. expatriates and certain former citizens or long-term residents of the United States.

In addition, this discussion does not address the tax treatment of partnerships (or entities or arrangements that are treated as partnerships for U.S. federal income tax purposes) or persons that hold their common stock through such partnerships or such entities or arrangements. If a partnership, including any entity or arrangement treated as a partnership for U.S. federal income tax purposes, holds shares of our common stock, the U.S. federal income tax treatment of a partner in such partnership will generally depend upon the status of the partner, the activities of the partnership and certain determinations made at the partner level. Such partners and partnerships should consult their own tax advisors regarding the tax consequences of the purchase, ownership and disposition of our common stock.

There can be no assurance that the Internal Revenue Service, which we refer to as the IRS, will not challenge one or more of the tax consequences described herein, and we have not obtained, nor do we intend to obtain, a ruling with respect to the U.S. federal income tax consequences with respect to the matters discussed below.

### **Distributions on our common stock**

Distributions, if any, on our common stock generally will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. If a distribution exceeds our current and accumulated earnings and profits, the excess will be treated as a tax-free return of the non-U.S. holder's investment, up to such holder's adjusted tax basis in the common stock. Any remaining excess will be treated as capital gain from the sale or exchange of such common stock, subject to the tax treatment described below in "–Gain on sale, exchange or other disposition of our common stock."

Subject to the discussion below regarding backup withholding and foreign accounts, dividends paid to a non-U.S. holder will generally be subject to withholding of U.S. federal income tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty. A non-U.S. holder that is eligible for a reduced rate of U.S. withholding tax under an income tax treaty may obtain a refund or credit of any excess amounts withheld by timely filing an appropriate claim for refund with the IRS. A non-U.S. holder of our common stock who claims the benefit of an applicable income tax treaty generally will be required to provide a properly executed IRS Form W-8BEN or W-8BEN-E (or successor form) and satisfy relevant certification and other requirements. Non-U.S. holders are urged to consult their tax advisors regarding their entitlement to benefits under a relevant income tax treaty.

Dividends that are treated as effectively connected with a trade or business conducted by a non-U.S. holder within the United States and, if an applicable income tax treaty so provides, that are attributable to a permanent establishment or a fixed base maintained by the non-U.S. holder within the United States are generally exempt from the 30% withholding tax if the non-U.S. holder satisfies applicable certification and disclosure requirements. To claim the exemption, the non-U.S. holder must furnish to us or the applicable withholding agent a valid IRS Form W-8ECI (or applicable successor form), certifying that the dividends are effectively connected with the non-U.S. holder's conduct of a trade or business within the United States. However, such U.S. effectively connected income, net of specified deductions and credits, is taxed at the same graduated U.S. federal income tax rates applicable to U.S. persons (as defined in the Code). Any U.S. effectively connected income received by a non-U.S. holder that is a corporation may also, under certain circumstances, be subject to an additional "branch profits tax" at a 30% rate or such lower rate as may be specified by an applicable income tax treaty.

## **Gain on sale, exchange or other disposition of our common stock**

Subject to the discussions below regarding backup withholding and foreign accounts, in general, a non-U.S. holder will not be subject to any U.S. federal income tax on any gain realized upon such holder's sale, exchange or other disposition of shares of our common stock unless:

- the gain is effectively connected with a U.S. trade or business of the non-U.S. holder and, if an applicable income tax treaty so provides, is attributable to a permanent establishment or a fixed base maintained in the United States by such non-U.S. holder, in which case the non-U.S. holder generally will be taxed at the graduated U.S. federal income tax rates applicable to U.S. persons (as defined in the Code) and, if the non-U.S. holder is a foreign corporation, the branch profits tax described above in "Distributions on our common stock" may also apply;
- the non-U.S. holder is a nonresident alien individual who is present in the United States for 183 days or more in the taxable year of the disposition and certain other conditions are met, in which case the non-U.S. holder will be subject to a 30% tax (or such lower rate as may be specified by an applicable income tax treaty) on the net gain derived from the disposition, which may be offset by U.S. source capital losses of the non-U.S. holder, if any (even though the individual is not considered a resident of the United States), provided the non-U.S. holder has timely filed U.S. federal income tax returns with respect to such losses; or
- our common stock constitutes a U.S. real property interest because we are, or have been, at any time during the five-year period preceding such disposition (or the non-U.S. holder's holding period, if shorter) a "U.S. real property holding corporation." Even if we are or become a U.S. real property holding corporation, provided that our common stock is regularly traded on an established securities market, our common stock will be treated as a U.S. real property interest only with respect to a non-U.S. holder that holds more than 5% of our outstanding common stock, directly or indirectly, actually or constructively, during the shorter of the five-year period ending on the date of the disposition or the period that the non-U.S. holder held our common stock. In such case, such non-U.S. holder generally will be taxed on its net gain derived from the disposition at the graduated U.S. federal income tax rates applicable to U.S. persons (as defined in the Code). Generally, a corporation is a U.S. real property holding corporation only if the fair market value of its U.S. real property interests equals or exceeds 50% of the sum of the fair market value of its worldwide real property interests plus its other assets used or held for use in a trade or business. Although there can be no assurance, we do not believe that we are, or have been, a U.S. real property holding corporation, or that we are likely to become one in the future. We expect that our common stock will be regularly traded on an established securities market, but no assurance can be provided that our common stock will be regularly traded.

## **Backup withholding and information reporting**

We must report annually to the IRS and to each non-U.S. holder the gross amount of the dividends on our common stock paid to such holder and the tax withheld, if any, with respect to such dividends. Non-U.S. holders will have to comply with specific certification procedures to establish that the holder is not a U.S. person (as defined in the Code) in order to avoid backup withholding at the applicable rate with respect to dividends on our common stock. U.S. backup withholding generally will not apply to a non-U.S. holder who provides a properly executed IRS Form W-8BEN or W-8BEN-E or otherwise establishes an exemption.

Information reporting and backup withholding will generally apply to the proceeds of a disposition of our common stock by a non-U.S. holder effected by or through the U.S. office of any broker, U.S. or foreign,



unless the holder certifies its status as a non-U.S. holder and satisfies certain other requirements, or otherwise establishes an exemption. Generally, information reporting and backup withholding will not apply to a payment of disposition proceeds to a non-U.S. holder where the transaction is effected outside the United States through a non-U.S. office of a broker. However, for information reporting purposes, dispositions effected through a non-U.S. office of a broker with substantial U.S. ownership or operations generally will be treated in a manner similar to dispositions effected through a U.S. office of a broker. Non-U.S. holders should consult their own tax advisors regarding the application of the information reporting and backup withholding rules to them.

Copies of information returns may be made available to the tax authorities of the country in which the non-U.S. holder resides or is incorporated under the provisions of a specific treaty or agreement.

Backup withholding is not an additional tax. Any amounts withheld under the backup withholding rules from a payment to a non-U.S. holder may be allowed as a credit against the non-U.S. holder's U.S. federal income tax liability, if any, and may entitle such holder to a refund, provided that the required information is timely furnished to the IRS.

## **Foreign accounts**

The Code generally imposes a U.S. federal withholding tax of 30% on dividends and the gross proceeds of a disposition of our common stock paid to a "foreign financial institution" (as specifically defined for this purpose), unless such institution enters into an agreement with the U.S. government to, among other things, withhold on certain payments and to collect and provide to the U.S. tax authorities substantial information regarding U.S. account holders of such institution (which may include certain equity and debt holders of such institution, as well as certain account holders that are foreign entities with U.S. owners). Foreign financial institutions located in jurisdictions that have an intergovernmental agreement with the United States governing these withholding and reporting requirements may be subject to different rules. This U.S. federal withholding tax of 30% also applies to dividends and the gross proceeds of a disposition of our common stock paid to a non-financial foreign entity, unless such entity provides the withholding agent with either a certification that it does not have any substantial direct or indirect U.S. owners or information regarding substantial direct and indirect U.S. owners of the entity. The withholding tax described above will not apply if the foreign financial institution or non-financial foreign entity otherwise qualifies for an exemption from the rules. The withholding provisions described above currently apply to dividends on our common stock and will apply with respect to gross proceeds of a sale or other disposition of our common stock on or after January 1, 2019. Under certain circumstances, a non-U.S. holder might be eligible for refunds or credits of such taxes. Non-U.S. holders are encouraged to consult with their own tax advisors regarding the possible implications of the legislation on their investment in our common stock.

**EACH PROSPECTIVE INVESTOR SHOULD CONSULT ITS OWN TAX ADVISOR REGARDING THE TAX CONSEQUENCES OF PURCHASING, HOLDING AND DISPOSING OF OUR COMMON STOCK, INCLUDING THE CONSEQUENCES OF ANY PROPOSED CHANGE IN APPLICABLE LAW, AS WELL AS TAX CONSEQUENCES ARISING UNDER ANY STATE, LOCAL, NON-U.S. OR U.S. FEDERAL NON-INCOME TAX LAWS.**

## Underwriting

We are offering the shares of common stock described in this prospectus through a number of underwriters. J.P. Morgan Securities LLC, Jefferies LLC and Leerink Partners LLC are acting as book-running managers of the offering and as representatives of the underwriters. We have entered into an underwriting agreement with the underwriters. Subject to the terms and conditions of the underwriting agreement, we have agreed to sell to the underwriters, and each underwriter has severally agreed to purchase, at the public offering price less the underwriting discounts and commissions set forth on the cover page of this prospectus, the number of shares of common stock listed next to its name in the following table:

| Name                                 | Number of shares |
|--------------------------------------|------------------|
| J.P. Morgan Securities LLC . . . . . |                  |
| Jefferies LLC . . . . .              |                  |
| Leerink Partners LLC . . . . .       |                  |
| Total . . . . .                      | 4,062,500        |

The underwriters are committed to purchase all the common shares offered by us if they purchase any shares. The underwriting agreement also provides that if an underwriter defaults, the purchase commitments of non-defaulting underwriters may also be increased or the offering may be terminated.

The underwriters propose to offer the common shares directly to the public at the initial public offering price set forth on the cover page of this prospectus and to certain dealers at that price less a concession not in excess of \$ \_\_\_\_\_ per share. Any such dealers may resell shares to certain other brokers or dealers at a discount of up to \$ \_\_\_\_\_ per share from the initial public offering price. After the initial offering of the shares to the public, the offering price and other selling terms may be changed by the underwriters. Sales of shares made outside of the United States may be made by affiliates of the underwriters.

The underwriters have an option to buy up to 609,375 additional shares of common stock from us to cover sales of shares by the underwriters which exceed the number of shares specified in the table above. The underwriters have 30 days from the date of this prospectus to exercise this option to purchase additional shares. If any shares are purchased with this option to purchase additional shares, the underwriters will purchase shares in approximately the same proportion as shown in the table above. If any additional shares of common stock are purchased, the underwriters will offer the additional shares on the same terms as those on which the shares are being offered.

The underwriting fee is equal to the public offering price per share of common stock less the amount paid by the underwriters to us per share of common stock. The underwriting fee is \$ \_\_\_\_\_ per share. The following table shows the per share and total underwriting discounts and commissions to be paid to the

underwriters assuming both no exercise and full exercise of the underwriters' option to purchase additional shares.

|                     | Without<br>option to<br>purchase<br>additional<br>shares<br>exercise | With full<br>option to<br>purchase<br>additional<br>shares<br>exercise |
|---------------------|--|--|
| Per Share . . . . . | \$   | \$   |
| Total . . . . .     | \$   | \$   |

We estimate that the total expenses of this offering, including registration, filing and listing fees, printing fees and legal and accounting expenses, but excluding the underwriting discounts and commissions, will be approximately \$1,900,000. We have agreed to reimburse the underwriters for expenses relating to the clearance of this offering with the Financial Industry Regulatory Authority up to \$35,000.

At our request, the underwriters have reserved for sale at the initial public offering price per share up to 406,250 shares of our common stock, or 10% of the shares of common stock offered pursuant to this prospectus, to certain individuals through a directed share program, including employees, directors and other persons associated with us. If purchased by these persons, these shares will not be subject to a lock-up restriction, except in the case of shares purchased by any director or officer, which will be subject to the lock-up restrictions described below. The number of shares of common stock available for sale to the general public will be reduced by the number of reserved shares sold to these individuals. Any reserved shares not purchased by these individuals will be offered by the underwriters to the general public on the same basis as the other shares of common stock offered pursuant to this prospectus. The directed share program will be arranged through J.P. Morgan Securities LLC.

A prospectus in electronic format may be made available on the web sites maintained by one or more underwriters, or selling group members, if any, participating in the offering. The underwriters may agree to allocate a number of shares to underwriters and selling group members for sale to their online brokerage account holders. Internet distributions will be allocated by the representatives to underwriters and selling group members that may make Internet distributions on the same basis as other allocations.

We have agreed that we will not (i) offer, pledge, announce the intention to sell, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase or otherwise dispose of, directly or indirectly, or file with the SEC a registration statement under the Securities Act relating to, any shares of our common stock or securities convertible into or exchangeable or exercisable for any shares of our common stock, or publicly disclose the intention to make any offer, sale, pledge, disposition or filing, or (ii) enter into any swap or other arrangement that transfers all or a portion of the economic consequences associated with the ownership of any shares of common stock or any such other securities (regardless of whether any of these transactions are to be settled by the delivery of shares of common stock or such other securities, in cash or otherwise), in each case without the prior written consent of J.P. Morgan Securities LLC for a period of 180 days after the date of this prospectus.

We have agreed that, subject to certain conditions, the foregoing restrictions shall not apply to:

- (i) the shares of our common stock to be sold in this offering;

- (ii) any shares of our common stock issued pursuant to equity compensation plans described in this prospectus, provided that no filing under the Exchange Act or other public announcement shall be made, subject to certain exceptions;
- (iii) any options and awards granted under an equity compensation plan described in this prospectus, provided that the recipient executes a lock-up agreement for the remainder of the 180-day period referred to above;
- (iv) the filing of a registration statement on Form S-8 relating to an equity compensation plan described in this prospectus; or
- (v) up to 5% of our outstanding securities by us in connection with certain strategic transactions, including acquisitions, provided that the recipient executes a lock-up agreement for the remainder of the 180-day period referred to above.

Our directors, executive officers and stockholders have entered into lock-up agreements prior to the commencement of this offering pursuant to which each of these persons or entities for a period of 180 days after the date of this prospectus, may not, without the prior written consent of J.P. Morgan Securities LLC, (1) offer, pledge, announce the intention to sell, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, any shares of our common stock or any securities convertible into or exercisable or exchangeable for our common stock (including, without limitation, common stock or such other securities which may be deemed to be beneficially owned by such directors, executive officers and stockholders in accordance with the rules and regulations of the SEC and securities which may be issued upon exercise of a stock option or warrant) or (2) enter into any swap or other agreement that transfers, in whole or in part, any of the economic consequences of ownership of the common stock or such other securities, whether any such transaction described in clause (1) or (2) above is to be settled by delivery of common stock or such other securities, in cash or otherwise, or (3) make any demand for or exercise any right with respect to the registration of any shares of our common stock or any security convertible into or exercisable or exchangeable for our common stock.

Each such director, executive officer and stockholder has agreed that, subject to certain conditions, the foregoing restrictions shall not apply to any shares of our common stock purchased in this offering or in the public market following this offering, or any transfers of shares of our common stock:

- (i) as a bona fide gift or gifts or by will, testamentary document or intestate succession;
- (ii) to any trust for the direct or indirect benefit of such directors, executive officers and stockholders or their immediate family;
- (iii) to partners, members, stockholders or trust beneficiaries of such directors, executive officers and stockholders;
- (iv) in the event such stockholder is a corporation, partnership, limited liability company, trust or other business entity, to any direct or indirect affiliate of such stockholder or any investment fund or other entity controlled or managed by such stockholder or any investment fund or other entity that controls such stockholder;
- (v) by operation of law;

(vi) to the Company pursuant to agreements under which the Company has the option to repurchase such shares or a right of first refusal with respect to transfers of such shares upon termination of service of the locked-up party;

(vii) pursuant to the conversion of outstanding shares of preferred stock of the Company outstanding as of the date of this registration statement into shares of common stock of the Company, provided that the shares of common stock received upon conversion will be subject to the same lock-up restrictions; or

(viii) pursuant to a bona fide third-party tender offer, merger, consolidation or other similar transaction made to all holders of the Company's securities involving a change of control of the Company, provided, that in the event that such tender offer, merger, consolidation or other such transaction is not completed, such securities held by such directors, executive officers and stockholders will remain subject to the lock-up provisions.

We have agreed to indemnify the underwriters against certain liabilities, including liabilities under the Securities Act.

We have applied to have our common stock approved for listing on The NASDAQ Global Market under the symbol "DOVA."

In connection with this offering, the underwriters may engage in stabilizing transactions, which involves making bids for, purchasing and selling shares of common stock in the open market for the purpose of preventing or retarding a decline in the market price of the common stock while this offering is in progress. These stabilizing transactions may include making short sales of the common stock, which involves the sale by the underwriters of a greater number of shares of common stock than they are required to purchase in this offering, and purchasing shares of common stock on the open market to cover positions created by short sales. Short sales may be "covered" shorts, which are short positions in an amount not greater than the underwriters' option to purchase additional shares referred to above, or may be "naked" shorts, which are short positions in excess of that amount. The underwriters may close out any covered short position either by exercising their option to purchase additional shares, in whole or in part, or by purchasing shares in the open market. In making this determination, the underwriters will consider, among other things, the price of shares available for purchase in the open market compared to the price at which the underwriters may purchase shares through the option to purchase additional shares. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market that could adversely affect investors who purchase in this offering. To the extent that the underwriters create a naked short position, they will purchase shares in the open market to cover the position.

The underwriters have advised us that, pursuant to Regulation M of the Securities Act, they may also engage in other activities that stabilize, maintain or otherwise affect the price of the common stock, including the imposition of penalty bids. This means that if the representatives of the underwriters purchase common stock in the open market in stabilizing transactions or to cover short sales, the representatives can require the underwriters that sold those shares as part of this offering to repay the underwriting discount received by them.

These activities may have the effect of raising or maintaining the market price of the common stock or preventing or retarding a decline in the market price of the common stock, and, as a result, the price of the common stock may be higher than the price that otherwise might exist in the open market. If the underwriters commence these activities, they may discontinue them at any time. The underwriters may carry out these transactions on The NASDAQ Global Market, in the over-the-counter market or otherwise.

Prior to this offering, there has been no public market for our common stock. The initial public offering price will be determined by negotiations between us and the representatives of the underwriters. In determining the initial public offering price, we and the representatives of the underwriters expect to consider a number of factors including:

- the information set forth in this prospectus and otherwise available to the representatives;
- our prospects and the history and prospects for the industry in which we compete;
- an assessment of our management;
- our prospects for future earnings;
- the general condition of the securities markets at the time of this offering;
- the recent market prices of, and demand for, publicly traded common stock of generally comparable companies; and
- other factors deemed relevant by the underwriters and us.

Neither we nor the underwriters can assure investors that an active trading market will develop for our common shares, or that the shares will trade in the public market at or above the initial public offering price.

## **Selling restrictions**

### ***General***

Other than in the United States, no action has been taken by us or the underwriters that would permit a public offering of the securities offered by this prospectus in any jurisdiction where action for that purpose is required. The securities offered by this prospectus may not be offered or sold, directly or indirectly, nor may this prospectus or any other offering material or advertisements in connection with the offer and sale of any such securities be distributed or published in any jurisdiction, except under circumstances that will result in compliance with the applicable rules and regulations of that jurisdiction. Persons into whose possession this prospectus comes are advised to inform themselves about and to observe any restrictions relating to the offering and the distribution of this prospectus. This prospectus does not constitute an offer to sell or a solicitation of an offer to buy any securities offered by this prospectus in any jurisdiction in which such an offer or a solicitation is unlawful.

### ***Notice to prospective investors in Canada***

The shares may be sold only to purchasers purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 Prospectus Exemptions or subsection 73.3(1) of the Securities Act (Ontario), and are permitted clients, as defined in National Instrument 31-103 Registration Requirements, Exemptions and Ongoing Registrant Obligations. Any resale of the shares must be made in accordance with an exemption from, or in a transaction not subject to, the prospectus requirements of applicable securities laws.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this prospectus (including any amendment thereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser's province or territory. The

purchaser should refer to any applicable provisions of the securities legislation of the purchaser's province or territory for particulars of these rights or consult with a legal advisor.

Pursuant to section 3A.3 of National Instrument 33-105 Underwriting Conflicts (NI 33-105), the underwriters are not required to comply with the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with this offering.

***Notice to prospective investors in the European Economic Area***

In relation to each Member State of the European Economic Area (each, a "Relevant Member State"), no offer of shares may be made to the public in that Relevant Member State other than:

- A. to any legal entity which is a qualified investor as defined in the Prospectus Directive;
- B. to fewer than 150 natural or legal persons (other than qualified investors as defined in the Prospectus Directive), as permitted under the Prospectus Directive, subject to obtaining the prior consent of the representatives; or
- C. in any other circumstances falling within Article 3(2) of the Prospectus Directive, provided that no such offer of shares shall require the Company or the representatives to publish a prospectus pursuant to Article 3 of the Prospectus Directive or supplement a prospectus pursuant to Article 16 of the Prospectus Directive.

Each person in a Relevant Member State who initially acquires any shares or to whom any offer is made will be deemed to have represented, acknowledged and agreed to and with each of the representatives and the Company that it is a "qualified investor" within the meaning of the law in that Relevant Member State implementing Article 2(1)(e) of the Prospectus Directive. In the case of any shares being offered to a financial intermediary as that term is used in Article 3(2) of the Prospectus Directive, each such financial intermediary will be deemed to have represented, acknowledged and agreed that the shares acquired by it in the offer have not been acquired on a non-discretionary basis on behalf of, nor have they been acquired with a view to their offer or resale to, persons in circumstances which may give rise to an offer of any shares to the public other than their offer or resale in a Relevant Member State to qualified investors as so defined or in circumstances in which the prior consent of the representatives has been obtained to each such proposed offer or resale.

For the purposes of this provision, the expression an "offer of shares to the public" in relation to any shares in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and the shares to be offered so as to enable an investor to decide to purchase or subscribe the shares, as the same may be varied in the Relevant Member State by any measure implementing the Prospectus Directive in the Relevant Member State and the expression "Prospectus Directive" means Directive 2003/71/EC (as amended by Directive 2010/73/EU), and includes any relevant implementing measure in the Relevant Member State.

***Notice to prospective investors in the United Kingdom***

In addition, in the United Kingdom, this document is being distributed only to, and is directed only at, and any offer subsequently made may only be directed at persons who are "qualified investors" (as defined in the Prospectus Directive) (i) who have professional experience in matters relating to investments falling within Article 19 (5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005, as amended (the "Order") and/or (ii) who are high net worth companies (or persons to whom it may otherwise be lawfully communicated) falling within Article 49(2)(a) to (d) of the Order (all such persons

together being referred to as “relevant persons”) or otherwise in circumstances which have not resulted and will not result in an offer to the public of the shares in the United Kingdom within the meaning of the Financial Services and Markets Act 2000.

Any person in the United Kingdom that is not a relevant person should not act or rely on the information included in this document or use it as basis for taking any action. In the United Kingdom, any investment or investment activity that this document relates to may be made or taken exclusively by relevant persons.

***Notice to prospective investors in Hong Kong***

The shares have not been offered or sold and will not be offered or sold in Hong Kong, by means of any document, other than (a) to “professional investors” as defined in the Securities and Futures Ordinance (Cap. 571) of Hong Kong and any rules made under that Ordinance; or (b) in other circumstances which do not result in the document being a “prospectus” as defined in the Companies (Winding Up and Miscellaneous Provisions) Ordinance (Cap. 32) of Hong Kong or which do not constitute an offer to the public within the meaning of that Ordinance. No advertisement, invitation or document relating to the shares has been or may be issued or has been or may be in the possession of any person for the purposes of issue, whether in Hong Kong or elsewhere, which is directed at, or the contents of which are likely to be accessed or read by, the public of Hong Kong (except if permitted to do so under the securities laws of Hong Kong) other than with respect to shares which are or are intended to be disposed of only to persons outside Hong Kong or only to “professional investors” as defined in the Securities and Futures Ordinance and any rules made under that Ordinance.

***Notice to prospective investors in Singapore***

This prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of shares may not be circulated or distributed, nor may the shares be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to persons in Singapore other than (i) to an institutional investor under Section 274 of the Securities and Futures Act, Chapter 289 of Singapore (the “SFA”), (ii) to a relevant person pursuant to Section 275(1), or any person pursuant to Section 275(1A), and in accordance with the conditions, specified in Section 275 of the SFA or (iii) otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA.

Where the shares are subscribed or purchased under Section 275 of the SFA by a relevant person which is:

- (a) a corporation (which is not an accredited investor (as defined in Section 4A of the SFA)) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor; or
- (b) a trust (where the trustee is not an accredited investor) whose sole purpose is to hold investments and each beneficiary of the trust is an individual who is an accredited investor,

securities (as defined in Section 239(1) of the SFA) of that corporation or the beneficiaries’ rights and interest (howsoever described) in that trust shall not be transferred within six months after that corporation or that trust has acquired the shares pursuant to an offer made under Section 275 of the SFA except:

- (a) to an institutional investor or to a relevant person defined in Section 275(2) of the SFA, or to any person arising from an offer referred to in Section 275(1A) or Section 276(4)(i)(B) of the SFA;



- (b) where no consideration is or will be given for the transfer;
- (c) where the transfer is by operation of law;
- (d) as specified in Section 276(7) of the SFA; or
- (e) as specified in Regulation 32 of the Securities and Futures (Offers of Investments) (Shares and Debentures) Regulations 2005 of Singapore

***Notice to prospective investors in Japan***

The shares have not been and will not be registered pursuant to Article 4, Paragraph 1 of the Financial Instruments and Exchange Act. Accordingly, none of the shares nor any interest therein may be offered or sold, directly or indirectly, in Japan or to, or for the benefit of, any resident of Japan (which term, as used herein, means any person resident in Japan, including any corporation or other entity organized under the laws of Japan), or to others for re-offering or resale, directly or indirectly, in Japan or to or for the benefit of a resident of Japan, except pursuant to an exemption from the registration requirements of, and otherwise in compliance with, the Financial Instruments and Exchange Act and any other applicable laws, regulations and ministerial guidelines of Japan in effect at the relevant time.

**Other relationships**

Certain of the underwriters and their affiliates have provided in the past to us and our affiliates and may provide from time to time in the future certain commercial banking, financial advisory, investment banking and other services for us and such affiliates in the ordinary course of their business, for which they have received and may continue to receive customary fees and commissions. For example, Jefferies LLC acted as financial advisor in connection with our sale of Series A preferred stock. In addition, from time to time, certain of the underwriters and their affiliates may effect transactions for their own account or the account of customers, and hold on behalf of themselves or their customers, long or short positions in our debt or equity securities or loans, and may do so in the future.

## Legal matters

The validity of the shares of common stock offered hereby will be passed upon for us by Cooley LLP, New York, New York. As of the date of this prospectus, a partner of Cooley LLP beneficially owns an aggregate of 13,668 shares of our common stock. Certain legal matters will be passed upon for the underwriters by Davis Polk & Wardwell LLP.

## Experts

The consolidated financial statements of Dova Pharmaceuticals, Inc. as of December 31, 2016, and for the period from March 24, 2016 (inception) through December 31, 2016 have been included herein and in the registration statement in reliance on the report of KPMG LLP, independent registered public accounting firm, appearing elsewhere herein, and upon the authority of said firm as experts in accounting and auditing.

The audit report covering the December 31, 2016 consolidated financial statements contains an explanatory paragraph that states we have suffered recurring losses from operations that raises substantial doubt about our ability to continue as a going concern. The consolidated financial statements do not include any adjustments that might result from the outcome of that uncertainty.

## Where you can find more information

We have filed with the SEC a registration statement on Form S-1 under the Securities Act, with respect to the shares of common stock being offered by this prospectus. This prospectus, which constitutes part of the registration statement, does not contain all of the information in the registration statement and its exhibits. For further information with respect to our company and the common stock offered by this prospectus, we refer you to the registration statement and its exhibits. Statements contained in this prospectus as to the contents of any contract or any other document referred to are not necessarily complete, and in each instance, we refer you to the copy of the contract or other document filed as an exhibit to the registration statement. Each of these statements is qualified in all respects by this reference.

You can read our SEC filings, including the registration statement, over the internet at the SEC's website at [www.sec.gov](http://www.sec.gov). You may also read and copy any document we file with the SEC at its public reference room at 100 F Street, N.E., Room 1580, Washington, D.C. 20549. You may also obtain copies of these documents at prescribed rates by writing to the Public Reference Section of the SEC at 100 F Street, N.E., Washington, D.C. 20549. Please call the SEC at 1-800-SEC-0330 for further information on the operation of the public reference facilities.

Upon completion of this offering, we will be subject to the information reporting requirements of the Exchange Act, and we will file reports, proxy statements and other information with the SEC. These reports, proxy statements and other information will be available for inspection and copying at the public reference room and website of the SEC referred to above. We also maintain a website at [www.dova.com](http://www.dova.com), at which you may access these materials free of charge as soon as reasonably practicable after they are electronically filed with, or furnished to, the SEC. The information contained in, or that can be accessed through, our website is not part of, and is not incorporated into, this prospectus.

# Dova Pharmaceuticals, Inc.

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## Report of independent registered public accounting firm

The Board of Directors  
Dova Pharmaceuticals, Inc.:

We have audited the accompanying consolidated balance sheet of Dova Pharmaceuticals, Inc. and subsidiary (the Company) as of December 31, 2016, and the related consolidated statements of operations, stockholders' equity, and cash flows for the period from March 24, 2016 (inception) to December 31, 2016. These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these consolidated financial statements based on our audit.

We conducted our audit in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements. An audit also includes assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall financial statement presentation. We believe that our audit provides a reasonable basis for our opinion.

In our opinion, the consolidated financial statements referred to above present fairly, in all material respects, the financial position of Dova Pharmaceuticals, Inc. and subsidiary as of December 31, 2016, and the results of their operations and their cash flows for the period from March 24, 2016 (inception) to December 31, 2016 in conformity with U.S. generally accepted accounting principles.

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 1 to the consolidated financial statements, the Company has suffered recurring losses from operations that raises substantial doubt about the Company's ability to continue as a going concern. Management's plans in regard to these matters are also described in Note 1. The consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty. Our opinion is not modified with respect to this matter.

/s/ KPMG LLP

Richmond, Virginia

April 21, 2017

Except as to Note 8, which is as of  
June 16, 2017

**Dova Pharmaceuticals, Inc.**  
**Consolidated balance sheet**  
(in thousands, except share and per share amounts)

|  | December 31,<br>2016   | Pro forma<br>liabilities and<br>stockholders'<br>equity<br>December 31,<br>2016<br><br>(unaudited) |
|--|------------------------|--|
| <b>ASSETS</b>  |                        |  |
| Current assets   |                        |  |
| Cash and cash equivalents . . . . .  | \$ 28,709              | \$ 28,709  |
| Prepaid expenses . . . . .   | 37                     | 37   |
| Total current assets . . . . .   | <u>28,746</u>          | <u>28,746</u>  |
| <b>Total assets . . . . .</b>  | <b><u>\$28,746</u></b> | <b><u>\$28,746</u></b>   |
| <b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>  |                        |  |
| Current liabilities  |                        |  |
| Accounts payable . . . . .   | \$ 157                 | \$ 157   |
| Accrued expenses . . . . .   | 7,918                  | 7,918  |
| Accrued interest . . . . .   | 151                    | 151  |
| Due to related party . . . . .   | 85                     | 85   |
| Total current liabilities . . . . .  | <u>8,311</u>           | <u>8,311</u>   |
| Note payable, long-term . . . . .  | <u>13,640</u>          | <u>13,640</u>  |
| <b>Total liabilities . . . . .</b>   | <b><u>21,951</u></b>   | <b><u>21,951</u></b>   |
| Commitments and contingencies  |                        |  |
| Stockholders' equity   |                        |  |
| Series A preferred stock, \$0.001 par value; 1,400,000 shares authorized;<br>982,714 shares issued and outstanding as of December 31, 2016 . . . . . | 1                      | —  |
| Common stock, \$0.001 par value; 22,110,000 shares authorized;<br>17,332,257 shares issued and outstanding as of December 31, 2016 . . . . .         | 17                     | 21   |
| Additional paid-in capital . . . . .   | 33,967                 | 33,963   |
| Accumulated deficit . . . . .  | <u>(27,190)</u>        | <u>(27,190)</u>  |
| Total stockholders' equity . . . . .   | <u>6,795</u>           | <u>6,795</u>   |
| <b>Total liabilities and stockholders' equity . . . . .</b>  | <b><u>\$28,746</u></b> | <b><u>\$28,746</u></b>   |

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

**Dova Pharmaceuticals, Inc.**  
**Consolidated statement of operations**  
(in thousands, except share and per share amounts)

|  | For the period from<br>March 24, 2016<br>(Inception) to<br>December 31, 2016 |
|--|--|
| <b>Operating expenses:</b>   |  |
| Research and development . . . . .   | \$ 20,842  |
| Research and development—licenses acquired . . . . .                               | 5,000  |
| General and administrative . . . . .   | 1,201  |
| Total operating expenses . . . . .   | <u>27,043</u>  |
| <b>Loss from operations . . . . .</b>  | <b><u>(27,043)</u></b>   |
| <b>Other income (expenses)</b>   |  |
| Other income, net . . . . .  | 9  |
| Interest expense—related party . . . . .   | (4)  |
| Interest expense . . . . .   | (152)  |
| Total other expenses, net . . . . .  | <u>(147)</u>   |
| <b>Net loss . . . . .</b>  | <b><u>\$ (27,190)</u></b>  |
| Net loss per share, basic and diluted . . . . .                                    | <u>\$ (1.57)</u>   |
| Weighted average common shares outstanding, basic and diluted . . . . .            | <u>17,332,257</u>  |
| Pro forma net loss per share, basic and diluted, unaudited . . . . .               | <u>\$ (1.32)</u>   |
| Pro forma weighted average common shares outstanding, basic and diluted, unaudited | <u>20,575,207</u>  |

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

**Dova Pharmaceuticals, Inc.**  
**Consolidated statement of stockholders' equity**  
(in thousands, except share amounts)

|   | Member units |            | Series A preferred stock |             | Common stock      |             | Additional paid-in capital | Accumulated deficit | Total stockholders' equity |
|---|--------------|------------|--------------------------|-------------|-------------------|-------------|----------------------------|---------------------|----------------------------|
|   | Shares       | Amount     | Shares                   | Amount      | Shares            | Amount      |                            |                     |                            |
| <b>Balance as of March 24, 2016</b>   |              |            |                          |             |                   |             |                            |                     |                            |
| (Inception) . . . . .   | —            | \$—        | —                        | \$—         | —                 | \$—         | \$ —                       | \$ —                | \$ —                       |
| Capital contributions—PBM   |              |            |                          |             |                   |             |                            |                     |                            |
| Capital . . . . .   | 52,522       | —          | —                        | —           | —                 | —           | 696                        | —                   | 696                        |
| Capital contribution—PBM Capital Investments, LLC—payment of AkaRx upfront purchase price . . . . . | —            | —          | —                        | —           | —                 | —           | 5,000                      | —                   | 5,000                      |
| Conversion from LLC to Corporation . . . . .  | (52,522)     | —          | —                        | —           | 17,332,257        | 17          | (17)                       | —                   | —                          |
| Issuance of Series A preferred stock for cash, net of cost of \$711 . . . . .                       | —            | —          | 982,714                  | 1           | —                 | —           | 28,288                     | —                   | 28,289                     |
| Net loss . . . . .  | —            | —          | —                        | —           | —                 | —           | —                          | (27,190)            | (27,190)                   |
| <b>Balance as of December 31, 2016 . . . . .</b>  | <b>—</b>     | <b>\$—</b> | <b>982,714</b>           | <b>\$ 1</b> | <b>17,332,257</b> | <b>\$17</b> | <b>\$33,967</b>            | <b>\$(27,190)</b>   | <b>\$ 6,795</b>            |

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

**Dova Pharmaceuticals, Inc.**  
**Consolidated statement of cash flows**  
(in thousands)

|  | For the period from<br>March 24, 2016<br>(inception) to<br>December 31, 2016 |
|--|--|
| <b>Cash flows from operating activities</b>  |  |
| Net loss . . . . .   | \$(27,190)   |
| Adjustments to reconcile net loss to net cash used in operating activities:                            |  |
| Research and development-licenses acquired, expensed . . . . .   | 5,000  |
| Research and development expenses under Eisai Note . . . . .   | 13,640   |
| Changes in operating assets and liabilities:   |  |
| Prepaid expenses . . . . .   | (37)   |
| Accounts payable . . . . .   | 157  |
| Accrued expenses . . . . .   | 7,209  |
| Accrued interest . . . . .   | 151  |
| Due to related party . . . . .   | 83   |
| Net cash used in operating activities . . . . .  | (987)  |
| <b>Cash flows from financing activities</b>  |  |
| Proceeds from issuance of Series A preferred stock . . . . .   | 29,000   |
| Capital contribution—PBM Capital . . . . .   | 696  |
| Net cash provided by financing activities . . . . .  | 29,696   |
| Net increase in cash and cash equivalents . . . . .  | 28,709   |
| Cash and cash equivalents at the beginning of the period . . . . .                                     | —  |
| <b>Cash and cash equivalents at the end of the period . . . . .</b>                                    | <b>\$28,709</b>  |
| <b>Supplemental disclosure of cash flow information:</b>   |  |
| Cash paid for interest . . . . .   | \$ 3   |
| <b>Supplemental disclosure of noncash investing and financing activities:</b>                          |  |
| Issuance of Series A preferred stock—unpaid offering cost . . . . .                                    | \$ 709   |
| Issuance of Series A preferred stock—offering cost paid by PBM Capital . . . . .                       | \$ 2   |
| Conversion from LLC to Corporation . . . . .   | \$ 5   |
| Research and development expenses under Eisai Note . . . . .   | \$ 13,640  |
| Capital contribution—PBM Capital Investments, LLC—payment of AkaRx upfront<br>purchase price . . . . . | \$ 5,000   |

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



# Dova Pharmaceuticals, Inc.

## Notes to consolidated financial statements

### 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 undergoing a non-emergent minimally to moderately invasive medical procedure. 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. AkaRx is the Company’s only subsidiary.

The consolidated financial statements of Dova and its wholly owned subsidiary AkaRx (the “Company”) include the results of operations from Inception through December 31, 2016.

#### *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 December 31, 2016, the Company had an accumulated deficit of \$27.2 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 \$29.0 million (at a purchase price of \$29.51 per share).

The Company expects to use the proceeds from the above transaction primarily for general corporate purposes, which may include financing the Company’s growth, conducting clinical trials for additional indications for avatrombopag, regulatory filings for avatrombopag, preparing for commercialization of avatrombopag, if approved, developing new or existing drug candidates, making payments on the Eisai Note and funding capital expenditures, acquisitions and investments.

The accompanying financial statements have been prepared on a going concern basis, which contemplates the realization of assets and satisfaction of liabilities in the normal course of business. The financial statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might result from the outcome of this uncertainty. The Company anticipates incurring additional losses until such time, if ever, that it can obtain marketing approval to sell, and then generate significant sales, of its drug candidate that is currently in development. Substantial additional financing will be needed by the Company to fund its operations and to develop and commercialize its drug candidate. These factors raise substantial doubt about the Company's ability to continue as a going concern.

The Company will seek to obtain additional capital through the sale of debt or equity financings or other arrangements to fund operations; however, there can be no assurance that the Company 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. Issued debt securities may contain covenants and limit the Company's ability to pay dividends or make other distributions to stockholders. If the Company is unable to obtain such additional financing, future operations would need to be scaled back or discontinued.

## **Note 2—Significant accounting policies**

### ***Basis of presentation and principles of consolidation***

The Company's 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.

### ***Unaudited Pro Forma Information***

The unaudited pro forma balance sheet data as of December 31, 2016 gives effect to the automatic conversion of all outstanding shares of the Company's preferred stock on an 3.3-for-one basis into an aggregate of 3,242,950 shares of common stock, which will occur immediately prior to the Company's planned initial public offering. The unaudited pro forma basic and diluted net loss per share for the period from March 24, 2016 to December 31, 2016 gives effect to such automatic conversion as if it had occurred as of the beginning of the period.

### ***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 consolidated financial statements and the reported amounts of expenses during the reporting period. The most significant estimates in the Company's consolidated financial statements relate to the valuation of preferred and common stock 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.

### ***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 unpaid service fees and out-of-pocket costs due under the TSA. Once such expenses 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 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.

### ***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 notes 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.
- 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 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 over the requisite service period based on the estimated grant-date fair value of the awards. For stock-based compensation awards to non-employees, the Company re-measures the fair value of the non-employee awards at each reporting period prior to vesting and finally at the vesting date of the award. Changes in the estimated fair value of these non-employee awards are recognized as compensation expense in the period of change. 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. For the period from March 24, 2016 to December 31, 2016, the Company did not pay any employee compensation or issue any stock-based compensation to any employee, director or consultant. At December 31, 2016, no stock options were authorized.

#### ***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 December 31, 2016.

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 period ended December 31, 2016 did not include the 982,714 shares of Series A preferred stock as the inclusion of these securities would have been antidilutive.

#### ***Recent accounting pronouncements***

In August 2014, the Financial Accounting Standards Board ("FASB") issued ASU No. 2014-15, *Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern*, that requires management to evaluate whether there are conditions and events that raise substantial doubt about the Company's ability to continue as a going concern within one year after the financial statements are issued or available to be issued on both an interim and annual basis. Management is required to provide certain footnote disclosures if it concludes that substantial doubt exists or when its plans alleviate substantial doubt about the Company's ability to continue as a going concern. The Company adopted ASU 2014-15 in 2016, and has disclosed the results of its evaluation in Note 1.

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. In connection with the adoption of this ASU, the Company elected to account for forfeitures as they occur. Other provisions of ASU 2016-09 had no impact on the Company's consolidated financial statements.

In January 2017, the FASB issued ASU No. 2017-01, "*Business Combinations (Topic 805) Clarifying the Definition of a Business*". The amendments in this ASU clarify the definition of a business with the objective of adding guidance to assist entities with evaluating whether transactions should be accounted for as acquisitions (or disposals) of assets or businesses. The definition of a business affects many areas of accounting including acquisitions, disposals, goodwill, and consolidation. The guidance is effective for annual periods beginning after December 15, 2017, including interim periods within those periods. Early adoption is permitted, including for interim or annual periods for which the financial statements have not been issued or made available for issuance. The Company adopted this guidance upon its Inception in 2016. See Note 3 regarding the adoption of ASU 2017-01.

## **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 include a license to avatrombopag, other associated intellectual property, inventory, documentation and records, and related materials. Because avatrombopag has not yet received regulatory approval, the \$5.0 million purchase price paid to date for these assets has been expensed in the Company's statement of operations for the period ended December 31, 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 December 31, 2016.

### ***Transition services agreement***

Pursuant to the terms and conditions of the TSA, Eisai has 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 a specified amount 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 December 31, 2016, the Company had outstanding borrowings of \$13.6 million under the Note and the Company owed Eisai \$0.2 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 are 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 will be required to make aggregate milestone payments of up to \$5.0 million to Astellas if certain 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.

### **Note 4—Related party agreements**

#### ***Dova and AkaRx 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 period ended December 31, 2016, the Company incurred aggregate expenses under the SAs of \$450,000, which were included in general and administrative expenses.

From April 1, 2016 to September 15, 2016, PBM Capital and its affiliates advanced approximately \$634,000 to the Company. The Company re-paid the advance including approximately \$3,000 of accrued interest on September 15, 2016. Interest accrued at a rate of 3% per annum.

As of December 31, 2016, the Company owed PBM Capital Group, LLC and its affiliates approximately \$85,000.

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 2,522.20 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.

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, has sole voting and dispositive power over all outstanding shares of the Company's common stock.

At December 31, 2016, the Company was authorized to issue 22,110,000 shares of common stock with a par value of \$0.001 per share; 17,332,257 shares were issued and outstanding.

### ***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 pays 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 Company's board of directors.

In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Company or a Deemed Liquidation Event (certain mergers, consolidations, reorganizations or recapitalizations, or the sale, lease, transfer, exclusive license or other disposition of all or substantially all the assets of the Company), the holders of shares of Series A preferred stock will be entitled to be paid out of the assets of the Company available for distribution to its stockholders before any payment shall be made to the holders of common stock. The amount payable per share to the holders of the Series A preferred stock will be equal to the greater of (i) one and a half times the Series A Original Issue Price, plus any dividends declared but unpaid, or (ii) such amount per share as would have been payable had all shares of Series A preferred stock been converted into common stock immediately prior to such liquidation, dissolution, winding up or Deemed Liquidation Event.

Each holder of outstanding shares of Series A preferred stock is entitled to cast the number of votes equal to the number of whole shares of common stock into which the shares of Series A preferred stock held are convertible as of the record date for determining stockholders entitled to vote on a matter. Holders of the Series A preferred stock vote together with the holders of common stock as a single class.

The holders of Series A preferred stock, voting as a separate class, are entitled to elect by majority vote (with each share of Series A preferred stock entitled to one vote) one individual to the Company's board of directors. The Series A preferred stockholders also have certain protective rights.

Each share of Series A preferred stock is 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 is mandatorily convertible under certain conditions (i) when the Company issues shares of common stock in a public offering generating gross proceeds of at least \$60.0 million to the Company, at a 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 is \$8.94, and is 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. In the event the Company issues additional shares of common stock for no consideration or for consideration per share less than the Series A preferred stock



conversion price then in effect, the conversion price is reduced based on a weighted average anti-dilution formula.

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

Further, the Series A preferred stock is 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 is not clearly and closely related to the associated preferred stock host instrument but does not meet the definition of a derivative and therefore was not bifurcated from the equity host.

At December 31, 2016, the Company was authorized to issue 1,400,000 preferred shares with a par value of \$0.001 per share and 982,714 shares of preferred stock were issued and outstanding.

## Note 6—Income taxes

A reconciliation of the statutory U.S. federal rate to the Company's effective tax rate is as follows:

|  | 2016  |
|--|-------|
| Statutory federal tax rate . . . . .                   | 35%   |
| Income not subject to corporate income taxes . . . . . | (7)%  |
| Change in valuation allowance . . . . .                | (28)% |
| Income Tax Provision/(Benefit) . . . . .               | —%    |

The components of the net deferred tax asset as of December 31, 2016 are as follows (in thousands):

|  | 2016     |
|--|----------|
| Deferred tax assets:                                     |          |
| Net operating loss carryforwards . . . . .               | \$ 8,983 |
| Amortization of intangible assets . . . . .              | 333      |
| Charitable Contributions . . . . .                       | 4        |
| Total Deferred tax assets . . . . .                      | 9,320    |
| Less valuation allowance . . . . .                       | (9,320)  |
| Deferred tax asset, net of valuation allowance . . . . . | \$ —     |

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 December 31, 2016.

The Company has determined, based upon available evidence, that it is more likely than not that the net deferred tax asset will not be realized and, accordingly, has provided a full valuation allowance against its net deferred tax asset. A valuation allowance of approximately \$9.3 million was recorded for the period ended December 31, 2016.

As of December 31, 2016, the Company had federal and state net operating loss carryforwards of approximately \$21.9 million. The federal and state net operating loss carryforwards will begin to expire, if not utilized, by 2036. Future changes in ownership may limit the utilization of the net operating loss carryforward due to the ownership change limitations provided by Section 382 of the Internal Revenue Code of 1986, as amended, and similar provisions. Additionally, the Company may be entitled to claim federal and state income tax credits for its research and development activities (“R&D Credits”) occurring during 2016 which have not yet been determined. Any R&D Credits generated by the Company in 2016 would result in an additional deferred tax asset that would be subject to a full valuation allowance.

At December 31, 2016, the Company did not have any significant uncertain tax positions. The Company will recognize interest and penalties related to uncertain tax positions in income tax expense. As of December 31, 2016, the Company had no accrued interest or penalties related to uncertain tax positions and no amounts have been recognized in the Company’s consolidated statement of operations. The Company does not anticipate a material change to unrecognized tax benefits in the next twelve months.

The 2013 and subsequent federal and state tax years for AkaRx remain open for the assessment of income taxes. Dova’s initial tax year was 2016, which remains open for the assessment of income taxes.

## **Note 7—Commitments and contingencies**

### *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.

## **Note 8—Subsequent events**

### *Option grants*

In March 2017, the Company’s board of directors approved an increase in the number of shares of common stock authorized to 23,100,000 shares of common stock, and approved the 2017 Equity Incentive Plan (the “2017 Plan”) which authorized the Company to issue up to 2,285,250 shares of common stock at the discretion of the board of directors to provide equity awards to employees, directors and consultants.

In March 2017, options to purchase 1,332,375 shares of common stock under the 2017 Plan were awarded to certain employees of the Company. The Company performed a valuation of its common stock in order to determine the fair value as of the award date. On June 2, 2017, the Company’s non-employee director committee of the board of directors approved the award date valuation which set the exercise price for options awarded in March 2017 at \$3.73 per share and established an accounting grant date.

In April 2017, options to purchase 284,466 shares of common stock under the 2017 Plan were awarded to two employees of the Company. On June 2, 2017, the Company’s non-employee director committee of the board of directors approved the award date valuation which set the exercise price for the options awarded in April 2017 at \$3.73 per share and established an accounting grant date.

In May 2017, options to purchase 108,900 shares of common stock under the 2017 Plan were awarded to one employee and three non-employee directors of the Company. On June 2, 2017, the Company’s non-employee director committee of the board of directors approved the award date valuation which set the exercise price for options awarded in May 2017 at \$7.32 and established an accounting grant date.

***Forward stock split***

On June 16, 2017, the Company effected a 3.3-for-one forward stock split of 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 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 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.

**Dova Pharmaceuticals, Inc.**  
**Condensed consolidated balance sheets**  
(in thousands, except share and per share amounts)

|  | December 31,<br>2016   | March 31,<br>2017<br>(Unaudited) | Pro forma<br>liabilities and<br>stockholders'<br>equity<br>March 31, 2017<br>(Unaudited) |
|--|------------------------|----------------------------------|--|
| <b>ASSETS</b>  |                        |                                  |  |
| Current assets   |                        |                                  |  |
| Cash and cash equivalents . . . . .  | \$ 28,709              | \$ 26,645                        | \$ 26,645  |
| Prepaid expenses . . . . .   | 37                     | 26                               | 26   |
| Total current assets . . . . .   | <u>28,746</u>          | <u>26,671</u>                    | <u>26,671</u>  |
| Deferred offering costs . . . . .  | —                      | 169                              | 169  |
| <b>Total assets . . . . .</b>  | <b><u>\$28,746</u></b> | <b><u>\$26,840</u></b>           | <b><u>\$26,840</u></b>   |
| <b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>  |                        |                                  |  |
| Current liabilities  |                        |                                  |  |
| Accounts payable . . . . .   | \$ 157                 | \$ 126                           | \$ 126   |
| Accrued expenses . . . . .   | 7,918                  | 4,756                            | 4,756  |
| Accrued interest . . . . .   | 151                    | —                                | —  |
| Due to related party . . . . .   | 85                     | 50                               | 50   |
| Note payable, short-term . . . . .   | —                      | 20,537                           | 20,537   |
| Total current liabilities . . . . .  | <u>8,311</u>           | <u>25,469</u>                    | <u>25,469</u>  |
| Note payable, long-term . . . . .  | 13,640                 | —                                | —  |
| <b>Total liabilities . . . . .</b>   | <b><u>21,951</u></b>   | <b><u>25,469</u></b>             | <b><u>25,469</u></b>   |
| <b>Commitments and contingencies</b>   |                        |                                  |  |
| <b>Stockholders' equity</b>  |                        |                                  |  |
| Series A preferred stock, \$0.001 par value;<br>1,400,000 shares authorized; 982,714 shares<br>issued and outstanding as of December 31, 2016<br>and March 31, 2017 . . . . .  | 1                      | 1                                | —  |
| Common stock, \$0.001 par value; 22,110,000 and<br>23,100,000 shares authorized as of<br>December 31, 2016 and March 31, 2017; 17,332,257<br>shares issued and outstanding as of December 31,<br>2016 and March 31, 2017 . . . . . | 17                     | 17                               | 21   |
| Additional paid-in capital . . . . .   | 33,967                 | 33,967                           | 33,963   |
| Accumulated deficit . . . . .  | (27,190)               | (32,614)                         | (32,614)   |
| Total stockholders' equity . . . . .   | <u>6,795</u>           | <u>1,371</u>                     | <u>1,371</u>   |
| <b>Total liabilities and stockholders' equity . . . . .</b>  | <b><u>\$28,746</u></b> | <b><u>\$26,840</u></b>           | <b><u>\$26,840</u></b>   |

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

**Dova Pharmaceuticals, Inc.**  
**Condensed consolidated statements of operations**  
(in thousands, except share and per share amounts)  
(Unaudited)

|  | For the period from<br>March 24, 2016<br>(Inception) to<br>March 31, 2016 | For the three<br>months ended<br>March 31, 2017 |
|--|---|---|
| <b>Operating expenses:</b>   |   |   |
| Research and development . . . . .   | \$ 150  | \$ 4,276  |
| Research and development—licenses acquired . . . . .                                 | 5,000   | —   |
| General and administrative . . . . .   | 12  | 955   |
| Total operating expenses . . . . .   | <u>5,162</u>  | <u>5,231</u>                                    |
| <b>Loss from operations . . . . .</b>  | <b><u>(5,162)</u></b>   | <b><u>(5,231)</u></b>                           |
| <b>Other income (expenses)</b>   |   |   |
| Other income, net . . . . .  | —   | 33  |
| Interest expense . . . . .   | —   | (226)   |
| Total other expenses, net . . . . .  | <u>—</u>  | <u>(193)</u>                                    |
| <b>Net loss . . . . .</b>  | <b><u>\$ (5,162)</u></b>  | <b><u>\$ (5,424)</u></b>                        |
| Net loss per share, basic and diluted . . . . .                                      | <u>\$ (0.31)</u>  | <u>\$ (0.31)</u>                                |
| Weighted average common shares outstanding, basic and diluted . . .                  | <u>16,500,000</u>   | <u>17,332,257</u>                               |
| Pro forma net loss per share, basic and diluted . . . . .                            |   | <u>\$ (0.26)</u>                                |
| Pro forma weighted average common shares outstanding, basic and<br>diluted . . . . . |   | <u>20,575,207</u>                               |

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

**Dova Pharmaceuticals, Inc.**  
**Condensed consolidated statement of stockholders' equity**  
(in thousands, except share amounts)  
(Unaudited)

|                                | Series A<br>preferred stock |        | Common stock |        | Additional<br>paid-in<br>capital | Accumulated<br>deficit | Total<br>stockholders'<br>equity |
|--------------------------------|-----------------------------|--------|--------------|--------|----------------------------------|------------------------|----------------------------------|
|                                | Shares                      | Amount | Shares       | Amount |                                  |                        |                                  |
| Balance as of December 31,     |                             |        |              |        |                                  |                        |                                  |
| 2016 . . . . .                 | 982,714                     | \$ 1   | 17,332,257   | \$ 17  | \$ 33,967                        | \$ (27,190)            | \$ 6,795                         |
| Net loss . . . . .             | —                           | —      | —            | —      | —                                | (5,424)                | (5,424)                          |
| Balance as of March 31, 2017 . | 982,714                     | \$ 1   | 17,332,257   | \$ 17  | \$ 33,967                        | \$ (32,614)            | \$ 1,371                         |

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

**Dova Pharmaceuticals, Inc.**  
**Condensed consolidated statements of cash flows**  
(in thousands)  
(Unaudited)

|   | For the period from<br>March 24, 2016<br>(Inception) to<br>March 31, 2016 | For the three<br>months ended<br>March 31, 2017 |
|---|---|---|
| <b>Cash flows from operating activities</b>   |   |   |
| Net loss . . . . .  | \$(5,162)   | \$ (5,424)                                      |
| Adjustments to reconcile net loss to net cash used in operating activities:                         |   |   |
| Research and development-licenses acquired, expensed . . . . .                                      | 5,000   | —   |
| Non-cash research and development expenses . . . . .  | —   | 4,267   |
| Changes in operating assets and liabilities:  |   |   |
| Prepaid expenses . . . . .  | —   | 11  |
| Accounts payable . . . . .  | —   | (31)  |
| Accrued expenses . . . . .  | 162   | 10  |
| Accrued interest . . . . .  | —   | (151)   |
| Due to related party . . . . .  | —   | (35)  |
| Net cash used in operating activities . . . . .   | —   | (1,353)   |
| <b>Cash flows from financing activities</b>   |   |   |
| Payment of offering cost . . . . .  | —   | (711)   |
| Net cash used in financing activities . . . . .   | —   | (711)   |
| Net decrease in cash and cash equivalents . . . . .   | —   | (2,064)   |
| Cash and cash equivalents at the beginning of the period . . . . .                                  | —   | 28,709  |
| <b>Cash and cash equivalents at the end of the period . . . . .</b>                                 | <b>\$ —</b>   | <b>\$26,645</b>                                 |
| <b>Supplemental disclosure of cash flow information:</b>  |   |   |
| Cash paid for interest . . . . .  | \$ —  | \$ 377  |
| <b>Supplemental disclosure of noncash investing and financing activities:</b>                       |   |   |
| Change in note payable . . . . .  | \$ —  | \$ 6,897  |
| Unpaid deferred offering cost . . . . .   | \$ —  | \$ 169  |
| Capital contribution—PBM Capital Investments, LLC—payment of AkaRx upfront purchase price . . . . . | \$5,000   | \$ —  |

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

# Dova Pharmaceuticals, Inc.

## Notes to condensed consolidated financial statements

### 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 undergoing a non-emergent minimally to moderately invasive medical procedure. 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. AkaRx is the Company’s only subsidiary.

The unaudited condensed consolidated financial statements of Dova and its wholly owned subsidiary AkaRx (the “Company”) include the results of operations for the period from Inception through March 31, 2016 and the three months ended March 31, 2017.

#### *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 March 31, 2017, the Company had an accumulated deficit of \$32.6 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).

The Company expects to use the proceeds from the above transaction primarily for general corporate purposes, which may include financing the Company’s growth, conducting clinical trials for additional indications for avatrombopag, regulatory filings for avatrombopag, preparing for commercialization of avatrombopag, if approved, developing new or existing drug candidates, making payments on the Eisai Note and funding capital expenditures, acquisitions and investments.



The accompanying financial statements have been prepared on a going concern basis, which contemplates the realization of assets and satisfaction of liabilities in the normal course of business. The financial statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might result from the outcome of this uncertainty. The Company anticipates incurring additional losses until such time, if ever, that it can obtain marketing approval to sell, and then generate significant sales, of its drug candidate that is currently in development. Substantial additional financing will be needed by the Company to fund its operations and to develop and commercialize its drug candidate. These factors raise substantial doubt about the Company's ability to continue as a going concern.

The Company will seek to obtain additional capital through the sale of debt or equity financings or other arrangements to fund operations; however, there can be no assurance that the Company 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. Issued debt securities may contain covenants and limit the Company's ability to pay dividends or make other distributions to stockholders. If the Company is unable to obtain such additional financing, future operations would need to be scaled back or discontinued.

## **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 months ended March 31, 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 appearing elsewhere in this prospectus.

### ***Unaudited pro forma information***

The unaudited pro forma balance sheet data as of March 31, 2017 gives effect to the automatic conversion of all outstanding shares of the Company's preferred stock on an 3.3-for-one basis into an aggregate of 3,242,950 shares of common stock, which will occur immediately prior to the Company's planned initial public offering. The unaudited pro forma basic and diluted net loss per share for the three months ended March 31, 2017 gives effect to such automatic conversion as if it had occurred as of the beginning of the period.

### ***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.

### ***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.

### ***Deferred offering costs***

Deferred offering costs consist of legal fees incurred through the balance sheet date that are directly related to the initial public offering ("IPO") and that will be charged to stockholder's equity upon the completion of the IPO. Should the IPO prove to be unsuccessful, these deferred costs, as well as additional expenses to be incurred, will be charged to operations.

### ***Accrued expenses***

Accrued expenses primarily consist of unpaid service fees and out-of-pocket costs due under the TSA. Once such expenses 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 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.

### ***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.
- 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 December 31, 2016 and March 31, 2017, 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 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 employees' roles within 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 March 31, 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 period ended March 31, 2017 did not include the 982,714 shares of Series A Preferred Stock as the inclusion of these securities would have been antidilutive.

#### ***Recent accounting pronouncements***

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.

### **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 March 24, 2016 (inception) to March 31, 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 March 31, 2017.

#### ***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 a specified amount 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 March 31, 2017, the Company had outstanding borrowings of \$20.5 million under the Note and the Company did not owe Eisai any 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 are 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 will be required to make aggregate milestone payments of up to \$5.0 million to Astellas if certain 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.

#### **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 months ended March 31, 2017, the Company incurred expenses under the SAs of \$150,000, which were included in general and administrative expenses.

As of March 31, 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.

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, has sole voting and dispositive power over all outstanding shares of the Company’s common stock.

In March 2017, the Company's board of directors approved an increase in the number of shares of common stock authorized to 23,100,000 shares of common stock, and approved the 2017 Equity Incentive Plan (the "2017 Plan") which authorized the Company to issue up to 2,285,250 shares of common stock at the discretion of the board of directors to provide equity awards to employees, directors and consultants.

#### *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 pays 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 Company's board of directors.

In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Company or a Deemed Liquidation Event (certain mergers, consolidations, reorganizations or recapitalizations, or the sale, lease, transfer, exclusive license or other disposition of all or substantially all the assets of the Company), the holders of shares of Series A preferred stock will be entitled to be paid out of the assets of the Company available for distribution to its stockholders before any payment shall be made to the holders of common stock. The amount payable per share to the holders of the Series A preferred stock will be equal to the greater of (i) one and a half times the Series A Original Issue Price, plus any dividends declared but unpaid, or (ii) such amount per share as would have been payable had all shares of Series A preferred stock been converted into common stock immediately prior to such liquidation, dissolution, winding up or Deemed Liquidation Event.

Each holder of outstanding shares of Series A preferred stock is entitled to cast the number of votes equal to the number of whole shares of common stock into which the shares of Series A preferred stock held are convertible as of the record date for determining stockholders entitled to vote on a matter. Holders of the Series A preferred stock vote together with the holders of common stock as a single class.

The holders of Series A preferred stock, voting as a separate class, are entitled to elect by majority vote (with each share of Series A preferred stock entitled to one vote) one individual to the Company's board of directors. The Series A preferred stockholders also have certain protective rights.

Each share of Series A preferred stock is 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 is mandatorily convertible under certain conditions (i) when the Company issues shares of common stock in a public offering generating gross proceeds of at least \$60.0 million to the Company, at a 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 is \$8.94, and is 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. In the event the Company issues additional shares of common stock for no consideration or for consideration per share less than the Series A preferred stock conversion price then in effect, the conversion price is reduced based on a weighted average anti-dilution formula.

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

Further, the Series A preferred stock is 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 is not clearly and closely related to the associated preferred stock host instrument but does not meet the definition of a derivative and therefore was not bifurcated from the equity host.

At March 31, 2017, the Company was authorized to issue 1,400,000 preferred shares with a par value of \$0.001 per share and 982,714 shares of preferred stock were issued and outstanding.

## **Note 6—Commitments and contingencies**

### ***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.

## **Note 7—Subsequent events**

### ***Option grants***

In March 2017, options to purchase 1,332,375 shares of common stock under the 2017 Plan were awarded to certain employees of the Company. The Company performed a valuation of its common stock in order to determine the fair value as of the award date. On June 2, 2017, the Company's non-employee director committee of the board of directors approved the award date valuation which set the exercise price for options awarded in March 2017 at \$3.73 per share and established an accounting grant date.

In April 2017, options to purchase 284,466 shares of common stock under the 2017 Plan were awarded to two employees of the Company. On June 2, 2017, the Company's non-employee director committee of the board of directors approved the award date valuation which set the exercise price for the options awarded in April 2017 at \$3.73 per share and established an accounting grant date.

In May 2017, options to purchase 108,900 shares of common stock under the 2017 Plan were awarded to one employee and three non-employee directors of the Company. On June 2, 2017, the Company's non-employee director committee of the board of directors approved the award date valuation which set the exercise price for options awarded in May 2017 at \$7.32 and established an accounting grant date.

### ***Forward stock split***

On June 16, 2017, the Company effected a 3.3-for-one forward stock split of 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 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 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.



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*4,062,500 shares*



**Dova Pharmaceuticals, Inc.**

*Common Stock*

## **Prospectus**

**J.P. Morgan**

**Jefferies**

**Leerink Partners**

, 2017

Through and including \_\_\_\_\_, 2017 (the 25th day after the date of this prospectus), all dealers effecting transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This is in addition to a dealer's obligation to deliver a prospectus when acting as an underwriter and with respect to an unsold allotment or subscription.

