ENANTA PHARMACEUTICALS INC
Form 10-K
December 11, 2017

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

Form 10-K

ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the fiscal year ended September 30, 2017

OR

TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
Commission File Number 001-35839

ENANTA PHARMACEUTICALS, INC.

(Exact name of registrant as specified in its charter)

| DELAWARE | 2834 | 04-3205099 |
|---|---|---|
| (State or other jurisdiction of incorporation or organization) | (Primary Standard Industrial Classification Code Number) | (I.R.S. Employer Identification Number) |

500 Arsenal Street

Watertown, Massachusetts 02472

(617) 607-0800

(Address, including zip code, and telephone number, including area code, of registrant’s principal executive offices)

Securities registered pursuant to Section 12(b) of the Act:

| Title of each class: | Name of each exchange on which registered: |
|---|---|

Common Stock, $0.01 Par Value The NASDAQ Stock Market LLC (NASDAQ Global Select Market)
Securities registered pursuant to Section 12(g) of the Act: None

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes No

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes No

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

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

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K.

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

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

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

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

The aggregate market value of the registrant's common stock held by non-affiliates of the registrant as of the last business day of the registrant's most recently completed second fiscal quarter, March 31, 2017, based on the last reported sale price of the registrant's common stock of $30.80 per share was $491,133,874. The number of shares of the registrant's Common Stock, $0.01 par value, outstanding as of December 1, 2017, was 19,126,006 shares.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's Definitive Proxy Statement for its 2018 Annual Meeting of Stockholders scheduled to be held on February 28, 2018, which Definitive Proxy will be filed with the Securities and Exchange Commission not

later than 120 days after the registrant's fiscal year end of September 30, 2017, are incorporated by reference into Part III of this Form 10-K.

As used in this Form 10-K, “Enanta,” “the Company,” “we,” “our,” and “us” refer to Enanta Pharmaceuticals, Inc., and “MAVYRET/MAVIRET” refers to AbbVie’s new HCV regimen consisting of tablets of glecaprevir/pibrentasvir, except where the context otherwise requires or as otherwise indicated.

NOTE REGARDING FORWARD LOOKING STATEMENTS

This Annual Report on Form 10-K contains forward-looking statements concerning our business, operations and financial performance and condition, as well as our plans, objectives and expectations for our business operations and financial performance and condition. Any statements contained herein that are not statements of historical facts may be deemed to be forward-looking statements. In some cases, you can identify forward-looking statements by terminology such as “aim,” “anticipate,” “assume,” “believe,” “contemplate,” “continue,” “could,” “due,” “estimate,” “expect,” “may,” “objective,” “plan,” “predict,” “potential,” “positioned,” “seek,” “should,” “target,” “will,” “would,” and other similar e are predictions of or indicate future events and future trends, or the negative of these terms or other comparable terminology. These forward-looking statements include, but are not limited to, statements about overall trends, royalty revenue trends, research and clinical development plans, liquidity and capital needs and other statements of expectations, beliefs, future plans and strategies, anticipated events or trends and similar expressions. These forward-looking statements are based on our management’s current expectations, estimates, forecasts and projections about our business and the industry in which we operate and our management’s beliefs and assumptions. These forward-looking statements are not guarantees of future performance or development and involve known and unknown risks, uncertainties and other factors that are in some cases beyond our control. As a result, any or all of our forward-looking statements in this Annual Report on Form 10-K may turn out to be inaccurate. Factors that may cause actual results to differ materially from current expectations include, among other things, those listed under “Risk Factors” and discussed elsewhere in this Annual Report on Form 10-K. These forward-looking statements speak only as of the date of this Annual Report on Form 10-K. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future. You should, however, review the factors and risks we describe in the reports we will file from time to time with the SEC after the date of this Annual Report on Form 10-K.

ENANTA PHARMACEUTICALS, INC.

ANNUAL REPORT ON FORM 10-K

For the year ended September 30, 2017

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PART I

ITEM 1. BUSINESS
BUSINESS

Overview

We are a biotechnology company that uses our robust, chemistry-driven approach and drug discovery capabilities to create small molecule drugs primarily for the treatment of viral infections and liver diseases. We discovered glecaprevir, the second of two protease inhibitors discovered and developed through our collaboration with AbbVie and marketed as part of AbbVie's new direct-acting antiviral (DAA) regimen under the tradenames MAVYRET™ (U.S.) or MAVIRET™ (ex-U.S.) (glecaprevir/pibrentasvir) for the treatment of chronic hepatitis C virus, or HCV. The other protease inhibitor under our HCV collaboration is part of AbbVie's initial DAA regimens for the treatment of chronic HCV marketed under the tradenames VIEKIRA PAK® (paritaprevir/ritonavir/ombitasvir

/dasabuvir) (U.S.) or VIEKIRAX® (paritaprevir/ritonavir/ombitasvir) (ex-U.S.). Our royalties from our AbbVie collaboration and our existing financial resources provide us funding to support our wholly owned research and development efforts, which are currently focused on the following disease targets:

- non-alcoholic steatohepatitis, or NASH, a liver disease estimated to affect approximately 6 million individuals in the U.S. alone;
- primary biliary cholangitis, or PBC, a chronic liver disease that slowly destroys bile ducts in the liver, which affects an estimated 17,000 individuals in the U.S.;
- respiratory syncytial virus, or RSV, the most common cause of bronchiolitis and pneumonia in children under one year of age in the U.S., resulting in an estimated 75,000 to 125,000 hospitalizations each year in the U.S.; and
- hepatitis B virus, or HBV, the most prevalent chronic hepatitis, which is estimated to affect approximately 250 million individuals worldwide.

We had $293.7 million in cash and marketable securities at September 30, 2017. In fiscal 2017, we received and earned as revenue a total of $65.0 million in milestone payments for commercialization regulatory approvals of the glecaprevir/pibrentasvir combination in the U.S. in August 2017 and the EU in July 2017 and earned $37.8 million in per-product royalties on portions of AbbVie's net sales of its HCV regimens allocated to paritaprevir or glecaprevir. We earned the remaining $15.0 million milestone payment from AbbVie upon reimbursement approval for MAVIRET in Japan in November 2017. We expect our existing financial resources and quarterly royalty payments will allow us to continue to invest for the foreseeable future in our wholly owned research and development programs.

Our Wholly Owned Programs

Our wholly owned research and development programs are in liver disease (non-virology), namely NASH and PBC, and in virology, namely RSV and HBV:

- NASH and PBC: We are working on multiple compounds that selectively bind to and activate the farnesoid X receptor, or FXR. We plan to develop these compounds, referred to as FXR agonists, for use in the treatment of NASH and PBC, both of which are liver diseases with very few therapeutic options. Our lead FXR agonist, EDP-305, represents a new class of FXR agonist designed to take advantage of increased binding interactions with the receptor. We believe this class is significantly different from other FXR agonists in clinical development.
- In October 2017, we announced results of a Phase 1a/b clinical study of EDP-305, which was generally safe and well tolerated over a broad range of single and multiple doses with pharmacokinetic data supporting once daily oral dosing. The study included 98 healthy volunteer subjects, or HV subjects, and 48 subjects who were obese and with or without pre-diabetes or type

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2 diabetes, whom we refer to as subjects with presumptive non-alcoholic fatty liver disease, or PN subjects.
EDP-305 exhibited strong engagement of the FXR receptor as evidenced by increased levels of FGF19 and reduced levels of C4, both of which are monitored as downstream markers indicating FXR receptor activity.
Results support the ability to administer EDP-305 in future trials at doses that neither elicit clinically significant changes in lipids nor result in pruritus (itching).

- Since November 2016, we have presented data at the 2016 and 2017 annual meetings of the American Association for the Study of Liver Diseases (AASLD), the 2017 NASH-TAG conference and the 2017 International Liver Congress (ILC) that demonstrated that EDP-305 is a highly selective FXR agonist and shows more potent activity in a variety of in vitro and in vivo NASH models compared to the most advanced NASH candidate in development today, obeticholic acid, or OCA.
    - We plan to initiate a Phase 2 clinical study of EDP-305 in PBC patients by the end of calendar 2017.
    - We also plan to initiate a Phase 2 clinical study of EDP-305 in NASH patients in early 2018.
- EDP-305 has been granted Fast Track designation by the U.S. Food and Drug Administration (FDA) for the treatment of NASH patients with liver fibrosis and separately for the treatment of PBC.
- In addition, we are pursuing research in other classes of FXR agonists as well as other mechanisms that may provide therapeutic benefit in NASH and that either or both of these could be used as combination therapies for NASH.
- RSV: We have selected EDP-938, a potent N-protein inhibitor of activity of both major subgroups of RSV, referred to as RSV-A and RSV-B, as our first development candidate for RSV. We believe EDP-938 is differentiated from fusion inhibitors currently in development for RSV because N-protein inhibitors directly target the viral replication process of RSV and have demonstrated high barriers to resistance against RSV in vitro.
- In June 2017, we presented preclinical data demonstrating that EDP-938 is a potent inhibitor of both RSV-A and RSV-B activity, maintaining antiviral activity post-infection while presenting a high barrier to resistance in vitro. EDP-938 demonstrated a greater than 4-log reduction in viral load in an animal model challenged with RSV. Further, EDP-938 maintained antiviral potency across all clinical isolates tested in vitro, as well as virus that was resistant to fusion inhibitors. The compound inhibited RSV at a post-entry, replication step and maintained its activity in vitro when given 24 hours post infection. In addition, combination studies of EDP-938 with other types of RSV inhibitors, such as fusion inhibitors, showed synergistic antiviral effects.
    - We plan to initiate a Phase 1 clinical study of EDP-938 by the end of calendar 2017.
- HBV: We also have a program to discover and develop new chemical entities for the treatment of HBV. Our initial focus is on core inhibitors, a mechanism with early clinical validation. We believe that it may be necessary to utilize more than one compound/mechanism for the treatment of HBV and therefore we are pursuing multiple approaches. We continue to make progress in discovering, characterizing, and seeking patent protection for new core inhibitors of HBV with the goal of identifying a development candidate in 2018. In addition, we are conducting preclinical experiments with other mechanisms that target HBV.

We have utilized our internal chemistry and drug discovery capabilities to generate all of our development-stage programs.

Licensed Products

Through our Collaborative Development and License Agreement with AbbVie, we have developed and licensed to AbbVie two protease inhibitor compounds that have been clinically tested, manufactured, and commercialized by AbbVie. Royalties on AbbVie's net sales allocated to the protease inhibitors in these HCV regimens provided us $37.8 million and $57.7 million in royalty revenue in our 2017 and 2016 fiscal years, respectively. To date, we have earned a total of $330.0 million in milestone payments related to clinical development and commercialization regulatory approvals of these regimens in major markets, including a $15.0 million milestone earned in November 2017.

- Glecaprevir: Glecaprevir is the protease inhibitor we discovered that was developed by AbbVie in its new fixed-dose combination with its NS5A inhibitor, pibrentasvir, for the treatment of HCV. This combination, currently marketed under the brand name MAVYRET™ in the U.S. and MAVIRET™ ex-U.S. and referred to in this report as MAVYRET/MAVIRET, is a new, once daily, all oral, fixed-dose, ribavirin-free treatment for HCV genotypes 1-6, which is referred to as being pan-genotypic. In the EU, U.S. and Japan it is approved as an 8-week treatment for patients without cirrhosis and new to treatment. Today, these patients are estimated to represent the majority of HCV patients in the developed country markets.

Our economics from AbbVie's MAVYRET/MAVIRET consist of two components:

- We earned a total of $65.0 million in milestone payments in fiscal 2017 upon approval for the glecaprevir/pibrentasvir combination in the U.S. and the EU, and we earned the remaining $15.0 million milestone payment in November 2017 in connection with commercialization regulatory approval in Japan.
- We also receive annually tiered, double-digit, per-product royalties on 50% of the net sales of the 2-DAA glecaprevir/pibrentasvir combination in MAVYRET/MAVIRET, which are calculated separately from the royalties of paritaprevir-containing regimens.

The EU, U.S. and Japan authorizations for the MAVYRET/MAVIRET combination of glecaprevir and pibrentasvir, and AbbVie's applications for approval of MAVYRET/MAVIRET in other jurisdictions, are supported by the following studies:

- 8 weeks for treatment-naïve, non-cirrhotics: In November 2016, results from several Phase 3 studies of this combination demonstrated 97.5% of chronic HCV infected patients without cirrhosis and new to treatment across all major genotypes (GT1-6) achieved sustained virologic response at 12 weeks post-treatment, referred to as $SVR_{12}$, with just 8 weeks of MAVYRET/MAVIRET treatment.
- 8 weeks with chronic kidney disease: Results were also presented from AbbVie's EXPEDITION-4 study in chronic HCV patients with chronic kidney disease (CKD), in which 98% of patients (n=102/104) across all major genotypes (GT1-6) achieved $SVR_{12}$ with 12 weeks of treatment with MAVYRET/MAVIRET.
- 8 weeks for GT-3: Data from AbbVie's ENDURANCE-3 study were presented at the International Liver Congress, or ILC, demonstrating that 95% of patients with challenging-to-treat, genotype 3 (GT3) chronic HCV infection, without cirrhosis and new to treatment, achieved $SVR_{12}$ after 8 weeks of treatment with MAVYRET/MAVIRET.
- 12 weeks for compensated cirrhosis: Data from AbbVie's EXPEDITION-1 study were also presented at the ILC, demonstrating that 99% of HCV-infected patients with genotype 1, 2, 4, 5 or 6 and compensated cirrhosis (Child-Pugh A) achieved $SVR_{12}$ following 12 weeks of MAVYRET/MAVIRET treatment without ribavirin.
- Paritaprevir: Paritaprevir is the protease inhibitor contained in AbbVie's initial HCV treatment regimens currently marketed in the U.S., EU, Japan and other countries around the world under the trade names VIEKIRA PAK®, VIEKIRAX® VIEKIRAX XR™ and TECHNIVIE®. First approved and sold in the U.S. in December 2014 for treatment of genotype 1, or GT-1, HCV, AbbVie's HCV regimens containing paritaprevir are now also approved for genotype 4, or GT-4, HCV.

Our Strategy

Our primary objective is to become a leader in the field of viral infections and liver diseases in order to provide new treatments for patients with unmet medical needs. Our focus is on antiviral targets for viruses such as RSV and HBV as well as liver diseases, such as NASH and PBC. All of these disease areas involve significant market opportunities and have attracted the research and development efforts of many competitors. Our strategy includes the following key elements:

- Develop novel treatment options for NASH, PBC and RSV. We have potential candidates in clinical development for NASH, PBC and RSV. EDP-305, our lead FXR agonist, has completed a Phase 1 a/b clinical study and we expect to advance it by initiating a Phase 2 clinical study in PBC by the end of calendar 2017 and a Phase 2 clinical study in NASH in early 2018. We have also selected EDP-938 as our lead RSV development candidate and plan to initiate a Phase 1 clinical study of it by the end of calendar 2017.
- Invest in research and development of product candidates in HBV and additional product candidates in NASH, PBC and RSV. We are continuing to invest significant resources in our NASH, PBC, RSV and HBV research programs in an effort to identify and advance additional novel compounds that have the potential to address significant unmet medical needs in these disease areas. We are also continuing our efforts discovering HBV candidates and plan to identify a candidate in 2018. We may clinically explore other diseases where our assets could play a role. We are also seeking to identify a lead candidate in a second class of FXR agonists in 2018. In addition, we may seek to augment our product candidate pipeline through the acquisition or in-licensing of external assets and/or technologies in one or more of our disease areas of focus.
- Use our existing resources and future cash flow from our AbbVie collaboration to fund our research and development activities. Our existing financial resources and any future royalty payments from our AbbVie collaboration will provide us substantial resources to fund our research and development programs for the foreseeable future. These resources will allow us to advance up to several compounds into clinical development and progress the most promising ones at least through proof-of-concept for further development as a monotherapy or in combinations with other therapeutic agents when we believe such combinations will provide the most promising opportunities.
- Collaborate, where and when appropriate, with pharmaceutical partners to create combination therapies and accelerate the development and commercialization of our proprietary compounds. We are prepared to join forces, where and when appropriate, with collaborators with compounds targeting other mechanisms of action in diseases such as NASH and HBV, where there is the potential for better treatments with combination therapies. Our decisions regarding our proprietary programs will be based on the results of our early phase clinical studies and the potential for combinations with one or more drugs targeting other mechanisms of action in these diseases.

Our Research and Development Pipeline

The following table summarizes our product development pipeline in our virology and liver disease programs:

Our FXR Program in NASH and PBC

Background and Overview of NASH and PBC

Non-alcoholic fatty liver disease, or NAFLD, is the accumulation of excessive fat in liver cells in the form of triglycerides, a process known as hepatic steatosis, that is not associated with alcohol abuse. It is normal for the liver to contain some fat. However, if more than 5%-10% of the liver's weight is fat, then it is called a fatty liver. A subgroup of NAFLD patients have liver cell injury and inflammation (steatohepatitis) in addition to excessive fat. Progression of this condition leads to non-alcoholic steatohepatitis, or NASH. Patients with NASH can develop fibrosis, a fibrous scarring of the liver, and ultimately cirrhosis of the liver. Typically scored on a scale of 1-4, also referred to as F1-F4, fibrosis in its earlier stages has been shown to be reversible, but in its most advanced stage results in cirrhosis, which is understood to be a more advanced, irreversible scarring of the liver, potentially leading to hepatocellular carcinoma (HCC) or requiring a liver transplant. NASH is widely considered to be the liver expression of metabolic diseases related to type 2 diabetes, insulin resistance, obesity, hyperlipidemia and hypertension.

Stages of Liver Injury

According to the World Gastroenterology Organization Global Guidelines 2014, NASH is an increasingly common chronic liver disease with worldwide distribution that is closely associated with diabetes and obesity, which have both reached epidemic proportions. It is estimated that there are at least 1.46 billion obese adults worldwide. Approximately 6 million individuals in the U.S. are estimated to have progressed to NASH and some 600,000 to NASH-related cirrhosis. NASH and NAFLD are now considered the number one cause of liver disease in Western countries.

Currently, there are no approved treatments for NASH. While patients presenting with NASH are counseled on lifestyle modifications, new effective treatments are urgently needed, particularly in the setting of advanced fibrosis and cirrhosis. Currently, each of Intercept Pharmaceuticals, Genfit, Gilead and Allergan (Tobira) has a compound in one or more Phase 3 trials in NASH. In addition, many Phase 2 and earlier stage studies of other classes of compounds are underway by different companies.

Primary biliary cholangitis (formerly known as primary biliary cirrhosis), or PBC, is a chronic, or long-term, disease of the liver that slowly destroys the medium-sized bile ducts within the liver. Bile is a digestive liquid that is made in the liver. It travels through the bile ducts to the small intestine, where it helps digest fats and absorb fatty vitamins. In patients with PBC, the bile ducts are destroyed by inflammation. This causes bile to remain in the liver, where gradual injury damages liver cells and causes cirrhosis, or scarring of the liver. As cirrhosis progresses and the amount of scar tissue in the liver increases, the liver loses its ability to function, leading to potential liver failure, liver transplantation or hepatocellular carcinoma. While PBC is a relatively rare disease (the incidence in Europe, North America, Asia and Australia ranges from 0.33-5.8 cases per 100,000, and is 10 times more common in women than in men), it remains one of the major causes of liver failure and/or the need for liver transplant.

Agonists of the farnesoid X receptor, referred to as FXR agonists, have shown promising activity in many preclinical models of liver disease. One FXR agonist, obeticholic acid, or OCA (brand name Ocaliva ®), which was approved by the FDA in May 2016 for the treatment of PBC, has already demonstrated favorable clinical results in NASH. We believe that new FXR agonists may provide substantial therapeutic benefit in NASH and PBC and may overcome some of the potential shortcomings of OCA, including limited effects on resolution of NASH, elevation of low-density lipoprotein, or LDL, and itching, also called pruritis.

Scientific Background

FXR is a nuclear hormone receptor that functions to modulate gene expression in response to various metabolic stimuli. FXRs are expressed at high levels in the liver and intestine. Bile acids have been identified as important physiological ligands for FXRs, able to bind and activate the receptor. The downstream gene modulation resulting from bile acid engagement of FXRs not only contribute to the regulation of bile acid synthesis and metabolism, but is also involved in a number of other metabolic processes, in particular lipid metabolism. More recently, it has been discovered that bile acids, via FXR, are able to promote insulin sensitivity and decrease lipid synthesis in the liver. In addition, studies have shown that bile acid-dependent FXR activation is able to provide beneficial effects on fibrosis in the liver as well. For these reasons, FXR is considered to be a viable target for NASH. Recent Phase 2b

trials with OCA, a synthetic analog of natural bile acids known to activate FXR, demonstrated efficacy in NASH patients. In PBC, improved outcomes would be expected due to the reduction of bile acid synthesis by activation of FXR. OCA demonstrated efficacy in a Phase 3 trial in PBC, which was the basis for its conditional approval in the U.S. in May 2016 for the treatment of PBC in combination with first line therapy ursodeoxycholic acid (UDCA) in adults with an inadequate response to UDCA, or as monotherapy in adults unable to tolerate UDCA.

EDP-305 and Our Approach to the Treatment of NASH and PBC

Even though there has been clinical validation demonstrated by the FXR agonist, OCA, we believe that there is an opportunity for the development of a treatment that shows improvements in potency and efficacy and reductions in potential safety liabilities for the treatment of NASH and PBC. Using our strong chemistry capabilities, we have undertaken the discovery and development of new FXR agonists that we believe may provide substantial improvements over the FXR agonists currently in advanced clinical development.

EDP-305, our lead FXR agonist candidate, represents a new class of FXR agonists that has been designed to take advantage of increased binding interactions with the receptor. Further, this non-bile acid class contains steroidal and non-steroidal components, and does not contain the carboxylic acid group that can lead to the formation of taurine and glycine conjugates normally associated with bile acids, which may also be present in other classes of FXR agonists.

We reported the results of our Phase 1 a/b clinical study of EDP-305 in October 2017. Our double-blind, placebo-controlled Phase 1 study was designed to evaluate the safety, tolerability and pharmacokinetics of single ascending doses (SAD) and multiple ascending doses (MAD) of EDP-305 in adult healthy volunteer subjects, or HV subjects, and subjects with presumptive NAFLD, or PN subjects. By presumptive NAFLD, we mean adults who are obese, with or without pre-diabetes or type 2 diabetes.

In this Phase 1 study, EDP-305 was shown to be generally safe and well tolerated over a broad range of single and multiple doses with pharmacokinetic (PK) data supporting once daily oral dosing. EDP-305 exhibited strong engagement of the FXR receptor as evidenced by increased FGF19 levels and reduced C4 levels which can be monitored as downstream markers indicating FXR activity. The results of the study support the ability to administer EDP-305 in future trials at doses that neither elicit clinically significant changes in lipids nor result in pruritus.

- A total of 146 subjects received at least one dose of EDP-305 (n=110) or placebo (n=36) including 50 HV subjects in the SAD phases of the study and 96 (48 HV, and 48PN) subjects in the MAD phases of the study. Overall, mean BMI in the PN cohort was 32 (29, 35). SAD had 6 cohorts at doses of 1, 5, 10, 20, 40 and 80 mg EDP-305/placebo, and MAD had 6 cohorts at doses of 0.5, 1, 2.5, 5, 10 and 20 mg EDP-305/placebo for 14 days.
- Strong FXR target engagement was demonstrated, with doses of EDP-305 > 1 mg increasing FGF19 and reducing C4 in all subjects, while PN subjects were even more sensitive with significant effects also observed in both parameters at the lowest multiple doses of 0.5 and 1 mg.
    - No serious adverse events (SAEs) were reported, and EDP-305 was generally well tolerated at all doses tested.
    - Treatment-emergent adverse events occurring in ≥ 2 EDP-305 treated subjects in MAD cohorts were: headache and pruritus in HV subjects, and constipation and pruritus in PN subjects.
    - Of the cases of pruritus noted (9% for EDP-305, 3% in placebo), the majority were mild or moderate and occurred at multiple doses of 20 mg, with no cases below 10mg. Notably, EDP-305 demonstrated potent engagement of the FXR receptor across the lower dose range where there was no pruritus.
    - Two subjects discontinued treatment in the MAD phase at the 20 mg dose level, one for a transient grade 2 elevation of ALT/AST liver enzymes, and one for moderate pruritus.

- No dose-related changes in lipids were observed in HV subjects at any doses; and no dose-related changes in lipids were observed in PN subjects except for reductions of total cholesterol and high-density

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lipoprotein, or HDL, cholesterol at the multiple 20mg dose, with no concomitant increase in low-density lipoprotein, or LDL, cholesterol.

We plan to initiate a Phase 2 dose-ranging study in PBC patients by the end of 2017 and a Phase 2 dose-ranging study in NASH patients in early 2018.

Our RSV Program

Background Overview of RSV

Respiratory syncytial virus, or RSV, is a virus that infects the lungs and represents a serious unmet medical need in infants and children, as well as immune-compromised individuals and the elderly. RSV is the most common cause of bronchiolitis (inflammation of the small airways in the lung) and pneumonia in children under 1 year of age in the United States. Each year, 75,000 to 125,000 children in this age group are hospitalized due to RSV infection. In one large U.S.-based study, RSV infection in children was associated with 20% of hospitalizations, 18% of emergency department visits, and 15% of pediatric office visits for acute respiratory infections in the November-April timeframe. Though a prophylactic monoclonal antibody-based treatment is available for infants considered at high risk for RSV infection, this study found that most young children affected by RSV infection were previously healthy, and thus would not normally be considered for prophylaxis. There is currently no safe and effective treatment available for treating RSV infection.

Scientific Background

RSV is a member of the Paramyxoviridae family, and is a single-stranded, negative-sense RNA virus. The RSV genome consists of ten genes that encode for 11 proteins, namely NS1, NS2, N, P, M, SH, G, F, M2-1, M2-2, and L. The F and G proteins are the predominant target proteins for RSV vaccines. Similarly, small molecule therapeutics have focused primarily on the F (or fusion) protein, while some efforts have targeted the N and L proteins. There are two major subgroups of RSV, designated RSV-A and RSV-B, each of which contains numerous genotypes. Both groups are viewed as capable of causing RSV infections that can result in hospitalization.

EDP-938 and Our approach to the treatment of RSV

While a number of companies are developing potential approaches geared towards the F protein (or fusion protein, responsible for mediating viral entry of RSV into host cells), we are focused on the N-protein mechanism that targets the replication process of RSV. It is possible that N-protein inhibitors may also be effective at later stages of infection. Through our internal chemistry efforts, we have identified a clinical candidate, EDP-938, a potent inhibitor of RSV-A and RSV-B, and plan to initiate a Phase 1 clinical study by the end of calendar 2017.

Our HBV Program

Background and Overview of HBV

Hepatitis B virus, or HBV, can cause potentially life-threatening liver infection. The virus is transmitted through contact with the blood or other bodily fluids of an infected person. It is estimated that approximately 250 million people worldwide are chronically infected, and 15-25% of patients with chronic HBV infection develop chronic liver disease, including cirrhosis, liver cancer, or liver decompensation. It is also estimated that more than 885,000 people worldwide died in 2015 due to complications of HBV. Estimates for the total number of persons chronically infected with HBV in the U.S. vary but generally range between 0.5 million and 2.0 million. Combining U.S., Japan, and major EU populations, estimates of HBV prevalence have been as high as 4.9 million.

Current approaches to treatment include interferon therapy and/or inhibitors of HBV reverse transcriptase, the enzyme responsible for viral DNA synthesis, which is necessary for HBV replication. Treatment with interferon offers modest cure rates, and is accompanied by serious side effects, including flu-like symptoms, fatigue, headache and nausea. Reverse transcriptase inhibitors can be very effective at suppressing the virus but often require lifelong therapy and rarely result in full eradication of the virus from the liver. New treatments that can provide functional cures to chronically-infected patients are urgently needed.

Scientific Background

HBV is a partially double-stranded DNA virus, with a complex life cycle. There are multiple mechanisms associated with HBV replication that could potentially be targeted with new drugs, including mechanisms of entry, capsid assembly, cccDNA formation, transcription and secretion, as well as reverse transcriptase. In addition, host factor and immunological mechanisms could play a role as well.

Our approach to the treatment of HBV

We are initially focusing on new core inhibitors that we expect to have an impact on capsid assembly and possibly interfere with other viral processes. This approach is supported by early clinical validation, with the core inhibitor NVR 3-778 from Novira and JNJ-56136379 from Janssen demonstrating clinical reduction of viral DNA in chronic HBV patients in short-term Phase 2 clinical studies. We are making significant progress in discovering, characterizing, and securing patent protection for new core inhibitors.

Our goal is to identify a development candidate from our HBV program in 2018. In addition, we are conducting preclinical experiments with other mechanisms that target HBV.

Our Licensed HCV Protease Inhibitor Products

Background and Overview of HCV Market

HCV is a virus that is a common cause of viral hepatitis, an inflammation of the liver. HCV is typically contracted by contact with the blood or other body fluids of another individual infected with HCV. HCV is a leading cause of chronic liver disease, including cirrhosis, liver failure and cancer, and the leading cause of death from liver disease in the United States. HCV disease progression occurs over a period of 20 to 30 years, with the majority of HCV-infected individuals generally exhibiting no major symptoms in the early stages of the disease. Therefore, until a major symptom is diagnosed, many individuals are unaware they are infected and live undiagnosed without seeking treatment. For that reason, combined with the new availability of effective treatments for HCV, the United States Centers for Disease Control and Prevention, or CDC, issued new guidelines in 2013 recommending screening for all Americans born between the years 1945 and 1965 so that HCV-infected individuals will be aware of their condition and can consider treatment options.

An estimated 180 million people worldwide are chronically infected with HCV and have an increased risk of eventually developing liver cirrhosis or liver cancer. More than 350,000 people die every year from HCV-related liver diseases. The CDC estimated in 2016 that 2.7-3.9 million people in the United States are chronically infected with HCV, with an estimated 33,900 new infections in 2015, the most recent year for which the CDC has published data. We believe that the chronically infected population remains significantly untreated, even with the introduction of several new regimens beginning in 2013. With the introduction of Gilead's Harvoni® and AbbVie's VIEKIRA PAK® in late 2014, the reported worldwide sales of the five leading HCV therapies in 2015 totaled approximately $23 billion. After the introduction of Merck's 2-DAA regimen (brand name ZEPATIER™) and Gilead's pan-genotypic 2-DAA regimen (brand name Epclusa™), reported worldwide sales of the seven leading HCV therapies in 2016 totaled approximately $19 billion. Reported worldwide net sales of the eight leading HCV therapies through the nine months ended September 30, 2017 totaled approximately $10 billion. In the quarter ending September 30, 2017, AbbVie's new, pan-genotypic regimen, MAVYRET/MAVIRET, was approved in the U.S., the EU, Japan and other jurisdictions, including approval for 8-week treatment of treatment-naïve, non-cirrhotic HCV patients, and Gilead's new 3-DAA regimen, VOSEVI, was also approved as a 12-week regimen, including approval in specified DAA-experienced patients.

HCV sales have declined since their peak in 2015 due to competitive pricing pressures and a decline in the number of patients treated annually after the initial wave of diagnosed chronic HCV patients who had urgency for treatment. After the regulatory approvals of MAVYRET and VOSEVI, Johnson & Johnson and Merck announced they had terminated their development of additional HCV treatments.

The approved treatments for HCV have provided significant benefit to HCV patients. To date, these treatments have cure rates approaching 100% in several subpopulations. Medical practice defines a “cure” as the point at which there

is no quantifiable virus in a patient's blood for a sustained period of time after cessation of therapy, which is often referred to as a sustained virologic response, or SVR.

Scientific Background

Most of the currently approved HCV therapies targeting HCV focus directly on the viral life cycle and proteins that are critical to HCV replication. Replication of the HCV genome occurs on intracellular membranes and requires the participation of multiple viral proteins, some of which have enzymatic activities. Agents, often referred to as inhibitors, that target viral proteins directly are generally referred to as direct acting antivirals, or DAAs. All currently approved DAA therapies include one or a combination of two or more inhibitors of the NS3 protease, the NS5A protein, and the NS5B polymerase.

NS3 Protease. As HCV replicates, it generates long strands of protein that must be processed into many individual active functional proteins that are referred to as non-structural proteins with the designated abbreviation NS, including NS3 and NS5A. The NS3 protease is responsible for most of this protein processing of the newly translated HCV protein, and plays an essential role in the viral life cycle. Inhibition of the protease prevents these new critical proteins from forming and therefore prevents replication and survival of the virus.

NS5A. The NS5A protein has key roles in both the RNA replication of HCV and modulation of the physiology of its host cell in the body. Research has shown that targeting NS5A gives rise to profound antiviral activity, and as a result, this protein has emerged as an additional important DAA target for anti-HCV drugs.

NS5B Polymerase. HCV is a single-stranded RNA virus, and NS5B is an HCV RNA polymerase responsible for synthesis of new HCV RNA, allowing the HCV genome to be copied and the virus to survive and replicate. Two separate classes of DAA inhibitors of NS5B polymerase are used as treatments for HCV. Nucleoside/nucleotide inhibitors of NS5B directly inhibit the active site of that enzyme and prevent further elongation of the RNA, and thus are equally active against all HCV genotypes. A second class, known as non-nucleoside inhibitors, affects replication of the RNA by altering the shape of the enzyme at remote sites on the enzyme surface, with the result being that any given non-nucleoside inhibitor is usually only active against certain HCV genotypes.

Our Licensed Products in AbbVie's Marketed Therapies

Paritaprevir - The first protease inhibitor developed through our collaboration with AbbVie, paritaprevir is one DAA in AbbVie's 3-DAA regimen marketed as VIEKIRA PAK® in the U.S. (containing paritaprevir/ritonavir/ombitasvir/dasabuvir). Co-administration of paritaprevir with ritonavir, which we refer to together as paritaprevir/r, combines paritaprevir with a commonly used boosting agent that increases the blood concentrations of many protease inhibitors and has enabled once-daily dosing of paritaprevir. AbbVie developed paritaprevir/r in the 3-DAA combination with its non-nucleoside polymerase and NS5A inhibitors, with or without ribavirin, for treatment of genotype 1 HCV patients This 3-DAA combination has been sold as VIEKIRA PAK in the U.S. since December 2014, and as VIEKIRAX®+EXVIERA® in the EU since January 2015, for non-cirrhotic patients and those with early stage, or compensated, cirrhosis. AbbVie also markets a 2-DAA combination of paritaprevir with the same NS5A inhibitor for genotype 4 HCV patients in the U.S. and EU under the name VIEKIRAX, which is also approved for genotype 1 patients in Japan.

Glecaprevir - Our second protease inhibitor, glecaprevir, was developed by AbbVie in combination with pibrentasvir, AbbVie's second NS5A inhibitor. This co-formulated combination, marketed as MAVYRET (U.S.) or MAVIRET (ex-U.S.), contains two new DAAs that target and inhibit proteins essential for the replication of the hepatitis C virus. MAVYRET/MAVIRET is approved in the U.S. and the EU as an 8-week, pan-genotypic, fixed-dose combination treatment, dosed once-daily as three oral tablets, taken with food, for chronic HCV patients without

cirrhosis and new to treatment. MAVYRET/MAVIRET is also approved as a treatment for patients with specific treatment challenges, including those GT-1 patients not cured by prior treatment experience with either a protease inhibitor or an NS5A inhibitor (but not both), and in patients with limited treatment options, such as those with severe chronic kidney disease (CKD) or those with genotype 3 chronic HCV. MAVYRET/MAVIRET is approved for use in patients across all stages of CKD with any of the major HCV genotypes (GT1-6).

- The approvals of MAVYRET/MAVIRET are supported by data from nine registrational studies in AbbVie's clinical development program, which evaluated more than 2,300 patients in 27 countries across all major HCV genotypes (GT1-6) and special populations. The key studies are summarized below:

| Study Name | Patient Population | Treatment Duration | $SVR_{12}$ Rate |
|---|---|---|---|
| ENDURANCE-1 | GT1 without cirrhosis, new to treatment or not cured with previous IFN-based treatments (pegIFN +/- RBV or SOF/RBV +/- pegIFN), and patients co-infected with HIV-1 | 8 weeks | 99% (n=348/351) |
| ENDURANCE-3 | GT3 without cirrhosis, new to treatment | 8 weeks | 95% (n=149/157) |
| SURVEYOR-2 (Part 4) | GT2, 4, 5, or 6 without cirrhosis, new to treatment or not cured with previous IFN-based treatments (pegIFN, SOF/RBV or pegIFN/SOF) | 8 weeks | 97% (n=196/203) |
| EXPEDITION-1 | GT1, 2 with compensated cirrhosis (Child-Pugh A) | 12 weeks | 99% (n=145/146) |
| EXPEDITION-4 | GT1-6 chronic HCV infection and chronic kidney disease (CKD), including those on dialysis, not cured with previous treatment with sofosbuvir (SOF) plus ribavirin (RBV) or with interferon (IFN) plus RBV, with or without SOF | 12 weeks | 98% (n=102/104) |

Drug Discovery

We have internally discovered all of the compounds in our research and development programs. Our scientists have expertise in the areas of medicinal chemistry, molecular virology and pharmacology, with highly developed sets of skills in compound generation, target selection, screening and pharmacology, preclinical development and lead optimization. We are utilizing these skills and capabilities in our discovery and development of virology and liver disease product candidates.

We focus on virology and liver disease indications representing large and growing market opportunities with significant unmet medical needs. Our selection of a particular therapeutic target within those disease indications takes into consideration the experience and expertise of our scientific team and includes our ability to generate robust medicinal chemistry structure-activity relationships to assist lead optimization and secure relevant intellectual property rights. Once we have identified lead compounds, they are tested using in vitro and in vivo pharmacology studies and in vivo research models of antiviral or antibacterial efficacy.

Collaboration and License Agreement with AbbVie

We entered into a Collaborative Development and License Agreement with Abbott Laboratories in November 2006 to develop and commercialize HCV NS3 and NS3/4A protease inhibitors. The agreement, which was amended in January and December 2009, was then assigned to AbbVie Inc. on January 1, 2013 in connection with Abbott's transfer of its research-based pharmaceuticals business to AbbVie. Under the agreement, we have granted AbbVie an exclusive, worldwide, royalty-bearing license, including a right to grant sublicenses, to specified intellectual property, including several issued U.S. patents, relating to protease inhibitors. We also granted AbbVie access to our drug discovery capabilities in the HCV NS3 and NS3/4A protease inhibitor field. AbbVie granted us a co-exclusive (together with AbbVie), royalty-free, fully paid license, without the right to grant sublicenses, to certain of AbbVie's intellectual property, AbbVie's interest in joint intellectual property and improvements discovered by AbbVie, for the purpose of allowing us to conduct certain development and commercialization activities in the United States relating to protease

inhibitors. AbbVie is responsible for and has funded all costs associated with the development, manufacturing and commercialization of paritaprevir, glecaprevir and any other compounds under this agreement. Under the agreement, we are eligible to receive milestone payments and royalties with respect to these compounds. So long as a product candidate is being developed or commercialized under the agreement, we undertake not to conduct any activity, or grant licenses to a third party, relating to protease inhibitors.

A joint steering committee was established under the agreement with review and oversight responsibilities for all research, development and commercialization activities. The joint steering committee is comprised of three of our senior personnel and three senior personnel from AbbVie; however, AbbVie has final authority to make all decisions regarding development and commercialization activities.

The research program and the evaluation period, which was performed by both parties, ended in June 2011. The first commercialized compound was paritaprevir, and glecaprevir is now the second commercialized protease inhibitor. AbbVie's MAVYRET/MAVIRET, which is the fixed-dose combination of glecaprevir with pibrentasvir, received regulatory approval in the U.S., EU and Japan in the quarter ending September 30, 2017. Through September 30, 2017, we have received a total of $500.0 million from AbbVie as part of our collaboration, consisting of license payments, proceeds from a sale of preferred stock, research funding payments, milestone payments and royalties. We earned a $15.0 million milestone payment upon reimbursement approval of MAVIRET in Japan in November 2017.

We are receiving annually tiered royalties on each protease product developed under the agreement, ranging from the low double digits up to twenty percent, or on a blended basis from the low double digits up to the high teens, based on AbbVie's calendar year net sales of its HCV regimens that are allocated to the protease product in each regimen. However, if a product is determined to be a combination product, as is the case for both paritaprevir and glecaprevir, the net sales of the combination product are adjusted on a country-by-country and product-by-product basis to reflect a good faith determination of the relative value of each pharmaceutically active ingredient, based on the estimated fair market value. This means that a portion of AbbVie's worldwide annual net sales of a combination product or regimen is first allocated to one of our protease inhibitors and then that royalty-bearing portion is multiplied by the annually tiered royalty rates to determine our actual royalty for the protease product in that regimen in a given period. In October 2014, we entered into an amendment to our agreement with AbbVie to finalize the net sales allocations for regimens containing paritaprevir, as well as for any regimen containing glecaprevir. Under the terms of the agreement as amended, 50% of AbbVie's net sales of the MAVYRET/MAVIRET regimen containing glecaprevir are allocated to glecaprevir net sales for purposes of calculating royalties, whereas only 30% of net sales of a 3-DAA regimen containing paritaprevir and 45% of net sales of a 2-DAA regimen containing paritaprevir are allocated to paritaprevir net sales. These royalties are calculated separately for each protease product, and the tiered royalty rates return to the lowest tier at the start of each calendar year.

Royalties owed to us under the agreement can be reduced by AbbVie in certain circumstances, including (i) if AbbVie exercises its right to license or otherwise acquire rights to intellectual property controlled by a third party where a product could not be legally developed or commercialized in a country without the third-party intellectual property right, (ii) where a product developed under the collaboration agreement is sold in a country and not covered by a valid patent claim in such country, or (iii) where sales of a generic product are equal to at least a specified percentage of AbbVie's market share of a product in a country.

AbbVie's obligation to pay royalties on products developed under the agreement expires on a country-by-country and product-by-product basis upon the later of (i) the date of expiration of the last of the licensed patents with a valid claim covering the product in the applicable country, and (ii) ten years after the first commercial sale of the product in the applicable country.

Our intellectual property existing as of the effective date of the agreement remains our property. Any intellectual property jointly developed is jointly owned. We will have the unilateral right to enforce our patent rights on any covered product following the first commercial sale of such product, as will AbbVie. In the event of infringement related to any of our patents, we will have the first right and option to initiate legal proceedings or take other actions. In the event of infringement related to any AbbVie patents, AbbVie will have the first right and option to initiate legal proceedings or take other actions. In the event of infringement of a joint patent right, we will discuss with

AbbVie whether to initiate legal proceedings or take other actions. AbbVie will have the obligation to defend at its sole expense any actions brought against either party alleging infringement of third-party rights by reason of the activities conducted under the agreement and we will have the right to obtain separate counsel at our own expense. Additionally, AbbVie, at its sole expense, will be responsible for all trademark prosecution.

Subject to the exceptions described above, a party's rights and obligations under the agreement continue until: (i) such time as AbbVie is no longer developing a product candidate or (ii) if, as of the time AbbVie is no longer developing any product candidates, AbbVie is commercializing any other protease inhibitor product, such time as all royalty terms for all covered products and all co-development terms for all co-developed products have ended. Accordingly, the final expiration date of the agreement is currently indeterminable.

Either party may terminate the agreement for cause in the event of a material breach, subject to prior notice and the opportunity to cure, or in the event of the other party's bankruptcy. Additionally, AbbVie may terminate the agreement for any reason upon specified prior notice.

If we terminate the agreement for cause or AbbVie terminates without cause, any licenses and other rights granted to AbbVie will terminate and AbbVie will be deemed to have granted us (i) a non-exclusive, perpetual, fully paid, worldwide, royalty-free license, with the right to sublicense, under AbbVie's intellectual property used in any product candidate and (ii) an exclusive (even as to AbbVie), perpetual, fully paid, worldwide, royalty-free license, with the right to sublicense, under AbbVie's interest in joint intellectual property rights to develop product candidates resulting from covered compounds and to commercialize any products derived from such compounds. Upon our request, AbbVie will also transfer to us all right, title and interest in any related product trademarks, regulatory filings and clinical trials.

If AbbVie terminates the agreement for our uncured breach, the milestone and royalty payments payable by AbbVie may be reduced, the licenses granted to AbbVie will remain in place, we will be deemed to have granted AbbVie an exclusive license under our interest in joint intellectual property, AbbVie will continue to have the right to commercialize any covered products, and all rights and licenses granted to us by AbbVie will terminate.

Competition

We are engaged in segments of the pharmaceutical industry that are highly competitive and rapidly changing. Many large pharmaceutical and biotechnology companies, academic institutions, governmental agencies and other public and private research organizations are commercializing or pursuing the development of products that target HCV, NASH, PBC, RSV and HBV and other viral infections or liver diseases that we may target in the future.

Many of our competitors have substantially greater commercial infrastructures and financial, technical and personnel resources than we have, as well as drug candidates in late-stage clinical development. We will not be able to compete successfully unless we are able to:

- design and develop products that are superior to other products in the market;
- attract qualified scientific, medical, sales and marketing and commercial personnel;
- obtain patent and/or other proprietary protection for our processes and product candidates;
- obtain required regulatory approvals; and
- collaborate with others in the development and commercialization of new products.

Established competitors may invest heavily to quickly discover and develop novel compounds that could make our product candidates obsolete. In addition, any new product that competes with an approved product must demonstrate compelling advantages in efficacy, convenience, tolerability and safety, or some combination of these factors, in order to overcome price competition and to be commercially successful.

We expect AbbVie's HCV treatment regimens containing any of our licensed protease inhibitors to continue to face intense competition from existing approved products in the HCV market. AbbVie's HCV treatment regimens currently face competition in various world markets and subpopulations of HCV. The principal competitive

treatments are Gilead's Sovaldi® (sofosbuvir), Harvoni® (a fixed-dose combination of sofosbuvir and ledipasir), Epclusa® (a fixed dose combination of sofosbuvir and velpatasvir) and Vosevi™ (a triple combination therapy of sofosbuvir, velpatasvir and voxilaprevir approved by the FDA in July 2017 for specified sofosbuvir -treatment failures and NS5A-inhibitor treatment failures); Merck's Zepatier® (a fixed-dose combination of grazoprevir and elbasvir); with limited competition in certain sub-populations in specific geographies from Bristol-Myers Squibb's Daklinza™ (daclatasvir) and daclatasvir in combination with asunaprevir; and Johnson & Johnson's Olysio® (simeprevir).

Competitive products in the form of other treatment methods or a vaccine for HCV may render AbbVie's products obsolete or noncompetitive. AbbVie's marketed HCV regimens that contain one of our collaboration's protease inhibitors will face competition based on their safety and effectiveness, reimbursement coverage, price, patent position, marketing and sales capabilities, and other factors. If any of the products developed under our collaboration agreement with AbbVie face competition from generic products, the collaboration agreement provides that the royalty rate applicable to such product is reduced significantly by a specified percentage on a product-by-product, country-by-country basis. If AbbVie is not able to compete effectively against current and future competitors for AbbVie's HCV products, our financial condition, operations and stock price will suffer.

We also expect our product candidates in other disease areas to face intense and increasing competition as new products continue to enter the NASH and antiviral markets and advanced technologies become available. Though there is currently no approved treatment for NASH, we expect significant competition from other companies in the development of new treatments for NASH and related conditions. We are aware of several companies with programs that are significantly more advanced than ours, including companies with compounds in Phase 3 in NASH, namely Intercept, Genfit, Gilead, and Allergan (Tobira). In May 2016, the FDA granted conditional approval for Intercept's FXR agonist (brand name Ocaliva®) for the treatment of primary biliary cholangitis (PBC) in combination with first line therapy UDCA in adults with an inadequate response to UDCA, or as monotherapy in adults unable to tolerate UDCA. In addition, a number of companies are in Phase 2 clinical trials for NASH or related conditions. These companies include Alberio, Arisaph, Astra-Zeneca, BMS, Boehringer Ingelheim, Conatus, Cirius, Galectin, Galmed, Gilead, GlaxoSmithKline, Immuron, Inventiva, Madrigal, Medicinova, Novartis, NGM, Novo Nordisk, Pfizer, Shire, and Viking. A significant number of other companies are conducting earlier clinical trials that may be applicable in NASH and other cholestatic diseases, including Bird Rock Bio, Arena Pharmaceuticals, Fast Forward Pharmaceuticals, Can-Fite Biopharma, Cymabay, Durect, Genkyotex, Ionis, and Islet. There are also additional companies conducting preclinical studies in these disease areas.

Similarly, HBV and RSV represent competitive therapeutic areas. While there are effective antiviral medications prescribed for HBV, they generally have low true cure rates. Many companies are seeking to develop new HBV drugs that alone or in combination with other mechanisms could lead to a functional cure of HBV. Arbutus, Gilead, HEC, Ionis, Johnson & Johnson, Maxwell, Replicor, Roche and Spring Bank have Phase 2 programs in progress, with many of these companies conducting earlier stage programs as well. In addition, a number of companies have Phase 1 or earlier stage HBV programs, including Aicuris, Alnylam, Altimmune, Assembly, Enyo and Transgene.

For RSV, there are currently no safe and effective therapies for already established RSV infection. Several companies are seeking new antiviral treatments for RSV infection in adult and pediatric settings. Ark Bioscienes, Johnson & Johnson, Gilead and ReViral have compounds in Phase 2 development, as does Ablynx with a potential therapeutic antibody. Earlier stage, small molecule programs have also been reported by Medivir and Pulmocide. A prophylactic, monoclonal-antibody-based treatment from MedImmune, which is commercialized by AbbVie outside of the U.S., is approved for infants considered at high risk for RSV infection; however studies have found that most young children affected by RSV infection were previously healthy, and thus would not normally be prescribed prophylactic treatment. In addition, a number of companies have RSV vaccines in development, primarily directed at prevention of RSV infection, and some companies are also evaluating vaccines in a therapeutic mode for treatment of established RSV infection.

If we are not able to develop new products that can compete effectively against our current and future competitors, our business will not grow and our financial condition, operations and stock price will suffer.

Intellectual Property

As part of our business strategy, we actively seek patent protection for our product candidates in the United States and certain major foreign jurisdictions and file additional patent applications, when appropriate, to cover improvements to our compounds. We also rely on trade secrets, internal know-how, technological innovations and agreements with third parties to develop, maintain and protect our competitive position. Our ability to be competitive will depend on the success of this strategy.

Each of our major programs, including HCV, NASH, PBC, HBV and RSV, typically has several issued patents and pending patent claims in the program area containing claims to compounds, methods of use and processes for synthesis. However, only a few of the issued patents and/or pending patent applications cover the lead product candidate in a given program.

NASH, HBV and RSV Programs. Our patent portfolio directed to FXR agonists for NASH, PBC and fibrosis, core inhibitors for HBV and N-protein inhibitors for RSV includes pending U.S. patent applications as well as numerous foreign patent applications.

HCV NS3 Protease Inhibitor Program. The patent portfolio directed to the HCV protease inhibitor program with AbbVie includes U.S. patents and foreign patents, as well as non-provisional applications. The issued U.S. composition-of-matter patent covering paritaprevir is expected to expire in 2031. The issued U.S. composition-of-matter patent covering glecaprevir is expected to expire in 2032. AbbVie is a joint owner of a number of these patent applications. AbbVie also has rights to some or all of these patents and patent applications pursuant to its collaboration agreement with us.

We may obtain patents for certain compounds many years before we obtain marketing approval for products containing such compounds. Because patents have a limited life, which usually begins to run well before the first commercial sale of the related product, the commercial value of the patent may be limited. However, we may be able to apply for patent term extensions in the United States and in a number of European countries, compensating in part for delays in obtaining marketing approval, but we cannot be certain we will obtain such extensions.

It is also very important that we do not infringe patents or other proprietary rights of others. If we do infringe such patents or other proprietary rights, we could be prevented from developing or selling products or from using the processes covered by those patents, could be required to pay substantial damages, or could be required to obtain a license from the third party to allow us to use their technology, which may not be available on commercially reasonable terms or at all. If we were not able to obtain a required license or develop alternative technologies, we may be unable to develop or commercialize some or all of our products, and our business could be adversely affected.

In addition, we jointly own patent applications, together with AbbVie, that claim paritaprevir and glecaprevir as a chemical entity. However, there is no guarantee that such applications will issue. Further, the existence of issued patents does not guarantee our right to practice the patented technology or commercialize the patented product. Third parties may have already or could obtain rights to patents that could be used to prevent or attempt to prevent us from commercializing our product candidates. If these other parties are successful in obtaining valid and enforceable patents, and establishing our infringement of those patents, we could be prevented from commercializing our product candidates unless we were able to obtain a license under such patents, which may not be available on commercially reasonable terms or at all.

Much of our scientific capabilities depend upon the knowledge, experience and skills of key scientific and technical personnel. To protect our rights to our proprietary know-how and technology, we endeavor to require all employees, as well as our consultants and advisors, when feasible, to enter into confidentiality agreements that require disclosure

and assignment to us of ideas, developments, discoveries and inventions made by these employees, consultants and advisors in the course of their service to us.

We may be unable to obtain, maintain and protect the intellectual property rights necessary to conduct our business, and we may be subject to claims that we infringe or otherwise violate the intellectual property rights of others,

which could materially harm our business. For more information, see "Risk Factors—Risks Related to Our Intellectual Property Rights."

Government Regulation

Government authorities in the United States, at the federal, state and local level, and in other countries extensively regulate, among other things, the research, development, testing, manufacture, quality control, approval, labeling, packaging, storage, record-keeping, promotion, advertising, distribution, post-approval monitoring and reporting, marketing and export and import of products such as those we develop. Any pharmaceutical candidate that we develop must be approved by the FDA before it may be legally marketed in the United States and by the appropriate foreign regulatory agency before it may be legally marketed in foreign countries.

United States Drug Development Process

In the United States, the FDA regulates drugs under the Federal Food, Drug and Cosmetic Act, or FDCA, and implementing regulations. Drugs are also subject to other federal, state and local statutes and regulations. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources. Failure to comply with the applicable United States requirements at any time during the product development process, approval process or after approval, may subject an applicant to administrative or judicial sanctions. FDA sanctions could include refusal to approve pending applications, withdrawal of an approval, a clinical hold, warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement or civil or criminal penalties. Any agency or judicial enforcement action could have a material adverse effect on us.

The process required by the FDA before a drug may be marketed in the United States generally involves the following:

- Completion of preclinical laboratory tests, animal studies and formulation studies according to Good Laboratory Practice, or GLPs, or other applicable regulations;
- Submission to the FDA of an Investigational New Drug Application, or an IND, which must become effective before human clinical trials may begin;
- Performance of adequate and well-controlled human clinical trials according to the FDA's current Good Clinical Practice, or GCPs, to establish the safety and efficacy of the proposed drug for its intended use;
- Submission to the FDA of a New Drug Application, or an NDA, for a new drug product;
- Satisfactory completion of an FDA inspection of the manufacturing facility or facilities where the drug is to be produced to assess compliance with the FDA's current Good Manufacturing Practice standards, or cGMP, to assure that the facilities, methods and controls are adequate to preserve the drug's identity, strength, quality and purity;
- Potential FDA audit of the nonclinical and clinical trial sites that generated the data in support of the NDA; and
- FDA review and approval of the NDA.

The lengthy process of seeking required approvals, which can often take anywhere from six months from the time the NDA is filed if there is a priority review for a breakthrough therapy to twelve months for a standard review, and the continuing need for compliance with applicable statutes and regulations require the expenditure of substantial resources. There can be no certainty that approvals will be granted.

Before testing any compounds with potential therapeutic value in humans, the product candidate enters the preclinical testing stage. Preclinical tests include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies to assess the potential safety and activity of the product candidate. The conduct of the preclinical tests must comply with GLP and other federal regulations and requirements. The sponsor

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must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and a proposed clinical protocol, to the FDA as part of the IND. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA places the clinical trial on a clinical hold within that 30-day time period. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. The FDA may also impose clinical holds on a drug at any time before or during clinical trials due to safety concerns or non-compliance. Accordingly, we cannot assure that submission of an IND will result in the FDA allowing clinical trials to begin, or that, once begun, issues will not arise that result in suspension or termination of such trial.

Clinical trials involve the administration of the product candidate to healthy volunteers or patients having the disease being studied under the supervision of qualified investigators, generally physicians not employed by or under the trial sponsor's control. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection and exclusion criteria, and the parameters to be used to monitor subject safety. Each protocol must be submitted to the FDA as part of the IND. Clinical trials must be conducted in accordance with the FDA's GCP requirements. Further, each clinical trial must be reviewed and approved by an independent institutional review board, or IRB, at or servicing each institution at which the clinical trial will be conducted. An IRB is charged with protecting the welfare and rights of trial participants and considers such items as whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the informed consent form that must be provided to each clinical trial subject or his or her legal representative and must monitor the clinical trial until it is completed.

Human clinical trials prior to approval are typically conducted in three sequential phases that may overlap or be combined:

- Phase 1. The drug is initially introduced into healthy humans and tested for safety, dosage tolerance, absorption, metabolism, distribution and excretion. In the case of some products for severe or life-threatening diseases, especially when the product may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted only in patients having the specific disease.
- Phase 2. The drug is evaluated in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance, optimal dosage and dosing schedule for patients having the specific disease.
- Phase 3. Clinical trials are undertaken to further evaluate dosage, clinical efficacy and safety in an expanded patient population at geographically dispersed clinical trial sites. These clinical trials, which usually involve more patients than earlier trials, are intended to establish the overall risk/benefit ratio of the product and provide an adequate basis for product labeling. Generally, at least two adequate and well-controlled Phase 3 clinical trials are required by the FDA for approval of an NDA.

Post-approval studies, or Phase 4 clinical trials, may be conducted after initial marketing approval. These studies are used to gain additional experience from the treatment of patients in the intended therapeutic indication and may be required by the FDA as part of the approval process.

Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and written IND safety reports must be submitted to the FDA by the investigators for serious and unexpected adverse events or any finding from tests in laboratory animals that suggests a significant risk for human patients. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, if at all. The FDA, or the sponsor or its data safety monitoring board, may suspend a clinical trial at any time on various grounds, including a finding that the research patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug has been associated with unexpected serious harm to patients.

Concurrent with clinical trials, companies usually complete additional animal studies and develop additional information about the chemistry and physical characteristics of the drug as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things,

must include methods for testing the identity, strength, quality and purity of the final drug. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

U.S. Review and Approval Processes

The results of product development, preclinical studies and clinical trials, along with descriptions of the manufacturing process, analytical tests conducted on the chemistry of the drug, proposed labeling and other relevant information are submitted to the FDA as part of an NDA requesting approval to market the product. The submission of an NDA is subject to the payment of substantial user fees by the applicant; a waiver of such fees may be obtained under certain limited circumstances.

In addition, under the Pediatric Research Equity Act, or PREA, an NDA or supplement to 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 product is safe and effective. The FDA may grant deferrals for submission of pediatric data or full or partial waivers.

The FDA reviews all NDAs submitted before it accepts them for filing and may request additional information rather than accepting an NDA for filing. Once the submission is accepted for filing, the FDA begins an in-depth review of the NDA. Under the goals and policies agreed to by the FDA under the Prescription Drug User Fee Act, or PDUFA, the FDA has twelve months in which to complete its initial review of a standard NDA and respond to the applicant, and six months for a priority NDA. The FDA does not always meet its PDUFA goal dates for standard and priority NDAs.

After the NDA submission is accepted for filing, the FDA reviews the NDA to determine, among other things, whether the proposed product is safe and effective for its intended use and whether the product is being manufactured in accordance with cGMP to assure and preserve the product's identity, strength, quality and purity. In addition to its own review, the FDA may refer applications for novel drug products or drug products that present 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 and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions. During the approval process, the FDA also will determine whether a Risk Evaluation and Mitigation Strategy, or REMS, is necessary to assure the safe use of the drug. If the FDA concludes that a REMS is needed, the sponsor of the NDA must submit a proposed REMS; the FDA will not approve the NDA without a REMS, if required.

Before approving an NDA, the FDA will inspect the facilities at which the product is to be manufactured. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and are adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA will typically inspect one or more clinical sites to assure compliance with GCP. If the FDA determines that the application, manufacturing process or manufacturing facilities are not acceptable it will outline the deficiencies in the submission and often will request additional testing or information.

The NDA review and approval process is lengthy and difficult, and the FDA may refuse to approve an NDA if the applicable regulatory criteria are not satisfied or may require additional clinical data or other data and information. Even if such data and information is submitted, the FDA may ultimately decide that the NDA does not satisfy the criteria for approval. Data obtained from clinical trials are not always conclusive and may be susceptible to varying interpretations, which could delay, limit or prevent regulatory approval. The FDA will issue a "complete response" letter

if the agency decides not to approve the NDA. The complete response letter usually describes all of the specific deficiencies in the NDA identified by the FDA. The deficiencies identified may be minor, for example, requiring labeling changes, or major, for example, requiring additional clinical trials. Additionally, the complete response letter may include recommended actions that the applicant might take to place the application in a condition for approval. If a complete response letter is issued, the applicant may either resubmit the NDA, addressing all of the deficiencies identified in the letter, or withdraw the application.

If a product receives regulatory approval, the approval may be limited to specific diseases and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling. In addition, the FDA may require Phase 4 testing, which involves clinical trials designed to further assess a product's safety and effectiveness and may require testing and surveillance programs to monitor the safety of approved products that have been commercialized.

Expedited Development and Review Programs

The FDA has four programs intended to expedite the development and review of new drugs addressing unmet medical needs or treating serious or life-threatening conditions: fast track, breakthrough therapy, priority review, and accelerated approval.

The FDA "fast track" program is intended to expedite or facilitate the process for reviewing new products to treat serious or life-threatening conditions and address unmet medical needs. Fast track designation applies to the combination of the product and the specific indication for which it is being studied. Under the fast track program, the sponsor will have more frequent interactions with the FDA during drug development, and may also submit sections of the NDA on a rolling basis to the FDA for review before submitting the complete application. Fast track does not guarantee that a product will be reviewed more quickly or receive FDA approval.

The FDA "breakthrough therapy" program is intended to expedite the development and review of drugs for serious or life-threatening conditions. Preliminary clinical evidence must show that the drug may have substantial improvement over existing therapies on one or more clinically significant endpoints. Although the drug does not have to address an unmet medical need, designation of breakthrough therapy status carries all the "fast track" program features. Additionally, the breakthrough therapy program entitles the sponsor to earlier and more frequent interaction with the FDA review team regarding development of nonclinical and clinical data, and allows the FDA to offer product development and regulatory advice necessary to shorten the time for product approval. The breakthrough therapy status does not guarantee a quicker development or review of the product, and does not ensure FDA approval.

The FDA also has a "priority review" program for products offering significant improvement in the treatment, diagnosis or prevention of a disease. The goal of the priority review program is to shorten the review period to six months from the ten months required for standard review. Any drug with breakthrough therapy, accelerated approval designation, or fast track can be granted priority review if it meets the necessary criteria.

The FDA "accelerated approval" program is intended to expedite the development and review of products with the potential to treat serious or life-threatening illnesses and provide meaningful therapeutic benefit over existing treatments. The program allows approval of a product on the basis of adequate and well-controlled clinical studies establishing that the product has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or on the basis of an effect on a clinical endpoint that can be measured earlier than survival or irreversible morbidity. As a condition of approval, the FDA generally requires that a sponsor of the product perform adequate and well-controlled post-marketing clinical studies to establish safety and efficacy for the approved indication. Failure to conduct such studies or failure of the studies to establish required safety and efficacy may result in revocation of approval. The FDA also requires, as a condition for accelerated approval, pre-approval of promotional materials, which could adversely impact the timing of the commercial launch or subsequent marketing of the product.

Post-Approval Requirements

Any drug products for which we receive FDA approvals are subject to continuing regulation by the FDA. Certain requirements include, among other things, record-keeping requirements, reporting of adverse experiences with the

product, providing the FDA with updated safety and efficacy information on an annual basis or more frequently for specific events, product sampling and distribution requirements, complying with certain electronic records and signature requirements and complying with FDA promotion and advertising requirements. These promotion and advertising requirements include, among others, standards for direct-to-consumer advertising, prohibitions against promoting drugs for uses or in patient populations that are not described in the drug's approved labeling (known as

"off-label use"), rules for conducting industry-sponsored scientific and educational activities and promotional activities involving the internet. Failure to comply with FDA requirements can have negative consequences, including the immediate discontinuation of noncomplying materials, adverse publicity, enforcement letters from the FDA, mandated corrective advertising or communications with doctors, and civil or criminal penalties. Although physicians may prescribe legally available drugs for off-label uses, manufacturers may not market or promote such off-label uses.

We rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of our product candidates. Manufacturers of our product candidates are required to comply with applicable FDA manufacturing requirements contained in the FDA's cGMP regulations. These regulations require, among other things, quality control and quality assurance as well as the corresponding maintenance of comprehensive records and documentation. Drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are also required to register their establishments and list any products they make with the FDA and to comply with related requirements in certain states. These entities are further subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP and other laws. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain cGMP compliance. Discovery of problems with a product after approval may result in serious and extensive restrictions on a product, manufacturer or holder of an approved NDA. These restrictions may include suspension of a product until the FDA is assured that quality standards can be met, continuing oversight of manufacturing by the FDA under a "consent decree," which frequently includes the imposition of costs and continuing inspections over a period of many years, as well as possible withdrawal of the product from the market. In addition, changes to the manufacturing process generally require prior FDA approval before being implemented. Other types of changes to the approved product, such as adding new indications and additional labeling claims, are also subject to further FDA review and approval.

The FDA also may require post-marketing testing, known as Phase 4 testing, as well as risk minimization action plans and surveillance to monitor the effects of an approved product or place conditions on an approval that could otherwise restrict the distribution or use of the product.

U.S. Patent Term Restoration and Marketing Exclusivity

Drug Price Competition and Patent Term Restoration Act of 1984

Depending upon the timing, duration and specifics of the FDA approval of the use of our product candidates, some of our United States patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during federal regulatory review preceding the FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period is generally one-half the time between the effective date of an IND and the submission date of an NDA plus the time between the submission date of an NDA and the approval of that application. Only one patent applicable to an approved drug is eligible for the extension and the application for the extension must be submitted within 60 days of approval, prior to the expiration of the patent. The United States Patent and Trademark Office, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may apply for restoration of patent term for one of our currently owned or licensed patents to add patent life beyond its current expiration date, depending on the expected length of the clinical trials and other factors involved in the filing of the relevant NDA. However, there is no guarantee that any such application will be approved.

Federal Food, Drug and Cosmetic Act ("FDCA")

Market exclusivity provisions under the FDCA, which are independent of patent status and any patent related extensions, can also delay the submission or the approval of certain applications of other companies seeking to reference another company's NDA. If the new drug is a new chemical entity subject to an NDA, the FDCA provides a five-year period of non-patent marketing exclusivity within the United States to the first applicant to obtain approval of an NDA for a new chemical entity. A drug is a new chemical entity if the FDA has not previously

approved any other new drug containing the same active moiety, which is the molecule or functional group of a molecule responsible for the action of the drug substance. During the exclusivity period, the FDA may not accept for review an abbreviated new drug application, or ANDA, or a so-called Section 505(b)(2) NDA, submitted by another company for another version of such drug where the applicant does not own or have a legal right of reference to all the data required for approval. However, such an application may be submitted after four years if it contains a certification of patent invalidity or non-infringement to one of the patents listed with the FDA by the innovator NDA holder. The FDCA also provides three years of marketing exclusivity for an NDA, or supplement to an existing NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example new indications, dosages or strengths of an existing drug. This three-year exclusivity covers only the conditions associated with the new clinical investigations and does not prohibit the FDA from approving ANDAs for drugs containing the original active agent. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA. However, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

Other U.S. Healthcare Laws and Compliance Requirements

In the United States, our activities are potentially subject to regulation by various federal, state and local authorities in addition to the FDA, including the Centers for Medicare and Medicaid Services (formerly the Health Care Financing Administration), other divisions of the United States Department of Health and Human Services (e.g., the Office of Inspector General), the United States Department of Justice and individual United States Attorney offices within the Department of Justice, state attorney generals and state and local governments. For example, sales, marketing and scientific/educational grant programs must comply with the anti-fraud and abuse provisions of the Social Security Act, the False Claims Act, the privacy and security provisions of the Health Insurance Portability and Accountability Act, or HIPAA, and similar state laws, each as amended. Pricing and rebate programs must comply with the Medicaid rebate requirements of the Omnibus Budget Reconciliation Act of 1990 and the Veterans Health Care Act of 1992, each as amended. If products are made available to authorized users of the Federal Supply Schedule of the General Services Administration, additional laws and requirements apply. Under the Veterans Health Care Act, or VHCA, drug companies are required to offer certain pharmaceutical products at a reduced price to a number of federal agencies including the United States Department of Veterans Affairs and United States Department of Defense, the Public Health Service and certain private Public Health Service—designated entities in order to participate in other federal funding programs including Medicare and Medicaid. Recent legislative changes purport to require that discounted prices be offered for certain United States Department of Defense purchases for its TRICARE program via a rebate system. Participation under the VHCA requires submission of pricing data and calculation of discounts and rebates pursuant to complex statutory formulas, as well as the entry into government procurement contracts governed by the Federal Acquisition Regulations.

In order to distribute products commercially, we must comply with state laws that require the registration of manufacturers and wholesale distributors of pharmaceutical products in a state, including, in certain states, manufacturers and distributors who ship products into the state even if such manufacturers or distributors have no place of business within the state. Some states also impose requirements on manufacturers and distributors to establish the pedigree of product in the chain of distribution, including some states that require manufacturers and others to adopt new technology capable of tracking and tracing product as it moves through the distribution chain. Several states have enacted legislation requiring pharmaceutical companies to establish marketing compliance programs, file periodic reports with the state, make periodic public disclosures on sales, marketing, pricing, clinical trials and other activities, and/or register their sales representatives, as well as to prohibit pharmacies and other healthcare entities from providing certain physician prescribing data to pharmaceutical companies for use in sales and marketing, and to prohibit certain other sales and marketing practices. All of our activities are potentially subject to federal and state consumer protection and unfair competition laws.

Europe / Rest of World Government Regulation

In addition to regulations in the United States, we will be subject to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales and distribution of our products.

Whether or not we obtain FDA approval for a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. Certain countries outside of the United States have a similar process that requires the submission of a clinical trial application much like the IND prior to the commencement of human clinical trials. In the European Union, for example, a clinical trial application, or CTA, must be submitted to each country's national health authority and an independent ethics committee, much like the FDA and IRB, respectively. Once the CTA is approved in accordance with a country's requirements, clinical trials may proceed.

The requirements and process governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, the clinical trials are conducted in accordance with International Conference on Harmonisation (ICH) / WHO Good Clinical Practice standards and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

To obtain regulatory approval of an investigational drug under European Union regulatory systems, we must submit a marketing authorization application to the European Medicines Agency, or the EMA. The application used to file an NDA in the United States is similar to that required in the European Union, with the exception of, among other things, country-specific document requirements.

For other countries outside of the European Union, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, again, the clinical trials are conducted in accordance with GCPs and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Pharmaceutical Coverage, Pricing and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any product candidates for which we obtain regulatory approval. In the United States and markets in other countries, sales of any products for which we receive regulatory approval for commercial sale will depend in part on the availability of reimbursement from third-party payors. Third-party payors include government health administrative authorities, managed care providers, private health insurers and other organizations. The process for determining whether a payor will provide coverage for a drug product may be separate from the process for setting the price or reimbursement rate that the payor will pay for the drug product. Third-party payors may limit coverage to specific drug products on an approved list, or formulary, which might not include all of the FDA-approved drug products for a particular indication. Third-party payors are increasingly challenging the price and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. We may need to conduct expensive pharmaco-economic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain the FDA approvals. Our product candidates may not be considered medically necessary or cost-effective. A payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development.

In 2003, the United States government enacted legislation providing a partial prescription drug benefit for Medicare recipients, which became effective at the beginning of 2006. Government payment for some of the costs of prescription drugs may increase demand for any products for which we receive marketing approval. However, to obtain payments under this program, we would be required to sell products to Medicare recipients through

prescription drug plans operating pursuant to this legislation. These plans will likely negotiate discounted prices for our products. Federal, state and local governments in the United States continue to consider legislation to limit the growth of healthcare costs, including the cost of prescription drugs. Future legislation could limit payments for pharmaceuticals such as the product candidates that we are developing.

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, collectively known as the Affordable Care Act, substantially changed the way healthcare is financed by both governmental and private insurers, and significantly impacted the pharmaceutical industry.

The comprehensive overhaul has extended coverage to approximately 20 million previously uninsured Americans. Since its adoption, the Affordable Care Act contains a number of provisions, including those governing enrollment in federal healthcare programs, reimbursement changes and fraud and abuse, which have affected existing government healthcare programs and have resulted in the development of new programs, including Medicare payment for performance initiatives and improvements to the physician quality reporting system and feedback program. Additionally, the Affordable Care Act, as limited by the United States Supreme Court's decision in June 2012:

- increases the minimum level of Medicaid rebates payable by manufacturers of brand-name drugs from 15.1% to 23.1%;
- requires collection of rebates for drugs paid by Medicaid managed care organizations;
- requires manufacturers to participate in a coverage gap discount program, under which they 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, beginning January 2011; and
- imposes a non-deductible annual fee on pharmaceutical manufacturers or importers who sell "branded prescription drugs" to specified federal government programs.

There have been proposals by President Trump and the Republican majorities in both houses of the U.S. Congress to repeal or replace all or portions of the Affordable Care Act but to date no such legislation has been agreed upon. At this time, it remains unclear what legislation, if any, to repeal or replace the Affordable Care Act will become law, or what impact any such legislation may have on our existing product candidates, any of our future product candidates or AbbVie's commercialization of its HCV regimens. The full impact that the Affordable Care Act and other new laws will have on our business remains uncertain.

Different pricing and reimbursement schemes exist in other countries. In the European Union, governments influence the price of pharmaceutical products through their pricing and reimbursement rules and control of national healthcare systems that fund a large part of the cost of those products to consumers. Some jurisdictions operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed. To obtain reimbursement or pricing approval, some of these countries may require the completion of clinical trials that compare the cost-effectiveness of a particular product candidate to currently available therapies. Other member states allow companies to fix their own prices for medicines, but monitor and control company profits. The downward pressure on healthcare costs in general, particularly prescription drugs, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross-border imports from low-priced markets exert a commercial pressure on pricing within a country.

The marketability of any drug candidates for which we receive regulatory approval for commercial sale may suffer if the government and third-party payers fail to provide adequate coverage and reimbursement. In addition, emphasis on managed care in the United States has increased and we expect will continue to increase the pressure on pharmaceutical drug pricing. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

Manufacturing

We do not have our own manufacturing capabilities, except with respect to limited amounts of active pharmaceutical ingredients needed for preclinical development. In the past, we have relied on third-party manufacturers for supply of

active pharmaceutical ingredients, and we expect that in the future we will rely on such manufacturers for the supply of ingredients that will be used in clinical trials of our product candidates that we are developing ourselves. Manufacturing for paritaprevir and glecaprevir are conducted by AbbVie. We do not expect to establish our own

manufacturing facilities and we will continue to rely on third-party manufacturers to produce commercial quantities of any product candidates that we commercialize ourselves. We believe that all of the materials required for the manufacture of those product candidates could be obtained from more than one source.

Sales and Marketing

We currently do not have any commercialization or sales and marketing capabilities, and currently have no fixed plans to invest in or build such capabilities internally. We have partnered our products for HCV with AbbVie. We may also partner or collaborate with, or license commercial rights to, other larger pharmaceutical or biopharmaceutical companies to support the development of one or more of our wholly owned product candidates through late-stage clinical development and, if successful, commercialization. However, we still retain all commercial rights to our independent programs and we will continue to evaluate our alternatives for commercializing them once they are more advanced in their clinical development.

Employees

As of September 30, 2017, we had 89 full-time employees, 53 of whom hold Ph.D. or M.D. degrees. None of our employees are subject to a collective bargaining agreement or represented by a trade or labor union. We consider our relations with our employees to be good.

Available Information

Our Internet website address is http://www.enanta.com. Through our website, we make available, free of charge, our annual report on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K and any amendments to those reports, as well as proxy statements, and, from time to time, other documents as soon as reasonably practicable after we electronically file such material with, or furnish it to, the Securities and Exchange Commission, or SEC. These SEC reports can be accessed through the “Investors” section of our website. The information found on our website is not part of this or any other report we file with or furnish to the SEC.

Investors may read and copy any materials we file with the SEC at the SEC’s Public Reference Room at 100 F Street, NE, Washington, DC 20549. You may obtain information on the operation of the Public Reference Room by calling the SEC at 1-800-SEC-0330. The SEC also maintains an Internet website that contains reports, proxy and information statements, and other information regarding Enanta Pharmaceuticals, Inc. and other issuers that file electronically with the SEC. The SEC’s Internet website address is http://www.sec.gov.

ITEM 1A. RISK FACTORS

RISK FACTORS

Our business faces significant risks and uncertainties. Certain factors may have a material adverse effect on our business prospects, financial condition and results of operations, and you should carefully consider them. Accordingly, in evaluating our business, we encourage you to consider the following discussion of risk factors, in its entirety, in addition to other information contained in or incorporated by reference into this Annual Report on Form 10-K and our other public filings with the SEC. Other events that we do not currently anticipate or that we currently deem immaterial may also affect our business, prospects, financial condition and results of operations.

Risks Related to Our Business

Our financial prospects for the next several years are dependent upon the development and commercialization efforts of AbbVie for combination therapies incorporating our protease inhibitors paritaprevir or glecaprevir for the treatment of HCV. AbbVie may act in its best interest rather than in our best interest, which could adversely affect our business.

We rely on AbbVie to fund and conduct the clinical development and commercialization of regimens containing paritaprevir or glecaprevir (our second protease inhibitor, which is one of the two DAAs in AbbVie's MAVYRET/MAVIRET treatment), over which we have granted AbbVie complete control. Our ability to generate significant revenue in the near term will depend primarily on the success of AbbVie's continued efforts to commercialize its paritaprevir-containing regimens in markets worldwide, as well as the successful marketing launch and commercialization by AbbVie of MAVYRET/MAVIRET. Such successes are subject to significant uncertainty, and we have no control over the resources, time and effort that AbbVie may devote to its regimens containing paritaprevir or glecaprevir. Any of several events or factors could have a material adverse effect on our ability to generate revenue from AbbVie's commercialization of paritaprevir or glecaprevir in combination therapies. For example, AbbVie:

- may not achieve satisfactory levels of market acceptance and reimbursement by physicians, patients and third-party payers for the MAVYRET/MAVIRET regimen in the various markets of the world where it is being introduced and sold by AbbVie;
- may not compete successfully with its MAVYRET/MAVIRET regimen against other products and therapies for HCV;
- may experience different competitive challenges and market opportunity for its paritaprevir-containing regimens as it begins to commercialize its MAVYRET/MAVIRET HCV regimen containing glecaprevir;
- may have to comply with additional requests and recommendations from the FDA, including label restrictions for its regimens containing paritaprevir or glecaprevir;
- may not make all regulatory filings and obtain all necessary approvals from foreign regulatory agencies and all commercially necessary reimbursement approvals;
- may not commit sufficient resources to the marketing and distribution of MAVYRET/MAVIRET, whether for competitive or strategic reasons or otherwise due to a change in business priorities;
- may cease to perform its obligations under the terms of our collaboration agreement;
- may unilaterally terminate our collaboration agreement on specified prior notice without any reason and without any further commitment; and

- may not be able to manufacture paritaprevir or glecaprevir in compliance with requirements of the FDA and similar foreign regulatory agencies and in commercial quantities sufficient to meet market demand

We do not have access to all information regarding the HCV regimens being commercialized by AbbVie, including certain information about spontaneous safety reports for any marketed product, regulatory affairs, process development, manufacturing, marketing, sales and other areas known by AbbVie. Thus, our ability to keep our stockholders informed about the status of products licensed under our collaboration is limited by the degree to which AbbVie keeps us informed. If AbbVie does not perform in the manner we expect or fulfill its responsibilities in a timely manner, or at all, the global commercialization of MAVYRET/MAVIRET could be delayed or terminated or be commercially unsuccessful. In addition, AbbVie has the right to make decisions regarding the development and commercialization of licensed product without consulting us, and may make decisions with which we do not agree. If AbbVie acts in a manner that is not in our best interest, then it could adversely affect our business and prospects.

Our royalty revenues are derived from AbbVie's net sales of regimens to treat HCV. If AbbVie is unable to increase and maintain sales of these regimens above current levels of sales, our royalty revenues and results of operations would be adversely affected.

Our quarterly royalty revenue from AbbVie's net sales of HCV treatment regimens containing paritaprevir has declined since those sales peaked in the first quarter of our fiscal 2016. AbbVie has priced the MAVYRET/MAVIRET regimen well below the pricing of its first HCV regimens, and below that of its principal competitor, which means AbbVie will need to capture significant increases in market share to maintain or increase its HCV net sales and our royalty revenues. While commercialization of these regimens is exclusively in AbbVie's control without any input from us, we believe it is possible that prices will decline further due to payers obtaining additional discounts, and there may be a decline in AbbVie's market share over time due to competitive actions by its principal competitor. We also note that AbbVie's principal competitor in HCV, Gilead, has reported a decline year over year across all major geographic markets in the number of new patients starting on DAA treatments for HCV.

In addition, in light of continued fiscal crises experienced by several countries in the European Union and Japan, governments have announced or implemented measures to manage and reduce healthcare expenditures. AbbVie may experience global pricing pressure for its HCV regimens from such measures, which may be reflected in larger discounts or rebates on its regimens or delayed reimbursement. Also, private and public payers may choose to exclude AbbVie's regimens from their formulary coverage lists or limit the types of patients for whom coverage will be provided. Any such change in formulary coverage, discounts or rebates or reimbursement for AbbVie's HCV regimens would negatively affect the demand for such regimens and our royalty revenues from them.

We and AbbVie face substantial competition in the markets for HCV drugs, and there are many companies developing potential therapies for NASH, PBC, HBV and RSV, as well as other liver diseases and viral infections, which may result in others discovering, developing or commercializing products before we do or doing so more successfully than we do.

The pharmaceutical and biotechnology industries are intensely competitive and rapidly changing. Many large pharmaceutical and biotechnology companies, academic institutions, governmental agencies and other public and private research organizations are commercializing or pursuing the development of products that target HCV, NASH, PBC, HBV, RSV and other viral infections or liver diseases that we may target in the future.

Many of our competitors have substantially greater commercial infrastructure and greater financial, technical and personnel resources than we have, as well as drug candidates in late-stage clinical development.

In all the disease areas currently under the focus of our research and development efforts, there are other companies with more product candidates that are more advanced than ours. Our competitors may succeed in developing these product candidates or others and obtaining regulatory approval before we can do so with any of our product candidates. If we are not “first to market” with one of our product candidates in one or more of these disease indications, our competitive position could be compromised because it may be more difficult for us to obtain

marketing approval for that product candidate and market acceptance of that product candidate as a follow-on competitor. In addition, any new product that competes with an approved product typically must demonstrate compelling advantages in efficacy, convenience, tolerability or safety, or some combination of these factors, in order to gain regulatory approvals, overcome price competition and be commercially successful.

We expect AbbVie's HCV treatment regimens containing any of our licensed protease inhibitors to continue to face intense competition due to existing approved products in the HCV market. AbbVie's HCV treatment regimens currently face competition in various world markets and subpopulations of HCV from Gilead's Sovaldi® (sofosbuvir), Harvoni® (a fixed-dose combination of sofosbuvir and ledipasvirm), Epclusa® (a fixed dose combination of sofosbuvir and velpatasvir) and Vosevi™ (a triple combination therapy of sofosbuvir, velpatasvir and voxilaprevir approved by the FDA in July 2017 for specified sofosbuvir -treatment failures and NS5A-inhibitor treatment failures); Merck's Zepatier® (a fixed-dose combination of grazoprevir and elbasvir); Bristol-Myers Squibb's Daklinza™ (daclatasvir) and daclatasvir in combination with asunaprevir; and Johnson & Johnson's Olysio® (simeprevir). Competitive products in the form of other treatment methods or a vaccine for HCV may render AbbVie's HCV regimens obsolete or noncompetitive. AbbVie's regimens that contain one of our collaboration's protease inhibitors will face competition based on their safety and effectiveness, reimbursement coverage, price, patent position, AbbVie's marketing and sales capabilities, and other factors. If any of AbbVie's HCV regimens face competition from generic products, the collaboration agreement provides that the royalty rate applicable to our protease product contained in the regimen is reduced significantly by a specified percentage on a product-by-product, country-by-country basis. If AbbVie is not able to compete effectively against its competitors in HCV, our business will not grow and our financial condition, operations and stock price will suffer.

We also expect our other product candidates to face intense and increasing competition as new products continue to enter the NASH and antiviral markets and advanced technologies become available. Though there is currently no approved treatment for NASH, we expect significant competition from other companies in the development of new treatments for NASH and related conditions. We are aware of several companies with programs that are significantly more advanced than ours, including companies with compounds in Phase 3 in NASH, namely Intercept, Genfit, Gilead, and Tobira (Allergan). In May 2016, the FDA granted conditional approval for Intercept's FXR agonist (brand name Ocaliva®) for the treatment of primary biliary cholangitis (PBC) in combination with first line therapy UDCA in adults with an inadequate response to UDCA, or as monotherapy in adults unable to tolerate UDCA. In addition, a number of companies are in Phase 2 clinical trials for NASH or related conditions. These companies include Alberio, Arisaph, Astra-Zeneca, BMS, Boehringer Ingelheim, Conatus, Cirius, Galectin, Galmed, Gilead, GlaxoSmithKline, Immuron, Inventiva, Madrigal, Medicinova, Novartis, NGM, Novo Nordisk, Pfizer, Shire, and Viking. A significant number of other companies are conducting earlier clinical trials that may be applicable in NASH and other cholestatic diseases, including Bird Rock Bio, Arena Pharmaceuticals, Fast Forward Pharmaceuticals, Can-Fite Biopharma, Cymabay, Durect, Genkyotex, Ionis, and Islet. There are also additional companies conducting preclinical studies in these disease areas.

Similarly, HBV and RSV represent competitive therapeutic areas. While there are effective antiviral medications prescribed for HBV, they generally have low true cure rates. Many companies are seeking to develop new HBV drugs that alone or in combination with other mechanisms could lead to a functional cure of HBV. Arbutus, Gilead, HEC, Ionis, Johnson & Johnson, Maxwell, Replicor, Roche and Spring Bank have Phase 2 programs in progress, with many of these companies conducting earlier stage programs as well. In addition, a number of companies have Phase 1 or earlier stage HBV programs, including Aicuris, Alnylam, Altimmune, Assembly, Enyo and Transgene.

For RSV, there are currently no safe and effective therapies for already established RSV infection. Several companies are seeking new antiviral treatments for RSV infection in adult and pediatric settings. Ark Bioscienes, Johnson & Johnson, Gilead and ReViral each have compounds in Phase 2 development, as does Ablynx with a potential therapeutic antibody. Earlier stage, small molecule programs have also been reported by Medivir and Pulmocide. A

prophylactic, monoclonal-antibody-based treatment from MedImmune, which is commercialized by AbbVie outside of the U.S., is approved for infants considered at high risk for RSV infection; however studies have found that most young children with RSV infection were previously healthy, and thus would not normally be prescribed prophylactic treatment. In addition, a number of companies have RSV vaccines in development, primarily directed at prevention of RSV infection, and some companies are also evaluating vaccines in a therapeutic mode for treatment of established RSV infection.

If we are not able to develop new products that can compete effectively against our current and future competitors, our business will not grow and our financial condition, operations and stock price will suffer.

We have not developed independently any approved products and we have limited clinical development experience, which makes it difficult to assess our ability to develop and commercialize our product candidates.

AbbVie has been and will continue to be responsible for all of the clinical development of our paritaprevir and glecaprevir protease inhibitor product candidates. We have not yet demonstrated an ability to address successfully many of the risks and uncertainties associated with late stage clinical development, regulatory approval and commercialization of therapeutic products such as the ones we plan to develop independently. For example, to execute our business plan for development of our independent NASH, PBC, HBV and RSV programs, we will need to successfully:

- execute clinical development of our product candidates and demonstrate acceptable safety and efficacy for them alone or in combination with other drugs or drug candidates;
- obtain required regulatory approvals for the development and commercialization of our product candidates;
- develop and maintain any future collaborations we may enter into for any of these programs;
- obtain and maintain patent protection for our product candidates and freedom from infringement of intellectual property of others;
- establish acceptable commercial manufacturing arrangements with third-party manufacturers;
- build and maintain robust sales, distribution and marketing capabilities, either independently or in collaboration with future collaborators;
- gain market acceptance for our product candidates among physicians, payers and patients; and
- manage our spending as costs and expenses increase due to clinical trials, regulatory approvals and commercialization.

If we are unsuccessful in accomplishing these objectives, we may not be able to successfully develop and commercialize our product candidates and expand our business or continue our operations.

If we are not successful in discovering further product candidates in addition to EDP-305 and EDP-938, our ability to expand our business and achieve our strategic objectives will be impaired.

Much of our internal research is at preclinical stages. Research programs designed to identify product candidates require substantial technical, financial and human resources, whether or not any product candidates are ultimately identified. Our research programs may initially show promise in identifying additional potential product candidates, yet fail to yield product candidates for clinical development or commercialization for many reasons, including the following:

- the research methodology used may not be successful in identifying additional potential product candidates;
- competitors may develop alternatives that render our product candidates less commercially viable or obsolete;
- competitors may obtain intellectual property protection that effectively prevents us from developing a product candidate;
- a product candidate may, on further study, be shown not to be an effective treatment in humans or to have harmful side effects or other characteristics that indicate it is unlikely to be effective or otherwise does not meet applicable regulatory criteria; and
- a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all.

Additional drug candidates that we may develop will require significant research, preclinical and clinical studies, regulatory approvals and commitments of resources before they can be commercialized. We cannot give assurance that our research will lead to the discovery of any additional drug candidates that will generate additional revenue for us. If we are unable to identify additional compounds suitable for preclinical and clinical development, we may not be able to obtain sufficient product revenue in future periods, which likely would result in significant harm to our financial position and adversely impact our stock price.

Expenses associated with development of our product candidates may cause our results of operations to fluctuate from period to period, which may result in losses.

Many of the preclinical and clinical development activities required for our product candidates have to be contracted out to contract research organizations (CROs) at significant expense. We expect these expenses to increase substantially in the coming year as we advance compounds and conduct more clinical studies. It is difficult to accurately predict the timing and amounts of these expenses, and we expect that they will vary from quarter to quarter. In addition, the FDA or other regulatory agencies may require more preclinical or clinical testing than we originally anticipated for any of our product candidates. We may also be required to purchase expensive competitor drugs for use in our trials, either to demonstrate potential treatment combinations or as comparators to our product candidates. As a result, the expenses of our development programs and our operating results may fluctuate significantly from quarter to quarter, and our stock price may be adversely affected.

If we fail to attract and keep senior management and key scientific personnel, we may be unable to successfully develop our product candidates, conduct our clinical trials and commercialize our product candidates.

Our success depends in part on our continued ability to attract, retain and motivate highly qualified management, clinical and scientific personnel. We are highly dependent upon our senior management, particularly Jay R. Luly, Ph.D., our Chief Executive Officer and President, Yat Sun Or, Ph.D., our Senior Vice President, Research and Development and Chief Scientific Officer, and Nathalie Adda, M.D., our Senior Vice President, Chief Medical Officer, as well as other employees and consultants. Although none of these individuals has informed us to date that he or she intends to retire or resign in the near future, the loss of the services of any of these individuals or one or more of our other members of senior management could delay or prevent the successful development of our product candidates.

Although we have not historically experienced unique difficulties attracting and retaining qualified employees, we could experience such problems in the future. For example, competition for qualified personnel in the biotechnology and pharmaceutical fields is intense. In addition, we will need to hire additional personnel as we expand our clinical development and commercial activities. We may not be able to attract and retain quality personnel on acceptable terms.

We may encounter difficulties in managing our growth and expanding our operations successfully.

As we expand our research efforts and seek to advance our product candidates through clinical trials, we will need to expand our development, regulatory, manufacturing, marketing and sales capabilities or contract with third parties to provide these capabilities for us. As our operations expand, we expect that we will need to manage additional relationships with various strategic partners, suppliers and other third parties. Future growth will impose significant added responsibilities on members of management. Our future financial performance and our ability to commercialize our product candidates and to compete effectively will depend, in part, on our ability to manage any future growth effectively. To that end, we must be able to manage our development efforts and clinical trials effectively and hire, train and integrate additional management, administrative and sales and marketing personnel. We may not be able to accomplish these tasks, and our failure to accomplish any of them could prevent us from successfully growing

our company.

To date, our principal sources of revenue have been our collaboration agreements, including our current agreement with AbbVie. Future levels of royalties under the AbbVie agreement are uncertain. We have had no other products approved for commercial sale by us. Therefore, it is possible that we may incur operating losses in one or more years in the future, and our ability to achieve sustained profitability is unproven.

In each of our 2017, 2016, 2015 and 2014 fiscal years our net income resulted primarily from license payments, including milestone payments we earned from AbbVie and royalties we earned since December 2014 on net sales of AbbVie's HCV regimens allocated to our protease inhibitors included in those regimens. There is no assurance, however, that we will report net income in subsequent years. To date, we have not commercialized any products ourselves.

Our principal source of revenue historically has been our collaboration agreements, including our current agreement with AbbVie. The level of future royalties on products containing paritaprevir or glecaprevir are uncertain given the competitive nature of the market for HCV therapies, the emergence of new therapies for HCV, price competition, the changing nature of payer contracts of AbbVie and others, and the varying rates of reimbursement in different countries. At any time, AbbVie may choose not to continue its commercialization activities for the MAVYRET/MAVIRET regimen or that regimen may not be accepted in the market. If we are unable to develop and commercialize any more of our product candidates, either alone or with a collaborator, or if any such product candidate does not achieve market acceptance, we may not generate sufficient product sales or product royalties. In addition, for any of our product candidates included in a treatment regimen with more than one active compound, it would be uncertain what portion of net sales of the regimen would be allocated to our product candidate. Even if we do generate significant product royalties or product sales, we may not be able to sustain profitability on a quarterly or annual basis. Our failure to sustain profitability could depress the market price of our common stock and ultimately could impair our ability to raise capital, expand our business, diversify our product offerings or continue our operations. A decline in the market price of our common stock also could cause you to lose all or a part of your investment.

We may require substantial additional financing in the longer term to achieve our goals if the further commercialization of paritaprevir containing regimens is curtailed or if any launch or commercialization of MAVYRET/MAVIRET is delayed or is not successful. A failure to obtain this necessary capital when needed could force us to delay, limit, reduce or terminate some or all of our product development efforts.

Since our inception, most of our resources have been dedicated to the discovery and preclinical development of our product candidates. In particular, we have expended, and believe that we will continue to expend for the foreseeable future, substantial resources discovering and developing our proprietary product candidates. These expenditures will include costs associated with research and development, preclinical manufacturing of product candidates, conducting preclinical experiments and clinical trials and obtaining regulatory approvals, as well as commercializing any products later approved for sale. For the foreseeable future, we expect to incur substantial additional costs associated with research and development for our internally developed programs, exclusive of costs incurred by AbbVie in developing MAVYRET/MAVIRET. In addition, we may seek opportunities to in-license or otherwise acquire new therapeutic candidates and therapies.

Our future capital requirements depend on many factors, including:

- whether our existing collaboration continues to generate substantial royalties to us;
- the number and characteristics of the product candidates we pursue;
- the scope, progress, results and costs of researching and developing any of our product candidates on our own, including conducting preclinical research and clinical trials;
- opportunities to in-license or otherwise acquire new therapeutic candidates and therapies;

- the timing, receipt and amount of royalties on paritaprevir and glecaprevir and any sales of our product candidates, if any, or royalties thereon;
- the timing of, and the costs involved in, obtaining regulatory approvals for any product candidates we develop independently;

- the cost of commercialization activities, if any, of any product candidates we develop independently that are approved for sale, including marketing, sales and distribution costs;
- the cost of manufacturing our product candidates and any products we successfully commercialize independently, including manufacturing for clinical development;
- our ability to maintain our existing collaboration and to establish new collaborations, licensing or other arrangements and the financial terms of such agreements; and
- the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing patents, including any litigation costs and the outcomes of any such litigation.

Additional funds may not be available if and when we need them, on terms that are acceptable to us, or at all. Our ability to raise funds will depend on financial, economic and market conditions and other factors, many of which are beyond our control. If adequate funds are not available to us on a timely basis, we may be required to delay, limit, reduce or terminate preclinical studies, clinical trials or other research and development activities for one or more of our product candidates.

Our government funded contract for our antibiotic program, which was concluded in fiscal 2015, is subject to audit and adjustments that could affect our previously reported revenues.

Our contract with the National Institute of Allergy and Infectious Diseases, or NIAID, a division of the National Institutes of Health, an agency of the United States Department of Health and Human Services, to support our antibiotic program, was completed in fiscal 2015. Our contract-related costs and fees, including allocated indirect costs, are subject to audits and adjustments by negotiation between us and the U.S. government. As part of the audit process, the government audit agency verifies that all charges made by a contractor against a contract are legitimate and appropriate. Audits may result in recalculation of contract revenues and non-reimbursement of some contract costs and fees. Any audits of our contract related costs and fees could result in material adjustments to our reported revenue and require payments by us to the U.S. government.

Risks Related to Development, Clinical Testing and Regulatory Approval of Our Product Candidates

Clinical drug development involves a lengthy and expensive process with uncertain timelines and uncertain outcomes. If clinical trials of any of our proprietary product candidates are prolonged or delayed, we may be unable to commercialize our product candidates on a timely basis.

Clinical testing is expensive and, depending on the stage of development, can take a substantial time period to complete. Its outcome is inherently uncertain, and failure can occur at any time during clinical development. None of our product candidates in our pipeline other than paritaprevir and glecaprevir, which have been clinically developed by AbbVie, has yet to advance beyond Phase 2 clinical trials. Any future clinical trials of our product candidates may fail to demonstrate sufficient safety and efficacy. Moreover, regulatory and administrative delays for any product candidate in our pipeline may adversely affect our or any future collaborator’s clinical development plans and jeopardize our or any future collaborator’s ability to attain product approval, commence product sales and compete successfully against other therapies.

Clinical trials can be delayed for a variety of reasons, including delays related to:

- reaching agreement on acceptable terms with prospective contract research organizations, or CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- failure of third-party contractors, such as CROs, or investigators to comply with regulatory requirements;
- delay or failure in obtaining the necessary approvals from regulators or institutional review boards, or IRBs, in order to commence a clinical trial at a prospective trial site, or their suspension or termination of a clinical trial once

commenced;
- difficulty in recruiting suitable patients to participate in a trial;

- difficulty in having patients complete a trial or return for post-treatment follow-up;
- clinical sites deviating from trial protocol or dropping out of a trial;
- problems with drug product or drug substance storage and distribution;
- adding new clinical trial sites;
- our inability to manufacture, or obtain from third parties, adequate supply of drug product sufficient to complete our preclinical studies and clinical trials;
- governmental or regulatory delays and changes in regulatory requirements, policy and guidelines, including guidelines specifically addressing requirements for the development of treatments for NASH, PBC, RSV or HBV;
- program discontinuations or clinical holds for a program of a competitor, which could increase the level of regulatory scrutiny or delay data review or other response times by regulators with respect to one of our programs in the same class as the competitor's program; or
- varying interpretations of data by the FDA, the EMA and similar foreign regulatory agencies.

We could also encounter delays if a clinical trial is suspended or terminated by us, by the IRBs of the institutions in which such trial is being conducted, by any Data Safety Monitoring Board, or DSMB, for such trial, or by the FDA, the EMA 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. In addition, delays can occur due to safety concerns arising from trials or other clinical data regarding another company's product candidate in the same compound class as one of ours. If we or any future collaborators experience delays in the completion of, or termination of, any clinical trial of one of our product candidates, the commercial prospects of the product candidate will be harmed, and our ability to commence product sales and generate product revenues from the product candidate will be delayed. In addition, any delays in completing our clinical trials will increase our costs and slow down our product candidate development and approval process. Any of these occurrences may harm our business, financial condition and prospects significantly. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

We may choose to test any of our clinical candidates preclinically and/or clinically in combination with other compounds with different mechanisms of action, and any adverse results from such testing may have adverse consequences for the further development potential of not only the combination but also the clinical candidate itself as a monotherapy or in combination with other mechanisms of action.

We expect that the further development of successful therapies in our principal disease areas of NASH, RSV and HBV may require combining one or more of our compounds with other compounds with different mechanisms of action. To advance our programs and achieve favorable opportunities for any such combinations we may conduct preclinical testing, as well as clinical testing, with one of our other compounds or with a compound of a third party, with or without a longer-term collaboration with any such party. We may choose to disclose such testing in advance, but we can anticipate that some of the testing would be done without any public disclosure. If any such testing produces adverse results, we may have to disclose it to regulatory authorities as part of the data available with respect to our product candidate and the data may have adverse consequences for the further development and the ultimate conditions attached to any approved use of the product candidate, whether in the combination tested or even as a monotherapy or in combination with other mechanisms.

EDP-305, EDP-938 or any other product candidate emerging from our current NASH, PBC, RSV and HBV programs may have undesirable side effects which may delay or prevent marketing approval, or, if approval is received, require our product candidate to be taken off the market, require us to include safety warnings or otherwise limit sales.

In our NASH/PBC program, we are developing agonists of the farnesoid X receptor, or FXR, that are designed to bind to that receptor and then trigger a response from it. The adverse effects from long-term exposure to the FXR drug class are not well known since within this class only two drugs have been approved by the FDA—Ocaliva®, approved in May 2016 for PBC, and an older drug not commonly used but approved to treat cholesterol gallstones (by dissolving them) and a rare lipid storage disease. Unforeseen side effects from any of our product candidates could arise either during clinical development or, if approved, after the approved product has been marketed. The range and potential severity of possible side effects from systemic therapies like FXR agonists could be significant.

In addition, our drug candidates for NASH may be developed as a potential treatment for a severe disease that commonly occurs in patients with other serious conditions, including metabolic syndrome and diabetes. Any clinical trials in NASH will necessarily be conducted in patient populations that may be more prone than the general population to exhibit certain disease states or adverse events. It may be difficult to discern whether certain events or symptoms observed during our trials were due to our drug candidates or placebo, resulting in our company and our development programs being negatively affected even if such events or symptoms are ultimately determined to be unlikely related to our drug candidates.

If any of our product candidates receives marketing approval and we or others later identify undesirable or unacceptable side effects caused by such products:

- regulatory authorities may require the addition of labeling statements, specific warnings, a contraindication or field alerts to physicians and pharmacies;
- we may be required to change instructions regarding the way the product is administered, conduct additional clinical trials or change the labeling of the product;
- we may be subject to limitations on how we may promote the product;
- sales of the product may decrease significantly;
    - regulatory authorities may require us to take our approved product off the market;
- we may be subject to litigation or product liability claims; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the affected product or could substantially increase commercialization costs and expenses, which in turn could delay or prevent us from generating significant revenue from the sale of any product we develop.

If we are required to suspend or discontinue clinical trials due to side effects or other safety risks associated with our product candidates, or if we are required to conduct studies on the long-term effects associated with the use of any of those product candidates, commercialization any of those product candidates could be delayed or halted.

Clinical trials involving our product candidates may be suspended or terminated at any time for a number of safety-related reasons. For example, we may voluntarily suspend or terminate clinical trials if at any time one of our product candidates, or a combination therapy including any of them, presents an unacceptable safety risk to the clinical trial patients. In addition, IRBs or regulatory agencies may order the temporary discontinuation or termination of clinical trials at any time if they believe that the clinical trials are not being conducted in accordance with applicable regulatory requirements, including if they present an unacceptable safety risk to patients. Administering any product candidate to humans may produce undesirable side effects. The existence of undesirable side effects resulting from any of our product candidates, or a combination therapy including any of them, could cause us or

regulatory authorities, such as the FDA or EMA, to interrupt, delay or halt clinical trials of our product candidates and could result in the FDA or EMA or other regulatory agencies denying further development or

approval of our product candidates for any or all targeted indications. This, in turn, could prevent us from commercializing our product candidates.

Results of earlier clinical trials may not be predictive of the results of later-stage clinical trials.

The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials, if any. In addition, results of Phase 3 clinical trials in one or more ethnic groups are not necessarily indicative of results in other ethnic groups. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy results despite having progressed through preclinical studies and initial clinical trials. Many companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to adverse safety profiles or lack of efficacy, notwithstanding promising results in earlier studies. Similarly, future clinical trial results may not be successful for these or other reasons.

Product candidate development risk is heightened by any changes in the planned clinical trials compared to the completed clinical trials. As product candidates are developed through preclinical early and late stage clinical trials towards approval and commercialization, it is customary that various aspects of the development program, such as manufacturing and formulation, are altered along the way in an effort to optimize processes and results. Such changes carry the risk that they will not achieve these intended objectives. Any of these changes could make the results of planned clinical trials or other future clinical trials we may initiate less predictable and could cause our product candidates to perform differently, which could delay completion of clinical trials, delay approval of our product candidates and/or jeopardize our ability to commence product sales and generate revenues.

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

The regulatory approval process is expensive and, while the time required to gain FDA and foreign regulatory approval is uncertain, it may take years. Regulatory approvals are unpredictable and depend upon numerous factors, including the substantial discretion of the regulatory authorities. In addition, approval policies, regulations, or the type and amount of preclinical and clinical data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions. We may be required to undertake and complete certain additional preclinical studies to generate toxicity and other data required to support the submission of a New Drug Application, or NDA, to the FDA or comparable application to other regulatory authorities. AbbVie obtained all regulatory approvals for its paritaprevir-containing regimens and for MAVYRET/MAVIRET, which contains glecaprevir. We have not obtained regulatory approval by ourselves for any of our wholly owned product candidates and it is possible that none of our existing product candidates or any of our future product candidates will ever obtain regulatory approval. Furthermore, approval in the United States by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA.

Our product candidates could fail to receive regulatory approval for many reasons, including the following:

- the FDA, the EMA or other comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- we may be unable to demonstrate to the satisfaction of the FDA, the EMA or other comparable foreign regulatory authorities that a product candidate is safe and effective for its proposed indication;
- the results of clinical trials may not meet the level of statistical significance required by the FDA, the EMA or other comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;

- the FDA, the EMA or other comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials;

- the data collected from clinical trials of our product candidates may not be sufficient to support the submission of an NDA or other submissions or to obtain regulatory approval in the United States or elsewhere;
- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies of any of our product candidates; and
- the approval policies or regulations of the FDA, the EMA or other comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

We cannot be assured that after spending substantial time and resources, we will obtain regulatory approvals in any desired jurisdiction. Even if we were to obtain approval, regulatory authorities may grant approval contingent on the performance of costly post-marketing clinical trials, or may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate. Significant clinical trial delays could allow our competitors to obtain marketing approval before we do or could in effect shorten the patent protection period during which we may have the exclusive right to commercialize our product candidates. In addition, it may ultimately not be possible to achieve the prices intended for our products. In many foreign countries, including those in the European Union, a product candidate must be approved for reimbursement before it can be approved for sale in that country. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates and our business.

Even if we receive regulatory approval for any of our product candidates we develop independently, we will be subject to ongoing FDA obligations and continued regulatory review in other jurisdictions, which may result in significant additional expense. Additionally, our product candidates, if approved, could be subject to labeling and other restrictions and market withdrawal and we may be subject to penalties if we or our collaborators fail to comply with regulatory requirements or experience unanticipated problems with our products.

Any regulatory approvals that we receive for our product candidates we develop independently may be subject to limitations on the approved indicated uses for which the product may be marketed or subject to certain conditions of approval, or may contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the safety and efficacy of the product candidate.

In addition, if the FDA approves any of our product candidates, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion and recordkeeping for the product will be subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, as well as continued compliance with current good manufacturing practices, or cGMP, and good clinical practices, or GCP, for any clinical trials that we or our collaborators conduct post-approval. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in, among other things:

- restrictions on the marketing or manufacturing of the product, withdrawal of the product from the market or voluntary or mandatory product recalls;
- fines, warning letters or holds on any post-approval clinical trials;
- refusal by the FDA to approve pending applications or supplements to approved applications filed by us, or suspension or revocation of product license approvals;
- product seizure or detention, or refusal to permit the import or export of products; and
- injunctions or the imposition of civil or criminal penalties.

We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we, or AbbVie in the case of any licensed HCV product, are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if

we or AbbVie are not able to maintain regulatory compliance, our product candidates or AbbVie's

licensed HCV products may lose any marketing approval that may have been obtained and we may not achieve or sustain profitability, which would adversely affect our business.

We may delay or terminate the development of a product candidate at any time if we believe the perceived market or commercial opportunity does not justify further investment, which could materially harm our business and adversely affect our stock price.

Even though the results of preclinical studies and clinical trials that we have conducted or may conduct in the future may support further development of one or more of our product candidates, we may delay, suspend or terminate the future development of a product candidate at any time for strategic, business, financial or other reasons, including the determination or belief that the emerging profile of the product candidate is such that it may not receive regulatory approvals in key markets, gain meaningful market acceptance, otherwise provide any competitive advantages in its intended indication or market or generate a significant return to stockholders. Such a delay, suspension or termination could materially harm our business, results of operations or financial condition. In addition, AbbVie has the right to make decisions regarding the development and commercialization of paritaprevir and glecaprevir without consulting us, and may make decisions with which we do not agree.

Risks Related to Commercialization of Our Product Candidates

Even if AbbVie successfully commercializes MAVYRET/MAVIRET, or even if we are able to commercialize any other treatment regimen containing one of our product candidates from any of our proprietary discovery programs, MAVYRET/MAVIRET or the resulting products, as the case may be, may become subject to unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives in the United States, which would harm our business.

The regulations that govern marketing approvals, pricing and reimbursement for new drug products vary widely from country to country. In the United States, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act of 2010, collectively referred to as the ACA, is significantly changing the way healthcare is financed by both governmental and private insurers. While we cannot predict what impact on federal reimbursement policies this law or any amendment to it will continue to have in general or specifically on any product or regimen that we may commercialize, the ACA or any such amendment may result in downward pressure on pharmaceutical reimbursement, which could negatively affect market acceptance of new products. In addition, although the United States Supreme Court has upheld the constitutionality of most of the ACA, several states have not implemented certain sections of the ACA, including 19 that have rejected the expansion of Medicaid eligibility for low income citizens, and some members of the U.S. Congress are still working to repeal the ACA. More recently, President Trump and the Republican majorities in both houses of the U.S. Congress have been seeking to repeal or replace all or portions of the ACA but to date they have been unable to agree on any such legislation. We cannot predict what legislation, if any, to repeal or replace the ACA will become law, or what impact any such legislation may have on us or on AbbVie’s commercialization of its HCV regimens.

Our ability to commercialize any product candidate successfully, as well as AbbVie’s commercialization of MAVYRET/MAVIRET, will also depend in part on the extent to which reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A primary trend in the U.S. healthcare industry is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. In the case of HCV, limitations of coverage have recently been used to limit access to HCV treatments for only those patients with more advanced fibrosis. Increasingly, third-party payors are requiring that drug companies provide them with predetermined

discounts from list prices and, in many cases involving HCV drugs, seeking discounts in exchange for greater patient access to a particular HCV drug. In addition, there are private and public payors challenging the prices charged for medical products. We cannot be sure that reimbursement will be available for any product that we may commercialize and, if reimbursement is available, the level of reimbursement. In addition, reimbursement may impact the demand for, or the price of, MAVYRET/MAVIRET or any product candidate for which we may obtain marketing approval. If reimbursement is not available or is available only to limited levels, AbbVie may not be successful in commercializing MAVYRET/MAVIRET and we may not be able to successfully commercialize any product candidate for which we may seek marketing approval.

There may be significant delays in obtaining reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the drug is approved by the FDA or comparable authorities in other jurisdictions. Moreover, eligibility for reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Interim reimbursement levels for new drugs, if applicable, may also be insufficient to cover our and any collaborator's costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. AbbVie's inability to promptly obtain coverage and profitable payment rates from both government-funded and private payors for MAVYRET/MAVIRET, or our inability to do the same for any product candidate that we develop, could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

In general, the United States and several other jurisdictions are considering a number of legislative and regulatory proposals to change the healthcare system in ways that could affect our ability to sell our products profitably. 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 to healthcare. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives. We expect to experience pricing pressures in connection with the sale of any products that we develop or that are being commercialized under our collaboration with AbbVie. The implementation of cost containment measures or other healthcare reforms may limit our ability to generate revenue, maintain profitability or commercialize our product candidates.

If, in the future, we are unable to establish our own sales, marketing and distribution capabilities or enter into licensing or collaboration agreements for these purposes, we may not be successful in commercializing any product candidates.

We do not have a sales or marketing infrastructure and have no sales, marketing or distribution experience. We will seek to either build our own commercial infrastructure to commercialize any products if and when they are approved, or enter into licensing or collaboration agreements where our collaborator is responsible for commercialization, as in the case of our collaboration with AbbVie, or where we have the right to assist in the future development and commercialization of such products.

To develop internal sales, distribution and marketing capabilities, we will have to invest significant amounts of financial and management resources, some of which will be committed prior to any confirmation that any of our proprietary product candidates will be approved. For product candidates for which we decide to perform sales, marketing and distribution functions ourselves, we could face a number of additional risks, including:

- our inability to recruit and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe any products;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

Where and when appropriate, we may elect to utilize contract sales forces or distribution partners to assist in the commercialization of our product candidates. If we enter into arrangements with third parties to perform sales,

marketing and distribution services for our products, the resulting revenues or the profitability from these revenues to us are likely to be lower than if we had sold, marketed and distributed our products ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell, market and distribute our product

candidates or may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties, and any of these third parties may fail to devote the necessary resources and attention to sell, market and distribute our products effectively.

If we do not establish sales, marketing and distribution capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates.

Our commercial success depends upon significant market acceptance among physicians, patients and healthcare payors of MAVYRET/MAVIRET, as well as similar market acceptance of any product candidates we are developing independently.

MAVYRET/MAVIRET, as well as EDP-305, EDP-938, or any other product candidate that we may develop in the future, whether as part of a combination therapy or as a monotherapy, may not gain market acceptance among physicians, healthcare payors, patients and the medical community. The degree of market acceptance of MAVYRET/MAVIRET or of any product candidate for which we obtain approval for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of treatment regimens containing one of our product candidates, as demonstrated in clinical trials, and the degree to which these regimens represent a clinically meaningful improvement in care as compared with other available therapies;
- the clinical indications for which any treatment regimen containing one of our product candidates become approved;
- acceptance among physicians, major operators of clinics, payors and patients of any treatment regimen containing one of our product candidates;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the potential and perceived advantages of treatment regimens containing one of our product candidates over alternative treatments;
- the cost of treatment of regimens containing one of our product candidates in relation to the cost of alternative treatments;
- the availability of adequate reimbursement and pricing by third parties and government authorities and successful negotiation of favorable agreements with payors by us or any collaborator of ours, as well as the impact of any agreements among any of the foregoing and one or more of our competitors limiting access to our product in favor of one or more competitive products;
- the continued longevity of the HCV drug market or growth and longevity of any other market for which we develop a drug;
- the levels of funding provided by government-funded healthcare for HCV treatment or treatment of any other disease for which we develop a drug;
- the relative convenience and ease of administration of any treatment regimen containing one of our product candidates compared to competitive regimens;
- the prevalence and severity of adverse side effects, whether involving the use of treatment regimens containing one of our products candidates or similar, competitive treatment regimens; and
- the effectiveness of our sales and marketing efforts and those of AbbVie in the case of MAVYRET/MAVIRET.

If treatment regimens containing one of our product candidates are approved and then fail to achieve market acceptance, we may not be able to generate significant additional revenue. Further, if new, more favorably received therapies are introduced after any such regimen achieves market acceptance, then we may not be able to maintain that market acceptance over time.

Foreign governments tend to impose strict price controls, which may adversely affect our future profitability.

In most foreign countries, particularly in the European Union, prescription drug pricing and/or reimbursement is subject to governmental control. In those countries that impose price controls, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies.

Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we (or AbbVie in the case of MAVYRET/MAVIRET) might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenues that are generated from the sale of the product in that country. If reimbursement of MAVYRET/MAVIRET or of any of our product candidates is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, or if there is competition from lower priced cross-border sales, our results of operations will be negatively affected.

Risks Related to Our Dependence on Third Parties

We may not be successful in establishing new product collaborations, which could adversely affect our ability to develop and commercialize one or more of our product candidates. If we are unsuccessful in maintaining or forming alliances on favorable terms, our business may not succeed.

We may seek to enter into additional product collaborations in the future, including alliances with other biotechnology or pharmaceutical companies, to enhance and accelerate the development and commercialization of one or more of our product candidates. We face significant competition in seeking appropriate collaborators and the negotiation process is time-consuming and complex. Moreover, we may not be successful in our efforts to establish other product collaborations or other alternative arrangements for any product candidates and programs because our research and development pipeline may be insufficient, our product candidates and programs may be deemed to be at too early of a stage of development for collaborative effort and/or third parties may not view our product candidates and programs as having the requisite potential to demonstrate safety and efficacy. Even if we are successful in our efforts to establish product collaborations, the terms that we agree upon may not be favorable to us and we may not be able to maintain such product collaborations if, for example, development or approval of a product candidate is delayed or sales of an approved product are disappointing.

If our existing collaboration agreement with AbbVie is terminated, or if we determine that entering into other product collaborations is in our best interest but we either fail to enter into, delay in entering into or fail to maintain such collaborations:

- the development of certain of our product candidates may be terminated or delayed;
- our cash expenditures related to development of certain of our product candidates would increase significantly and we may need to seek additional financing;
- we may be required to hire additional employees or otherwise develop expertise, such as clinical, regulatory, sales and marketing expertise, which we do not currently have;
- we will bear all of the risk related to the development of any such product candidates; and
- the competitiveness of any product candidate that is commercialized could be reduced.

We intend to rely on third-party manufacturers to produce our development-stage product candidate supplies and any commercial supplies of any approved product candidates. Any failure by a third-party manufacturer to produce

acceptable supplies for us may delay or impair our ability to initiate or complete our clinical trials or sell any resulting product.

We do not currently own or operate any manufacturing facilities. We plan to continue to work with third-party contract manufacturers to produce sufficient quantities of any product candidates for preclinical testing, clinical

trials and commercialization. If we are unable to arrange for such a third-party manufacturing source for any of our product candidates, or fail to do so on commercially reasonable terms, we may not be able to successfully produce, develop and market one or more of our product candidates, or we may be delayed in doing so.

Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured product candidates ourselves, including reliance on the third party for regulatory compliance and quality control and assurance, volume production, the possibility of breach of the manufacturing agreement by the third party because of factors beyond our control (including a failure to synthesize and manufacture our product candidates in accordance with our product specifications) and the possibility of termination or nonrenewal of the agreement by the third party at a time that is costly or damaging to us. In addition, the FDA and other regulatory authorities require that our product candidates be manufactured according to cGMP and similar foreign standards. Pharmaceutical manufacturers and their subcontractors are required to register their facilities and/or products manufactured at the time of submission of the marketing application and then annually thereafter with the FDA and certain state and foreign agencies. They are also subject to periodic unannounced inspections by the FDA, state and other foreign authorities. Any subsequent discovery of problems with a product, or a manufacturing or laboratory facility used by us or our collaborators, may result in restrictions on the product or on the manufacturing or laboratory facility, including marketed product recall, suspension of manufacturing, product seizure, or a voluntary withdrawal of the drug from the market. Any failure by our third-party manufacturers to comply with cGMP or failure to scale up manufacturing processes, including any failure to deliver sufficient quantities of product candidates in a timely manner, could lead to a delay in, or failure to obtain, regulatory approval of any of our product candidates.

We plan to rely on third-party manufacturers to purchase from third-party suppliers the materials necessary to produce our product candidates for our clinical studies. There are a small number of suppliers for certain capital equipment and materials that we plan to use to manufacture our drugs. Such suppliers may not sell these materials to our manufacturers at the times we need them or on commercially reasonable terms. Moreover, we currently do not have any agreements for the production of these materials. Although we do not intend to begin a clinical trial unless we believe we have a sufficient supply of a product candidate to complete the clinical trial, any significant delay in the supply of a product candidate or the material components thereof for an ongoing clinical trial due to the need to replace a third-party manufacturer could considerably delay completion of our clinical studies, product testing and potential regulatory approval of our product candidates. If our manufacturers or we are unable to purchase these materials after regulatory approval has been obtained for our product candidates, the commercial launch of our product candidates would be delayed or there would be a shortage in supply, which would impair our ability to generate revenue from the sale of our product candidates.

Contract manufacturers may not be able to manufacture our product candidates at a cost or in quantities or in a timely manner necessary to develop and commercialize them. If we successfully commercialize any of our product candidates, to meet our projected needs we may need to find third parties that will increase their scale of production, or we may have to establish or access large-scale commercial manufacturing capabilities. We may require additional funds, personnel and other resources to build, lease or operate any manufacturing facility.

A portion of our research and a portion of our manufacturing of certain key intermediates used in the manufacture of the active pharmaceutical ingredients for our product candidates takes place in China through third-party researchers and manufacturers. A significant disruption in the operation of those researchers or manufacturers or political unrest in China could materially adversely affect our business, financial condition and results of operations.

Although manufacturing for MAVYRET/MAVIRET is being conducted by AbbVie, we have relied on third parties located in China to manufacture and supply certain key intermediates used in the manufacture of our active pharmaceutical ingredients, or API, for our current product candidates, and we expect to continue to use such third party manufacturers for such intermediates for any product candidates we develop independently. Any disruption in

production or inability of our manufacturers in China to produce adequate quantities to meet our needs, whether as a result of a natural disaster or other causes, could impair our ability to operate our business on a day-to-day basis and to continue our research and development of our product candidates. We also use contract researchers in China to conduct a portion of our research for our early stage programs. Any disruption in the team conducting that research could cause delays in one or more of our research programs and could require us to curtail one or more programs, at least until we could contract for that research to be done elsewhere. Furthermore, since these researchers and

manufacturers are located in China, we are exposed to the possibility of product supply disruption and increased costs in the event of changes in the policies of the Chinese government, political unrest or unstable economic conditions in China. Any of these matters could materially and adversely affect our business and results of operations. Any recall of the manufacturing lots or similar action regarding our API used in clinical trials could delay the trials or detract from the integrity of the trial data and its potential use in future regulatory filings. In addition, manufacturing interruptions or failure to comply with regulatory requirements by any of these manufacturers could significantly delay clinical development of potential products and reduce third-party or clinical researcher interest and support of proposed trials. These interruptions or failures could also impede commercialization of our product candidates and impair our competitive position. Further, we may be exposed to fluctuations in the value of the local currency in China. Future appreciation of the local currency could increase our costs. In addition, our labor costs could continue to rise as wage rates increase due to increased demand for skilled laborers and the availability of skilled labor declines in China.

We will rely on third parties to monitor, support, conduct and/or oversee clinical trials of our product candidates that we develop independently and, in some cases, to maintain regulatory files for those product candidates. If we are not able to maintain or secure agreements with such third parties on acceptable terms, if these third parties do not perform their services as required, or if these third parties fail to timely transfer any regulatory information held by them to us, we may not be able to obtain regulatory approval for, or commercialize, our product candidates.

We will rely on CROs, hospitals, clinics, academic institutions and other third-party collaborators who are outside our control to monitor, support, conduct and/or oversee preclinical and clinical studies of our product candidates. We will also rely on third parties to perform clinical trials of our product candidates when they reach that stage. As a result, we have less control over the timing and cost of these studies and the ability to recruit trial subjects than if we conducted these trials wholly by ourselves. If we are unable to maintain or enter into agreements with these third parties on acceptable terms, or if any such engagement is terminated, we may be unable to enroll patients on a timely basis or otherwise conduct our trials in the manner we anticipate. In addition, there is no guarantee that these third parties will devote adequate time and resources to our studies or perform as required by a contract or in accordance with regulatory requirements, including maintenance of clinical trial information regarding our product candidates. If these third parties fail to meet expected deadlines, fail to timely transfer to us any regulatory information, fail to adhere to protocols or fail to act in accordance with regulatory requirements or our agreements with them, or if they otherwise perform in a substandard manner or in a way that compromises the quality or accuracy of their activities or the data they obtain, then clinical trials of our product candidates may be extended, delayed or terminated, or our data may be rejected by the FDA or regulatory agencies.

To the extent we elect to enter into additional licensing or collaboration agreements to partner our product candidates, our dependence on such relationships may adversely affect our business.

Our commercialization strategy for some of our product candidates may depend on our ability to enter into collaboration agreements with other companies to obtain access to other compounds for use in combination with any of our product candidates or for assistance and funding for the development and potential commercialization of any of these product candidates, similar to what we have done with AbbVie. Supporting diligence activities conducted by potential collaborators and negotiating the financial and other terms of a collaboration agreement are long and complex processes with uncertain results. Even if we are successful in entering into one or more additional collaboration agreements, collaborations can involve greater uncertainty for us, as we may have limited or no control over certain aspects of our collaborative programs. We may determine that continuing a collaboration under the terms provided is not in our best interest, and we may terminate the collaboration. Our collaborators could delay or terminate their agreements with us, and our product candidates subject to collaborative arrangements may never be successfully commercialized.

Further, our collaborators may develop alternative products or pursue alternative technologies either on their own or in collaboration with others, including our competitors, and the priorities or focus of our collaborators may shift such that our programs receive less attention or resources than we would like, or they may be terminated altogether. Any such actions by our collaborators may adversely affect our business prospects and ability to earn revenue. In addition, we could have disputes with our collaborators, such as the interpretation of terms in our agreements. Any

such disagreements could lead to delays in the development or commercialization of any potential products or could result in time-consuming and expensive litigation or arbitration, which may not be resolved in our favor.

Even with respect to programs that we intend to commercialize ourselves, we may enter into agreements with collaborators to share in the burden of conducting clinical trials, manufacturing and marketing our product candidates or products. In addition, our ability to apply our proprietary technologies to develop proprietary compounds will depend on our ability to establish and maintain licensing arrangements or other collaborative arrangements with the holders of proprietary rights to such compounds. We may not be able to establish such arrangements on favorable terms or at all, and our collaborative arrangements may not be successful.

Risks Related to Our Intellectual Property Rights

We could be unsuccessful in obtaining or maintaining adequate patent protection for one or more of our product candidates.

Our commercial success will depend, in large part, on our ability to obtain and maintain patent and other intellectual property protection with respect to our product candidates. We cannot be certain that patents will be issued or granted with respect to our patent applications that are currently pending, or that issued or granted patents will not later be found to be invalid and/or unenforceable, be interpreted in a manner that does not adequately protect our products, or otherwise provide us with any competitive advantage. The patent position of biotechnology and pharmaceutical companies is generally uncertain because it involves complex legal and factual considerations. The standards applied by the United States Patent and Trademark Office and foreign patent offices in granting patents are not always applied uniformly or predictably. For example, there is no uniform worldwide policy regarding patentable subject matter or the scope of claims allowable in biotechnology and pharmaceutical patents. Consequently, patents may not issue from our pending patent applications. As such, we do not know the degree of future protection that we will have on our proprietary products and technology, if any, and a failure to obtain adequate intellectual property protection with respect to our product candidates and proprietary technology could have a material adverse impact on our business.

In addition, certain of our activities have been funded, and may in the future be funded, by the United States federal government. For example, the preclinical and early clinical development of the lead antibiotic product candidate in our former antibiotic program, which we are no longer developing, was funded under a contract with NIAID, an entity of the United States federal government. When new technologies are developed with United States federal government funding, the government obtains certain rights in any resulting patents, including a nonexclusive license authorizing the government to use the invention for non-commercial purposes. These rights may permit the government to disclose our confidential information to third parties and to exercise “march-in” rights to use or allow third parties to use our patented technology. The government can exercise its march-in rights if it determines that action is necessary because we fail to achieve practical application of the United States government-funded technology, or because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations or to give preference to United States industry. In addition, United States government-funded inventions must be reported to the government and United States government funding must be disclosed in any resulting patent applications. In addition, our rights in such inventions are subject to certain requirements to manufacture products in the United States.

Issued patents covering one or more of our product candidates could be found invalid or unenforceable if challenged in court.

Despite measures we take to obtain patent and other intellectual property protection with respect to our product candidates and proprietary technology, any of our intellectual property rights could be challenged or invalidated. For example, if we were to initiate legal proceedings against a third party to enforce a patent covering one of our product

candidates, the defendant could counterclaim that our patent is invalid and/or unenforceable. In patent litigation in the United States and in some other jurisdictions, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, for example, lack of novelty, obviousness or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the United States Patent and Trademark Office, or the applicable foreign counterpart, or

made a misleading statement, during prosecution. Although we believe that we have conducted our patent prosecution in accordance with the duty of candor and in good faith, the outcome following legal assertions of invalidity and unenforceability during patent litigation 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 and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on a product candidate. Even if a defendant does not prevail on a legal assertion of invalidity and/or unenforceability, our patent claims may be construed in a manner that would limit our ability to enforce such claims against the defendant and others. Any loss of patent protection could have a material adverse impact on one or more of our product candidates and our business.

Enforcing our intellectual property rights against third parties may also cause such third parties to file other counterclaims against us, which could be costly to defend and could require us to pay substantial damages, cease the sale of certain products or enter into a license agreement and pay royalties (which may not be possible on commercially reasonable terms or at all). Any efforts to enforce our intellectual property rights are also likely to be costly and may divert the efforts of our scientific and management personnel.

Claims that our product candidates or the sale or use of our products infringe the patent or other intellectual property rights of third parties could result in costly litigation or could require substantial time and money to resolve, even if litigation is avoided.

Our commercial success depends upon our ability to develop, manufacture, market and sell our product candidates and use our proprietary technology without infringing the intellectual property rights of others. We cannot guarantee that our product candidates or any uses of our product candidates do not and will not in the future infringe third-party patents or other intellectual property rights. Third parties might allege that we or our collaborators are infringing their patent rights or that we have misappropriated their trade secrets, or that we are otherwise violating their intellectual property rights, whether with respect to the manner in which we have conducted our research or to the composition, use or manufacture of the compounds we have developed or are developing with our collaborators. Such third parties might resort to litigation against us or other parties we have agreed to indemnify, which litigation could be based on either existing intellectual property or intellectual property that arises in the future.

It is also possible that we failed to identify, or may in the future fail to identify, relevant patents or patent applications held by third parties that cover our product candidates. For example, applications filed before November 29, 2000 and certain applications filed after that date that will not be filed outside the United States remain confidential until patents issue. Other patent applications in the United States and several other jurisdictions are published approximately 18 months after the earliest filing for which priority is claimed, with such earliest filing date being commonly referred to as the priority date. Furthermore, publication of discoveries in the scientific or patent literature often lags behind actual discoveries. Therefore, we cannot be certain that we or our collaborators were the first to invent, or the first to file patent applications on, our product candidates or for their uses, or that our product candidates will not infringe patents that are currently issued or that are issued in the future. In the event that a third party has also filed a patent application covering one of our product candidates or a similar invention, we may have to participate in an adversarial proceeding, known as an interference, declared by the U.S. Patent and Trademark Office or its foreign counterpart to determine priority of invention. Additionally, pending patent applications which have been published can, subject to certain limitations, be later amended in a manner that could cover our products or their use.

In order to avoid or settle potential claims with respect to any patent or other intellectual property rights of third parties, we may choose or be required to seek a license from a third party and be required to pay license fees or royalties or both, which could be substantial. These licenses may not be available on acceptable terms, or at all. Even if we were able to obtain a license, the rights may be nonexclusive, which could result in our competitors gaining access to the same intellectual property. Ultimately, we could be prevented from commercializing a product, or be

forced, by court order or otherwise, to cease some or all aspects of our business operations, if, as a result of actual or threatened patent or other intellectual property claims, we are unable to enter into licenses on acceptable terms. Further, we could be found liable for significant monetary damages as a result of claims of intellectual property infringement. For example, we have received, and may in the future receive, offers to license and demands to license from third parties claiming that we are infringing their intellectual property or owe license fees and, even if such claims are without merit, there can be no assurance that we will successfully avoid or settle such claims.

In addition, if AbbVie licenses or otherwise acquires rights to intellectual property controlled by a third party in various circumstances, for example, where a product could not be legally developed or commercialized in a country without the third-party intellectual property right, it is entitled under our collaboration agreement to decrease payments payable to us on a product-by-product basis and, in certain cases, on a country-by-country basis. Any of the foregoing events could harm our business significantly.

Defending against claims of patent infringement, misappropriation of trade secrets or other violations of intellectual property rights could be costly and time consuming, regardless of the outcome. Thus, even if we were to ultimately prevail, or to settle at an early stage, such litigation could burden us with substantial unanticipated costs. In addition, litigation or threatened litigation could result in significant demands on the time and attention of our management team, distracting them from the pursuit of other company business. Claims that our product candidates or the sale or use of our future products infringe, misappropriate or otherwise violate third-party intellectual property rights could therefore have a material adverse impact on our business.

Unfavorable outcomes in intellectual property litigation could limit our research and development activities and/or our ability to commercialize certain products.

If third parties successfully assert their intellectual property rights against us, we might be barred from using certain aspects of our technology, or barred from developing and commercializing certain products. Prohibitions against using certain technologies, or prohibitions against commercializing certain products, could be imposed by a court or by a settlement agreement between us and a plaintiff. In addition, if we are unsuccessful in defending against allegations that we have infringed, misappropriated or otherwise violated patent or other intellectual property rights of others, we may be forced to pay substantial damage awards to the plaintiff. There is inevitable uncertainty in any litigation, including intellectual property litigation. There can be no assurance that we would prevail in any intellectual property litigation, even if the case against us is weak or flawed. If litigation leads to an outcome unfavorable to us, we may be required to obtain a license from the intellectual property owner in order to continue our research and development programs or to market any resulting product. It is possible that the necessary license will not be available to us on commercially acceptable terms, or at all. Alternatively, we may be required to modify or redesign our products in order to avoid infringing or otherwise violating third-party intellectual property rights. This may not be technically or commercially feasible, may render our products less competitive, or may delay or prevent the entry of our products to the market. Any of the foregoing could limit our research and development activities, our ability to commercialize one or more product candidates, or both.

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 intellectual property litigation longer than we could. In addition, the uncertainties associated with litigation could have a material adverse effect on our ability to raise the funds necessary to conduct our clinical trials, continue our internal research programs, in-license needed technology, or enter into strategic partnerships that would help us bring our product candidates to market.

In addition, any future intellectual property litigation, interference or other administrative proceedings will result in additional expense and distraction of our personnel. An adverse outcome in such litigation or proceedings may expose us or any future strategic partners to loss of our proprietary position, expose us to significant liabilities, or require us to seek licenses that may not be available on commercially acceptable terms, if at all, each of which could have a material adverse effect on our business.

Intellectual property litigation may lead to unfavorable publicity that harms our reputation and causes the market price of our common stock to decline.

During the course of any intellectual property litigation, there could be public announcements of the results of hearings, rulings on motions, and other interim proceedings in the litigation. If securities analysts or investors regard these announcements as negative, the perceived value of our products, programs or intellectual property could be diminished. Accordingly, the market price of our common stock may decline. Such announcements could also harm our reputation or the market for our future products, which could have a material adverse effect on our business.

Confidentiality agreements with employees and third parties may not prevent unauthorized disclosure of trade secrets and other proprietary information.

In addition to patents, we rely on trade secrets, technical know-how and proprietary information concerning our business strategy and product candidates in order to protect our competitive position in the field of HCV, other antivirals and liver disease. In the course of our research and development activities and our business activities, we often rely on confidentiality agreements to protect our proprietary information. Such confidentiality agreements are used, for example, when we talk to vendors of laboratory or clinical development services or potential strategic partners. In addition, each of our employees is required to sign a confidentiality agreement and invention assignment agreement upon joining our company. We take steps to protect our proprietary information, and our confidentiality agreements and invention assignment agreements are carefully drafted to protect our proprietary interests. Nevertheless, there can be no guarantee that an employee or an outside party will not make an unauthorized disclosure of our proprietary confidential information. This might happen intentionally or inadvertently. It is possible that a competitor will make use of such information, and that our competitive position will be compromised, in spite of any legal action we might take against persons making such unauthorized disclosures. In addition, to the extent that our employees, consultants or contractors 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.

Trade secrets are difficult to protect. Although we use reasonable efforts to protect our trade secrets, our employees, consultants, contractors, business partners or outside scientific collaborators might intentionally or inadvertently disclose our trade secret information to competitors or our trade secrets may otherwise be misappropriated. Enforcing a claim that a third party illegally obtained and is using any of our trade secrets is expensive and time consuming, and the outcome is unpredictable. In addition, courts outside the United States sometimes are less willing than United States courts to protect trade secrets. Moreover, our competitors may independently develop equivalent knowledge, methods and know-how.

Our collaborators may have rights to publish data and other information to which we have rights. In addition, we sometimes engage individuals or entities to conduct research relevant to our business. The ability of these individuals or entities to publish or otherwise publicly disclose data and other information generated during the course of their research is subject to certain contractual limitations. These contractual provisions may be insufficient or inadequate to protect our confidential information. If we do not apply for patent protection prior to such publication, or if we cannot otherwise maintain the confidentiality of our proprietary technology and other confidential information, then our ability to obtain patent protection or to protect our trade secret information may be jeopardized, which could adversely affect our business.

Intellectual property rights do not necessarily protect us from all potential threats to our competitive advantage.

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

- others may be able to make compounds that are similar to our product candidates but that are not covered by the claims of the patents that we own or have exclusively licensed;
- we might not have been the first to make the inventions covered by the issued patents or pending patent applications that we own or may in the future exclusively license, which could result in the patent applications not issuing or being invalidated after issuing;
- we might not have been the first to file patent applications covering certain of our inventions, which could result in the patent applications not issuing or being invalidated after issuing;
-

- 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 may not provide us with any competitive advantages, or may be held invalid or unenforceable, as a result of legal challenges by our competitors; we may obtain patents for certain compounds many years before we obtain marketing approval for products containing such compounds, and because patents have a limited life, which may begin to run prior to the commercial sale of the related product, the commercial value of our patents may be limited;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may fail to develop additional proprietary technologies that are patentable;
- the laws of certain foreign countries may not protect our intellectual property rights to the same extent as the laws of the United States, or we may fail to apply for or obtain adequate intellectual property protection in all the jurisdictions in which we operate; and
- the patents of others may have an adverse effect on our business, for example by preventing us from marketing one or more of our product candidates for one or more indications.

Any of the aforementioned threats to our competitive advantage could have a material adverse effect on our business.

Changes in patent law could diminish the value of patents in general, thereby impairing our ability to protect our products.

As is the case with many other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining, maintaining and enforcing patents in the biopharmaceutical industry involves both technological complexity and legal complexity. Therefore, the process of obtaining, maintaining and enforcing biopharmaceutical patents is costly, time-consuming and inherently uncertain. In addition, recent legislative and judicial developments in the United States and elsewhere have in some cases narrowed the protection afforded to patent owners, made patents more difficult to obtain, or increased the uncertainty regarding the ability to obtain, maintain and enforce patents. For example, Congress recently passed patent reform legislation, and may pass patent reform legislation in the future. The United States Supreme Court has ruled on several patent cases in recent years, and in certain circumstances has narrowed the scope of patent protection available or otherwise weakened the rights of patent owners. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions and actions by the United States Congress, the federal courts, the United States Patent and Trademark Office, and their respective foreign counterparts, the laws and regulations governing patents could change in unpredictable ways that could weaken our ability to obtain new patents or to maintain and enforce our existing patents and patents that we might obtain in the future.

Risks Related to Our Industry

If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of our product candidates.

We face an inherent risk of product liability as a result of the clinical testing of our product candidates, and we will face an even greater risk if we commercialize any product candidates. For example, we may be sued if any of our product candidates, including any that are developed in combination therapies, allegedly causes injury or is found to be otherwise unsuitable during product testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability and a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our product candidates. Even successful defense would require significant financial and management resources. There is also risk that third parties we have agreed to indemnify

could incur liability.

Regardless of the merits or eventual outcome, liability claims may result in:

- decreased demand for our product candidates or any resulting products;
- injury to our reputation;
- withdrawal of clinical trial participants;
- costs to defend the related litigation;
- a diversion of management's time and our resources;
- substantial monetary awards to trial participants or patients;
- product recalls, withdrawals or labeling, marketing or promotional restrictions;
- loss of revenue;
- the inability to commercialize our product candidates; and
- a decline in our stock price.

Our inability to obtain and retain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of products we develop. We currently carry product liability insurance covering our clinical studies in the amount of $10.0 million in the aggregate. Although we maintain such insurance, any claim that may be brought against us could result in a court judgment or settlement in an amount that is not covered, in whole or in part, by our insurance or that is in excess of the limits of our insurance coverage. Our insurance policies also have various exclusions, and we may be subject to a product liability claim for which we have no coverage. We will have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be able to obtain, sufficient capital to pay such amounts.

Our internal computer systems, or those of our collaborator, CROs or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of development programs for our product candidates.

Despite the implementation of security measures, our internal computer systems and those of our collaborators, CROs, and other contractors and consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. Information security risks have significantly increased in recent years in part due to the proliferation of new technologies and the increased sophistication and activities of organized crime, hackers, terrorists and other external parties, including foreign state actors. As cyber threats continue to evolve, we may be required to expend significant additional resources to continue to modify or enhance our protective measures or to investigate and remediate any information security breaches.

While we have not experienced any such system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our independent drug development programs. For example, the loss of clinical trial data from ongoing or future clinical trials for any of our product candidates could result in delays in regulatory approval efforts and significantly increase costs to recover or reproduce the data. Our information security systems are also subject to laws and regulations requiring that we take measures to protect the privacy and security of certain information we gather and use in our business. For example, HIPAA and its implementing regulations impose, among other requirements, certain regulatory and contractual requirements regarding the privacy and security of personal health information. In addition to HIPAA, numerous other federal and state laws, including, without limitation, state security breach notification laws, state health information privacy laws and federal and state consumer protection laws, govern the collection, use, disclosure and storage of personal information. To the extent that any disruption or security breach were to result in a loss of or damage to data or applications, or inappropriate disclosure of confidential or proprietary information or personal health information, we could incur substantial liability, our reputation would be damaged, and the further development of our product candidates could be delayed.

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Our relationships with customers and third-party payors in the United States and elsewhere will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and third-party payors in the United States and elsewhere play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute our products for which we obtain marketing approval. Restrictions under applicable federal, state and foreign healthcare laws and regulations include the following:

- the federal healthcare anti-kickback statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward 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;
- the federal False Claims Act imposes criminal and civil penalties, including civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, 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, as amended by the Health Information Technology for Economic and Clinical Health Act, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program and also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- the federal false statements statute prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services;
- the federal transparency requirements under the Patient Protection and Affordable Care Act of 2010 requires manufacturers of drugs, devices, biologics and medical supplies to report to the Department of Health and Human Services information related to physician payments and other transfers of value and physician ownership and investment interests;
- analogous state laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, and some state laws require pharmaceutical companies to comply with the pharmaceutical industry’s voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other healthcare providers or marketing expenditures; and
- analogous anti-kickback, fraud and abuse and healthcare laws and regulations in foreign countries.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices 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, damages, fines, exclusion from government funded healthcare programs, such as Medicare and Medicaid, 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 providers or entities with whom we expect to do business, including our collaborators, are found not to be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs, which could also materially affect our business.

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If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials or our or third parties' disposal of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

We may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

We maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials. This insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of hazardous or radioactive materials.

Our insurance policies are expensive and only protect us from specified business risks, which will leave us exposed to significant uninsured liabilities.

We do not carry insurance for all categories of risk that our business may encounter. Some of the policies we currently maintain include general liability, employment practices liability, property, auto, workers' compensation, products liability and directors' and officers' insurance. We do not know, however, if we have adequate levels of coverage for any liability we may incur, or whether we will always be able to continue to maintain such insurance. Any significant uninsured liability may require us to make substantial payments, which would adversely affect our financial position and results of operations. Furthermore, any increase in the volatility of our stock price may result in us being required to pay substantially higher premiums for our directors' and officers' liability insurance than those to which we are currently subject, and may even cause one or more of our underwriters to be unwilling to insure us.

Risks Related to Our Common Stock

Our stock price has been, and is likely to continue to be, volatile, and thus our stockholders could incur substantial losses.

Our stock price has been volatile and could be subject to wide fluctuations in response to various factors, many of which are beyond our control. Since our initial public offering in March 2013, the price of our common stock on the NASDAQ Global Select Market has ranged from $16.18 to $52.58. The stock market in general and the market for biopharmaceutical companies, and for those developing potential therapies for HCV in particular, have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, you may not be able to sell your common stock at or above your purchase price, if at all. The market price for our common stock may be influenced by many factors, including:

•

actions by AbbVie regarding HCV treatment regimens containing paritaprevir or the MAVYRET/MAVIRET regimen containing glecaprevir as approved in the U.S., EU and Japan, including announcements regarding clinical, regulatory or commercial developments or our collaboration;

- market expectations about and response to the levels of sales or scripts achieved by, or the announced prices or discounts for, AbbVie's paritaprevir-containing HCV treatment regimens or competitive HCV drugs;

- failure of AbbVie's paritaprevir-containing HCV treatment regimens to maintain their sales levels or AbbVie's MAVYRET/MAVIRET regimen to achieve commercial success;
- results from or delays of clinical trials of our other product candidates, as well as results of regulatory reviews relating to the approval of our product candidates;
- new products, product candidates or new uses for existing products or technologies introduced or announced by our competitors and the timing of these introductions or announcements;
- the results of our efforts to discover or develop additional product candidates;
- our dependence on third parties, including our collaborators, CROs, clinical trial sponsors and clinical investigators;
- regulatory, political or legal developments in the United States or other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key scientific or management personnel;
- our ability to commercialize our product candidates we develop independently, if approved;
- the level of expenses related to any of our product candidates or clinical development programs;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- period-to-period variations in our financial results or those of companies that are perceived to be similar to us;
- sales of common stock by us or our stockholders in the future, as well as the overall trading volume of our common stock;
- changes in the structure of healthcare payment systems or other actions that affect the effective reimbursement rates for treatment regimens containing our products or for competitive regimens;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions and other factors that may be unrelated to our operating performance or the operating performance of our competitors, including changes in market valuations of similar companies; and
- the other factors described in this "Risk Factors" section.

Provisions in our corporate charter documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our corporate charter and our bylaws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which they might otherwise receive a premium for their shares.

These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified or staggered board of directors such that not all members of the board are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;

- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- provide that the state courts or, in certain circumstances, the federal courts, in Delaware shall be the sole and exclusive forum for certain actions involving us, our directors, officers, employees and stockholders;
- provide our board of directors with the authority to designate the terms of and issue a new series of preferred stock without stockholder approval, which could be used to institute a "poison pill" that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 66 2/3% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibit a person who owns 15% or more of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner. Any provision in our corporate charter or our bylaws or Delaware law that has the effect of delaying or deterring a change in control could limit the opportunity for our stockholders to receive a premium for their shares of our common stock, and could also affect the price that some investors are willing to pay for our common stock.

Our employment agreements with our executive officers may require us to pay severance benefits to any of those persons who are terminated in connection with a change of control of us, which could harm our financial condition or results.

Our executive officers are parties to employment agreements that provide for aggregate cash payments of up to approximately $4.1 million for severance and other non-equity-based benefits in the event of a termination of employment in connection with a change of control of our company. In addition, based on the closing price of our common stock as of September 30, 2017 of $46.80 per common share, the aggregate intrinsic value of unvested stock options and other equity awards subject to accelerated vesting upon these events was $6.6 million. The accelerated vesting of awards options could result in dilution to our stockholders and harm the market price of our common stock. The payment of these severance benefits could harm our company's financial condition and results. In addition, these potential severance payments may discourage or prevent third parties from seeking a business combination with us.

We are an "emerging growth company," and we cannot be certain if the reduced reporting requirements applicable to "emerging growth companies" will make our common stock less attractive to investors.

We are an "emerging growth company," as defined in the JOBS Act. For as long as we continue to be an "emerging growth company," we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not "emerging growth companies," including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. As an "emerging growth company" we are required to report periodic financial results and selected financial data related to two fiscal years compared to three and five years, respectively, for comparable data required to be reported by other public companies in selected SEC reports. We may take advantage of these exemptions until we are no longer an "emerging growth company." Based on the market value of our common stock at March 31, 2017 and the number of shares of our common stock held by non-affiliates, we will continue to be

an “emerging growth company” until the end of our 2018 fiscal year. We cannot

predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

If we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial results or prevent fraud. As a result, stockholders could lose confidence in our financial and other public reporting, which would harm our business and the trading price of our common stock.

Effective internal controls over financial reporting are necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, are designed to prevent fraud. Any failure to implement required new or improved controls, or difficulties encountered in their implementation could cause us to fail to meet our reporting obligations. In addition, any testing by us conducted in connection with Section 404 of the Sarbanes-Oxley Act of 2002, or any subsequent testing by our independent registered public accounting firm, may reveal deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses or that may require prospective or retroactive changes to our financial statements or identify other areas for further attention or improvement. Inferior internal controls could also cause investors to lose confidence in our reported financial information, which could have a negative effect on the trading price of our common stock.

We are required to disclose changes made in our internal controls and procedures on a quarterly basis and our management is required to assess the effectiveness of these controls annually. However, for as long as we are an "emerging growth company" under the JOBS Act, our independent registered public accounting firm will not be required to attest to the effectiveness of our internal controls over financial reporting pursuant to Section 404. We will continue to be an "emerging growth company" until our fiscal year ending September 30, 2018. An independent assessment of the effectiveness of our internal controls could detect problems that our management's assessment might not. Undetected material weaknesses in our internal controls could lead to financial statement restatements and require us to incur the expense of remediation.

Because we do not anticipate paying cash dividends on our common stock for the foreseeable future, investors in our common stock may never receive a return on their investment.

You should not rely on an investment in our common stock to provide dividend income. We do not anticipate that we will pay any cash dividends to holders of our common stock for the foreseeable future. Instead, we plan to retain any earnings to maintain and expand our existing operations.

Accordingly, investors must rely on sales of their common stock after price appreciation, which may never occur, as the only way to realize any return on their investment. As a result, investors seeking cash dividends should not invest in our common stock.

A sale of a substantial number of shares of our common stock in the public market 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. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. As of September 30, 2017, we had 19.1 million shares of common stock outstanding. In addition, as of September 30, 2017, 2.3 million and 0.2 million shares of common stock that are subject to outstanding options or restricted stock unit awards, respectively, under our equity plan are eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules, and Rules 144 and 701 under the Securities Act. If these additional shares of common stock are sold, or it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

If securities or industry analysts do not publish research, or publish inaccurate or unfavorable research, about our business, our stock price and trading volume could decline.

The trading market for our common stock will depend in part on the research and reports that securities or industry analysts publish about us or our business. If those analysts are unable to predict accurately the demand and net sales

of AbbVie's HCV regimens, that could result in our reported revenues and earnings being lower than the so-called "market consensus" of our projected revenues, which could negatively affect our stock price. In addition, if too few securities or industry analysts cover our company, the trading price for our stock would likely be negatively impacted. In the event that one or more of the analysts who cover us downgrade our stock or publish inaccurate or unfavorable research about our business, our stock price would likely decline. If one or more of these analysts cease coverage of our company or fail to publish reports on us regularly, demand for our stock could decrease, which might cause our stock price and trading volume to decline.

ITEM 1B. UNRESOLVED STAFF COMMENTS
None.

ITEM 2. PROPERTIES
Our corporate headquarters is located in Watertown, Massachusetts, where we lease approximately 49,000 square feet of office and laboratory space. The term of our current lease expires on September 1, 2022.

ITEM 3. LEGAL PROCEEDINGS
We are not a party to any material legal proceedings.

ITEM 4. MINE SAFETY DISCLOSURES
Not applicable.

PART II

ITEM 5. MARKET FOR THE COMPANY'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES
Market and Stockholder Information

Our common stock has been listed on The NASDAQ Global Select Market under the symbol "ENTA" since March 21, 2013. The following table shows the high and low sales price for our common stock as reported by The NASDAQ Global Select Market for the quarterly periods in the fiscal years ended September 30, 2017 and 2016:

| | Fiscal 2017 | |
|---|---|---|
| | High | Low |
| First Quarter | $34.53 | $22.32 |
| Second Quarter | $36.05 | $27.72 |
| Third Quarter | $37.54 | $29.45 |
| Fourth Quarter | $46.80 | $33.42 |

| | Fiscal 2016 | |
|---|---|---|
| | High | Low |
| First Quarter | $40.68 | $23.90 |
| Second Quarter | $34.19 | $21.52 |
| Third Quarter | $32.41 | $21.55 |
| Fourth Quarter | $26.81 | $21.00 |

As of December 1, 2017, there were 30 stockholders of record of our common stock, which excludes stockholders whose shares were held in nominee or street name by brokers.

We have never declared or paid cash dividends on our common stock, and we do not expect to declare or pay any cash dividends for the foreseeable future.

Performance Graph[(1)]

The following graph shows a comparison from March 21, 2013 through September 30, 2017 of cumulative total return on assumed investments of $100.00 in cash in each of our common stock, the NASDAQ Composite Index and the NASDAQ Biotechnology Index. Such returns are based on historical results and are not intended to suggest future performance. Data for the NASDAQ Composite Index and the NASDAQ Biotechnology Index assume reinvestment of dividends.

COMPARISON OF FIFTY FOUR MONTH CUMULATIVE TOTAL RETURN

Among Enanta Pharmaceuticals, Inc., the NASDAQ Composite Index,

and the NASDAQ Biotechnology Index

(1) This performance graph shall not be deemed to be "soliciting material" or to be "filed" with the SEC for purposes of Section 18 of the Securities and Exchange Act of 1934, as amended, or otherwise subject to the liabilities under that Section, and shall not be deemed incorporated by reference into any filing of Enanta Pharmaceuticals, Inc. under the Securities Act of 1933, as amended.

ITEM 6. SELECTED CONSOLIDATED FINANCIAL DATA

We have derived the consolidated statements of operations data for the years ended September 30, 2017, 2016, and 2015 and the consolidated balance sheet data as of September 30, 2017 and 2016 from our audited financial statements included elsewhere in this Annual Report on Form 10-K. The selected statements of operations data for the years ended September 30, 2014 and 2013 and the balance sheet data as of September 30, 2015, 2014 and 2013 are derived from our audited financial statements not included in this Annual Report on Form 10-K. Our historical results for any prior period are not necessarily indicative of any results to be expected for any future period.

| | Years Ended September 30, | | | | |
|---|---|---|---|---|---|
| | 2017 | 2016 | 2015 | 2014 | 2013 |
| | (in thousands, except per share data) | | | | |
| Consolidated Statements of Operations Data: | | | | | |
| Revenue | $102,814 | $88,268 | $160,880 | $47,741 | $32,053 |
| Operating expenses: | | | | | |
| Research and development | 57,451 | 40,461 | 23,189 | 18,740 | 16,841 |
| General and administrative | 20,749 | 16,966 | 13,543 | 10,016 | 6,183 |
| Total operating expenses | 78,200 | 57,427 | 36,732 | 28,756 | 23,024 |
| | | | | | |
| Income from operations | 24,614 | 30,841 | 124,148 | 18,985 | 9,029 |
| Other income (expense), net | 2,333 | 1,719 | 1,307 | 283 | 598 |
| | | | | | |
| Net income before income tax | 26,947 | 32,560 | 125,455 | 19,268 | 9,627 |
| Income tax (expense) benefit | (9,237) | (10,894) | (46,463) | 15,170 | — |
| Net income | 17,710 | 21,666 | 78,992 | 34,438 | 9,627 |
| Accretion of redeemable convertible preferred stock to redemption value | — | — | — | — | (2,526) |
| Net income attributable to participating securities | — | — | — | — | (13,670) |
| Net income (loss) attributable to common stockholders | $17,710 | $21,666 | $78,992 | $34,438 | $(6,569) |
| | | | | | |
| Net income (loss) per share attributable to common stockholders: | | | | | |
| Basic | $0.93 | $1.14 | $4.23 | $1.88 | $(0.67) |
| Diluted | $0.91 | $1.13 | $4.09 | $1.80 | $(0.67) |
| Weighted average common shares outstanding: | | | | | |
| Basic | 19,066 | 18,929 | 18,673 | 18,355 | 9,788 |
| Diluted | 19,407 | 19,224 | 19,295 | 19,185 | 9,788 |

| | As of September 30, | | | | |
|---|---|---|---|---|---|
| | 2017 | 2016 | 2015 | 2014 | 2013 |
| | (in thousands) | | | | |

| | | | | | |
|---|---|---|---|---|---|
| Consolidated Balance Sheet Data: | | | | | |
| Cash, cash equivalents and marketable securities | $293,707 | $242,203 | $209,443 | $131,767 | $112,183 |
| Working capital | 216,837 | 224,267 | 163,937 | 103,229 | 100,187 |
| Total assets | 326,637 | 281,277 | 246,013 | 155,415 | 116,973 |
| Capital lease obligation | 458 | 531 | 598 | — | — |
| Warrant liability | 807 | 1,251 | 1,276 | 1,584 | 1,620 |
| Series 1 nonconvertible preferred stock | 762 | 159 | 163 | 202 | — |
| Total stockholders’ equity | 301,676 | 269,936 | 236,157 | 148,654 | 110,468 |

ITEM 7.MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of financial condition and results of operations together with the section entitled "Selected Consolidated Financial Data" and our consolidated financial statements and related notes included elsewhere in this Annual Report on Form 10-K. This discussion and other parts of this Annual Report on Form 10-K contain forward-looking statements that involve risks and uncertainties, such as statements regarding our plans, objectives, expectations, intentions and projections. Our actual results could differ materially from those discussed in these forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those discussed in the "Risk Factors" section of this Annual Report on Form 10-K.

Overview

We are a biotechnology company that uses our robust, chemistry-driven approach and drug discovery capabilities to create small molecule drugs primarily for the treatment of viral infections and liver diseases. We discovered glecaprevir, the second of two protease inhibitors discovered and developed through our collaboration with AbbVie and marketed as part of AbbVie's new direct-acting antiviral (DAA) regimen under the tradenames MAVYRET™ (U.S.) or MAVIRET™ (ex-U.S.) (glecaprevir/pibrentasvir) for the treatment of chronic hepatitis C virus, or HCV. The other protease inhibitor under our HCV collaboration is part of AbbVie's initial DAA regimens for the treatment of chronic HCV marketed under the tradenames VIEKIRA PAK® (paritaprevir/ritonavir/ombitasvir/

dasabuvir) (U.S.) or VIEKIRAX® (paritaprevir/ritonavir/ombitasvir) (ex-U.S.). Our royalties from our AbbVie collaboration and our existing financial resources provide us funding to support our wholly owned research and development efforts, which are currently focused on the following disease targets:

- non-alcoholic steatohepatitis, or NASH, a liver disease estimated to affect approximately 6 million individuals in the U.S. alone;
- primary biliary cholangitis, or PBC, a chronic liver disease that slowly destroys bile ducts in the liver, which affects an estimated 17,000 individuals in the U.S.;
- respiratory syncytial virus, or RSV, the most common cause of bronchiolitis and pneumonia in children under one year of age in the U.S., resulting in an estimated 75,000 to 125,000 hospitalizations each year in the U.S.; and
- hepatitis B virus, or HBV, the most prevalent chronic hepatitis, which is estimated to affect approximately 250 million individuals worldwide.

We had $293.7 million in cash and marketable securities at September 30, 2017. In fiscal 2017, we received and earned as revenue a total of $65.0 million in milestone payments for commercialization regulatory approvals of the glecaprevir/pibrentasvir combination in the U.S. in August 2017 and the EU in July 2017 and earned $37.8 million in per-product royalties on portions of AbbVie's net sales of its HCV regimens allocated to paritaprevir or glecaprevir. We earned the remaining $15.0 million milestone payment from AbbVie upon reimbursement approval for MAVIRET in Japan in November 2017. We expect our existing financial resources and quarterly royalty payments will allow us to continue to invest for the foreseeable future in our wholly owned research and development programs.

Our Wholly Owned Programs

Our wholly owned research and development programs are in liver disease (non-virology), namely NASH and PBC, and in virology, namely RSV and HBV:

-

NASH and PBC: We are working on multiple compounds that selectively bind to and activate the farnesoid X receptor, or FXR. We plan to develop these compounds, referred to as FXR agonists, for use in the treatment of NASH and PBC, both of which are liver diseases with very few therapeutic options. Our lead FXR agonist, EDP-305, represents a new class of FXR agonist designed to take advantage of increased binding interactions with the receptor. We believe this class is significantly different from other FXR agonists in clinical development.

- In October 2017, we announced results of a Phase 1a/b clinical study of EDP-305, which was generally safe and well tolerated over a broad range of single and multiple doses with pharmacokinetic data supporting once daily oral dosing. The study included 98 healthy volunteer subjects, or HV subjects, and 48 subjects who were obese and with or without pre-diabetes or type 2 diabetes, whom we refer to as subjects with presumptive non-alcoholic fatty liver disease, or PN subjects.
EDP-305 exhibited strong engagement of the FXR receptor as evidenced by increased levels of FGF19 and reduced levels of C4, both of which are monitored as downstream markers indicating FXR receptor activity.
Results support the ability to administer EDP-305 in future trials at doses that neither elicit clinically significant changes in lipids nor result in pruritus (itching).
- Since November 2016, we have presented data at the 2016 and 2017 annual meetings of the American Association for the Study of Liver Diseases (AASLD), the 2017 NASH-TAG conference and the 2017 International Liver Congress (ILC) that demonstrated that EDP-305 is a highly selective FXR agonist and shows more potent activity in a variety of in vitro and in vivo NASH models compared to the most advanced NASH candidate in development today, obeticholic acid, or OCA.
    - We plan to initiate a Phase 2 clinical study of EDP-305 in PBC patients by the end of calendar 2017.
    - We also plan to initiate a Phase 2 clinical study of EDP-305 in NASH patients in early 2018.
- EDP-305 has been granted Fast Track designation by the U.S. Food and Drug Administration (FDA) for the treatment of NASH patients with liver fibrosis and separately for the treatment of PBC.
- In addition, we are pursuing research in other classes of FXR agonists as well as other mechanisms that may provide therapeutic benefit in NASH and that either or both of these could be used as combination therapies for NASH.
- RSV: We have selected EDP-938, a potent N-protein inhibitor of activity of both major subgroups of RSV, referred to as RSV-A and RSV-B, as our first development candidate for RSV. We believe EDP-938 is differentiated from fusion inhibitors currently in development for RSV because N-protein inhibitors directly target the viral replication process of RSV and have demonstrated high barriers to resistance against RSV in vitro.
- In June 2017, we presented preclinical data demonstrating that EDP-938 is a potent inhibitor of both RSV-A and RSV-B activity, maintaining antiviral activity post-infection while presenting a high barrier to resistance in vitro. EDP-938 demonstrated a greater than 4-log reduction in viral load in an animal model challenged with RSV. Further, EDP-938 maintained antiviral potency across all clinical isolates tested in vitro, as well as virus that was resistant to fusion inhibitors. The compound inhibited RSV at a post-entry, replication step and maintained its activity in vitro when given 24 hours post infection. In addition, combination studies of EDP-938 with other types of RSV inhibitors, such as fusion inhibitors, showed synergistic antiviral effects.
    - We plan to initiate a Phase 1 clinical study of EDP-938 by the end of calendar 2017.
- HBV: We also have a program to discover and develop new chemical entities for the treatment of HBV. Our initial focus is on core inhibitors, a mechanism with early clinical validation. We believe that it may be necessary to utilize more than one compound/mechanism for the treatment of HBV and therefore we are pursuing multiple approaches. We continue to make progress in discovering, characterizing, and seeking patent protection for new core inhibitors of HBV with the goal of identifying a development candidate in 2018. In addition, we are conducting preclinical experiments with other mechanisms that target HBV.

We have utilized our internal chemistry and drug discovery capabilities to generate all of our development-stage programs.

Licensed Products

Through our Collaborative Development and License Agreement with AbbVie, we have developed and licensed to AbbVie two protease inhibitor compounds that have been clinically tested, manufactured, and commercialized by AbbVie. Royalties on AbbVie's net sales allocated to the protease inhibitors in these HCV regimens provided us $37.8 million and $57.7 million in royalty revenue in our 2017 and 2016 fiscal years, respectively. To date, we have earned a total of $330.0 million in milestone payments related to clinical development and commercialization regulatory approvals of these regimens in major markets, including a $15.0 million milestone earned in November 2017.

- Glecaprevir: Glecaprevir is the protease inhibitor we discovered that was developed by AbbVie in its new fixed-dose combination with its NS5A inhibitor, pibrentasvir, for the treatment of HCV. This combination, currently marketed under the brand name MAVYRET™ in the U.S. and MAVIRET™ ex-U.S. and referred to in this report as MAVYRET/MAVIRET, is a new, once daily, all oral, fixed-dose, ribavirin-free treatment for HCV genotypes 1-6, which is referred to as being pan-genotypic. In the EU, U.S. and Japan it is approved as an 8-week treatment for patients without cirrhosis and new to treatment. Today, these patients are estimated to represent the majority of HCV patients in the developed country markets.

Our economics from AbbVie's MAVYRET/MAVIRET consist of two components:

- We earned a total of $65.0 million in milestone payments in fiscal 2017 upon approval for the glecaprevir/pibrentasvir combination in the U.S. and the EU, and we earned the remaining $15.0 million milestone payment in November 2017 in connection with commercialization regulatory approval in Japan.
- We also receive annually tiered, double-digit, per-product royalties on 50% of the net sales of the 2-DAA glecaprevir/pibrentasvir combination in MAVYRET/MAVIRET, which are calculated separately from the royalties of paritaprevir-containing regimens.

The EU, U.S. and Japan authorizations for the MAVYRET/MAVIRET combination of glecaprevir and pibrentasvir, and AbbVie's applications for approval of MAVYRET/MAVIRET in other jurisdictions, are supported by the following studies:

- 8 weeks for treatment-naïve, non-cirrhotics: In November 2016, results from several Phase 3 studies of this combination demonstrated 97.5% of chronic HCV infected patients without cirrhosis and new to treatment across all major genotypes (GT1-6) achieved sustained virologic response at 12 weeks post-treatment, referred to as $SVR_{12}$, with just 8 weeks of MAVYRET/MAVIRET treatment.
- 8 weeks with chronic kidney disease: Results were also presented from AbbVie's EXPEDITION-4 study in chronic HCV patients with chronic kidney disease (CKD), in which 98% of patients (n=102/104) across all major genotypes (GT1-6) achieved $SVR_{12}$ with 12 weeks of treatment with MAVYRET/MAVIRET.
- 8 weeks for GT-3: Data from AbbVie's ENDURANCE-3 study were presented at the International Liver Congress, or ILC, demonstrating that 95% of patients with challenging-to-treat, genotype 3 (GT3) chronic HCV infection, without cirrhosis and new to treatment, achieved $SVR_{12}$ after 8 weeks of treatment with MAVYRET/MAVIRET.
- 12 weeks for compensated cirrhosis: Data from AbbVie's EXPEDITION-1 study were also presented at the ILC, demonstrating that 99% of HCV-infected patients with genotype 1, 2, 4, 5 or 6 and compensated cirrhosis (Child-Pugh A) achieved $SVR_{12}$ following 12 weeks of MAVYRET/MAVIRET treatment without ribavirin.
- Paritaprevir: Paritaprevir is the protease inhibitor contained in AbbVie's initial HCV treatment regimens currently marketed in the U.S., EU, Japan and other countries around the world under the trade names VIEKIRA PAK®, VIEKIRAX® VIEKIRAX XR™ and TECHNIVIE®. First approved and sold in the U.S. in December 2014 for treatment of genotype 1, or GT-1, HCV, AbbVie's HCV regimens containing paritaprevir are now also approved for genotype 4, or GT-4, HCV.

Financial Operations Overview

We are currently funding all research and development for our internal programs. We expect to incur substantially greater expenses as we continue to advance our FXR agonist program for NASH and PBC. We have completed a Phase 1 study in 146 patients and plan to initiate additional studies, one in PBC patients by the end of calendar 2017 and one in NASH patients in early 2018. We also plan to initiate a Phase 1 clinical study of our lead RSV candidate, EDP-938, by the end of calendar 2017. We expect to increase expenses in fiscal 2018 as we conduct these clinical studies and advance other compounds into substantial preclinical development.

Since commencing our operations in 1995, we have devoted substantially all of our resources to the discovery and development of novel compounds for the treatment of viral infections and liver diseases. For the periods included in this report we have funded our operations primarily through payments received under collaboration agreements with pharmaceutical companies and one government research and development contract, as well as net proceeds that we received from our March 2013 IPO. Our revenue from our collaboration agreements has resulted in our reporting net income in each of our past six fiscal years. Our revenue in the near term will continue to be dependent on our royalty payments from our collaboration with AbbVie.

For its new MAVYRET/MAVIRET regimen, which in the majority of chronic HCV patients only requires 8 weeks of treatment compared to 12 weeks with VIEKIRA PAK, AbbVie has initially set a lower list price compared to its original HCV regimens and other HCV products on the market. As MAVYRET/MAVIRET replaces AbbVie's paritaprevir-containing regimens over the next several quarters, AbbVie is seeking to increase its HCV market share through this pricing and the favorable treatment characteristics of the new regimen. It is still too early to know how successful AbbVie's efforts will be. Given the uncertainty of AbbVie's future HCV sales that will generate our royalty payments and the development risks affecting our future expenditures for the advancement of our internally developed compounds, it is uncertain whether we will continue to report net income in fiscal 2018 and thereafter.

Revenue

Since our inception, our revenue has been derived from two primary sources: collaboration agreements with pharmaceutical companies and one government research and development contract. We have entered into three significant collaboration agreements and contracts since 2006, the most significant of which is our continuing collaboration agreement with AbbVie. In addition, from September 2011 through August 2015, we had a contract with the National Institute of Allergy and Infectious Diseases, or NIAID, which funded the preclinical and early clinical development of an antibiotic product candidate for potential use in biodefense.

Beginning in our fiscal year ended September 30, 2015, we generated royalty revenue from AbbVie's net sales allocable to paritaprevir, which is part of AbbVie's initial treatment regimens for HCV approved in the U.S. in December 2014 and in the EU and dozens of other countries since then. During the year ended September 30, 2017, AbbVie received approval for its new HCV regimen, marketed as MAVYRET™ in the U.S. and MAVIRET™ outside the U.S.

The following table is a summary of revenue recognized from our collaboration agreement and our government contract for the years ended September 30, 2017, 2016, and 2015:

| | Years Ended September 30, | | |
|---|---|---|---|
| | 2017 | 2016 | 2015 |
| | (in thousands) | | |
| AbbVie agreement: | | | |
| Milestones | $65,000 | $30,000 | $125,000 |
| Royalties | 37,814 | 57,692 | 34,077 |
| NIAID contract: | — | 576 | 1,803 |
| Total revenue | $102,814 | $88,268 | $160,880 |

AbbVie Agreement

Since all of our research obligations under the AbbVie agreement were concluded by June 30, 2011, all milestone payments received since then have been recognized as revenue upon achievement of each milestone by AbbVie. We earned and recognized as revenue milestone payments associated with commercialization regulatory approvals of HCV regimens under the AbbVie agreement totaling $65.0 million, $30.0 million and $125.0 million, during the years ended September 30, 2017, 2016, and 2015. Milestone revenue recognized in our fiscal 2017 was related to commercialization regulatory approvals of glecaprevir-containing regimens marketed as MAVYRET™ (U.S.) and MAVIRET™ (ex-U.S.) while milestone revenue recognized in our fiscal years 2016 and 2015 related to commercialization regulatory approvals of AbbVie's paritaprevir-containing regimens. In November 2017, we earned an additional $15.0 million milestone payment from AbbVie related to commercialization regulatory approval of MAVIRET™ in Japan.

We also receive annually tiered, double-digit royalties per protease inhibitor product on AbbVie's net sales allocable to either of our collaboration's protease inhibitors. Under the terms of our AbbVie agreement, as amended in October 2014, 30% of net sales of 3-DAA regimens containing paritaprevir and 45% of net sales of 2-DAA regimens containing paritaprevir are allocated to paritaprevir for purposes of calculating our annually tiered royalties. In the case of regimens containing glecaprevir, 50% of AbbVie's net sales are allocated to glecaprevir. Beginning with each January 1, the cumulative net sales of each royalty-bearing product start at zero for purposes of calculating the tiered royalties on a product-by-product basis.

We expect all of our revenue in 2018 to be generated from our collaboration agreement with AbbVie.

NIAID Contract

Under our NIAID contract, which ended in August 2015, we received research and development funding of approximately $20.6 million from NIAID through September 30, 2016. We recognized revenue of $0.6 million and $1.8 million under this agreement during the years ended September 30, 2016 and 2015, respectively.

Internal Programs

As our internal product candidates are currently in preclinical or early clinical development, we have not generated any revenue from our own product sales and do not expect to generate any revenue from product sales derived from these product candidates for at least the next several years. We expect that our revenue for the next several years will be derived from royalties and the remaining milestone payment (which was earned in November 2017) under our current collaboration agreement with AbbVie, as well as any additional collaboration that we may enter into in the future.

Operating Expenses

The following table summarizes our operating expenses for the years ended September 30, 2017, 2016, and 2015:

| | Years Ended September 30, | | |
|---|---|---|---|
| | 2017 | 2016 | 2015 |
| | (in thousands) | | |
| Research and development | $57,451 | $40,461 | $23,189 |
| General and administrative | 20,749 | 16,966 | 13,543 |

| | | | |
|---|---|---|---|
| Total operating expenses | $78,200 | $57,427 | $36,732 |

Research and Development Expenses

Research and development expenses consist of costs incurred to conduct basic research, such as the discovery and development of novel small molecules as therapeutics, as well as any external expenses of preclinical and clinical development activities. We expense all costs of research and development as incurred. These expenses consist primarily of:

- personnel costs, including salaries, related benefits and stock-based compensation expense for employees engaged in scientific research and development functions;
- third-party contract costs relating to research, formulation, manufacturing, preclinical study and clinical trial activities;
- third-party license fees;
- laboratory consumables; and
- allocated facility-related costs.

Project-specific expenses reflect costs directly attributable to our clinical development candidates and preclinical candidates nominated and selected for further development. Remaining research and development expenses are reflected in research and drug discovery, which represents early-stage drug discovery programs. At any given time, we typically have several active early stage research and drug discovery projects. Our internal resources, employees and infrastructure are not directly tied to any individual research or drug discovery project and are typically deployed across multiple projects. As such, we do not report information regarding costs incurred for our early-stage research and drug discovery programs on a project-specific basis. We expect that our research and development expenses will continue to increase in the future as we advance our NASH, PBC, RSV and HBV programs.

Our research and drug discovery programs are at early stages; therefore, the successful development of our product candidates is highly uncertain and may not result in approved products. Completion dates and completion costs can vary significantly for each product candidate and are difficult to predict. Given the uncertainty associated with clinical trial enrollments and the risks inherent in the development process, we are unable to determine the duration and completion costs of the current or future clinical trials of our product candidates or if, or to what extent, we will generate revenue from the commercialization and sale of any of our product candidates. We anticipate that we will make determinations as to which development programs to pursue and how much funding to direct to each program on an ongoing basis in response to the preclinical and clinical success and prospects of each product candidate, as well as ongoing assessments of the commercial potential of each product candidate.

General and Administrative Expenses

General and administrative expenses consist primarily of personnel costs, which include salaries, related benefits and stock-based compensation, of our executive, finance, business and corporate development and other administrative functions. General and administrative expenses also include travel expenses, allocated facility-related costs not otherwise included in research and development expenses, directors and officers liability insurance premiums, and professional fees for auditing, tax, and legal services and patent expenses.

We expect that general and administrative expenses will increase in the future primarily due to ongoing expansion of our operating activities in support of our own research and development programs, as well as potential additional costs associated with operating a growing public company.

Other Income (Expense), Net

Interest income. Interest income consists of interest earned on our cash equivalents and short-term and long-term marketable securities balances as well as interest earned for refunds received from tax authorities.

Interest expense. Interest expense consists of interest expense related to our capital lease obligation.

Change in fair value of warrant liability and Series 1 nonconvertible preferred stock. We have issued warrants for the purchase of our Series 1 nonconvertible preferred stock and we have issued Series 1 nonconvertible preferred stock, both of which are financial instruments that may require a transfer of assets because of the liquidation preference features of the underlying stock. Therefore, we have classified these warrants and Series 1 nonconvertible preferred stock as liabilities that we remeasure to fair value at each reporting period. We record the changes in the fair value of the warrants and Series 1 nonconvertible preferred stock as a component of other income (expense), net.

Income Tax Expense

Income tax expense is based on our best estimate of applicable income tax rates for the entire fiscal year applied to pre-tax profit reported for the year-to-date period. The income tax expense for the years ended September 30, 2017, 2016, and 2015 resulted from federal and state taxes attributable to our operating income.

Critical Accounting Policies

Our consolidated financial statements are prepared in accordance with accounting principles generally accepted in the United States of America. The preparation of our consolidated financial statements and related disclosures requires us to make estimates and assumptions that affect the reported amount of assets, liabilities, revenue, costs and expenses, and related disclosures. We believe that the estimates and assumptions involved in the accounting policies described below may have the greatest potential impact on our consolidated financial statements and, therefore, consider these to be our critical accounting policies. We evaluate our estimates and assumptions on an ongoing basis. Actual results may differ from these estimates under different assumptions and conditions. See also Note 2 to the consolidated financial statements included elsewhere in this Annual Report on Form 10-K for information about these critical accounting policies as well as a description of our other significant accounting policies.

Revenue Recognition

Our revenue has been generated primarily through collaborative research and license agreements. The terms of these agreements contain multiple deliverables which may include (i) licenses, (ii) research and development activities, and (iii) participation in joint research and development steering committees. The terms of these agreements may include nonrefundable upfront license fees, payments for research and development activities, payments based upon the achievement of certain milestones, and royalty payments based on product sales derived from the collaboration. The majority of revenue is derived under our agreement with AbbVie. Under this agreement, we have no ongoing deliverables and therefore, royalties and milestones received under this arrangement are recognized when earned. In all instances, revenue is recognized only when the price is fixed or determinable, persuasive evidence of an arrangement exists, delivery has occurred or the services have been rendered, collectibility of the resulting receivable is reasonably assured, and we have fulfilled our performance obligations under the contract.

We apply Accounting Standards Codification No. 605-25, Revenue Recognition Multiple-Deliverable Revenue Arrangements, or ASC 605-25, for multiple element arrangements entered into or materially modified on or after October 1, 2011. This guidance amends the criteria for separating and allocating consideration in a multiple-element arrangement by modifying the fair value requirements for revenue recognition and eliminating the use of residual value method. The selling prices of deliverables under the arrangement may be derived using third-party evidence, (“TPE”) or a best estimate of selling price (“BESP”), if vendor-specific objective evidence (“VSOE”), is not available. The objective of BESP is to determine the price at which we would transact a sale if the element within the license agreement was sold on a standalone basis. Establishing BESP involves management’s judgment and considers multiple factors, including market conditions and company-specific factors such as those factors contemplated in negotiating the agreements as well as internally developed models that include assumptions related to market opportunity, discounted cash flows, estimated development costs, probability of success, and the time needed to commercialize a

product candidate pursuant to the license. In validating our BESP, we consider whether changes in key assumptions used to determine the BESP will have a significant effect on the allocation of the arrangement consideration between the multiple deliverables. Deliverables under a multiple element arrangement are separated into multiple units if (i) the delivered item has value to the customer on a standalone basis and (ii) if the arrangement includes a general right of return relative to the delivered item, delivery or performance of the

undelivered item is considered probable and substantially within our control. In determining the separate units of accounting, we evaluate whether the license has standalone value to the collaborator based on consideration of the relevant facts and circumstances for each arrangement. Factors considered in this determination include the research and development capabilities of the collaborator and the availability of relevant research expertise in the marketplace. In addition, we consider whether or not (i) the collaborator can use the license for its intended purpose without the receipt of the remaining deliverables, (ii) the value of the license is dependent on the undelivered items, and (iii) the collaborator or other vendors can provide the undelivered items. We may exercise significant judgment in determining whether a deliverable is a separate unit of accounting. The arrangement consideration that is fixed or determinable at the inception of the arrangement is allocated to the separate units of accounting based on their relative selling prices. The appropriate revenue recognition model is applied to each element and revenue is accordingly recognized as each element is delivered.

Royalty revenue is recognized based on contractual terms when reported sales are reliably measurable and collectibility is reasonably assured, provided that there are no performance obligations remaining.

During the years ended September 30, 2016 and 2015, we also generated revenue from a government contract under which we were reimbursed for certain allowable costs incurred for the funded project. Revenue from the government contract was recognized when the related service was performed. The related costs incurred by us under the government contract were included in research and development expense in the consolidated statements of operations. This contract was completed in August 2015.

Amounts received prior to satisfying all revenue recognition criteria are recorded as deferred revenue in the accompanying consolidated balance sheets. Amounts not expected to be recognized as revenue within the next twelve months of the consolidated balance sheet date are classified as long-term deferred revenue.

In the event that a collaborative research and license agreement is terminated and we then have no further performance obligations, we recognize as revenue any amounts that had not previously been recorded as revenue but were classified as deferred revenue at the date of such termination.

Stock-Based Compensation - Stock Options and Restricted Stock Unit Awards

We measure stock awards with service-based conditions granted to employees and directors at fair value on the date of grant and recognize the corresponding stock-based compensation expense of those awards, net of estimated forfeitures, over the requisite service period, which is generally the vesting period of the respective award. Stock awards granted with service-based vesting conditions, which include restricted stock units and stock options, are recorded as an expense using the straight-line method. In the case of performance-based options, we recognize stock-based compensation expense related to these awards when the performance-based targets are deemed probable of being achieved.

The fair value of each restricted stock unit granted is based on the fair value of our common stock on the date of grant. The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model. We have granted stock options with exercise prices equivalent to the fair value of our common stock on the date of grant. Generally, our expected volatility has been measured based on a combination of our historical stock volatility since our March 2013 IPO and the historical volatility of our publicly traded peer companies. We expect to continue to utilize this method until such time as we have adequate historical data regarding the volatility of our traded stock price following our March 2013 IPO. The expected term of our options has been determined utilizing the “simplified” method for awards that qualify as “plain-vanilla” options and we expect to continue to do so until such time as we have adequate historical data regarding our employee exercise patterns. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately

equal to the expected term of the award. Our expected dividend yield is 0 and is based on the fact that we have never paid cash dividends and do not expect to pay any cash dividends in the foreseeable future.

We recognize stock-based compensation expense for only the portion of awards that are expected to vest. In developing a forfeiture rate estimate, we have considered our historical experience to estimate pre-vesting forfeitures

for service-based options. If our actual forfeiture rate is materially different from the estimate, our stock-based compensation expense could be different from what we have recorded in the current period.

These assumptions represent our best estimates, but the estimates involve inherent uncertainties and the application of our judgment. As a result, if factors change and we use significantly different assumptions or estimates, our stock-based compensation expense could be materially different.

Stock-Based Compensation - Market and Performance-based Stock Unit Awards

In addition to awards with service-based vesting conditions, we also have granted performance share units, or PSUs, and relative stockholder return units, or rTSRUs, to certain of our executives. The number of units represents the target number of shares of common stock that may be earned; however, the actual number of shares that may be earned ranges from 0% to 200% of the target number. The fair value of PSUs is based on the fair value of our common stock on the date of grant. The fair value of rTSRUs is based on a Monte Carlo simulation model. Assumptions and estimates utilized in the calculation of the fair value of the rTSRUs include the risk-free interest rate, dividend yield, average closing price, expected volatility based on the historical volatility of publicly traded peer companies and the remaining performance period of the award.

The PSUs vest and result in issuance, at settlement, of common shares for each recipient based upon the recipient's continued employment with us through the settlement date of the award and our achievement of specified research and development milestones. The requisite service period of the PSUs is generally 2 years. In the case of PSUs, we recognize stock-based compensation expense based on the grant date fair value of the award only when performance-based targets are deemed probable of being achieved.

The rTSRUs vest and result in the issuance of common stock based upon the recipient's continuing employment with us through the settlement date of the award and the relative ranking of the total stockholder return, or TSR, of our common stock in relation to the TSR of the component companies in the NASDAQ Biotech Index, generally over a two-year period based on a comparison of average closing stock prices in specified periods noted in the award agreement. The fair value related to the rTSRUs is recorded as stock-based compensation expense over the period from date of grant to the settlement date regardless of whether the related target relative total stockholder return is achieved.

Income Taxes

Income taxes are provided for tax effects of transactions reported in the consolidated financial statements and consist of income taxes currently due plus deferred income taxes related to timing differences between the basis of certain assets and liabilities for financial statement reporting purposes and the basis for income tax reporting purposes. Deferred taxes are determined based on the difference between the financial reporting and tax basis of assets and liabilities using enacted tax rates in effect in the years in which the differences are expected to reverse. Changes in deferred tax assets and liabilities are recorded in the provision for income taxes. A valuation allowance is 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. At each balance sheet date, we assess the likelihood that deferred tax assets will be realized, and recognize a valuation allowance if it is more likely than not that some portion of the deferred tax assets will not be realized. Assessment of the potential recovery of deferred tax assets requires judgment and is evaluated by estimating the future taxable income expected and considering prudent and feasible tax planning strategies. As of September 30, 2017, we continue to believe it is more likely than not that we will be able to realize our deferred tax assets and therefore no valuation allowance has been recorded in either period.

Uncertain tax positions represent tax positions for which reserves have been established. We account for uncertainty in income taxes recognized in the consolidated financial statements by applying a two-step process to determine the amount of tax benefit to be recognized. First, the tax position must be evaluated to determine the likelihood that it will be sustained upon external examination by the taxing authorities. If the tax position is deemed more likely than not to be sustained, the tax position is then assessed to determine the amount of benefit to be recognized in the financial statements. The amount of the benefit that may be recognized is the largest amount that has a greater than 50% likelihood of being realized upon ultimate settlement. The provision for income taxes includes the effects of

any resulting tax reserves, or unrecognized tax benefits, that are considered appropriate as well as the related net interest and penalties.

JOBS Act

The JOBS Act was signed into law on April 5, 2012 and contains provisions that, among other things, reduce certain reporting requirements for an "emerging growth company." As an "emerging growth company," we are electing not to take advantage of the extended transition period afforded by the JOBS Act for the implementation of new or revised accounting standards, and as a result, we will comply with new or revised accounting standards on the relevant dates on which adoption of such standards is required for non-emerging growth companies. Section 107 of the JOBS Act provides that our decision not to take advantage of the extended transition period is irrevocable. Subject to certain conditions set forth in the JOBS Act, as an "emerging growth company", we are not required to, among other things, (i) provide an auditor's attestation report on our system of internal controls over financial reporting pursuant to Section 404, (ii) provide all of the compensation disclosures that may be required of non-emerging growth public companies under the Dodd-Frank Wall Street Reform and Consumer Protection Act, (iii) comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements (auditor discussion and analysis), and (iv) disclose certain executive compensation-related items such as the correlation between executive compensation and performance and comparisons of the Chief Executive Officer's compensation to median employee compensation. Based on the market value of our common stock at March 31, 2017 and the number of shares of our common stock held by non-affiliates, we continued to be an "emerging growth company" for our fiscal 2017. Therefore, these exemptions will apply until the end of our fiscal year 2018, which includes the fifth anniversary of the completion of our initial public offering in March 2013.

Results of Operations

Comparison of Years Ended September 30, 2017, 2016, and 2015

| | Years Ended September 30, | | |
|---|---|---|---|
| | 2017 | 2016 | 2015 |
| | (in thousands) | | |
| Revenue | $102,814 | $88,268 | $160,880 |
| Research and development | 57,451 | 40,461 | 23,189 |
| General and administrative | 20,749 | 16,966 | 13,543 |
| Other income (expense), net: | | | |
| Interest income | 2,532 | 1,735 | 968 |
| Interest expense | (40) | (45) | (8) |
| Change in fair value of warrant liability and Series 1 nonconvertible preferred stock, net | (159) | 29 | 347 |
| Income tax expense | (9,237) | (10,894) | (46,463) |

Revenue. We recognized revenue of $102.8 million during the year ended September 30, 2017, as compared to $88.3 million during the year ended September 30, 2016. The increase in revenue of $14.5 million year over year was primarily due to timing of milestone payments earned under our AbbVie agreement and was partially offset by lower royalties earned on AbbVie's net sales of HCV regimens allocated to paritaprevir during 2017 as compared to 2016.

During the years ended September 30, 2017 and 2016, we recognized milestone revenue of $65.0 million and $30.0 million, respectively. In fiscal 2017, we earned and received milestone payments as a result of commercialization regulatory approvals for AbbVie's glecaprevir-containing regimens in the EU and U.S. In fiscal 2016, we earned and received a milestone payment as a result of commercialization regulatory approval for AbbVie's paritaprevir-containing regimen in Japan. We earned royalties of $37.8 million and $57.7 million during the years ended September 30, 2017 and 2016, respectively, primarily based on the portion of AbbVie's net sales of its HCV regimens allocable to paritaprevir. We recognized lower royalty revenue in 2017 compared to 2016 due to lower sales of paritaprevir-containing regimens as a result of increased competition from other HCV products on the

market. We began earning royalties on the portion of AbbVie's net sales of glecaprevir-containing regimens in the fourth quarter of fiscal 2017.

We recognized revenue of $88.3 million during the year ended September 30, 2016 as compared to $160.9 million during the year ended September 30, 2015. The decrease in revenue of $72.6 million year over year was primarily due to a decrease in milestone payments received from AbbVie and was partially offset by higher royalties earned on the portions of AbbVie's net sales of HCV regimens allocated to paritaprevir during 2016. During the years ended September 30, 2016 and 2015, we recognized milestone revenue of $30.0 million and $125.0 million, respectively. In fiscal 2016, we earned and received a milestone payment as a result of commercialization regulatory approval for AbbVie's paritaprevir-containing regimen in Japan. In fiscal 2015, we earned and received milestone payments as a result of commercialization regulatory approvals for AbbVie's paritaprevir-containing regimen in the U.S. and EU We earned royalties of $57.7 million and $34.1 million during the years ended September 30, 2016 and 2015, respectively, based on the portion of AbbVie's net sales of its HCV regimens allocable to paritaprevir. We recognized higher royalty revenue in fiscal 2016 compared to 2015 due to the timing of regulatory approvals of paritaprevir-containing regimens which started in fiscal 2015 and continued in fiscal 2016.

Research and development expenses.

| | Years Ended September 30, | | |
|---|---|---|---|
| | 2017 | 2016 | 2015 |
| | (in thousands) | | |
| R&D programs: | | | |
| Liver disease | $34,750 | $17,840 | $9,096 |
| Virology | 22,399 | 21,692 | 10,403 |
| Other | 302 | 929 | 3,690 |
| Total research and development expenses | $57,451 | $40,461 | $23,189 |

Research and development expense increased by $17.0 million for the year ended September 30, 2017 as compared to the same period in 2016 due to progression of preclinical and clinical activities in our liver disease and virology programs. Increases were driven by an increase in headcount to support our clinical and preclinical activities and an increase in external costs for those activities.

Research and development expenses increased by $17.3 million for the year ended September 30, 2016 as compared to the same period in 2015 due to progression of preclinical and clinical activities in our liver disease and virology programs. Increases were driven by an increase in headcount to support our preclinical activities, expansion of our research facility and an increase in external costs for clinical and preclinical activities.

We expect that our research and development expenses will continue to increase in the future as we advance our NASH, PBC, RSV and HBV programs.

General and administrative expenses. General and administrative expenses increased by $3.8 million for the year ended September 30, 2017 as compared to the same period in 2016 primarily due to an increase in compensation

expense due to increased headcount as well as the achievement of milestones in 2017 under existing performance-based stock awards in 2017.

General and administrative expenses increased by $3.4 million for the year ended September 30, 2016 as compared to the same period in 2015 primarily due to an increase in stock-based compensation expense related to an increase in headcount, additional stock option grants to employees, and achievement of performance-based stock awards in 2016.

Other income (expense), net. Changes in components of other income (expense), net were as follows:

Interest income. Interest income increased by $0.8 million for the year ended September 30, 2017 as compared to the same period in 2016 due to higher average investment balances and changes in interest rates for fiscal 2017 as compared to the same period in 2016.

Interest income increased by $0.8 million for the year ended September 30, 2016 as compared to the same period in 2015 due to higher average investment balances in fiscal 2016 as compared to 2015.

Interest expense. Interest expense represents interest related to our capital lease obligation.

Change in fair value of warrant liability and Series 1 nonconvertible preferred stock. We recognized other expense of $0.2 million and other income of less than $0.1 million for the years ended September 30, 2017 and 2016, respectively. We recognized expense in our fiscal 2017 due to an increase in fair value of the outstanding liabilities year over year, and recognized income in our fiscal 2016 due to a decrease in fair value of the outstanding liabilities year over year. During the year ended September 30, 2017, a total of 0.7 million warrants were exercised, resulting in the issuance of 0.7 million shares of Series 1 nonconvertible preferred stock.

Subsequent to September 30, 2017, a total of 0.9 million warrants were exercised prior to warrant expiration, resulting in 0.9 million shares of Series 1 nonconvertible preferred stock issued. On October 4, 2017, less than 0.1 million of outstanding warrants expired unexercised. The liability associated with these expired warrants will be reversed, resulting in income in 2018.

We recognized other income of less than $0.1 million and $0.3 million for the years ended September 30, 2016 and 2015 as a result of the remeasurement of the warrant liability and Series 1 nonconvertible preferred stock year over year due to a decrease in fair value of the outstanding liabilities.

Income tax expense. Income tax expense was $9.2 million and $10.9 million for the years ended September 30, 2017 and 2016, respectively. The effective tax rate for the years ended September 30, 2017 and 2016 was 34.3% and 33.5%, respectively. The decrease in income tax expense was primarily due to lower profit before taxes as well as an increase in federal research and development tax credits which were deductible for tax purposes. The increase in the effective tax rate was primarily due to an increase in non-deductible stock-based compensation expense. This increase was partially offset by an increase in federal research and development credits as part of our advancement of our wholly owned research and development programs in 2017.

Income tax expense was $10.9 million and $46.5 million for the years ended September 30, 2016, and 2015, respectively. The effective tax rate for the years ended September 30, 2016 and 2015 was 33.5%, and 37.0%, respectively. The decrease in income tax expense was primarily due to lower profit before taxes as well as the reinstatement in fiscal 2016 of the federal research and development tax credits for prior years, which were deductible for tax purposes and included in the Company’s annual effective tax rate for 2016. The decrease in the effective tax rate was primarily due to an increase in federal research and development credits as part of our advancement of our wholly owned research and development programs in 2016. This increase was partially offset by an increase in non-deductible stock-based compensation expense.

Income tax expense for all periods presented was attributable to the tax provision on the earnings of our operations, all of which are domestic.

Liquidity and Capital Resources

From our inception through September 30, 2017, we have financed our operations primarily through payments under our collaborations, government research and development contracts and grants, and the net proceeds from our initial public offering of our equity in March 2013.

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The following table shows a summary of our cash flows for each of the years ended September 30, 2017, 2016, and 2015:

| | Years Ended September 30, | | |
|---|---|---|---|
| | 2017 | 2016 | 2015 |
| | (in thousands) | | |
| Cash provided by (used in): | | | |
| Operating activities | $52,653 | $35,809 | $76,673 |
| Investing activities | $(4,572) | $(43,663) | $(88,186) |
| Financing activities | $1,017 | $2,705 | $2,540 |
| Net increase (decrease) in cash and cash equivalents | $49,098 | $(5,149) | $(8,973) |

Net cash provided by operating activities

The increase in cash provided by operating activities of $16.8 million for the year ended September 30, 2017 as compared to the same period in 2016 was primarily driven by an increase in cash receipts under our collaboration with AbbVie of $15.9 million year over year. We received $105.1 million during 2017, including royalties and $65.0 million in milestone payments, compared to $89.2 million in cash from AbbVie during 2016, including royalties and a $30.0 million milestone payment. In addition, our cash taxes paid, net of refunds received, decreased by $17.5 million, due to lower profit before tax and timing of tax payments and refunds. These increases were partially offset by increased cash spending on research and development during 2017 in order to progress clinical development and preclinical research in our proprietary programs.

The decrease in cash provided by operating activities of $40.9 million for the year ended September 30, 2016 as compared to the same period in 2015 was primarily driven by a decrease in cash receipts under our collaboration with AbbVie of $55.5 million year over year. We received $89.2 million in cash from AbbVie during 2016, including royalties and a $30.0 million milestone payment, compared to $144.7 million in cash from AbbVie during 2015, including royalties and $125.0 million in milestone payments. In addition, we increased cash spending on research and development during 2016 in order to progress clinical development and preclinical research in our proprietary programs. These decreases to cash used in operating activities were partially offset by a decrease in income tax payments of $27.5 million based on lower income before income tax in 2016 as compared to 2015.

Net cash used in investing activities

The increase in cash used in investing activities of $39.1 million for the year ended September 30, 2017 as compared to the same period in 2016 was driven by timing of purchases, sales and maturities of marketable securities. In addition, our capital asset outlay decreased by $2.2 million year over year due to the expansion of our research facility which was completed in fiscal 2016.

The decrease in cash used in investing activities of $44.5 million for the year ended September 30, 2016 as compared to the same period in 2015 was driven by timing of purchases, sales and maturities of marketable securities. The decrease was partially offset by an increase in capital expenditure outlay in 2016 of $2.4 million compared to 2015 due to the expansion of our research facility in 2016.

Net cash provided by financing activities

The decrease in cash provided by financing activities of $1.7 million for the year ended September 30, 2017 as compared to the same period in 2016 was driven primarily by tax withholding payments for the vesting of performance stock units in 2017 and a lower income tax benefit for stock options exercised in fiscal 2017 as compared to fiscal 2016.

The increase in cash provided by financing activities of $0.2 million for the year ended September 30, 2016 as compared to the same period in 2015 was driven by an increase in proceeds from exercises of stock options.

Funding Requirements

As of September 30, 2017, we had $293.7 million in cash, cash equivalents and short-term and long-term marketable securities. We believe that our existing cash, cash equivalents and marketable securities as of September 30, 2017 will be sufficient to meet our anticipated cash requirements for the foreseeable future. However, our forecast that the use of our financial resources will be adequate to support our operations is a forward-looking statement that involves risks and uncertainties, and actual results could vary materially.

Our future capital requirements are difficult to forecast and will depend on many factors, including:

- whether our existing collaboration continues to generate substantial royalties to us;
- the number and characteristics of the product candidates we pursue;
- the scope, progress, results and costs of researching and developing any of our product candidates on our own, including conducting preclinical research and clinical trials;
- opportunities to in-license or otherwise acquire new therapeutic candidates and therapies;
- the timing, receipt and amount of royalties on paritaprevir and glecaprevir and any sales of our product candidates, if any, or royalties thereon;
- the timing of, and the costs involved in, obtaining regulatory approvals for any product candidates we develop independently;
- the cost of commercialization activities, if any, of any product candidates we develop independently that are approved for sale, including marketing, sales and distribution costs;
    - the cost of manufacturing our product candidates and any products we successfully commercialize independently, including manufacturing for clinical development;
- our ability to maintain our existing collaboration and to establish new collaborations, licensing or other arrangements and the financial terms of such agreements; and
- the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing patents, including any litigation costs and the outcomes of any such litigation.

We do not believe that inflation had a material effect on our business, financial condition or results of operations in the last three fiscal years. If our costs were to become subject to significant inflationary pressures, we could not offset such higher costs through revenue increases because our revenues are substantially outside of our control. Our inability to do so could harm our business, financial condition and results of operations.

Off-Balance Sheet Arrangements

We do not engage in any off-balance sheet financing activities. We do not have any interest in entities referred to as variable interest entities, which include special purpose entities and other structured finance entities.

Recently Issued Accounting Pronouncements

A description of recently issued accounting pronouncements that may potentially impact our financial position and results of operations is set forth in Note 2 to the consolidated financial statements included in this Annual Report on Form 10-K.

Contractual Obligations and Commitments

We lease office space in Watertown, Massachusetts under a lease that commenced on October 1, 2011 and was amended in 2015 to expand the rented space and extend the lease term through September 2022. In conjunction with the amendment of the lease, the Company entered into a capital lease agreement to fund certain leasehold improvements and the purchase of lab equipment. The following table summarizes our contractual obligations at

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September 30, 2017 and the effect such obligations are expected to have on our liquidity and cash flow in future periods:

| | Payments Due by Period | | | | |
|---|---|---|---|---|---|
| | 2018 | 2019-2020 | 2021-2022 | 2023 and later | Total |
| | (in thousands) | | | | |
| Operating leases | $2,062 | $ 4,289 | $ 4,322 | $ — | $10,673 |
| Capital leases | 79 | 179 | 200 | — | 458 |
| Total contractual commitments and obligations | $2,141 | $ 4,468 | $ 4,522 | $ — | $11,131 |

As of September 30, 2017, we had 1.0 million outstanding warrants for the purchase of Series 1 nonconvertible preferred stock and 1.0 million outstanding shares of Series 1 nonconvertible preferred stock, all of which we classified as long-term liabilities on our consolidated balance sheet and recorded at fair value of $1.6 million. The fair value of both of these classes of instruments was measured based on significant inputs not observable in the market, which represented a Level 3 measurement within the fair value hierarchy. The fair value of these instruments represents less than 10% of liabilities measured at fair value as of September 30, 2017. The Series 1 nonconvertible preferred stock issued combined with the outstanding warrants as of September 30, 2017, would require the payment of $2.0 million upon a qualifying merger or sale of our company. The table above does not include this liability because we are unable to estimate the timing of this required payment, or if it will be required at all.

## ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

### Interest Rate Sensitivity

We had cash, cash equivalents and marketable securities of $293.7 million at September 30, 2017, which consisted of cash, money market funds, agency securities, commercial paper, treasury notes and corporate bonds. Interest income is sensitive to changes in the general level of interest rates; however, due to the nature of these investments, a 1% change in market interest rates would not be expected to have a material impact on our financial condition or results of operations. Other than our capital lease obligation, we had no debt outstanding as of September 30, 2017.

## ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

Our consolidated financial statements, together with the report of our independent registered public accounting firm, appear on pages F-1 through F-31 of this Annual Report on Form 10-K.

## ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE

None.

## ITEM 9A. CONTROLS AND PROCEDURES

### Conclusion Regarding the Effectiveness of Disclosure Controls and Procedures

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in the Company’s reports under the Securities Exchange Act of 1934, as amended (the Exchange Act), is recorded, processed, summarized and reported within the time periods specified in the SEC’s rules and forms, and that such

information is accumulated and communicated to management, including our Chief Executive Officer (CEO) and Chief Financial Officer (CFO), as appropriate, to allow timely decisions regarding required disclosure. In designing and evaluating the disclosure controls and procedures, management recognized that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control

objectives, as the Companies are designed to do, and management necessarily was required to apply its judgment in evaluating the risk related to controls and procedures.

In connection with the preparation of this Form 10-K, as of September 30, 2017, an evaluation was performed under the supervision and with the participation of our management, including the CEO and CFO, of the effectiveness of the design and operation of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act). Based on that evaluation, our management concluded that our disclosure controls and procedures were effective at a reasonable assurance level as of September 30, 2017. These conclusions were communicated to the Audit Committee.

Management’s Report on Internal Control Over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting as defined in Rule 13a-15(f) under the Exchange Act. Our internal control system is designed to provide reasonable assurance to the Company’s management and Board of Directors regarding the preparation and fair presentation of published financial statements. All internal control systems, no matter how well designed, have inherent limitations. Therefore, even those systems determined to be effective can provide only reasonable assurance with respect to financial statement preparation and presentation.

Our management has assessed the effectiveness of our internal control over financial reporting as of September 30, 2017. In making this assessment, management used the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission (COSO) in its 2013 Internal Control—Integrated Framework. Based on this assessment, our management has concluded that as of September 30, 2017 our internal control over financial reporting is effective.

As an Emerging Growth Company, as defined under the terms of the Jobs Act of 2012, the Company’s independent registered accounting firm is not required to issue an attestation report on the internal control over financial reporting.

Change in Internal Control over Financial Reporting—There were no changes in our internal control over financial reporting that occurred during our last quarter that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

ITEM 9B. OTHER INFORMATION
None.

PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

Portions of the response to this item are incorporated herein by reference from the discussion responsive thereto under the captions “Proposal 1 - Election of Directors—Nominees for Director and Current Directors”, “Section 16(a) Beneficial Ownership Reporting Compliance”, “Executive Officers” and “Corporate Governance—Board and Committee Matters” in the Company’s Definitive Proxy Statement relating to the 2018 Annual Meeting of Stockholders, also referred to as the 2018 Proxy Statement.

We have adopted a Code of Business Conduct and Ethics (the code of ethics) that applies to all of our directors, officers and employees. The code of ethics is available on our website at http://www.enanta.com. In addition, if we make any substantive amendments to the code of ethics or grant any waiver, including any implicit waiver, from a provision of the code to any of our executive officers or directors, we will disclose the nature of such amendment or waiver as required by applicable law.

ITEM 11. EXECUTIVE COMPENSATION

The response to this item is incorporated herein by reference from the discussion responsive thereto under the following captions in the 2018 Proxy Statement: “Executive Compensation” and “Corporate Governance—Certain Relationships and Related Transactions.”

ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS

The response to this item is incorporated herein by reference in part from the discussion responsive thereto under the caption “Beneficial Ownership of Common Stock” in the 2018 Proxy Statement.

The following table provides information about the securities authorized for issuance under the Company’s equity compensation plans as of September 30, 2017:

Equity Compensation Plan Information

(in thousands, except per share information)

| Plan Category | Number of securities to be issued upon exercise of outstanding options, warrants and rights (a) | | Weighted average exercise price of outstanding options, warrants and rights (b) | Number of securities remaining available for future issuance under equity compensation plans (excluding securities reflected in column (a)) (c) | |
|---|---|---|---|---|---|
| Equity compensation plans approved by | 3,567 | (2) | $ 19.56 | 460 | (3) |

| | | |
|---|---|---|
| security holders [1] | | |
| Equity compensation plans not approved by security holders | | |
| Totals | 3,567 | 460 |

(1) Consists of the Company's Amended and Restated 1995 Equity Incentive Plan, as amended, the Company's 2012 Equity Incentive Plan, as amended, and the Company's Employee Stock Purchase Plan.
(2) Consists of shares of the Company's common stock issuable upon exercise of outstanding options issued under the Company's Amended and Restated 1995 Equity Incentive Plan and the Company's 2012 Equity Incentive Plan as well as Series 1 nonconvertible preferred stock issuable upon exercise of outstanding warrants.
(3) Consists of shares of the Company's common stock reserved for future issuance under the Company's 2012 Equity Incentive Plan and the Company's Employee Stock Purchase Plan. This does not include 574 shares

that were automatically added to the Company's 2012 Equity Incentive Plan by its terms as of October 1, 2017.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE

The response to this item is incorporated herein by reference from the discussion responsive thereto under the caption "Corporate Governance—Certain Relationships and Related Transactions" and "Corporate Governance—Board and Committee Matters" in the 2018 Proxy Statement.

ITEM 14. PRINCIPAL ACCOUNTANT FEES AND SERVICES

The response to this item is incorporated herein by reference from the discussion responsive thereto under the captions "Corporate Governance—Board and Committee Matters" and "Audit Committee Report—Audit Fees" in the 2018 Proxy Statement.

PART IV

ITEM 15. EXHIBITS, FINANCIAL STATEMENT SCHEDULES

(a) 1. FINANCIAL STATEMENTS

The financial statements are included under Part II, Item 8 of this Report.

2. FINANCIAL STATEMENTS SCHEDULE

Schedules are omitted because they are not applicable, or are not required, or because the information is included in the consolidated financial statements and notes thereto.

3. EXHIBITS –

The exhibits are listed below under Part IV, Item 15(b) of this Report.

(b) EXHIBITS

| Exhibit | | Incorporated by Reference | | | | |
|---|---|---|---|---|---|---|
| Number | Exhibit Description | Form | Date | Exhibit Number | File Number | Filed Herewith |
| 3.1 | Restated Certificate of Incorporation of Enanta Pharmaceuticals, Inc. | 8-K | 03/28/2013 | 3.1 | 001-35839 | |
| 3.2 | Amended and Restated Bylaws of Enanta Pharmaceuticals, Inc. (as amended and restated in August 2015). | 8-K | 08/18/2015 | 3.2 | 001-35839 | |
| 4.1 | Specimen certificate evidencing shares of common stock. | S-1/A | 02/05/2013 | 4.1 | 333-184779 | |
| 4.2 | Form of Series 1 Non-Convertible Preferred Stock Warrant. | S-1 | 11/06/2012 | 4.2 | 333-184779 | |
| 4.3 | Specimen certificate evidencing shares of Series 1 Non-Convertible Preferred Stock | | | | | X |
| 10.1# | Form of Indemnification Agreement for directors and officers. | S-1/A | 02/05/2013 | 10.7 | 333-184779 | |
| 10.2# | | S-1/A | 03/05/2013 | 10.5 | 333-184779 | |

| | | | | | |
|---|---|---|---|---|---|
| | Amended and Restated Employment Agreement between the Company and Jay R. Luly, Ph.D., dated as of March 4, 2013. | | | | |
| 10.3# | Form of Amended and Restated Employment Agreement for Executive Officers other than Chief Executive Officer. | S-1/A | 03/05/2013 | 10.17 | 333-184779 |

| Exhibit Number | Exhibit Description | Incorporated by Reference Form | Date | Exhibit Number | File Number | Filed Herewith |
|---|---|---|---|---|---|---|
| 10.4† | Collaborative Development and License Agreement between the Company and Abbott Laboratories, dated November 27, 2006; as amended by a First Amendment to Collaborative Development and License Agreement dated January 27, 2009 and a Second Amendment to Collaborative Development and License Agreement dated December 9, 2009 (assigned to AbbVie Inc. as of January 1, 2013). | 10-Q | 02/09/2016 | 10.1 | 001-35839 | |
| 10.5† | Third Amendment to Collaborative Development and License Agreement between the Company and AbbVie dated October 20, 2014. | 10-K | 12/11/2014 | 10.5 | 001-35839 | |
| 10.6 | Fourth Amendment to Collaborative Development and License Agreement between the Company and AbbVie dated as of March 3, 2015. | 10-Q | 05/08/2015 | 10.1 | 001-35839 | |
| 10.7 | Lease Agreement between Company and ARE-500 Arsenal Street LLC, dated as of April 15, 2011. | S-1 | 11/06/2012 | 10.6 | 333-184779 | |
| 10.8 | First Amendment to Lease Agreement made as of March 5, 2015 between the Company and ARE-500 Arsenal Street LLC. | 10-Q | 05/08/2015 | 10.2 | 001-35839 | |
| 10.9 | Third Amended and Restated Registration Rights Agreement, dated as of August 23, 2012. | S-1/A | 11/06/2012 | 10.4 | 333-184779 | |
| 10.10# | Amended and Restated 1995 Equity Incentive Plan. | S-1/A | 03/05/2013 | 10.8 | 333-184779 | |
| 10.11# | Form of Incentive Stock Option Certificate under Amended and Restated 1995 Equity Incentive Plan. | S-1/A | 03/05/2013 | 10.9 | 333-184779 | |
| 10.12# | Form of Non-Statutory Stock Option Certificate under Amended and Restated 1995 Equity Incentive Plan. | S-1/A | 03/05/2013 | 10.10 | 333-184779 | |
| 10.13# | Form of Non-Statutory Stock Option Certificate for directors under Amended and Restated 1995 Equity Incentive Plan. | S-1/A | 03/05/2013 | 10.11 | 333-184779 | |
| 10.14# | 2012 Equity Incentive Plan (As adjusted to reflect the application of the 1-for-4.31 reverse stock split of the Company's common stock effected on March 1, 2013). | 10-K/A | 01/06/2017 | 10.14 | 001-35839 | |
| 10.15# | Form of Incentive Stock Option Agreement under 2012 Equity Incentive Plan. | S-1/A | 03/05/2013 | 10.13 | 333-184779 | |
| 10.16# | Form of Non-Statutory Stock Option Agreement under 2012 Equity Incentive Plan. | S-1/A | 03/05/2013 | 10.14 | 333-184779 | |
| 10.17# | Form of Non-Statutory Stock Option Certificate for directors under 2012 Equity Incentive Plan. | S-1/A | 03/05/2013 | 10.15 | 333-184779 | |
| 10.18# | Form of Performance Share Unit Certificate under 2012 Equity Incentive Plan. | | | | | X |
| 10.19# | Form of Relative Total Stockholder Return Unit Certificate under 2012 Equity Incentive Plan. | | | | | X |
| 10.20# | Employee Stock Purchase Plan. | S-1/A | 02/05/2013 | 10.16 | 333-184779 | |
| 21.1 | Subsidiaries of the Company. | | | | | X |
| 23.1 | | | | | | X |

Consent of PricewaterhouseCoopers LLP, Independent Registered Public Accounting Firm.

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| Exhibit Number | Exhibit Description | Incorporated by Reference | | | | Filed Herewith |
|---|---|---|---|---|---|---|
| | | Form | Date | Exhibit Number | File Number | |
| 31.1 | Certification of the Chief Executive Officer pursuant to Rule 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934. | | | | | X |
| 31.2 | Certification of Chief Financial Officer pursuant to Rule 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934. | | | | | X |
| 32.1 | Certification of the Chief Executive Officer and Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002. | | | | | X |
| 101 | The following materials from the Annual Report of Enanta Pharmaceuticals, Inc. on Form 10-K for the year ended September 30, 2017, formatted in XBRL (eXtensible Business Reporting Language): (i) Consolidated Balance Sheets as of September 30, 2017 and September 30, 2016 of Enanta Pharmaceuticals, Inc., (ii) Consolidated Statements of Operations for the years ended September 30, 2017, 2016, and 2015 of Enanta Pharmaceuticals, Inc., (iii) Consolidated Statements of Comprehensive Income for the years ended September 30, 2017, 2016, and 2015 of Enanta Pharmaceuticals, Inc., (iv) Consolidated Statements of Stockholders' Equity (Deficit) for the years ended September 30, 2017, 2016, and 2015 of Enanta Pharmaceuticals, Inc., (v) Consolidated Statements of Cash Flows for the years ended September 30, 2017, 2016, and 2015 of Enanta Pharmaceuticals, Inc., and (vi) Notes to Consolidated Financial Statements of Enanta Pharmaceuticals, Inc. | | | | | X |

#Management contract or compensatory plan, contract or agreement.

†Confidential treatment granted as to portions of this Exhibit. The confidential portions of this Exhibit have been omitted and are marked by asterisks.

††This Exhibit has been filed separately with the commission pursuant to an application for confidentiality treatment. The confidential portions of this Exhibit have been omitted and are marked by asterisks.

ITEM 16. FORM 10-K SUMMARY

None.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the Company has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized, this 11th day of December, 2017.

ENANTA PHARMACEUTICALS, INC.

By: /s/ Jay R. Luly, Ph.D.
Jay R. Luly, Ph.D.
Chief Executive Officer

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the Company in the capacities and on the dates indicated.

| Signature | Title | Date |
|---|---|---|
| /s/ Jay R. Luly, Ph.D. | President and Chief Executive Officer and Director (Principal Executive Officer) | December 11, 2017 |
| Jay R. Luly, Ph.D. | | |
| /s/ Paul J. Mellett | Chief Financial Officer (Principal Financial and Accounting Officer) | December 11, 2017 |
| Paul J. Mellett | | |
| /s/ Stephen Buckley, Jr. | Director | December 11, 2017 |
| Stephen Buckley, Jr. | | |
| /s/ Bruce L.A. Carter, Ph.D. | Director | December 11, 2017 |
| Bruce L.A. Carter, Ph.D. | | |
| /s/ George S. Golumbeski, Ph.D. | Director | December 11, 2017 |

George S. Golumbeski, Ph.D.

| | | |
|---|---|---|
| /s/ Kristine Peterson | Director | December 11, 2017 |

Kristine Peterson

| | | |
|---|---|---|
| /s/ Lesley Russell, MB. Ch.B., MRCP | Director | December 11, 2017 |

Lesley Russell, MB. Ch.B., MRCP

| | | |
|---|---|---|
| /s/ Terry Vance | Director | December 11, 2017 |

Terry Vance

ENANTA PHARMACEUTICALS, INC.

INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

Report of Independent Registered Public Accounting Firm

To the Board of Directors and Stockholders of

Enanta Pharmaceuticals, Inc.

In our opinion, the accompanying consolidated balance sheets and the related consolidated statements of operations, comprehensive income, changes in stockholders' equity (deficit) and cash flows present fairly, in all material respects, the financial position of Enanta Pharmaceuticals, Inc. and its subsidiary as of September 30, 2017 and 2016, and the results of their operations and their cash flows for each of the three years in the period ended September 30, 2017 in conformity with accounting principles generally accepted in the United States of America. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits. We conducted our audits of these financial statements 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, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

/s/ PricewaterhouseCoopers LLP

Boston, Massachusetts

December 11, 2017

ENANTA PHARMACEUTICALS, INC.

CONSOLIDATED BALANCE SHEETS

(in thousands, except per share data)

| | September 30, 2017 | September 30, 2016 |
|---|---|---|
| Assets | | |
| Current assets: | | |
| Cash and cash equivalents | $ 65,675 | $ 16,577 |
| Short-term marketable securities | 157,994 | 193,507 |
| Accounts receivable | 10,614 | 12,841 |
| Prepaid expenses and other current assets | 3,536 | 9,231 |
| Total current assets | 237,819 | 232,156 |
| Property and equipment, net | 8,049 | 8,004 |
| Long-term marketable securities | 70,038 | 32,119 |
| Deferred tax assets | 10,123 | 8,390 |
| Restricted cash | 608 | 608 |
| Total assets | $ 326,637 | $ 281,277 |
| Liabilities and Stockholders' Equity | | |
| Current liabilities: | | |
| Accounts payable | $ 3,714 | $ 3,377 |
| Accrued expenses and other current liabilities | 7,970 | 4,512 |
| Income taxes payable | 9,298 | — |
| Total current liabilities | 20,982 | 7,889 |
| Warrant liability | 807 | 1,251 |
| Series 1 nonconvertible preferred stock | 762 | 159 |
| Other long-term liabilities | 2,410 | 2,042 |
| Total liabilities | 24,961 | 11,341 |
| Commitments and contingencies (Note 12) | | |
| Stockholders' equity: | | |
| Common stock; $0.01 par value per share, 100,000 shares authorized; 19,120 and 19,036 shares issued and outstanding at September 30, 2017 and September 30, 2016, respectively | 191 | 190 |
| Additional paid-in capital | 256,241 | 242,081 |
| Accumulated other comprehensive income (loss) | (112) | 19 |
| Retained earnings | 45,356 | 27,646 |
| Total stockholders' equity | 301,676 | 269,936 |
| Total liabilities and stockholders' equity | $ 326,637 | $ 281,277 |

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

ENANTA PHARMACEUTICALS, INC.

CONSOLIDATED STATEMENTS OF OPERATIONS

(in thousands, except per share data)

| | Years Ended September 30, | | |
|---|---|---|---|
| | 2017 | 2016 | 2015 |
| Revenue | | | |
| Milestones | $65,000 | $30,000 | $125,000 |
| Royalties | 37,814 | 57,692 | 34,077 |
| Other | — | 576 | 1,803 |
| Total revenue | 102,814 | 88,268 | 160,880 |
| Operating expenses: | | | |
| Research and development | 57,451 | 40,461 | 23,189 |
| General and administrative | 20,749 | 16,966 | 13,543 |
| Total operating expenses | 78,200 | 57,427 | 36,732 |
| Income from operations | 24,614 | 30,841 | 124,148 |
| Other income (expense): | | | |
| Interest income | 2,532 | 1,735 | 968 |
| Interest expense | (40) | (45) | (8) |
| Change in fair value of warrant liability and Series 1 nonconvertible preferred stock | (159) | 29 | 347 |
| Total other income (expense), net | 2,333 | 1,719 | 1,307 |
| Income before income taxes | 26,947 | 32,560 | 125,455 |
| Income tax expense | (9,237) | (10,894) | (46,463) |
| Net income | $17,710 | $21,666 | $78,992 |
| Net income per share: | | | |
| Basic | $0.93 | $1.14 | $4.23 |
| Diluted | $0.91 | $1.13 | $4.09 |
| Weighted average shares outstanding: | | | |
| Basic | 19,066 | 18,929 | 18,673 |
| Diluted | 19,407 | 19,224 | 19,295 |

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

ENANTA PHARMACEUTICALS, INC.

CONSOLIDATED STATEMENTS OF COMPREHENSIVE INCOME

(in thousands)

| | Years Ended September 30, | | |
|---|---|---|---|
| | 2017 | 2016 | 2015 |
| Net income | $17,710 | $21,666 | $78,992 |
| Other comprehensive income (loss): | | | |
| Net unrealized gains (losses) on marketable securities, net of tax of ($78), ($9) and $20 | (131) | (14) | 133 |
| Total other comprehensive income (loss) | (131) | (14) | 133 |
| Comprehensive income | $17,579 | $21,652 | $79,125 |

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

ENANTA PHARMACEUTICALS, INC.

CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY (DEFICIT)

(in thousands)

| | Common Stock | | Additional Paid-In | Treasury Stock | | Accumulated Other Comprehensive Income | Retained Earnings (Accumulated | Total Stockholders' |
|---|---|---|---|---|---|---|---|---|
| | Shares | Amount | Capital | Shares | Amount | (Loss) | Deficit) | Equity |
| Balances at September 30, 2014 | 18,803 | $ 188 | $ 221,580 | (209) | $ (2) | $ (100) | $ (73,012) | $ 148,654 |
| Exercise of stock options | 123 | 1 | 725 | — | — | — | — | 726 |
| Stock-based compensation expense | — | — | 5,838 | — | — | — | — | 5,838 |
| Income tax benefit from stock option exercises | — | — | 1,814 | — | — | — | — | 1,814 |
| Other comprehensive income | — | — | — | — | — | 133 | — | 133 |
| Retirement of treasury stock | (209) | (2) | — | 209 | 2 | — | — | — |
| Net income | — | — | — | — | — | — | 78,992 | 78,992 |
| Balances at September 30, 2015 | 18,717 | $ 187 | $ 229,957 | — | $ — | $ 33 | $ 5,980 | $ 236,157 |
| Exercise of stock options | 319 | 3 | 1,023 | — | — | — | — | 1,026 |
| Stock-based compensation expense | — | — | 9,354 | — | — | — | — | 9,354 |
| Income tax benefit from stock option exercises | — | — | 1,747 | — | — | — | — | 1,747 |
| Other comprehensive loss | — | — | — | — | — | (14) | — | (14) |
| Net income | — | — | — | — | — | — | 21,666 | 21,666 |
| Balances at September 30, 2016 | 19,036 | $ 190 | $ 242,081 | — | $ — | $ 19 | $ 27,646 | $ 269,936 |
| Exercise of stock options | 72 | 1 | 1,078 | — | — | — | — | 1,079 |
| Vesting of restricted stock units, net of withholding | 12 | — | (202) | — | — | — | — | (202) |
| Stock-based compensation expense | — | — | 13,071 | — | — | — | — | 13,071 |
| Income tax benefit from stock option exercises | — | — | 213 | — | — | — | — | 213 |
| Other comprehensive loss | — | — | — | — | — | (131) | — | (131) |
| Net income | — | — | — | — | — | — | 17,710 | 17,710 |
| Balances at September 30, 2017 | 19,120 | $ 191 | $ 256,241 | — | $ — | $ (112) | $ 45,356 | $ 301,676 |

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

ENANTA PHARMACEUTICALS, INC.

CONSOLIDATED STATEMENTS OF CASH FLOWS

(in thousands)

| | Years Ended September 30, | | |
|---|---|---|---|
| | 2017 | 2016 | 2015 |
| Cash flows from operating activities | | | |
| Net income | $17,710 | $21,666 | $78,992 |
| Adjustments to reconcile net income to net cash provided by operating activities: | | | |
| Stock-based compensation expense | 13,071 | 9,354 | 5,838 |
| Depreciation and amortization expense | 2,137 | 1,661 | 639 |
| Deferred income taxes | (1,654) | (2,294) | 11,028 |
| Premium on marketable securities | (1,229) | (518) | (3,042) |
| Amortization of premium on marketable securities | 702 | 1,511 | 2,244 |
| Income tax benefit from exercise of stock options | (213) | (1,747) | (1,814) |
| Change in fair value of warrant liability and Series 1 nonconvertible preferred stock | 159 | (29) | (347) |
| Other non-cash items | — | 34 | (35) |
| Change in operating assets and liabilities: | | | |
| Accounts receivable | 2,227 | 2,448 | (13,565) |
| Unbilled receivables | — | 433 | 2,337 |
| Prepaid expenses and other current assets | 5,678 | (964) | (6,680) |
| Accounts payable | 633 | 1,451 | (543) |
| Accrued expenses | 3,443 | 1,858 | (227) |
| Income taxes payable | 9,511 | 548 | 1,199 |
| Other long-term liabilities | 478 | 397 | 649 |
| Net cash provided by operating activities | 52,653 | 35,809 | 76,673 |
| Cash flows from investing activities | | | |
| Purchase of marketable securities | (251,371) | (192,429) | (196,304) |
| Maturities of marketable securities | 239,287 | 153,504 | 108,407 |
| Sale of marketable securities | 10,018 | — | 2,210 |
| Purchase of property and equipment | (2,506) | (4,738) | (2,336) |
| Other investing activities | — | — | (163) |
| Net cash used in investing activities | (4,572) | (43,663) | (88,186) |
| Cash flows from financing activities | | | |
| Proceeds from exercise of stock options | 1,079 | 1,026 | 726 |
| Income tax benefit from exercise of stock options | 213 | 1,747 | 1,814 |
| Payments of withholding tax for share-based awards | (202) | — | — |
| Payments of capital lease obligations | (73) | (68) | — |
| Net cash provided by financing activities | 1,017 | 2,705 | 2,540 |
| Net increase (decrease) in cash and cash equivalents | 49,098 | (5,149) | (8,973) |
| Cash and cash equivalents at beginning of period | 16,577 | 21,726 | 30,699 |

| | | | |
|---|---|---|---|
| Cash and cash equivalents at end of period | $65,675 | $16,577 | $21,726 |
| Supplemental disclosure of cash flow information: | | | |
| Cash paid for income taxes | $1,588 | $12,616 | $40,072 |
| Non-cash items: | | | |
| Purchases of fixed assets included in accounts payable and accrued expenses | $318 | $637 | $1,562 |
| Fixed assets financed by landlord | $— | $— | $239 |
| Fixed assets purchased through capital lease | $— | $— | $598 |

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

ENANTA PHARMACEUTICALS, INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

(Amounts in thousands, except per share data)

1. Nature of the Business

Enanta Pharmaceuticals, Inc. (the "Company"), incorporated in Delaware in 1995, is a biotechnology company that uses its robust, chemistry-driven approach and drug discovery capabilities to create small molecule drugs primarily for the treatment of viral infections and liver diseases. The Company discovered glecaprevir, the second of two protease inhibitors discovered and developed through the Company's collaboration with AbbVie and marketed as part of AbbVie's new direct-acting antiviral (DAA) regimen under the tradenames MAVYRET™ (U.S.) or MAVIRET™ (ex-U.S.) (glecaprevir/pibrentasvir) for the treatment of chronic hepatitis C virus, or HCV. The other protease inhibitor under the Company's HCV collaboration is part of AbbVie's initial DAA regimens for the treatment of chronic HCV marketed under the tradenames VIEKIRA PAK® (paritaprevir/ritonavir/ombitasvir/dasabuvir) (U.S.) or VIEKIRAX® (paritaprevir/ritonavir/ombitasvir) (ex-U.S.). The royalties from the AbbVie collaboration and the Company's existing financial resources provides funding to support the Company's wholly owned research and development efforts, which are currently focused on the following disease targets: hepatitis B virus ("HBV"); non-alcoholic steatohepatitis ("NASH"); primary biliary cholangitis ("PBC"); and respiratory syncytial virus ("RSV").

The Company is subject to risks common to companies in the biotechnology industry including, but not limited to, the uncertainties of research and development, competition from technological innovations of others, dependence on collaborative arrangements, protection of proprietary technology, dependence on key personnel and compliance with government regulations and the need to obtain additional financing. Product candidates currently under development will require significant additional research and development efforts, including extensive preclinical and clinical testing and regulatory approvals, prior to commercialization. These efforts require significant amounts of capital and adequate personnel infrastructure and compliance reporting capabilities.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying consolidated financial statements include those of the Company and its subsidiary, Enanta Pharmaceuticals Security Corporation, after elimination of all intercompany accounts and transactions. The accompanying consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP").

Use of Estimates

The preparation of consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the consolidated financial statements, and the reported amounts of revenues and expenses during the reporting period. Significant estimates and assumptions reflected in these consolidated financial statements include, but are not limited to, management's judgments of separate units of accounting and best estimate of selling price of those units of accounting within its revenue arrangements; valuation of warrants, Series 1 nonconvertible preferred stock and stock-based awards; and the accounting for income taxes, including uncertain tax positions and the valuation of net deferred tax assets. Estimates are periodically reviewed in light of changes in circumstances, facts and experience. Actual results could differ from the Company's estimates.

Cash Equivalents and Marketable Securities

The Company considers all short-term, highly liquid investments with original maturities of ninety days or less at acquisition date to be cash equivalents. Marketable securities with original maturities of greater than ninety days and remaining maturities of less than one year from the balance sheet date are classified as short-term marketable securities. Marketable securities with remaining maturities of greater than one year from the balance sheet date are classified as long-term marketable securities.

The Company classifies all of its marketable securities as available-for-sale. All marketable securities are held with one investment manager. The Company continually evaluates the credit ratings of its investment portfolio and underlying securities. The Company invests in accordance with its investment policy and invests at the date of purchase in securities with a rating of A3 or higher and A- or higher according to Moody's and S&P, respectively. The Company reports available-for-sale investments at fair value as of each balance sheet date and records any unrealized gains or losses as a component of stockholders' equity. The cost of securities sold is determined on a specific identification basis, and realized gains and losses are included in other income (expense), net within the consolidated statements of operations. If any adjustment to fair value reflects a decline in the value of the investment, the Company considers available evidence to evaluate the extent to which the decline is "other than temporary" and reduces the investment to fair value through a charge to the consolidated statements of operations. There were no such adjustments necessary during the years ended September 30, 2017, 2016, and 2015.

Restricted Cash

As of September 30, 2017 and 2016, the Company had an outstanding letter of credit collateralized by a money market account of $608 to the benefit of the landlord of the Company's current building lease. This amount was classified as long-term restricted cash as of September 30, 2017 and 2016.

Concentration of Credit Risk and of Significant Customers and Suppliers

Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash, cash equivalents, marketable securities, accounts receivable and unbilled receivables. The Company has all cash and investment balances at one accredited financial institution, including cash in amounts that exceed federally insured limits. The Company does not believe that it is subject to unusual credit risk beyond the normal credit risk associated with commercial banking relationships.

The Company has historically generated all of its revenue from its collaborative research and license agreements as well as a U.S. government contract (see Note 7). As of September 30, 2017 and 2016, accounts receivable consisted of amounts due from the Company's principal collaborator (see Note 7).

The Company is completely dependent on third-party manufacturers for product supply for preclinical and clinical research activities in its non-partnered programs. The Company relies and expects to continue to rely exclusively on several manufacturers to supply it with its requirements for the active pharmaceutical ingredients related to these programs. These research programs would be adversely affected by a significant interruption in the supply of its active pharmaceutical ingredients.

Fair Value Measurements

Certain assets and liabilities are carried at fair value under GAAP. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. A fair value hierarchy is based on three levels of inputs which are used to measure fair value, of which the first two levels are considered observable and the last is considered unobservable:

- Level 1—Quoted prices in active markets for identical assets or liabilities.
- Level 2—Observable inputs (other than Level 1 quoted prices) such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities, or other inputs that are observable or can be corroborated by observable market data.

- Level 3—Unobservable inputs that are supported by little or no market activity and that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

The Company's instruments that are carried at fair value are cash equivalents, marketable securities and the warrant and Series 1 nonconvertible preferred stock liabilities. The carrying values of accounts receivable and unbilled

receivables, accounts payable and accrued expenses approximate their fair value due to the short-term nature of these assets and liabilities.

Property and Equipment

Property and equipment are stated at cost less accumulated depreciation or amortization. Depreciation and amortization expense is recognized using the straight-line method over the following estimated useful lives:

| | |
|---|---|
| Laboratory and office equipment | 5 years |
| Leasehold improvements | Shorter of life of lease or estimated useful life |
| Purchased software | 3 years |
| Computer equipment | 3 years |
| Furniture | 7 years |

Expenditures for repairs and maintenance of assets are charged to expense as incurred. Costs of major additions and betterments are capitalized and depreciated on a straight-line basis over their useful lives. Upon retirement or sale, the cost and related accumulated depreciation or amortization of assets disposed are removed from the accounts and any resulting gain or loss is included in income from operations in the consolidated statements of operations.

Impairment of Long-Lived Assets

The Company reviews long-lived assets, including property and equipment, for impairment whenever events or changes in business circumstances indicate that the carrying amount of the assets may not be fully recoverable. Factors that the Company considers in deciding when to perform an impairment review include significant underperformance of the business in relation to expectations, significant negative industry or economic trends, and significant changes or planned changes in the use of the assets. If an impairment review is performed to evaluate a long-lived asset for recoverability, the Company compares forecasts of undiscounted cash flows expected to result from the use and eventual disposition of the long-lived asset to its carrying value. An impairment loss would be recognized when estimated undiscounted future cash flows expected to result from the use of an asset are less than its carrying amount. The impairment loss would be based on the excess of the carrying value of the impaired asset over its fair value, determined based on discounted cash flows. To date, the Company has not recorded any impairment losses on long-lived assets.

Revenue Recognition

The Company’s revenue has been generated primarily through collaborative research and license agreements. The terms of these agreements contain multiple deliverables, which may include (i) licenses, (ii) research and development activities, and (iii) participation in joint research and development steering committees. The terms of these agreements may include nonrefundable upfront license fees, payments for research and development activities, payments based upon the achievement of certain milestones, and royalty payments based on product sales derived from the collaboration. In all instances, revenue is recognized only when the price is fixed or determinable, persuasive evidence of an arrangement exists, delivery has occurred or the services have been rendered, collectibility of the resulting receivable is reasonably assured, and the Company has fulfilled its performance obligations under the contract.

On October 1, 2011, the Company adopted Accounting Standards Update No. 2009-13, Multiple-Deliverable Revenue Arrangements (“ASU 2009-13”). This guidance, which applies to multiple-element arrangements entered into or materially modified on or after October 1, 2011, amends the criteria for separating and allocating consideration in a multiple-element arrangement by modifying the fair value requirements for revenue recognition and eliminating the

use of the residual value method. The selling prices of deliverables under the arrangement may be derived using third-party evidence ("TPE") or a best estimate of selling price ("BESP"), if vendor-specific objective evidence ("VSOE") is not available. The objective of BESP is to determine the price at which the Company would transact a sale if the element within the license agreement was sold on a standalone basis. Establishing BESP involves management's judgment and considers multiple factors, including market conditions and company-specific factors including those factors contemplated in negotiating the agreements as well as internally developed models that include assumptions related to market opportunity, discounted cash flows,

estimated development costs, probability of success, and the time needed to commercialize a product candidate pursuant to the license. Deliverables under a multiple-element arrangement are separated into multiple units if (i) the delivered item has value to the customer on a standalone basis, and (ii) if the arrangement includes a general right of return relative to the delivered item, delivery or performance of the undelivered item is considered probable and substantially within the control of the Company. The arrangement consideration that is fixed or determinable at the inception of the arrangement is allocated to the separate units of accounting based on their relative selling prices derived using TPE or BESP. The appropriate revenue recognition model is applied to each element, and revenue is accordingly recognized as each element is delivered. The Company may exercise significant judgment in determining whether a deliverable is a separate unit of accounting.

In determining the separate units of accounting, the Company evaluates whether the license has standalone value to the collaborator based on consideration of the relevant facts and circumstances for each arrangement. Factors considered in this determination include the research and development capabilities of the collaborator and the availability of relevant research expertise in the marketplace. In addition, the Company considers whether or not (i) the collaborator can use the license for its intended purpose without the receipt of the remaining deliverables, (ii) the value of the license is dependent on the undelivered items, and (iii) the collaborator or other vendors can provide the undelivered items.

For all periods presented, whenever the Company determines that an element is delivered over a period of time, revenue is recognized using either a proportional performance model or a straight-line model over the period of performance, which is typically the research and development term. Full-time equivalents (“FTEs”) are typically used as the measure of performance. At each reporting period, the Company reassesses its cumulative measure of performance and makes appropriate adjustments, if necessary. The Company recognizes revenue using the proportional performance model whenever the Company can make reasonably reliable estimates of the level of effort required to complete its performance obligations under an arrangement. Revenue recognized under the proportional performance model at each reporting period is determined by multiplying the total expected payments under the contract (excluding royalties and payments contingent upon achievement of milestones) by the ratio of the level of effort incurred to date to the estimated total level of effort required to complete the performance obligations under the arrangement. Revenue is limited to the lesser of the cumulative amount of payments received or the cumulative amount of revenue earned, as determined using the proportional performance model as of each reporting period. Alternatively, if the Company cannot make reasonably reliable estimates of the level of effort required to complete its performance obligations under an arrangement, then revenue under the arrangement is recognized on a straight-line basis over the period expected to complete the Company’s performance obligations. If and when a contingent milestone payment is earned, the additional consideration to be received is allocated to the separate units of accounting in the arrangement based on their relative selling prices at the inception of the arrangement. Revenue is limited to the lesser of the cumulative amount of payments received or the cumulative amount of revenue earned, as determined on a straight-line basis as of the period end date. If the Company cannot reasonably estimate when its performance obligation period ends, then revenue is deferred until the Company can reasonably estimate when the performance obligation period ends.

Royalty revenue is recognized based on contractual terms when reported sales are reliably measurable and collectibility is reasonably assured, provided that there are no performance obligations remaining.

During the years ended September 30, 2016 and 2015, the Company also generated revenue from a government contract, under which the Company was reimbursed for certain allowable costs for the funded project. Revenue from the government contract was recognized when the related service was performed. The related costs incurred by the Company under the government contract were included in research and development expenses in the consolidated statements of operations.

Amounts received prior to satisfying all revenue recognition criteria are recorded as deferred revenue in the accompanying consolidated balance sheets. Amounts not expected to be recognized as revenue within the next twelve months of the consolidated balance sheet date are classified as long-term deferred revenue.

In the event that a collaborative research and license agreement is terminated and the Company then has no further performance obligations, the Company recognizes as revenue any amounts that had not previously been recorded as revenue but were classified as deferred revenue at the date of such termination.

Research and Development Costs

Research and development costs are expensed as incurred. Included in research and development costs are wages, stock-based compensation and benefits of employees, third-party license fees and other operational costs related to the Company's research and development activities, including facility-related expenses and external costs of outside contractors engaged to conduct both preclinical and clinical studies and manufacture quantities of product for preclinical and clinical studies. The Company also includes in research and development expense the costs to complete the Company's obligations under research collaborations.

Patent Costs

All patent-related costs incurred in connection with filing and prosecuting patent applications are recorded as general and administrative expenses as incurred, as recoverability of such expenditures is uncertain.

Stock-Based Compensation

The Company measures all stock options and other stock-based awards granted to employees at fair value on the date of grant. The Company uses the Black-Scholes option-pricing model in the valuation of its stock options. The fair value of performance-based awards and restricted stock units is based on intrinsic value of the stock on the date of grant. The Company uses the Monte-Carlo simulation in order to calculate the fair value of the market-based awards. The fair value of options is recognized as stock-based compensation expense, net of estimated forfeitures, over the requisite service period, which is generally the vesting period of the respective award. The straight-line method of expense recognition is applied to all awards with service-based and market-based conditions. The Company records stock-based compensation expense related to performance-based awards when the performance-based targets are probable of being achieved. The Company classifies stock-based compensation expense in the consolidated statements of operations in the same manner in which the award recipient's payroll costs are classified.

Income Taxes

The Company accounts for income taxes using the asset and liability method, which requires the recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been recognized in the financial statements or in the Company's tax returns. Deferred taxes are determined based on the difference between the financial reporting and tax basis of assets and liabilities using enacted tax rates in effect in the years in which the differences are expected to reverse. Changes in deferred tax assets and liabilities are recorded in the provision for income taxes. The Company assesses the likelihood that its deferred tax assets will be recovered from future taxable income and, to the extent it believes based upon the weight of available evidence, that it is more likely than not that all or a portion of deferred tax assets will not be realized, a valuation allowance is established through a charge to income tax expense. Potential for recovery of deferred tax assets is evaluated by estimating the future taxable profits expected and considering prudent and feasible tax planning strategies.

Uncertain tax positions represent tax positions for which reserves have been established. The Company accounts for uncertainty in income taxes recognized in the consolidated financial statements by applying a two-step process to determine the amount of tax benefit to be recognized. First, the tax position must be evaluated to determine the likelihood that it will be sustained upon external examination by the taxing authorities. If the tax position is deemed more likely than not to be sustained, the tax position is then assessed to determine the amount of benefit to be recognized in the financial statements. The amount of the benefit that may be recognized is the largest amount that has a greater than 50% likelihood of being realized upon ultimate settlement. The provision for income taxes includes the effects of any resulting tax reserves, or unrecognized tax benefits, that are considered appropriate as well as the related net interest and penalties.

Net Income per Share

Basic net income per common share is computed by dividing the net income by the weighted average number of shares of common stock outstanding for the period. Diluted net income per common share is computed by dividing net income by the weighted average number of common shares outstanding for the period, including potential dilutive common shares assuming the dilutive effect of outstanding stock options and unvested restricted stock units.

Market-based awards are included in diluted net income per common share to the extent they would have vested if the period end date was the market criteria measurement date.

Segment Data

The Company manages its operations as a single segment for the purposes of assessing performance and making operating decisions. The Company is a biotechnology company focused on discovering and developing small molecule drugs for the treatment of viral infections and liver diseases. Revenue is generated exclusively from transactions occurring with partners located in the United States and all assets are held in the United States.

Comprehensive Income

Comprehensive income includes net income as well as other changes in stockholders' equity that result from transactions and economic events other than those with stockholders. The Company's only element of other comprehensive income is unrealized gains and losses on available-for-sale marketable securities.

Going Concern

In August 2014, the Financial Accounting Standards Board (the "FASB") issued ASU 2014-15, Presentation of Financial Statements - Going Concern (Subtopic 205-40) ("ASU 2014-15"). The Company adopted this standard as of September 30, 2017. The standard requires the Company to assess its ability to continue as a going concern one year beyond the date of filing and, in certain circumstances, provide additional footnote disclosures. Based on a detailed cash forecast incorporating current research and development activities and related spending plans, the Company believes that current cash, cash equivalents and marketable securities on hand at September 30, 2017 should be sufficient to fund operations for the foreseeable future, including at least the next twelve months beyond the date of issuance of these financial statements. The amount of capital available will depend on the Company's management of its existing cash, cash equivalents and marketable securities, as well as the level of future royalties the Company earns under its collaboration with AbbVie. If the Company should require financing beyond these resources to fund its research and development efforts, it may not be able to obtain financing on acceptable terms, or at all.

Recently Issued Accounting Pronouncements

In March 2016, the FASB issued ASU No. 2016-09, Improvements to Employee Share-Based Payment Accounting ("ASU 2016-09"), which intends to simplify several aspects of accounting for share-based payment transactions, including the income tax consequences, classification of awards as either equity or liabilities, a choice to recognize gross stock compensation expense with actual forfeitures recognized as they occur, as well as certain classifications on the statement of cash flows. This amendment will be effective for the Company in the fiscal year beginning October 1, 2017. The Company expects to change its forfeiture rate policy on a prospective basis by recording forfeitures as they occur. Upon adoption, the cumulative impact of this policy change to retained earnings will not be material to the consolidated balance sheet. Once adopted, the consolidated statements of cash flows will present any excess tax benefits as a cash flow from operating activities. The Company has elected to adopt this change on a prospective basis. The adoption of the standard is also expected to create variability in the consolidated statements of operations on a prospective basis as the tax consequences of settled share-based payments will be recognized in income tax expense when share-based payment awards are settled.

In May 2014, the FASB issued ASU No. 2014-09, Revenue from Contracts with Customers (Topic 606) ("ASU 2014-09") which supersedes all existing revenue recognition requirements, including most industry-specific guidance. The new standard requires a company to recognize revenue when it transfers goods or services to customers in an amount that reflects the consideration that the company expects to receive for those goods or services. The FASB has

continued to issue accounting standards updates to clarify and provide implementation guidance related to Revenue from Contracts with Customers, including ASU 2016-08, Revenue from Contract with Customers: Principal versus Agent Considerations, ASU 2016-10, Revenue from Contracts with Customers: Identifying Performance Obligations and Licensing, and ASU 2016-12, Revenue from Contracts with Customers: Narrow-Scope Improvements and Practical Expedients. These amendments address a number of areas, including an

entity's identification of its performance obligations in a contract, collectibility, non-cash consideration, presentation of sales tax and an entity's evaluation of the nature of its promise to grant a license of intellectual property and whether or not that revenue is recognized over time or at a point in time. The new guidance must be adopted using either a modified retrospective approach or a full retrospective approach for all periods presented. Under the modified retrospective method, the cumulative effect of applying the new standard would be recognized at the adoption date in retained earnings on the consolidated balance sheet. Under the full retrospective approach, the new standard would be applied to each prior reporting period presented. These new standards will be effective for the Company beginning October 1, 2018. Currently, the Company has only one revenue-generating contract – the AbbVie Agreement. The Company has completed its substantial performance obligations under the contract and is eligible to earn annually tiered per-product royalties on the portion of AbbVie's net sales of HCV regimens allocable to the protease inhibitor in the regimen. The Company is in process of determining the method of adoption but under either method, the impact of adoption is not expected to have a material impact on the Company's consolidated financial statements as it only has one revenue-generating arrangement outstanding to date in which all performance obligations have been achieved.

In November 2016, the FASB issued ASU No. 2016-18, Statement of Cash Flows (Topic 230): Restricted Cash ("ASU 2016-18") that changes the presentation of restricted cash and cash equivalents on the statement of cash flows. Restricted cash and restricted cash equivalents will be included with cash and cash equivalents when reconciling the beginning-of-period and end-of-period total amounts shown on the statement of cash flows. This amendment is effective for the Company in the fiscal year beginning October 1, 2018, but early adoption is permissible. The Company is currently evaluating the potential impact that ASU 2016-18 may have on its statement of cash flows.

In February 2016, the FASB issued ASU No. 2016-02, Leases ("ASU 2016-02"), which will replace the existing guidance in ASC 840, "Leases." The updated standard aims to increase transparency and comparability among organizations by requiring lessees to recognize leased assets and leased liabilities on the consolidated balance sheets and requiring disclosure of key information about leasing arrangements. This amendment is effective for the Company in the fiscal year beginning October 1, 2019, but early adoption is permissible. The Company is currently evaluating the potential impact that ASU 2016-02 may have on its financial position and results of operations.

In March 2017, the FASB issued ASU No. 2017-08, Receivables—Nonrefundable Fees and Other Costs (Subtopic 310-20): Premium Amortization on Purchased Callable Debt Securities ("ASU 2017-08") which requires companies to amend the amortization period for premiums on debt securities with explicit call features to be the earliest call date rather than through the contractual life of the debt instrument. This amendment aims to more closely align the recognition of interest income with the manner in which market participants price such instruments. This amendment is effective for the Company in the fiscal year beginning October 1, 2019, but early adoption is permissible. The Company is currently evaluating the potential impact that ASU 2017-08 may have on its financial position and results of operations.

In May 2017, the FASB issued ASU No. 2017-09, Compensation—Stock Compensation (Topic 718) ("ASU 2017-09") which provides updated guidance about changes to the terms or conditions of a share-based payment award that require companies to apply modification accounting under Topic 718. This amendment is effective for the Company in the fiscal year beginning October 1, 2018, but early adoption is permissible. The Company is currently evaluating the potential impact that ASU 2017-09 may have on its financial position and results of operations.

In June 2016, the FASB issued ASU No. 2016-13, Financial Instruments—Credit Losses ("ASU 2016-13"), which introduces a new methodology for accounting for credit losses on financial instruments, including available-for-sale debt securities. The guidance establishes a new "expected loss model" that requires entities to estimate current expected credit losses on financial instruments by using all practical and relevant information. Any expected credit losses are to be reflected as allowances rather than reductions in the amortized cost of available-for-sale debt securities. This amendment is effective for the Company in the fiscal year beginning October 1, 2020. The Company is currently

evaluating the potential impact that ASU 2016-13 may have on its financial position and results of operations.

Other accounting standards that have been issued or proposed by the FASB or other standards-setting bodies that do not require adoption until a future date are not expected to have a material impact on the Company's consolidated financial statements upon adoption.

3. Fair Value of Financial Assets and Liabilities

The following tables present information about the Company's financial assets and liabilities that were subject to fair value measurement on a recurring basis as of September 30, 2017 and 2016 and indicate the fair value hierarchy of the valuation inputs utilized to determine such fair value:

| | Fair Value Measurements at September 30, 2017 Using: | | | |
|---|---|---|---|---|
| | Level 1 | Level 2 | Level 3 | Total |
| | (in thousands) | | | |
| Assets: | | | | |
| Cash equivalents: | | | | |
| Money market funds | $19,863 | $— | $— | $19,863 |
| Commercial paper | — | 29,756 | — | 29,756 |
| Corporate bonds | — | 3,000 | — | 3,000 |
| Marketable securities: | | | | |
| U.S. Treasury notes | 60,843 | — | — | 60,843 |
| Corporate bonds | — | 150,731 | — | 150,731 |
| Commercial paper | — | 12,458 | — | 12,458 |
| U.S. Agency bonds | — | 4,000 | — | 4,000 |
| | $80,706 | $199,945 | $— | $280,651 |
| Liabilities: | | | | |
| Warrant liability | $— | $— | $807 | $807 |
| Series 1 nonconvertible preferred stock | — | — | 762 | 762 |
| | $— | $— | $1,569 | $1,569 |

| | Fair Value Measurements at September 30, 2016 Using: | | | |
|---|---|---|---|---|
| | Level 1 | Level 2 | Level 3 | Total |
| | (in thousands) | | | |
| Assets: | | | | |
| Cash equivalents: | | | | |
| Money market funds | $15,295 | $— | $— | $15,295 |
| Marketable securities: | | | | |
| U.S. Treasury notes | 69,608 | — | — | 69,608 |
| Corporate bonds | — | 76,073 | — | 76,073 |
| Commercial paper | — | 49,900 | — | 49,900 |
| U.S. Agency bonds | — | 30,045 | — | 30,045 |
| | $84,903 | $156,018 | $— | $240,921 |
| Liabilities: | | | | |

| | | | | |
|---|---|---|---|---|
| Warrant liability | $— | $— | $1,251 | $1,251 |
| Series 1 nonconvertible preferred stock | — | — | 159 | 159 |
| | $— | $— | $1,410 | $1,410 |

Cash equivalents at September 30, 2017 and 2016 consist of money market funds, corporate bonds and commercial paper which are readily convertible to cash and with less than 90 days until maturity.

During the years ended September 30, 2017, 2016, and 2015, there were no transfers between Level 1, Level 2 and Level 3.

As of September 30, 2017 and 2016, the warrant liability was comprised of the value of the warrants for the purchase of Series 1 nonconvertible preferred stock measured at fair value. As of September 30, 2017 and 2016, the outstanding Series 1 nonconvertible preferred stock was also measured at fair value. The fair values of these instruments were based on significant inputs not observable in the market, which represented a Level 3 measurement within the fair value hierarchy. The Company utilized a probability-weighted valuation model which takes into consideration various outcomes that may require the Company to transfer assets upon exercise. Changes in the fair values of the warrant liability and Series 1 nonconvertible preferred stock are recognized in other income (expense), net, in the consolidated statements of operations.

The recurring Level 3 fair value measurements of the Company's warrant liability and Series 1 nonconvertible preferred stock using probability-weighted discounted cash flow include the following significant unobservable inputs:

| | Range (Weighted Average) September 30, | |
|---|---|---|
| Unobservable Input | 2017 | 2016 |
| Probabilities of payout | 0%-65% | 0%-60% |
| Discount rate | 5.25% | 4.50% |

The following table provides a rollforward of the aggregate fair values of the Company's warrants for the purchase of Series 1 nonconvertible preferred stock and the outstanding Series 1 nonconvertible preferred stock for which fair value is determined by Level 3 inputs:

| | Warrant Liability | Series 1 Nonconvertible Preferred Stock |
|---|---|---|
| | (in thousands) | |
| Balance, September 30, 2014 | $1,584 | $ 202 |
| Decrease in fair value | (308) | (39) |
| Balance, September 30, 2015 | $1,276 | $ 163 |
| Decrease in fair value | (25) | (4) |
| Balance, September 30, 2016 | $1,251 | $ 159 |
| Warrants exercised | (549) | 549 |
| Increase in fair value | 105 | 54 |
| Balance, September 30, 2017 | $807 | $ 762 |

4. Marketable Securities

As of September 30, 2017 and 2016, the fair value of available-for-sale marketable securities, by type of security, was as follows:

| | September 30, 2017 | | | |
|---|---|---|---|---|
| | Amortized Cost | Gross Unrealized Gains | Gross Unrealized Losses | Fair Value |
| | (in thousands) | | | |
| Corporate bonds | $150,841 | $ 9 | $ (119) | $150,731 |
| U.S. Treasury notes | 60,908 | — | (65) | 60,843 |
| Commercial paper | 12,458 | — | — | 12,458 |
| U.S. Agency bonds | 4,004 | — | (4) | 4,000 |
| | $228,211 | $ 9 | $ (188) | $228,032 |

| | September 30, 2016 | | | |
|---|---|---|---|---|
| | Amortized Cost | Gross Unrealized Gains | Gross Unrealized Losses | Fair Value |
| | (in thousands) | | | |
| Corporate bonds | $76,077 | $ 27 | $ (31) | $76,073 |
| U.S. Treasury notes | 69,579 | 38 | (9) | 69,608 |
| Commercial paper | 49,900 | — | — | 49,900 |
| U.S. Agency bonds | 30,040 | 15 | (10) | 30,045 |
| | $225,596 | $ 80 | $ (50) | $225,626 |

As of September 30, 2017 and 2016, marketable securities consisted of investments that mature within one year, with the exception of certain corporate bonds and U.S. Treasury notes, which have maturities between one and three years and an aggregate fair value of $70,038 and $32,119, respectively.

5. Property and Equipment

Property and equipment consisted of the following as of September 30, 2017 and 2016:

| | September 30, | |
|---|---|---|
| | 2017 | 2016 |
| | (in thousands) | |
| Laboratory and office equipment | $9,521 | $7,440 |
| Leasehold improvements | 3,717 | 3,715 |
| Purchased software | 775 | 705 |
| Furniture | 595 | 551 |
| Construction in progress | 250 | 504 |
| Computer equipment | 245 | 187 |
| | 15,103 | 13,102 |
| Less: Accumulated depreciation and amortization | (7,054) | (5,098) |
| | $8,049 | $8,004 |

Depreciation and amortization expense for property and equipment, including assets acquired under capital leases, was $2,137, $1,661 and $639 for the years ended September 30, 2017, 2016, and 2015, respectively.

6. Accrued Expenses, Other Current Liabilities and Other Long-Term Liabilities

Accrued expenses, other current liabilities and other long-term liabilities consisted of the following as of September 30, 2017 and 2016:

| | September 30, | |
|---|---|---|
| | 2017 | 2016 |
| Accrued expenses: | (in thousands) | |
| Accrued preclinical and clinical expenses | $3,156 | $899 |
| Accrued payroll and related expenses | 2,829 | 2,384 |
| Accrued vendor manufacturing | 1,130 | 459 |
| Accrued professional fees | 456 | 393 |
| Capital lease obligation | 79 | 73 |
| Accrued other | 320 | 304 |
| | $7,970 | $4,512 |
| | | |
| Other long-term liabilities: | | |
| Uncertain tax positions | $1,175 | $745 |
| Accrued rent expense | 676 | 696 |
| Capital lease obligation | 379 | 458 |
| Asset retirement obligation | 180 | 143 |
| | $2,410 | $2,042 |

7. Collaboration Agreements

AbbVie Collaboration

On November 27, 2006, the Company entered into a Collaborative Development and License Agreement (the “AbbVie Agreement”) with Abbott Laboratories to identify, develop and commercialize HCV NS3 and NS3/4A protease inhibitor compounds, including paritaprevir and glecaprevir. The agreement was assigned by Abbott to AbbVie Inc. on January 1, 2013 in connection with Abbott’s transfer of its research-based pharmaceuticals business to AbbVie.

Under the terms of the AbbVie Agreement, as amended, AbbVie paid the Company upfront license payments and FTE reimbursements to fund research activities. The Company is also eligible to receive milestone payments for the successful development by AbbVie of one or more HCV compounds, as well as annually tiered, per-product royalties on the portion of AbbVie’s net sales of its HCV treatment regimens allocated to the protease inhibitor product.

The Company determined that the deliverables under the AbbVie Agreement included (i) the non-exclusive, royalty-free, worldwide research license and the exclusive, royalty-bearing development and commercialization license, (ii) the research services, and (iii) a commitment to participate on a steering committee, all of which were to be delivered over a three-year period. The Company concluded that the license did not have standalone value as it was dependent, in part, upon the Company’s continuing involvement in the HCV protease inhibitor research and its involvement in the joint steering committee. Additionally, the undelivered items, including the Company’s participation in the joint steering committee, which was considered participatory due to its decision making responsibilities, and the research services, did not have VSOE or VOE of fair value. Therefore, the license, the research services, and the joint steering committee participation were treated as a single unit of accounting. Accordingly, all amounts received were deferred, and revenue was recognized using the proportional performance model over the period during which the Company performed research services in connection with the AbbVie Agreement, as amended.

Subsequent to the research and evaluation period, which ended in June 2011, all decisions related to the development, commercialization and marketing have been made by AbbVie. The Company has the right to continue to attend the joint steering committee meetings to monitor the development and marketing plans; however, the Company has no

decision-making rights. As such, the joint steering committee commitment became protective in nature as of June 16, 2011.

During the years ended September 30, 2017, 2016, and 2015, the Company received $65,000, $30,000, and $125,000, respectively, in milestone payments under the AbbVie Agreement as a result of AbbVie’s

commercialization regulatory approvals for regimens containing the collaboration's first protease inhibitor. From commencement of the collaboration through September 30, 2017, the Company received a total of $500,000 under the AbbVie Agreement consisting of an upfront license payment, research funding, milestone payments, royalties and preferred stock financing. As of September 30, 2017, the Company is eligible to receive an additional milestone payment of $15,000 upon AbbVie's achievement of commercialization regulatory approval in Japan for MAVIRET. Since the Company completed all its performance obligations under the AbbVie Agreement by the end of fiscal 2011, any milestone payments earned since then have been and will be recognized as revenue when the associated milestone is achieved by AbbVie.

The Company receives annually tiered royalties on each protease product developed under the AbbVie Agreement, ranging from the low double digits up to twenty percent, or on a blended basis from the low double digits up to the high teens, based on the portion of AbbVie's calendar year net sales of the corresponding HCV regimen that is allocated to the protease product contained in the regimen. The portion of a product that is determined to be a combination product, as is the case for both regimens containing paritaprevir or glecaprevir, the net sales of the combination product are adjusted on a country-by-country and product-by-product basis to reflect a good faith determination of the relative value of each pharmaceutically active ingredient, based on the estimated fair market value. Under the terms of the amended agreement, 50% of AbbVie's net sales of regimens containing glecaprevir are allocated to glecaprevir net sales for purposes of calculating royalties, whereas only 30% of net sales of a 3-DAA regimen containing paritaprevir and 45% of net sales of a 2-DAA regimen containing paritaprevir are allocated to paritaprevir net sales.

Royalties owed to the Company under the agreement can be reduced by AbbVie in certain circumstances, including (i) if AbbVie exercises its right to license or otherwise acquire rights to intellectual property controlled by a third party where a product could not be legally developed or commercialized in a country without the third-party intellectual property right, (ii) where a product developed under the collaboration agreement is sold in a country and not covered by a valid patent claim in such country, and (iii) where sales of a generic product are equal to at least a specified percentage of AbbVie's market share of its product in a country.

AbbVie's obligation to pay royalties on a product developed under the agreement expires on a country-by-country basis upon the later of (i) the date of expiration of the last of the licensed patents with a valid claim covering the product in the applicable country, or (ii) ten years after the first commercial sale of the product in the applicable country.

Subject to certain exceptions, a party's rights and obligations under the agreement continue until (i) such time as AbbVie is no longer developing a product candidate or (ii) if, as of the time AbbVie is no longer developing any product candidates, AbbVie is commercializing any other protease inhibitor product, such time as all royalty terms for all covered products have ended. Accordingly, the final expiration date of the agreement is currently indeterminable.

Either party may terminate the agreement for cause in the event of a material breach, subject to prior notice and the opportunity to cure, or in the event of the other party's bankruptcy. Additionally, AbbVie may terminate the agreement for any reason upon specified prior notice.

If the Company terminates the agreement for cause or AbbVie terminates without cause, any licenses and other rights granted to AbbVie will terminate and AbbVie will be deemed to have granted the Company (i) a non-exclusive, perpetual, fully-paid, worldwide, royalty-free license, with the right to sublicense, under AbbVie's intellectual property used in any product candidate, and (ii) an exclusive (even as to AbbVie), perpetual, fully-paid, worldwide, royalty-free license, with the right to sublicense, under AbbVie's interest in any joint intellectual property rights to develop product candidates resulting from covered compounds and to commercialize any products derived from such compounds. Upon the Company's request, AbbVie will also transfer to the Company all right, title and interest in any

related product trademarks, regulatory filings and clinical trials.

If AbbVie terminates the agreement for the Company's uncured breach, the milestone and royalty payments payable by AbbVie may be reduced, the licenses granted to AbbVie will remain in place, the Company will be deemed to have granted AbbVie an exclusive license under the Company's interest in joint intellectual property, AbbVie will

continue to have the right to commercialize any covered products, and all rights and licenses granted to the Company by AbbVie will terminate.

NIAID Contract

On September 30, 2011, the Company entered into a contract with the National Institute of Allergy and Infectious Diseases ("NIAID"), a division of the National Institutes of Health ("NIH"), providing development funding to the Company for the preclinical and clinical development of a bridged bicyclic antibiotic. The contract was completed in August 2015 upon the Company's delivery of the study report for the Phase 1 clinical study.

The Company recognized revenue under this contract as development services were performed in accordance with the funding agreement. During the years ended September 30, 2016 and 2015, the Company recognized revenue of $576 and $1,803, respectively, under this contract. The Company received aggregate payments of $20,637 under the NIAID contract from its commencement through January 31, 2016.

8. Stockholders' Equity

The Company is authorized to issue 100,000 shares of common stock at a par value of $0.01. Each share of common stock entitles the holder to one vote on all matters submitted to a vote of the Company's stockholders. Common stockholders are entitled to receive such dividends as may be declared by the board of directors, if any.

The Company previously had 209 shares of treasury stock outstanding, which were retired on September 30, 2015, resulting in a decrease in the same number of issued shares of common stock.

9. Series 1 Nonconvertible Preferred Stock and Warrants

The Company's Certificate of Incorporation authorizes the issuance of up to 2,000 shares of Series 1 nonconvertible preferred stock at a par value of $0.01 per share. Holders of Series 1 nonconvertible preferred stock are not entitled to receive dividends. In the event of any liquidation, deemed liquidation, dissolution or winding up of the Company, the Series 1 nonconvertible preferred stockholders are entitled to receive in preference to all other stockholders, an amount equal to $1.00 per share, adjusted for any stock dividends, stock splits or reclassifications. Series 1 nonconvertible preferred stockholders will not be entitled to vote unless required by the Company pursuant to the laws of the State of Delaware. The Company may redeem the Series 1 nonconvertible preferred stock with the approval of the holders of a majority of the outstanding shares of Series 1 nonconvertible preferred stock at a redemption price of $1.00 per share. The Company must redeem the stock within 60 days of such election. Shares that are redeemed will be retired or canceled and not reissued by the Company.

In October and November 2010, a total of 2,000 warrants to purchase Series 1 nonconvertible preferred stock were issued. The warrants had an expiration date of October 4, 2017. As these warrants are free-standing financial instruments that may require the Company to transfer assets upon exercise, up to a maximum of $2,000, these warrants are classified as liabilities on the Company's consolidated balance sheet.

The following table summarizes the Company's outstanding warrants:

| | Series 1 Nonconvertible Preferred Stock Warrants Exercisable (in thousands, except per share data) | Weighted Average Exercise Price |
|---|---|---|
| Outstanding, as of September 30, 2014 | 1,775 | $ 0.01 |
| Granted | — | — |
| Expired | — | — |
| Exercised | — | — |
| Outstanding, as of September 30, 2015 | 1,775 | $ 0.01 |
| Granted | — | — |
| Expired | — | — |
| Exercised | — | — |
| Outstanding, as of September 30, 2016 | 1,775 | $ 0.01 |
| Granted | — | — |
| Expired | — | — |
| Exercised | (745) | $ 0.01 |
| Outstanding, as of September 30, 2017 | 1,030 | $ 0.01 |

For the years ended September 30, 2017, 2016, and 2015, the remeasurement of the warrants and Series 1 nonconvertible preferred stock resulted in income (expense) recorded in the consolidated statements of operations of ($159), $29, and $347, respectively. As of September 30, 2017 and 2016, the total fair value of the Series 1 nonconvertible preferred stock was $762 and $159, respectively. As of September 30, 2017 and 2016, the total fair value of the Series 1 nonconvertible preferred stock warrants was $807 and $1,251, respectively.

Subsequent to September 30, 2017, warrants to purchase a total of 977 shares of Series 1 nonconvertible preferred stock were exercised prior to warrant expiration, resulting in the issuance of 970 shares of Series 1 nonconvertible preferred stock. On October 4, 2017, a total of 52 outstanding warrants expired unexercised. The liability associated with these expired warrants will be reversed, resulting in income in 2018.

10. Stock-Based Awards

The Company's 2012 Equity Incentive Plan (the "2012 Plan") permits the Company to sell or issue awards of common stock or restricted common stock or to grant awards of incentive stock options or nonqualified stock options for the

purchase of common stock, restricted stock units, performance units, stock appreciation rights or other cash incentive awards, to employees, members of the board of directors and consultants of the Company. The number of shares of common stock that may be issued under the 2012 Plan is subject to increase by the number of shares forfeited under any options terminated and not exercised under the 2012 Plan or a predecessor plan, known as the 1995 Equity Incentive Plan, as well as by the number of shares added on the first day of each fiscal year, which is the lowest amount among the following: (i) 3% of the Company's outstanding shares of common stock as of that date, (ii) 2,088 shares of common stock, or (iii) an amount determined by the Compensation Committee of the Board of Directors. On October 1, 2017, the number of shares of common stock that might be issued under the 2012 Plan was increased by 574 shares. As of September 30, 2017, 274 shares remained available for future award.

The 2012 Plan replaces and is the successor to the 1995 Equity Incentive Plan (the "1995 Plan"). The 1995 Plan provided for the Company to sell or issue awards of common stock or restricted common stock, or to grant awards of incentive stock options or nonqualified stock options for the purchase of common stock, to employees, members of the board of directors and consultants of the Company. Sales, issuances or grants of shares entitle the holder to purchase common stock from the Company, for a specified exercise price, during a period specified by the applicable equity award agreement. Upon the closing of the Company's initial public offering, all remaining shares

reserved for issuance under the 1995 Plan were transferred to the 2012 Plan and no further awards were or will be made under the 1995 Plan.

Under the Company's Employee Stock Purchase Plan ("ESPP") a total of 186 shares of common stock are reserved for issuance. As of September 30, 2017, the Company had not commenced any offering under the ESPP and no plan shares have been issued.

The Company applies the fair value recognition provisions for all stock-based awards granted or modified. In the case of service-based awards, the compensation cost is recorded over the requisite service period of the award on the straight-line method based on the grant-date fair value. The requisite service period for service-based option awards is generally four years. Options granted under the 2012 Plan to employees generally vest over four years and to non-employee directors over one year, and expire after ten years.

Stock Option Valuation

The fair value of each stock option award is estimated on the date of grant using the Black-Scholes option-pricing model. The Company estimates expected volatility based on the historical volatility of publicly traded peer companies. The Company expects to continue to do so until such time as adequate historical data regarding the volatility of the Company's traded stock price following our March 2013 IPO is available. The expected term of the Company's options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The Company expects to continue to utilize this method until such time as we have adequate historical data regarding our employee exercise patterns. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. The expected dividend yield is zero on the fact that the Company has never paid cash dividends and does not expect to pay any cash dividends in the foreseeable future. The relevant data used to determine the value of the stock option awards are as follows, presented on a weighted average basis:

| | Years Ended September 30, | | |
|---|---|---|---|
| | 2017 | 2016 | 2015 |
| Risk-free interest rate | 1.97 % | 1.77 % | 1.85 % |
| Expected term (in years) | 6.05 | 6.10 | 6.03 |
| Expected volatility | 60 % | 70 % | 74 % |
| Expected dividends | 0 % | 0 % | 0 % |

The Company recognizes stock-based compensation expense for only the portion of awards that are expected to vest. In developing a forfeiture rate estimate, the Company has considered its historical experience to estimate pre-vesting forfeitures for service-based awards. The impact of a forfeiture rate adjustment will be recognized in full in the period of adjustment, and if the actual forfeiture rate is materially different from the Company's estimate, the Company may be required to record adjustments to its stock-based compensation expense in future periods. Beginning in fiscal 2018, the Company will no longer utilize a forfeiture rate estimate in calculating stock-based compensation expense and will recognize forfeitures as they occur.

As required by the 1995 Plan and the 2012 Plan, the exercise price for awards granted is not to be less than the fair value of common shares as estimated by the Company as of the date of grant.

The following table summarizes stock option activity, including aggregate intrinsic value for the year ended September 30, 2017:

| | Shares Issuable Under Options (in thousands) | Weighted Average Exercise Price | Weighted Average Remaining Contractual Term in years | Aggregate Intrinsic Value (in thousands) |
|---|---|---|---|---|
| Outstanding as of September 30, 2016 | 1,895 | $ 28.75 | 7.6 | $ 7,369 |
| Granted | 613 | 30.93 | | |
| Exercised | (72) | 14.95 | | |
| Forfeited | (138) | 18.73 | | |
| Outstanding as of September 30, 2017 | 2,298 | $ 30.36 | 7.4 | $ 37,821 |
| Options vested and expected to vest as of September 30, 2017 | 2,283 | $ 30.35 | 7.4 | $ 37,596 |
| Options exercisable as of September 30, 2017 | 1,350 | $ 28.52 | 6.7 | $ 24,705 |

The aggregate intrinsic value of options is calculated as the difference between the exercise price of the options and the fair value of the Company's common stock. The following tables summarize additional exercise and grant date information:

| | Years Ended September 30, | | |
|---|---|---|---|
| | 2017 | 2016 | 2015 |
| | (in thousands) | | |
| Aggregate intrinsic value of stock options exercised | $1,503 | $7,705 | $4,827 |
| Proceeds from stock options exercised | $1,079 | $1,026 | $726 |

| | Years Ended September 30, | | |
|---|---|---|---|
| | 2017 | 2016 | 2015 |
| Weighted average grant date fair value of options granted (per share) | $17.52 | $19.12 | $27.77 |

Performance-Based Options

In March 2013, the Company granted to certain executives 167 options that vest upon the achievement of certain performance-based targets. The aggregate grant date fair value of these options was $2,479. During the years ended September 30, 2017 and 2016, certain performance-based targets were achieved and the Company recorded stock-based compensation expense of $413 and $620, respectively, related to achievement of those targets. No stock-based compensation expense related to these options was recognized during the year ended September 30, 2015, as none of the performance-based targets was deemed to have become probable of being achieved in that period. The performance period for these options ended during the year ended September 30, 2017.

Market and Performance-Based Stock Unit Awards

The Company awards both performance share units, or PSUs, and relative total stockholder return units, or rTSRUs, to its executive officers. The number of units represents the target number of shares of common stock that may be earned; however, the actual number of shares that may be earned ranges from 0% to 200% of the target number.

The PSUs will vest and result in issuance, or settlement, of common shares for each recipient, based upon the recipient’s continued employment with the Company through the settlement date of the award and the Company’s achievement of specified research and development milestones. The requisite service period of the PSUs is generally 2 years.

The rTSRUs will vest and result in the issuance of common stock based upon the recipient's continuing employment with the Company through the settlement date of the award and the relative ranking of the total stockholder return, or TSR, of the Company's common stock in relation to the TSR of the component companies in the NASDAQ Biotech Index over a two-year period based on a comparison of average closing stock prices in specified periods noted in the award agreement. The number of market-based rTSRUs awarded represents the target number of shares of common stock that may be earned; however, the actual number of shares that may be earned ranges from 0% to 200% of the target number. The Company used a Monte Carlo simulation model to estimate the grant-date fair value of the rTSRUs. Assumptions and estimates utilized in the calculation of the fair value of the rTSRUs include the risk-free interest rate, dividend yield, expected volatility based on the historical volatility of publicly traded peer companies and the remaining performance period of the award. The table below sets forth the weighted average grant date fair value assumptions used to value the rTSRUs:

| | Years Ended September 30, | | |
|---|---|---|---|
| | 2017 | 2016 | 2015 |
| Risk-free interest rate | 1.24% | 0.94% | 0.61% |
| Dividend yield | 0 % | 0 % | 0 % |
| Expected volatility | 66 % | 65 % | 56 % |
| Remaining performance period (years) | 1.99 | 1.93 | 1.86 |

The following table summarizes activity for the year ended September 30, 2017 based on a target achievement of 100%:

| | PSUs | Weighted Average Grant Date Fair Value - PSUs | rTSRUs | Weighted Average Grant Date Fair Value - rTSRUs |
|---|---|---|---|---|