ENANTA PHARMACEUTICALS INC
Form 10-K
December 18, 2013
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UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

Form 10-K

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

For the fiscal year ended September 30, 2013

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 x

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 x

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 x 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 x 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. x

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

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

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

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 28, 2013, based on the last reported sale price of the registrant s common stock of $18.20 per share was $221,157,646. The number of shares of the registrant s Common Stock, $0.01 par value, outstanding as of December 16, 2013, was 17,961,713 shares.

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DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant s Definitive Proxy Statement for its 2014 Annual Meeting of Stockholders scheduled to be held on February 6, 2014, 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, 2013, are incorporated by reference into Part III of this Form 10-K.

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As used in this Form 10-K, Enanta, the Company, we, our, and us refer to Enanta Pharmaceuticals, Inc., except w
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, may, objective, plan, predict, potential, positioned, seek, should, target, will, would, and other 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:

- the continued commitment of our collaborators, AbbVie and Novartis, with respect to the development of product candidates incorporating ABT-450, ABT-493 and EDP-239, respectively;
- the completion, success and timing of preclinical studies and clinical trials conducted by AbbVie, Novartis or us;
- our and our collaborators abilities to obtain and maintain regulatory approval of therapies involving our product candidates;
- the receipt and timing of any milestone payments or royalties from AbbVie, Novartis or any other collaborator;
- our ability to obtain and maintain collaborators for our development programs or to obtain additional funding;
- the success of competing HCV or MRSA drugs that are now or later become available or other developments or projections relating to our competitors and our industry;
- changes in our or our collaborators plans to develop and commercialize our product candidates;
- the rate and degree of market acceptance of any of our product candidates and any combination therapies developed by AbbVie, Novartis or us;

the size and growth of the potential markets for our product candidates and our collaborators and our abilities to serve those markets, including our belief that substantial opportunities exist for improved treatments in HCV and bacterial infections;

our ability to obtain and maintain intellectual property protection for our product candidates and operate our business without infringing on the intellectual property rights of others;

the loss of any of our key scientific or management personnel;

regulatory developments in the United States and foreign countries affecting disease indications for our product candidates or anti-infective drugs generally;

the performance of third-party manufacturers of our product candidates, including our collaborators;

the accuracy of our estimates regarding our expenses, future revenue, capital requirements and needs for additional financing;

our expectations regarding the time during which we will be an emerging growth company under the JOBS Act; and

our financial performance.

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

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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.

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ENANTA PHARMACEUTICALS, INC.

ANNUAL REPORT ON FORM 10-K

For the year ended September 30, 2013

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PART I

ITEM 1. BUSINESS

BUSINESS

Overview

We are a research and development-focused biotechnology company that uses its robust chemistry-driven approach and drug discovery capabilities to create small molecule drugs in the infectious disease field. We are discovering and developing inhibitors designed for use against the hepatitis C virus, referred to as HCV. We believe that a successful approach to a complete cure for HCV in most patients will likely require treatment with a combination of drugs that attack different mechanisms necessary for replication and survival of HCV. Further, as there are many variants of HCV, we are developing inhibitors that may be used in multiple combination therapies, each designed and tested for effectiveness against one or more of those variants. Our development of inhibitors for validated HCV target mechanisms, as well as our collaborations with AbbVie (which name refers to Abbott Laboratories for all periods before January 1, 2013) and Novartis, should allow us to participate in multiple drug combinations as we and our collaborators seek the best combination therapies for HCV in its various forms. We estimate that total worldwide sales of HCV therapies were over $4 billion in 2012, and we expect that sales will continue to grow with the anticipated introduction of new therapies. In addition to our HCV programs, we have used our internal research capabilities to discover a new class of antibiotics, which we are developing for the treatment of multi-drug resistant bacteria, including MRSA. We have utilized our internal chemistry and drug discovery capabilities and our collaborations to generate all of our development-stage programs, and we have active research efforts to broaden our infectious disease drug pipeline.

Our HCV portfolio includes inhibitors of four fundamental, validated HCV targets:

NS3 Protease Inhibitors: ABT-450 and ABT-493. Our lead product candidate, ABT-450, is an inhibitor of NS3 protease, which is a key protein in HCV viral replication. ABT-450 is being developed as part of our AbbVie collaboration that has yielded ABT-450 and our next-generation protease inhibitor, ABT-493. ABT-450 co-administered with ritonavir, which we refer to together as ABT-450/r, is being tested in seven all-oral, interferon-free Phase 3 studies in combination with one of AbbVie s non-nucleoside polymerase and one of its NS5A inhibitors, plus ribavirin, for the treatment of HCV in genotype 1-infected patients. Six of these trials are expected to be part of the initial registration package designed for a total of at least 2,200 patients using a combination of these three directing-acting antivirals, or DAAs, plus ribavirin. Three of these Phase 3 trials are using the three-DAA combination with and without ribavirin.

In November and December 2013 AbbVie released preliminary results from the first two of these clinical trials, SAPPHIRE-I and SAPPHIRE-II, which were the first announced Phase 3 trial results of any company for an all-oral, interferon-free therapy in genotype 1 HCV patients. SAPPHIRE-I was a 631-patient trial of previously untreated, or naïve, adult patients and SAPPHIRE-II was a 394-patient trial of treatment-experienced adult patients who had previously failed pegylated interferon and ribavirin treatment. Both of these trials demonstrated after 12 weeks of treatment that 96% of patients treated with the new regimen had no quantifiable virus in their blood 12 weeks after treatment, also known as SVR_{12}. AbbVie has guided that preliminary results from the remaining four phase 3 trials will be released during 2013 and into early 2014. AbbVie has publicly projected that its development plan would support a target commercial launch of a combination HCV therapy in early 2015. We believe that we, together with

AbbVie, will obtain exclusivity in ABT-450 in the United States and other major market jurisdictions based on pending composition and use patent claims for ABT-450, which we expect will continue at least into 2029, assuming all such patents issue.

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Our Enanta-AbbVie collaboration has also produced ABT-493, a next-generation protease inhibitor for HCV. In 2013 AbbVie initiated combination studies of ABT-493 with ABT-530 (AbbVie s next-generation NS5A inhibitor) in healthy volunteers, and we expect ABT-493 to begin dosing in HCV patients in late 2013.

NS5A Inhibitor: EDP-239. We have had a robust drug discovery effort directed at the NS5A protein, which plays a key role in HCV viral replication. In February 2012, we entered into a collaboration with Novartis for the worldwide development, manufacture and commercialization of NS5A inhibitors, including our lead NS5A product candidate, EDP-239. In November 2012, Novartis initiated a Phase 1 clinical trial for EDP-239, and in 2013 initiated a trial testing EDP-239 in HCV patients. We believe that we, together with Novartis, have exclusivity to EDP-239 in the United States based on issued patent composition and use claims, which we expect will continue at least into 2030.

Cyclophilin Inhibitors. Our research activities have also focused on a more recently validated target against HCV, cyclophilin, which is a protein in the human body that has been shown to be involved in HCV replication. By focusing on this human, or host, target rather than a viral target, we have selected a mechanism shown to be less susceptible to the HCV resistance that can occur due to viral mutation in response to therapy. Using our extensive chemistry expertise with small molecules, we have identified a series of active cyclophilin binders designed to disrupt the interactions of HCV with cyclophilin. We expect to advance a lead cyclophilin inhibitor into preclinical drug metabolism, pharmacokinetic, and safety studies in 2014.

Nucleotide Polymerase Inhibitor. We have a small-molecule drug discovery effort underway for inhibitors of nucleotide polymerase in a clinically validated mechanism that is less susceptible to HCV resistance. Our researchers have identified a promising lead series with significant antiviral potency *in vitro*. We expect to select a candidate to advance into preclinical studies on our own in 2014.

In addition to our HCV programs, we have used our internal research capabilities to discover a new class of antibiotics, called Bicyclolides, which we are developing for the treatment of multi-drug resistant bacteria, including MRSA. These new antibiotic candidates include intravenous and oral forms for treatment of hospital and community infections arising from MRSA. EDP-788 is our lead candidate for the treatment of MRSA. Our preclinical and early clinical development of EDP-788 is funded under a contract with NIAID. We are conducting IND-enabling studies and plan to initiate a clinical trial in the first quarter of 2014.

In connection with our collaboration efforts, AbbVie has funded all research and development of our protease inhibitors since we entered into the collaboration in November 2006, and is responsible for obtaining regulatory approvals and commercializing ABT-450 and any next-generation products worldwide. In 2006, we received $57.2 million from AbbVie in connection with our entry into the collaboration agreement and AbbVie s simultaneous purchase of preferred stock from us. We also received a $40.0 million milestone payment in December 2010 following AbbVie s successful completion of a Phase 2a clinical trial and a $15.0 million milestone payment in December 2012 based on AbbVie s initiation of dosing in a Phase 3 clinical trial that includes ABT-450. Assuming AbbVie s successful development of the first protease inhibitor product, we will also be eligible to receive $195 million of additional pre-commercialization milestone payments, as well as $80 million of additional milestone payments for each follow-on protease inhibitor product, if any, developed by AbbVie and tiered royalties per product ranging from the low double digits up to twenty percent, or up to the high teens on a blended basis, on AbbVie s net sales, if any, allocable to our collaboration s protease inhibitors.

Under our collaboration with Novartis, we received a $34.4 million upfront payment in March 2012, and an $11.0 million milestone payment in January 2013 based on Novartis initiation of dosing in a Phase 1 clinical trial that includes EDP-239. In addition, we are eligible to receive up to $395 million of additional milestone payments for the first NS5A inhibitor product for which the specified milestones are achieved. We are also eligible to receive tiered royalties ranging on a blended basis from the low double digits up to the high teens on

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Novartis net sales, if any, allocable to each of our collaboration s NS5A inhibitors. Novartis will fund all costs associated with further development, regulatory approvals and commercialization of any NS5A inhibitor product candidates in this collaboration and we retain co-detail rights in the United States.

Our Strategy

Our primary objective is to become a leader in the infectious disease field, with a focus on HCV and multi-drug resistant bacterial infections. Our strategy includes the following key elements:

Develop compounds against four fundamental, validated HCV targets to give us multiple opportunities to participate in one or more of the potentially successful combination therapies for HCV. We believe that a successful approach to a complete cure for HCV in most patients will likely require treatment with a combination of drugs that attack different mechanisms necessary for replication and survival of HCV. As there are many variants of HCV, we are developing inhibitors that may be used in multiple combination therapies, each to be designed and tested for effectiveness against one or more of those variants, or genotypes. Our HCV portfolio includes inhibitors of four fundamental, validated HCV targets that we believe will provide the necessary therapeutic compounds for combination therapy, with the goal of placing one or more of our compounds into the combination or combinations that will ultimately be approved and accepted as preferred treatments for one or more genotypes of HCV.

Collaborate with large pharmaceutical partners to accelerate the development and commercialization of our lead HCV compounds. Our strategic partnerships allow us to join forces with collaborators with substantially greater resources and late-stage development and commercialization expertise as we seek the right combination for a cure for one or more genotypes of HCV. After testing ABT-450 in various combinations in clinical trials, AbbVie is combining ABT-450 with one of AbbVie s own non-nucleoside inhibitors and one of its NS5A inhibitors. In addition, AbbVie is conducting combination studies of our collaboration s next-generation protease inhibitor, ABT-493, with AbbVie s next-generation NS5A inhibitor, ABT-530. At the same time, our own lead NS5A product candidate, EDP-239, can become part of combination therapies developed by Novartis. The result is that our product candidates are being tested in multiple regimens using different combinations of mechanisms, increasing our chances of participating in more than one commercially successful combination therapy for HCV in its various forms.

Develop independently our own next generation HCV compounds and combination therapies with lower susceptibility to viral resistance. We are independently developing a lead cyclophilin inhibitor and will be selecting a nucleotide polymerase inhibitor for development, both of which we are seeking to design with lower susceptibility to the viral resistance that is being generated by first-generation (currently marketed) and second-generation HCV products. We are considering potential development of a combination of these two types of inhibitors.

Continue to leverage and fortify our intellectual property portfolio. We believe we have a strong intellectual property position relating to the development and commercialization of HCV-targeted therapeutics and antibiotics for the treatment of resistant pathogens.

Invest in research and early-stage development of product candidates. We intend to continue to invest significant resources in research programs and early-stage development of product candidates in an effort to identify and advance additional compounds that have the potential to address significant unmet medical needs in the infectious disease field. We will continue to seek further innovations for the treatment of HCV and other viral infections, as well as antibiotics for the treatment of resistant bacteria, such as MRSA.

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Our Research and Development Pipeline

The following table summarizes our product development pipeline in HCV antivirals as well as MRSA antibiotics:

As detailed above, our only product candidate that has advanced beyond Phase 2 clinical trials is ABT-450. Phase 3 trials of ABT-450 in combination therapy started in October 2012, and the full registration program of six trials involving approximately 2,200 patients has been fully enrolled and the preliminary results of the first two trials have been reported. We estimate that it may be early 2015 before a New Drug Application, or NDA, for one of our collaborator s combination therapies that includes ABT-450 could be approved by the FDA.

Our HCV Programs

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, organ 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

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undiagnosed without seeking treatment. For that reason, new guidelines proposed by the United States Centers for Disease Control and Prevention, or CDC, and currently under review would recommend screening for all Americans born between the years 1945 and 1965 so that HCV-infected individuals would be aware of their condition and could consider treatment options.

An estimated 150 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. As of July 2008, the CDC estimated that approximately 3.2 million people in the United States are chronically infected with HCV, with an estimated 17,000 new infections each year. We believe that the chronically infected population remains largely untreated, even with the introduction of new regimens containing a protease inhibitor in 2011. Currently approved therapies for HCV, which include interferon, ribavirin and the new protease inhibitors, had aggregate worldwide sales of over $4 billion in 2012. We believe that annual worldwide sales of these therapies and future approved therapies could increase sales in this market to $10 to $20 billion within the next ten years.

HCV is a small, single-stranded RNA virus. The specific genetic makeup, or genotype, of the virus can vary and at least six genotypes have been characterized in HCV-infected patients, with over 50 sub-types identified. Genotypes are designated with numbers (genotypes 1-6) and subtypes with letters (*e.g.* genotype 1a). HCV genotypes 1, 2, 3, and 4 are found worldwide, but their prevalence varies among geographic regions. Genotype 1, including its subtypes 1a and 1b, is the most common genotype globally, accounting for approximately 74% of all HCV infections. It is estimated that patients with genotype 2 or 3 represent approximately 12% of the worldwide chronically infected HCV population, with approximately 6% comprised of genotypes 4 through 6 and the remaining 8% of patients in other undesignated categories. The specific genotype and subtype of HCV in a patient appears to play a significant role in the degree of efficacy of standard of care therapy. Genotype 1 is the most difficult genotype to treat and the most common in North America and Europe.

Since the discovery of the virus in the late 1980s, considerable progress has been made in the treatment of HCV-infected individuals. However, a protective vaccine is not yet available and current treatments remain ineffective in a large percentage of the HCV-treated population. The standard of care for HCV traditionally has consisted of weekly injections of interferon, a protein that interferes with viral replication, with twice-daily dosing of ribavirin for 24 to 48 weeks. Ribavirin is a broad-spectrum drug that prevents the replication of a number of DNA and RNA-based viruses. This regimen has been moderately effective in many patients, resulting in a cure in only about 50% of genotype 1-infected patients. 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.

Recently introduced treatment regimens contain direct acting antivirals, or DAAs, with initial approval coming for protease inhibitors. The first two protease inhibitors approved, telaprevir (Incivek , Vertex Pharmaceuticals) and boceprevir (Victrelis , Merck), have shown cure rates of approximately 70% in genotype 1-infected patients. Telaprevir and boceprevir were approved for use in combination with interferon and ribavirin in patients infected with genotype 1 virus in 2011, and combination therapy incorporating a protease inhibitor has emerged as a new standard of care for HCV patients. In November 2013 the FDA approved a new protease inhibitor from Janssen Therapeutics, simeprevir (Olysio), for the treatment of genotype 1 chronic hepatitis C in adult patients with compensated liver disease. Simeprevir has shown cure rates of up to 80% in genotype 1-infected patients. However, the once-daily treatment must still be combined with pegylated interferon and ribavirin. In December 2013, the FDA approved sofosbuvir (Sovaldi), a nucleotide analogue inhibitor of the HCV NS5B polymerase enzyme from Gilead. Sofosbuvir is a once-a-day treatment given in combination with ribavirin for the treatment of chronic hepatitis C in adult patients with genotype 2 or 3 infection. Sofosbuvir may also be given in combination with pegylated interferon and ribavirin

for the treatment of chronic hepatitis C in treatment-naïve adult patients with genotype 1 (the predominant genotype in the major world markets) and genotype 4 infection.

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These new treatment regimens have several limitations that highlight the need for improved HCV therapies, particularly for genotype 1 HCV, including:

Sub-Optimal Cure Rates. Current approved regimens containing a protease inhibitor lead to a cure rate of approximately 70%- 80% in previously untreated, genotype 1 patients. Clinical trial participants taking Sovaldi-based therapy (Sofosbuvir) achieved SVR_{12} rates of 50-90 percent. The cure rate is on average lower among patients who did not fully respond to prior treatments with interferon and ribavirin therapy. There is a need for a cure for the patients who have failed therapy, many of whom may have developed HCV variants that are resistant to the specific protease inhibitors used in the protease-based therapies.

Dependence on Interferon. Current HCV therapy for genotype 1 (the predominant genotype in the major world markets) still includes injected interferon as part of the treatment regimens, which produces adverse events in over 50% of patients. Interferon often causes flu-like symptoms, fatigue, headaches and nausea during treatment, which affects patients quality of life and can lead to abandonment of therapy over the standard 24 to 48 weeks of therapy. We believe this has led to many patients waiting for the availability of new, interferon-free therapies before undergoing treatment.

Side Effects Associated With Currently Approved Protease Inhibitors. Other serious side effects of the new regimens containing a protease inhibitor include rash, anemia, itching (known as pruritus), and gastrointestinal effects. Rash is observed in more than a quarter of the patients taking simeprevir and in approximately half of patients treated with telaprevir and telaprevir-containing therapy and requires strict adherence to a rash management plan in close collaboration with an experienced dermatologist. Boceprevir administration can worsen the anemia that is observed with interferon and ribavirin therapy alone.

Inconvenient Treatment Regimen. Though simeprevir and Sovaldi are once-a-day medicines, the pharmaceutical properties of telaprevir and boceprevir require that they be dosed approximately every 8 hours, thus resulting in a complex treatment regimen that also includes weekly injections of interferon. All currently approved protease inhibitors require co-administration with interferon for treatment of genotype 1 HCV. We believe that demanding dosing requirements such as these can often lead to poor compliance with the treatment regimen and can accelerate the development of HCV resistance.

While providing a step forward, we believe these new treatment regimens may only provide sub-optimal cure rates, will still require treatment with interferon for many patients, including genotype 1 patients, may carry other undesirable side effect profiles, may require inconvenient dosing regimens, may be ineffective in many patient populations and may often result in HCV resistance. Accordingly, we believe there remains a significant unmet medical need in the HCV field, with an urgent need for improved treatments for HCV.

Scientific Background

Many of the new approaches under development 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. Current DAA development efforts typically focus on the NS3 protease, the NS5A protein, and the NS5B polymerase. In addition to

targets in HCV itself, there are human host proteins that are critical to viral replication. Inhibitors that interfere with host targets resulting in antiviral activity are referred to as host-targeted antivirals, or HTAs. One of the most promising HTA approaches to HCV treatment focuses on the human host protein known as cyclophilin A, or cyclophilin.

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Key Proteins in the HCV Replication Complex

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. NS3 protease inhibition is the mechanism of action for the two most recently approved HCV drugs, telaprevir and boceprevir, both of which are DAAs.

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 drug development.

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 in development 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 so that any given inhibitor is usually only active against certain HCV genotypes.

Cyclophilin. Viral function requires an interaction of the viral protein NS5A with the human host protein known as cyclophilin. Inhibitors that interfere with this NS5A-cyclophilin interaction would essentially provide a treatment that protects the human host cells from infection by the virus. Several studies using the immunosuppressive drug cyclosporine A, a known cyclophilin inhibitor, support the clinical validation of cyclophilin as an HTA for treatment of HCV infection. However, the immunosuppressive activity of cyclosporine A and associated side effects limit its clinical use and thus efforts are now focused on new agents devoid of immunosuppressive activity. Alisporivir, a nonimmunosuppressive cyclosporine A derivative under development by Novartis, has demonstrated effectiveness against many HCV genotypes, a high barrier to HCV resistance and no cross-resistance with several DAAs.

The ultimate goal in HCV treatment is complete cure with total eradication of the virus, measured by SVR. We believe that combination therapy will improve overall cure rates and will reduce the probability of resistance arising to any single antiviral agent. In particular, a combination of target mechanisms that includes those with a

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high barrier to resistance (cyclophilin, polymerase) may prove to be the most effective combination against multiple genotypes of HCV. Unlike treatment for certain viruses, such as HIV, complete clearance of the HCV virus is possible with effective therapy. This exciting prospect suggests that the ultimate goal of a complete cure with total eradication of the virus is within reach.

Our Approach to the Treatment of HCV

We are pursuing four fundamental, validated targets within the HCV field that represent a broad approach to the disease and specifically address the urgent unmet medical needs in current HCV therapies. Our approach incorporates the main targets for future HCV therapy. Our DAA approach directly targets three critical proteins of HCV, incorporating inhibitors of NS3 protease, NS5A protein, and NS5B polymerase. Inhibitors in our HTA approach protect the human host protein cyclophilin from being co-opted into the viral replication machinery of HCV. We believe a combination of inhibitors from our programs may provide a truly effective all-oral interferon-free or interferon/ribavirin-free therapeutic approach to HCV, with complete eradication of virus, low resistance rates, convenient dosing and acceptable side effect profiles.

ABT-450, a Protease Inhibitor for HCV Infection

Our protease inhibitor, ABT-450, discovered through our collaboration with AbbVie and currently in Phase 3 clinical trials, is a potent DAA that has demonstrated *in vitro* potency against known resistant HCV mutants. In Phase 1 studies, ABT-450 co-administered with ritonavir, a commonly used boosting agent to increase the blood concentrations of many protease inhibitors, was shown to be safe and well tolerated. Co-administration of ABT-450 with ritonavir, which we refer to together as ABT-450/r, has enabled once-daily dosing of ABT-450. Phase 2 studies have demonstrated the efficacy of ABT-450/r in patients with chronic HCV, and other interferon-free Phase 2 studies of ABT-450-containing regimens continue. In addition, AbbVie is conducting Phase 3 trials of ABT-450/r in combination with AbbVie s non-nucleoside polymerase and NS5A inhibitors, with and without ribavirin. While AbbVie and other companies are developing interferon-free and interferon/ribavirin-free HCV therapies in clinical trials, the efficacy of this approach has not yet been proven conclusively, nor has it resulted yet in any product approved by the FDA.

We believe that a treatment regimen containing ABT-450/r may have significant advantages over currently approved genotype 1 HCV treatment regimens containing protease inhibitors because of the following key attributes:

Improved Antiviral Activity. Compared to the current leading protease inhibitor, telaprevir, ABT-450 has demonstrated superior antiviral activity against HCV in patients, including genotype1.

No Interferon. Current genotype 1 HCV therapy still includes injected interferon. Interferon is often associated with flu-like symptoms, fatigue, headaches and nausea during treatment. ABT-450/r is being developed for use in one or more interferon-free regimens.

Tolerability. As noted above, serious side effects of current regimens for genotype 1 HCV that contain protease inhibitors include rash, anemia, pruritus, or itchy skin, and gastrointestinal effects. In contrast, most side effects in clinical trials including ABT-450/r to date were mild to moderate.

Shorter Treatment Regimen. ABT-450/r is being tested in various treatment combinations that are only 12 weeks in duration, as compared to the 24 to 48 weeks of treatment required with some of the current interferon-containing regimens.

More Convenient Treatment Regimen. ABT-450/r is being developed for oral, once-daily dosing. All of the combinations including ABT-450/r that AbbVie is testing include only orally administered drugs dosed either once or twice daily without the use of interferon. By comparison, current treatment regimens containing a protease inhibitor require weekly interferon injections.

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Under the AbbVie collaboration, we have granted AbbVie an exclusive worldwide royalty-bearing license, including a right to grant sublicenses, to our intellectual property position for NS3 protease inhibitors. We also granted AbbVie access to our drug discovery capabilities in the NS3 protease inhibitor field. AbbVie is responsible for and funds all costs associated with the development, manufacturing and commercialization of ABT-450 and other compounds under this collaboration agreement. We will be eligible to receive milestone payments and royalties with respect to these compounds if such products are successfully commercialized by AbbVie.

In November 2013, AbbVie announced preliminary results of its SAPPHIRE-I trial, and in December 2013 it announced preliminary results of its SAPPHIRE-II trial. These are the first of six Phase 3 trials for an ABT-450-containing regimen for treating genotype 1 infected patients. Results of the other four trials are expected to continue to be reported through the end of 2013 and into the first calendar quarter of 2014. These six trials are expected to be part of the initial registration package designed for a total of 2,200 patients using a combination of three DAAs, including ABT-450/r, one of AbbVie s non-nucleoside polymerase inhibitors and one of AbbVie s NS5A inhibitors, plus ribavirin. Three of these Phase 3 trials are using the same three-DAA (3D) combination, with and without ribavirin. In addition, in November 2013 AbbVie announced topline results of a Phase 2 clinical study, known as PEARL-I, in genotype 1a- and 1b-infected patients using a two-DAA (2D) dosing regimen containing a combination of ABT-450/r and ABT-267 dosed once daily. In PEARL-I, 95% of treatment-naïve patients with genotype 1b and 90% of treatment-experienced patients who had been null responders had sustained viral response 12 weeks after treatment. AbbVie is also conducting a Phase 2 study of the 2D regimen in Japan in genotype 1b and genotype 2-infected patients.

AbbVie has announced that it expects regulatory filings in the second calendar quarter of 2014 for an ABT-450-containing treatment regimen for genotype 1 HCV patients. In addition, in May 2013 AbbVie announced that its investigational direct-acting antiviral (DAA) combination with and without ribavirin for the treatment of genotype 1 hepatitis C virus (HCV) infection was designated as a breakthrough therapy by the U.S. Food and Drug Administration, or FDA. According to the FDA, breakthrough therapy designation is intended to expedite the development and review of drugs for serious or life-threatening conditions. For a breakthrough therapy, once a New Drug Application, or NDA, is accepted by the FDA, the agency in its discretion can grant it priority review status, which would set a date for regulatory action within six months of the NDA filing.

AbbVie has also announced that its development plan would support a target commercial launch of such a combination therapy in early 2015. AbbVie projects that there will be a potential worldwide market opportunity of $12-14 billion for HCV therapies by 2016 based upon an assumed treatment rate of 300,000 to 350,000 patients per year across all genotypes of HCV in the U.S., Japan, Canada and four major European countries, or the G7 countries. In addition, AbbVie had previously projected that peak sales for the combination therapies AbbVie is developing could reach $2 billion or more worldwide. AbbVie s projections are subject to risks and uncertainties. The actual market opportunity may vary and there is no guarantee what portion, if any, of the resulting market opportunity will be captured by an ABT-450-containing regimen, assuming that AbbVie obtains approval of such a regimen. One or more Phase 3 trials containing ABT-450/r could take longer than anticipated to complete or could have unexpected results, the FDA could find that the results of these trials are not adequate to support marketing approval, the FDA could require additional clinical trials as a condition for approval, or other HCV products could come to market sooner or achieve greater market acceptance than any for which AbbVie ultimately obtains approval.

Clinical Development

Phase 1. An Investigational New Drug Application, or IND, was filed for ABT-450 in December 2008 and clinical testing began in early 2009. ABT-450 was evaluated in a Phase 1a single ascending dose trial in doses ranging from 25 mg to 900 mg, with and without ritonavir. Data from this trial showed that ritonavir co-administration significantly

boosted the ABT-450 plasma concentrations. ABT-450 is being developed with low

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dose ritonavir to enhance exposure and allow once-daily dosing of ABT-450. A 14-day multiple dose study showed that ABT-450/r was well tolerated and demonstrated pharmacokinetics consistent with once-daily dosing.

Phase 2. In the first quarter of 2010, we and AbbVie announced the advancement of ABT-450/r into Phase 2 clinical trials. The objective of the initial Phase 2 study was to assess the safety, tolerability, pharmacokinetics and antiviral activity of multiple dose strengths of ABT-450/r in treatment-naïve adults (*i.e.*, those who have not previously received treatment for HCV) infected with HCV genotype 1. These initial studies with ABT-450/r paved the way for additional Phase 2a and 2b combination studies that use interferon-free regimens. The Pilot and Co-Pilot trials, which were initiated in late 2010 and early 2011, respectively, included combination trials of ABT-450/r with one or the other of two of AbbVie s non-nucleoside polymerase inhibitors. The Aviator study, which was initiated in 2011, was a trial of ABT-450/r and various combinations of two or three of the following: one of AbbVie s non-nucleoside polymerase inhibitors, one of its NS5A inhibitors and ribavirin.

All of the Phase 2 combination regimens tested by AbbVie were interferon-free, with a significantly shorter treatment duration (12 weeks) and a simpler treatment paradigm compared to the currently approved protease inhibitor regimens for genotype 1, all of which include weekly injections of interferon, and some of which require daily oral doses of ribavirin for 24 to 48 weeks.

AbbVie Aviator Study. The Aviator study consisted of HCV genotype 1 non-cirrhotic patients enrolled in an open-label trial of a 12-week interferon-free regimen consisting of three DAAs, with and without ribavirin. One combination in the study consisted of ABT-450/r once daily, plus ABT-267 (AbbVie s NS5A inhibitor), plus ABT-333 (AbbVie s non-nucleoside polymerase inhibitor) twice daily, plus weight-based ribavirin twice daily, which is the same combination now being tested in Phase 3 trials. As reported in an initial data abstract from the ongoing study, ABT-450/r was evaluated in treatment-naïve patients and treatment-experienced patients who had little or no decrease in HCV during prior treatment with the standard of care, known as null responders. Results from this ongoing trial demonstrated SVR_{12} in 99% of treatment-naïve HCV genotype 1-infected patients and in 93% of previous null responders (as compared with 47% SVR_{12} seen in the Co-Pilot study). The most common AEs were fatigue (28% and 27%) and headache (28% and 31%) for treatment-naïve and previous null responders, respectively. Initial results from the Aviator Phase 2 studies provided compelling support for the potential development of an interferon-free combination containing ABT-450 for treatment of HCV.

Other Studies: AbbVie has announced that it is conducting additional exploratory clinical studies to determine if a combination of ABT-450/r and ABT-267 dosed once daily can provide high cure rates in specific HCV populations, including a Phase 2 study, known as PEARL-I, in genotype 4-, 1a- and 1b-infected patients and a study in Japan in genotype 1b- and 2-infected patients. Preliminary results announced in November 2013 from the PEARL-I study demonstrated SVR_{12} rates of 95% (40/42) in HCV genotype1b, treatment-naïve patients and 90% (36/40) among prior null responders.

Phase 3. In November 2013, AbbVie announced preliminary results of its SAPPHIRE-I trial, the first of six Phase 3 trials for an all-oral, interferon-free, ABT-450-containing regimen for treating genotype 1-infected patients. Results of SAPPHIRE-I, SAPPHIRE-II and of the other four Phase 3 trials are expected to be part of the initial registration package designed for a total of at least 2,200 patients using a combination of the same three DAAs, including ABT-450/r, one of AbbVie s non-nucleoside polymerase inhibitors and one of AbbVie s NS5A inhibitors, plus ribavirin. The trials are designed to produce placebo-controlled results for treatment-naïve patients (SAPPHIRE-I) and treatment-experienced patients (SAPPHIRE-II), as well as separate results for genotype 1a (PEARL-IV) and 1b (PEARL-II and PEARL-III) patients, and patients with compensated cirrhosis (TURQUOISE-II). Three of these Phase 3 trials (PEARL-II, PEARL-III and PEARL-IV) are using the three-DAA combination, with and without ribavirin.

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Study Name	Genotype/(Number of) Patients	Treatment Regimen	Treatment Duration	SVR_{12}[d]
SAPPHIRE-I	GT1, treatment-naïve (631)	ABT-450/r[b] +ABT-267[c]	12 weeks	GT1a = 95% GT1b = 98%
		ABT-333 Ribavirin Placebo	12 weeks, then active treatment for 12 weeks	
SAPPHIRE-II	GT1, treatment-experienced (394)	ABT-450/r +ABT-267 ABT-333 Ribavirin	12 weeks	GT1a = 96% GT1b = 97%
		Placebo	12 weeks, then active treatment for 12 weeks	
PEARL-II	GT1b, treatment-experienced (210 [a])	ABT-450/r +ABT-267 ABT-333 Ribavirin	12 weeks	
		ABT-450/r +ABT-267 ABT-333	12 weeks	
PEARL-III	GT1b, treatment-naïve (400 [a])	ABT-450/r +ABT-267 ABT-333 Ribavirin	12 weeks	
		ABT-450/r +ABT-267 ABT-333	12 weeks	

		Placebo	
PEARL-IV	GT1a, treatment-naïve (300 [a])	ABT-450/r +ABT-267	12 weeks
		ABT-333	
		Ribavirin	
		ABT-450/r +ABT-267	12 weeks
		ABT-333	
		Placebo	
TURQUOISE-II	GT1, treatment-naïve and treatment-experienced (with compensated cirrhosis) (380 [a])	ABT-450/r +ABT-267	12 weeks
		ABT-333	
		Ribavirin	
		ABT-450/r +ABT-267	24 weeks
		ABT-333	
		Ribavirin	

[a] projected study population

[b] ABT-450/ritonavir

[c] ABT-267 is co-formulated with ABT-450/r, administered as two pills once daily

[d] SVR_{12} (Sustained Virological Response at 12 weeks after treatment completion): Continued HCV virus RNA below lower limit of measurable quantitation (LLOQ) 12 weeks after end of treatment (EOT)

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AbbVie SAPPHIRE-I Trial. The SAPPHIRE-I Phase 3 trial used a three-DAA, or 3D, regimen, consisting of boosted protease inhibitor ABT-450/ritonavir, NS5A inhibitor ABT-267, and non-nucleoside polymerase inhibitor ABT-333. The SAPPHIRE-I study used this 3D regimen plus ribavirin. Results from the 631-patient SAPPHIRE-I trial demonstrated a sustained virologic response at 12 weeks post-treatment (SVR_{12}) of 96% in treatment-naïve adult patients chronically infected with genotype 1 HCV. The majority of patients were genotype 1a, which is considered the more difficult-to-treat subtype. The SVR_{12} rates of genotype 1a and genotype 1b were 95% and 98%, respectively. These results were based on an intent-to-treat analysis and were achieved after 12 weeks of treatment. The rate of virologic relapse or breakthrough was low, occurring in 1.7 percent of patients receiving the 3D regimen. The treatment regimen was well tolerated, with an equal percentage of patients in the active and placebo arms (0.6 percent) discontinuing treatment due to adverse events.

AbbVie SAPPHIRE-II Trial. The SAPPHIRE-II Phase 3 trial used the same 3D regimen as in SAPPHIRE-I, including ribavirin. Results from the 394-patient SAPPHIRE-II trial demonstrated a sustained virologic response at 12 weeks post-treatment (SVR_{12}) in 96% of the treatment-experienced adult patients in the trial, all of whom were chronically infected with genotype 1 HCV and had previously failed pegylated interferon and ribavirin treatment. The majority of patients were genotype 1a, which is considered the more difficult-to-treat subtype and approximately 49% of the patients were prior null responders, namely patients defined as not achieving a significant reduction in the HCV virus during their prior treatment. The SVR_{12} rates of genotype 1a and genotype 1b were 96% and 97%, respectively. These results were based on an intent-to-treat analysis and were achieved after 12 weeks of treatment. The rate of virologic relapse or breakthrough was low, occurring in 2 percent of patients receiving the 3D regimen plus ribavirin. The treatment regimen was well tolerated, with 1 percent of patients in the active arm and no patients in the placebo arm discontinuing treatment due to adverse events.

Additional information about AbbVie s Phase 3 studies can be found on www.clinicaltrials.gov.

Next-Generation HCV Protease Inhibitor

AbbVie is also developing a next-generation protease inhibitor, ABT-493, discovered within the Enanta-AbbVie collaboration. AbbVie has announced that this protease inhibitor has demonstrated activity in preclinical *in vitro* testing against a broad range of HCV genotypes, including variants that have shown strong resistance to first generation protease inhibitors. AbbVie has also announced that this next-generation protease inhibitor was designed to enable once-daily dosing without ritonavir and to be co-formulated with AbbVie s next-generation NS5A inhibitor, ABT-530. AbbVie initiated a Phase 1 clinical trial of this next-generation protease inhibitor in November 2012, and is expected to begin dosing ABT-493 in HCV patients late in 2013.

EDP-239, an NS5A Inhibitor for HCV Infection

EDP-239, another DAA, is our lead NS5A inhibitor. The EDP-239 compound has demonstrated potent activity against major genotypes in the replicon assay, which is a common *in vitro* test for determining potency of an active compound in reducing HCV replication.

In addition, EDP-239 has additive to synergistic antiviral activity when used in combination with other anti-HCV therapeutics (DAA and HTA) in reducing HCV replication. Preclinical studies support excellent permeability and absorption potentials in humans. The compound has preferential penetration to the liver, which is the target site of infection, across all preclinical models tested. Human pharmacokinetic and pharmacodynamic modeling suggests a low, once-daily clinical dose for future testing. Novartis has completed Phase 1 trials with EDP-239 and has initiated proof-of-concept studies in HCV patients.

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We discovered EDP-239 internally at Enanta and entered into a collaboration with Novartis in February 2012, granting Novartis exclusive, worldwide rights to develop, manufacture and commercialize EDP-239. Novartis will fund all costs associated with the development, manufacture and commercialization of EDP-239 and related NS5A products. In addition, through August 2013, Novartis was responsible for funding our drug discovery efforts on additional selected compounds targeting NS5A. Under the agreement, we received an upfront payment of $34.4 million, and in January 2013 we received an $11.0 million milestone payment based on Novartis initiation of dosing in a Phase 1 clinical trial that includes EDP-239. We are eligible to receive up to $395 million of additional payments if Novartis achieves specified clinical, regulatory, and commercial milestones. We are also eligible to receive tiered royalties ranging on a blended basis from the low double digits up to the high teens on worldwide net sales of products, and retain co-detail rights, which would allow us to staff up to a specified percentage of the sales force for a designated product in the United States.

In addition to EDP-239, we have developed other NS5A inhibitors in the discovery stage under our Novartis collaboration, which may become candidates to be follow-on NS5A inhibitors.

Cyclophilin (Cyp) Inhibitors for HCV Infection

In anticipation of resistance arising to DAA HCV therapy that targets viral proteins, we have been developing an alternative HTA approach that targets the human host protein, cyclophilin, which is essential for replication of HCV.

Interruption of Viral Replication of HCV RNA by Cyclophilin Inhibitor

Abbreviation: CypA refers to cyclophilin A

We have demonstrated in replicon assays that multiple lead cyclophilin targeting inhibitors are potent inhibitors of HCV replication and are more potent than the clinical stage cyclophilin inhibitor alisporivir. Typically, cyclophilin inhibitors are based on the structures of cyclosporine A, which is known to be immunosuppressant with associated side effects that limit its clinical use. Based on our understanding of the structural elements of cyclosporine A that contribute to immunosuppressive activity, we have designed those elements out of our cyclophilin inhibitors and have confirmed a lack of *in vitro* immunosuppressive activity. We are advancing our lead candidates in preclinical studies and are continuing to generate and characterize a number of additional cyclophilin inhibitors in the discovery phase. We plan to select the most promising candidate and conduct IND-enabling studies in 2014.

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Nucleotide Polymerase Inhibitor Program for HCV Infection

We also have a program to develop inhibitors to HCV polymerase, which is another DAA mechanism considered to have a high barrier to resistance. Our researchers have identified a promising nucleotide lead series with significant antiviral potency *in vitro*. One of our lead compounds has demonstrated better *in vitro* potency than a reference clinical stage nucleotide inhibitor, GS-7977 (Sofosbuvir), under development by Gilead Sciences. We have an ongoing discovery effort in this inhibitor class and are considering a number of compounds for further development. We plan to select a candidate in 2014 that is suitable for advancement into preclinical studies.

Our MRSA Antibacterial Program

Background of MRSA Antibiotics

The past three decades have witnessed a dramatic change in the epidemiology of resistant Gram-positive bacterial infections all over the world. Families of common Gram-positive organisms include *Streptococcus*, or *Strep*, *Staphylococcus*, or *Staph*, and *Enterococcus*. Among the conditions associated with these pathogens are skin infections, bacteremia and endocarditis. One of these pathogens, known as methicillin-resistant *Staph aureus,* or MRSA, was principally identified when resistance was observed to methicillin, an early antibiotic used for *Staph aureus* and other bacterial infections. Increasingly, strains of MRSA have been identified that are also resistant to many other antibiotics.

The recognition and spread of MRSA, as well as *Enterococci* resistant to the antibiotic vancomycin, referred to as VRE, in the community and in healthcare facilities represents a major healthcare challenge. Widespread reports of emerging bacterial resistance to existing antibiotics emphasize the need for continued research and development of novel antimicrobials to address possible life-threatening infections caused by Gram-positive resistant pathogens. MRSA was responsible for approximately 94,000 reported infections that resulted in over 19,000 deaths in the United States in 2005, compared to approximately 16,000 deaths from AIDS.

In addition to the high potential for large hospital outbreaks, MRSA and Gram-positive resistance are moving out from hospitals into the community. During the past decade, rates of MRSA in the community have increased rapidly. Thus, an urgent need exists for the development of new antibiotics that will be effective against Gram-positive organisms that are resistant to current antibiotics in the macrolide class, such as clarithromycin (BiaxinTM), azithromycin (ZithromaxTM) and telithromycin (KetekTM), as well as VRE and *Enterococci* that are resistant to the oxazolidinone class of antibiotics, such as linezolid (Zyvox). In addition, there exists a significant need for agents that would allow step-down dosing, wherein MRSA patients being treated in a hospital setting with intravenous treatment could be sent home on the same drug to be taken orally.

EDP-788 and Our Bicyclolide Antibiotics

Through our internal chemistry efforts, we have created a new family of macrolide antibiotics called Bicyclolides that overcomes resistance and possesses a significantly improved product profile compared to existing macrolides such as Zithromax and Biaxin™. The main focus of our antibiotic work is on new mechanisms targeting resistant Gram-positive pathogens, including MRSA and other *Staph aureus* bacteria resistant to currently marketed macrolides. Our initial therapeutic focus is on skin infections, namely Acute Bacterial Skin and Skin Structure Infections, or ABSSSI. Examples of ABSSSI are cellulitis/erysipelas, wound infection, major cutaneous abscess and burn infections. Major pathogens involved in skin infections are *Strep pyogenes* and *Staph aureus*.

Our lead Bicyclolide antibiotic product candidate is EDP-788, which we are developing for use as an intravenous drug in the hospital setting and for oral dosing in a home setting. EDP-788 is a prodrug, which means that it is inactive until it is converted in the body into an active compound. EDP-788 is a highly water-soluble molecule which, when administered, is cleanly and rapidly converted into the active compound.

The active compound generated from EDP-788 is EDP-322, a Bicyclolide we developed that demonstrates a broad spectrum of activity against many bacterial organisms, including MRSA. *In vitro*, EDP-322 had either

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comparable or superior activity to vancomycin (Vancocin™) or linezolid (Zyvox™) in MRSA clinical isolates. A prominent advantage of EDP-322 is activity against isolates with resistance, in comparison to vancomycin, linezolid and daptomycin (Cubicin™), the three therapies most often utilized as a last stand against resistant bacteria. EDP-322 has also shown good activity against linezolid-resistant *Enterococci*. Finally, EDP-322 demonstrates excellent efficacy in a number of preclinical *in vivo* infection models.

Preclinical safety studies performed with EDP-322 presented no significant concerns. EDP-322 was evaluated in normal healthy volunteers in two double-blind, randomized, placebo-controlled Phase 1 trials, evaluating pharmacokinetic and safety parameters. EDP-322 showed good pharmacokinetics and was well tolerated in all dose groups, with no findings of clinical significance in vital signs, physical exams, electrocardiograms or clinical chemistry. Adverse events were limited to gastrointestinal effects that we believe were attributable to inadequate water solubility of the drug, which we would not expect when dosing with the water-soluble EDP-788.

Owing to its high water solubility, EDP-788 has the significant benefit of allowing for an intravenous, or IV, formulation that has met the initial safety requirements for IV dosing. Preclinical testing has also demonstrated that oral dosing of the prodrug EDP-788 results in higher blood levels of the active compound EDP-322 than when EDP-322 is dosed orally itself. This makes EDP-788 ideally suited for stepdown dosing from IV administration in the hospital to oral administration in the home setting. Neither EDP-322, nor any other compound in the class of Bicyclolides, has yet been shown to be effective in pivotal clinical trials or resulted in any product approved by the FDA.

All current Bicyclolide development activities are focused on EDP-788 with additional IND-enabling studies in progress and the initiation of clinical trials planned for the first quarter of 2014. Our preclinical and early clinical development of EDP-788 is funded under our contract with NIAID.

Drug Discovery and Chemical Development

We have internally developed all of the initial compounds in our research programs, and have participated in the early development of these programs with our collaborators using our own internal research capabilities. 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 antiviral and antibacterial product candidates.

We focus on infectious diseases representing large and growing market opportunities with significant unmet medical needs. Our selection of a particular therapeutic target within those infectious diseases takes into consideration the experience and expertise of our scientific team. The final selection is based on the possibility of being able 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 Agreements

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 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.

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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 funds all costs associated with the development, manufacturing and commercialization of ABT-450 and other compounds under this 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 lead compound is ABT-450, and additional compounds are under development. The first clinical milestone for ABT-450 was achieved in 2010. To date, we have received upfront license payments, research funding, and milestone payments totaling $107.5 million from AbbVie, and additionally we have received an equity investment of $12.5 million from AbbVie.

We are eligible to receive future milestone payments totaling up to $40 million (exclusive of $55.0 million of milestone payments already received) upon AbbVie s achievement of regulatory filing milestones for the first protease inhibitor product resulting from our collaboration, as well as additional milestone payments totaling up to $155 million upon AbbVie s achievement of commercial regulatory approval milestones for such product in selected world markets. We are also eligible to receive additional milestone payments totaling up to $80 million upon AbbVie s achievement of similar commercial regulatory approval milestones for each additional product containing a protease inhibitor.

We are eligible to receive tiered royalties ranging from the low double digits up to twenty percent, or up to the high teens on a blended basis, based on the annual net sales of each product developed under the agreement. However, if a product is determined to be a combination product under our agreement, the royalties will be 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 a fair market value calculation.

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, and (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 a product developed under the agreement expires on a country-by-country basis upon the later of (i) the last date upon which the manufacture, use or sale of a product would infringe one of the licensed patents, and (ii) ten years after the first commercial sale of the product in the applicable country.

Under the agreement, we hold an option to fund 40% of U.S. development costs and U.S. commercialization efforts (sales and promotion costs), in exchange for 40% of any U.S. profits, allocable to any product candidate that ultimately achieves regulatory approval and commercialization. We did not exercise our option right with respect to

ABT-450, but we retain our option right for any next-generation products developed under the agreement, which must be exercised within a specified period after the successful completion of a Phase 2a trial of the next-generation product. If we exercise our co-development option right, we would be eligible for a different schedule of milestones and milestone payments than those described above, but would not be eligible to

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receive royalties on U.S. sales. If the first collaboration product that is approved is not ABT-450 and is instead a co-developed product, we would be eligible to receive future milestone payments totaling up to $120 million for clinical development and regulatory and reimbursement approval milestones. If any additional collaboration product containing a protease inhibitor is co-developed, we would be eligible to receive future milestone payments totaling up to $40 million for similar regulatory and reimbursement approval milestones.

Our intellectual property existing as of the effective date of the agreement remains our property. Any intellectual property jointly developed will be jointly owned. We will have unilateral right to enforce Enanta 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 Enanta 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.

Novartis

In February 2012, we entered into a Collaboration and License Agreement with Novartis granting Novartis exclusive, worldwide rights to develop, manufacture and commercialize EDP-239, our lead compound from our NS5A inhibitor program, and other NS5A inhibitor compounds. Under the agreement, we received an upfront payment of $34.4 million and an $11.0 million milestone payment in January 2013 based on Novartis initiation of dosing in a Phase 1 clinical trial that includes EDP-239. We are eligible to receive up to $395 million of additional milestone payments for

the first NS5A inhibitor for which Novartis achieves specified clinical, regulatory, and commercial milestones, including a payment of $15 million upon Novartis initiation of the first Phase 2 trial using a combination containing any NS5A inhibitor from our collaboration.

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We are also eligible to receive tiered royalties ranging on a blended basis from the low double digits up to the high teens on net sales allocable to our collaboration s NS5A inhibitors, subject to reduction in certain circumstances, and we retain an option for co-detail rights in the United States, which would allow us to staff up to a specified percentage of the sales force for a designated product. Under our agreement we must exercise these co-detail rights for a collaboration product before its expected commercial launch and then negotiate and finalize a co-detailing agreement with Novartis on reasonable and customary terms. During the term of the collaboration agreement we agree not to research, develop, manufacture or commercialize competing products, either alone or with other parties.

Novartis will fund all costs associated with the development, manufacture and commercialization of EDP-239, EDP-239-containing combinations and any follow-on NS5A inhibitors. Novartis was also responsible for funding our efforts to discover follow-on NS5A inhibitors through August 2013.

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 Novartis. However, Novartis has ultimate decision making authority with respect to the research, development and commercialization of collaboration products.

Our patents and know-how existing as of the effective date of the agreement remain our property. Any know-how or inventions jointly developed will be jointly owned, subject to the exclusive rights we grant to Novartis, and subject to such exclusive right may be licensed to any third party. Neither party will assign to any third party its interest in any jointly owned patent rights without the other party s prior written consent. Novartis will be responsible for filing, prosecuting and maintaining patents, at Novartis expense, relating to our intellectual property which is subject to the license, and all joint intellectual property. Novartis will also have the first right to prosecute any third-party infringement.

Subject to certain exceptions, on a country-by-country and product-by-product basis, a party s rights and obligations under the agreement continue until the later of: (i) the expiration of the last to expire patent rights of a covered product in the applicable country or (ii) ten years from the first commercial sale of a covered product in the applicable country. As a result, the final expiration date of the Novartis license is indeterminable at this time. Upon expiration of the agreement with respect to a particular product and country, the licenses granted to Novartis in the agreement with respect to such product and country will remain in effect and convert to a non-exclusive, perpetual, unrestricted, fully-paid, royalty-free, worldwide license.

We may terminate the agreement (i) in the event of a material breach by Novartis, subject to prior notice and the opportunity to cure, (ii) in the event Novartis fails to use commercially reasonable efforts to develop and commercialize covered products in its territory or (iii) in the event Novartis is subject to an insolvency event. Novartis may terminate the agreement (i) in the event of a material breach by us, subject to prior notice and the opportunity to cure, (ii) in the event we are subject to an insolvency event or (iii) for any reason upon 120 days prior written notice. In the case of a termination for cause by us or a termination without cause by Novartis, any licenses and other rights granted by either party to the other will terminate and revert back to the granting party and we will regain control of the prosecution of the patents relating to our intellectual property. If such termination occurs prior to the second anniversary of the end of the research term, we retain exclusive worldwide rights, with the right to sublicense under all collaboration intellectual property owned in whole or in part by Novartis, to research, develop and commercialize compounds and products contemplated by the collaboration. If such termination occurs after the second anniversary of the end of the research term, then Novartis agrees to negotiate with us to grant us a worldwide, exclusive, field-limited, royalty-bearing license, with right to sublicense, under all collaboration intellectual property owned in whole or in part by Novartis.

Royalties and milestones owed to us under the agreement can be reduced by Novartis in certain circumstances, including (i) where a product could not be legally developed or commercialized in a country without obtaining third-party intellectual property rights, (ii) where it is decided that it would be useful to license or otherwise acquire a third-party intellectual property right to develop or commercialize the product, (iii) where the net sales of a

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product in a country in one year decrease by a specified percentage when compared to the preceding year because of generic product competition, and (iv) where a product is not covered by a valid patent claim in the country of sale.

NIAID Contract

In September 2011, we were awarded a contract from NIAID to fund preclinical and early clinical development of a new class of bridged bicyclic antibiotics known as Bicyclolides. The Bicyclolides are to be used as medical countermeasures against multiple biodefense bacteria found in anthrax, plague and tularemia.

The contract has an initial term of 30 months ending on March 30, 2014. NIAID has the option to extend the contract up to 6 times. If each option period is exercised, the contract would be extended until September 29, 2016. The initial award under the initial term was $14.3 million, with the possibility of up to a total of $42.7 million if each option period is exercised by NIAID. In August 2013 NIAID exercised two such options which increased the contract amount awarded to date to $23.5 million.

Under the contract, all intellectual property rights held by us and any inventions, know-how or other intellectual property rights derived as a result of this contract will be our property, subject to certain rights of the United States federal government. See Risk Factors We could be unsuccessful in obtaining or maintaining adequate patent and other intellectual property protection for one or more of our product candidates. We also retain the right to use any data developed under the contract to enter into commercial transactions that are unrelated to the biodefense field.

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 viral diseases, including the same diseases we are targeting.

We expect our licensed product candidates and our future product candidates to face intense and increasing competition as new products enter the HCV and antibacterial markets and advanced technologies become available, particularly in the case of HCV therapies in combinations with existing products and other new products. Two drug products, Incivek (telaprevir) of Vertex and Victrelis (boceprevir) of Merck, were approved in 2011 by the FDA for the treatment of HCV in combination with the previous standard of care consisting of interferon in combination with ribavirin. A third protease inhibitor, simeprevir (Olysio) from Janssen Therapeutics was approved by the FDA in November 2013 for use in genotype 1 HCV patients only when used in combination with pegylated interferon and ribavirin. In December 2013, the FDA approved sofosbuvir (Sovaldi), a nucleotide analogue inhibitor of the HCV NS5B polymerase enzyme from Gilead for patients with genotype 2 or 3 HCV or in patients with genotypes 1 or 4 when combined with pegylated interferon and ribavirin. The evolving standard of care treatment regimens and the cure rates of patients using one of these approved drugs and future approved combinations of DAAs other than ones we have developed and are developing may be such that our development and discovery efforts in the area of HCV may be rendered noncompetitive.

We believe that a significant number of product candidates that are currently under development may become commercially available in the future for the treatment of HCV. We are aware that many competitors other than our collaborators have product candidates in Phase 2 or later stage clinical trials, including Achillion, Boehringer Ingelheim, Bristol-Myers Squibb, Gilead, Hoffman-La Roche, Idenix, Johnson & Johnson, Medivir, Merck and Vertex. Our competitors products may be more effective, have fewer side effects, have lower costs or be better marketed and sold than any product candidate that includes ABT-450, ABT-493 and EDP-239 or any of our future

compounds or than any of our future product candidates. Additionally, products that our competitors successfully develop for the treatment of HCV may be marketed prior to any HCV product that our collaborators or we may successfully develop.

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AbbVie has the right to market and sell products that compete with the product candidates that we have licensed to it and any competition by AbbVie could also have a material adverse effect on our future business.

Our lead antibiotic product candidate, EDP-788, is being developed as a broad-spectrum antibiotic with MRSA coverage for first line use in the hospital setting. In this treatment setting, if approved, EDP-788 would compete with a number of currently-marketed antibiotics, including Tygacil and Teflaro , and antibiotics currently in Phase 3 development, including omadocycline/PTK-0796, a tetracycline under development by Paratek Pharmaceuticals, as well as delafloxicin being developed by Rib-X Pharmaceuticals. We expect that EDP-788 would also compete with currently marketed antibiotics used for serious, Gram-positive infections, including vancomycin, a generic drug that is manufactured by a variety of companies, Zyvox , Cubicin and telavancin (Vibativ). In addition, a number of Gram-positive anti-infective product candidates currently in Phase 3 development could also compete with EDP-788 if they are approved, including dalbavancin (under development by Durata Therapeutics, Inc.), oritavancin (under development by The Medicines Company), tedizolid (under development by Cubist Therapeutics, Inc.) and Taksta (under development by Cempra, Inc.).

Competitive products may render our product candidates licensed to others, as well as any future product candidates we may develop ourselves for HCV or MRSA treatment, obsolete or noncompetitive. All of these product candidates will face competition based on their safety and effectiveness, the timing and scope of regulatory approvals, the availability and cost of supply, marketing and sales capabilities, reimbursement coverage, price, patent position and other factors. Our competitors may succeed in developing competing products before we do, obtaining regulatory approval for products or gaining acceptance for the same markets that we are targeting. If we or our collaborators are not first to market with one of our product candidates for a given disease indication or a given product profile, our competitive position could be compromised because it may be more difficult for us to obtain marketing approval for that product candidate and/or successfully market that product candidate as a second competitor.

Many of our competitors have substantially greater commercial infrastructures and financial, technical and personnel resources than we have. 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 in order to overcome price competition and to be commercially successful. If we and our collaborators are not able to compete effectively against current and future competitors for our product candidates, our business will not grow and our financial condition will be adversely

affected.

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 typically has several issued patents and pending patent claims in the program area containing claims to compounds, methods of use and processes for synthesis, but only a few of the issued patents and/or pending patent applications cover the lead product candidate in the program.

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HCV NS3 Protease Inhibitor Program. The patent portfolio directed to the HCV protease inhibitor program with AbbVie included as of September 30, 2013 more than 35 issued U.S. patents, with 13 U.S. non-provisional applications pending, as well as approximately 50 issued foreign patents with more than 225 non-provisional applications pending in foreign jurisdictions. The issued United States patents and the applications, if granted, will expire between 2023 and 2031 before taking into account any extensions that may be obtained through patent term extension or adjustment, or term reduction due to filing of terminal disclaimers. 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.

HCV NS5A Inhibitor Program. Our patent portfolio directed to our HCV NS5A inhibitor program with Novartis included as of September 30, 2013 eight issued U.S. patents, 17 U.S. non-provisional applications, one provisional U.S. application, one issued foreign patent as well as more than 75 non-provisional applications in foreign jurisdictions and five PCT applications pending. The issued United States patents and the applications, if granted, will expire between 2030 and 2032 before taking into account any extensions that may be obtained through patent term extension or adjustment, or term reduction due to filing of terminal disclaimers. Novartis has rights to some or all of these patents and patent applications pursuant to its collaboration agreement with us.

Cyclophilin Inhibitor Program. Our ongoing research activities include identifying compounds that inhibit cyclophilin, a protein in the human body that has been shown to be involved in HCV replication. Our current portfolio directed to cyclophilin binders for the treatment of HCV included as of September 30, 2013 at least five issued U.S. patents, three U.S. non-provisional applications, two U.S. provisional applications along with two granted patents and 16 non-provisional applications pending in foreign jurisdictions. The issued United States patents and patent applications, if granted, will expire between 2030 and 2031 before taking into account any extensions that may be obtained through patent term extension or adjustment, or term reduction due to filing of terminal disclaimers.

HCV Nucleotide Polymerase Inhibitor Program. Our patent portfolio directed to our HCV nucleotide polymerase inhibitor program, which is in the early stages of development, includes one issued patent with three pending U.S. non-provisional applications and one provisional application, and one pending PCT application.

Antibacterial Program. Our patent portfolio directed to antibacterials included as of September 30, 2013 more than 20 issued U.S. patents, and approximately five U.S. non-provisional applications, one U.S.provisional application pending, as well as more than 50 issued foreign patents and more than a dozen non-provisional foreign applications. These patents and patent applications, if granted, will expire between 2020 and 2032 before taking into account any extension that may be obtained through patent term extension or adjustment, or term reduction due to filing of terminal disclaimers.

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 may begin to run prior to the 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 ABT-450 as a chemical entity. However, there is no guaranty that such applications will issue. We also own one issued patent that claims EDP-

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239 as a chemical entity. 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 or 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 are developing. 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 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 Practices, 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;

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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 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 suspend or terminate 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.

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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 AEs 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

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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 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 need not 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

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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

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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.

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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.

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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 pharmacoeconomic 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.

In March 2010, the President signed one of the most significant healthcare reform measures in decades. 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 changes the way healthcare will be financed by both governmental and private insurers, and significantly impacts the pharmaceutical industry. The comprehensive $940 billion dollar overhaul is expected to extend coverage to approximately 32 million previously uninsured Americans. The Affordable Care Act contains a number of provisions, including those governing enrollment in federal healthcare programs, reimbursement changes and fraud and abuse, which will impact existing government healthcare programs and will

result in the development of new programs, including Medicare

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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 percent 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 proposed in Congress a number of legislative initiatives regarding healthcare, including possible repeal of the Affordable Care Act. At this time, it remains unclear whether there will be any changes made to the Affordable Care Act, whether to certain provisions or its entirety. The full impact that the Affordable Care Act and other new laws will have on our business is uncertain. Nor is it clear whether other legislative changes will be adopted, if any, or how such changes would affect the demand for our product candidates once commercialized.

Different pricing and reimbursement schemes exist in other countries. In the European Community, 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 payors 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 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 supply of ingredients that will be used in clinical trials of our product candidates that we are developing ourselves. Manufacturing for each of our two lead product candidates, namely ABT-450 and EDP-239, is being conducted by our collaborator for the respective product candidate. 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.

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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 already partnered our two lead candidates with AbbVie and Novartis, respectively. We may also partner or collaborate with, or license commercial rights to, other larger pharmaceutical or biopharmaceutical companies to support the development of our cyclophilin and nucleotide polymerase inhibitor 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, 2013, we had 44 full-time employees, 23 of whom hold Ph.D. degrees. None of our employees is subject to a collective bargaining agreement or represented by a trade or labor union. We consider our relations with our employees to be good.

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 substantially dependent upon the development and marketing efforts of AbbVie for combination therapies incorporating ABT-450 or ABT-493 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 ABT-450, ABT-493 (a next-generation protease inhibitor in clinical development) and any other protease inhibitors we develop, over which AbbVie has complete control. Our ability to generate significant revenue in the near term will depend primarily on the successful development, regulatory approval, marketing and commercialization by AbbVie of combination therapies incorporating ABT-450 or ABT -493. Such success is subject to significant uncertainty, and we have limited control over the resources, time and effort that AbbVie may devote to ABT-450 or ABT-493. Any of several events or factors could have a material adverse effect on our ability to generate revenue from AbbVie s potential commercialization of ABT-450 or ABT-493 in combination therapies. For example, AbbVie:

- may be unable to successfully complete the clinical development of an ABT-450-containing regimen;

may have to comply with additional requests and recommendations from the FDA, including additional clinical trials;

may not make all regulatory filings and obtain all necessary approvals from the FDA and similar foreign regulatory agencies;

may not commit sufficient resources to the development, regulatory approval, marketing and distribution of an ABT-450 (or ABT-493)-containing regimen, whether for strategic reasons or otherwise due to a change in business priorities;

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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 to continue development of any of our product candidates;

may not be able to manufacture our product candidate in compliance with requirements of the FDA and similar foreign regulatory agencies and in commercial quantities sufficient to meet market demand;

may not achieve market acceptance of combination therapies incorporating our product candidate by physicians, patients and third-party payors;

may not compete successfully with any such combination therapies against alternative products and therapies for HCV; and

may independently develop products that compete with our product candidate in the treatment of HCV.

We will not have access to all information regarding the products being developed and potentially commercialized by AbbVie, including information about clinical trial design and execution, safety reports from clinical trials, spontaneous safety reports if the product is later approved and marketed, regulatory affairs, process development, manufacturing, marketing and other areas known by AbbVie. Thus, our ability to keep our stockholders informed about the status of product candidates under our collaboration will be 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 clinical development, regulatory approval and commercialization efforts related to ABT-450 or ABT-493 could be delayed or terminated or be commercially unsuccessful. In addition, AbbVie has the right to make decisions regarding the development and commercialization of product candidates without consulting us, and may make decisions with which we do not agree. Conflicts between us and AbbVie may arise if there is a dispute about the progress of the clinical development of a product candidate, the achievement and payment of a milestone amount, or the ownership of intellectual property developed during the course of our collaboration agreement. It may be necessary for us to assume responsibility at our own expense for the development of ABT-450, ABT-493 or other protease inhibitors. In that event, we would likely be required to limit the size and scope of one or more of our independent programs or increase our expenditures and seek additional funding which may not be available on acceptable terms or at all, and our business would be materially and adversely affected.

Our prospects for successful development of EDP-239 or any other NS5A inhibitor are dependent upon the development and marketing efforts of Novartis. Novartis may act in its best interest rather than in our best interest, which could adversely affect our business.

We rely on Novartis to fund and conduct the clinical development of EDP-239 and any other NS5A inhibitor product candidates under our collaboration, and for the successful regulatory approval, marketing and commercialization of one or more of them. Such success will be subject to significant uncertainty, and we have limited control over the resources, time and effort that Novartis may devote to our NS5A inhibitors. Moreover, Novartis may terminate the collaboration without any reason on 120 days notice to us. As with our AbbVie collaboration, any of several events or factors could have a material adverse effect on our ability to generate revenue from Novartis development and commercialization of EDP-239, including ones similar to those described in the preceding risk factor regarding our

AbbVie collaboration.

If Novartis does not perform in the manner we expect or fulfill its responsibilities in a timely manner, or at all, the clinical development, regulatory approval and commercialization efforts related to EDP-239 could be delayed, terminated or be commercially unsuccessful. Conflicts between us and Novartis may arise if there is a dispute with Novartis similar to potential disputes with AbbVie about any of the matters mentioned in the preceding risk factor. It may become necessary for us to assume the responsibility at our own expense for the development of EDP-239 or other NS5A inhibitors. In that event, we would likely be required to limit the size and scope of one or more of our independent programs or increase our expenditures and seek additional funding which may not be available on acceptable terms or at all, and our business would be materially and adversely affected.

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We and our collaborators face substantial competition in the market for HCV drugs and for anti-infectives generally, which may result in others discovering, developing or commercializing products before us or doing so more successfully than we and our collaborators.

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, MRSA and other infectious diseases that we may target in the future.

We expect our current and future product candidates to face intense and increasing competition as new products enter the HCV and antibacterial markets and advanced technologies become available, particularly in the case of HCV in combinations with existing products and other new products. Two drug products, Incivek (telaprevir) of Vertex and Victrelis (boceprevir) of Merck, were approved in 2011 by the FDA for the treatment of HCV in combination with interferon and ribavirin, which in combination were the previous standard of care. A third protease inhibitor, simeprevir (Olysio) from Janssen Therapeutics was approved by the FDA in November 2013 for use in genotype 1 HCV patients only when used in combination with pegylated interferon and ribavirin. In December 2013, the FDA approved sofosbuvir (Sovaldi), a nucleotide analogue inhibitor of the HCV NS5B polymerase enzyme from Gilead for patients with genotype 2 or 3 HCV or in patients with genotypes 1 or 4 when combined with pegylated interferon and ribavirin. Other all-oral treatment regimens are under development and may obtain regulatory approvals for some forms of HCV before any combination including one of our compounds is approved. These other potential new treatment regimens may render our HCV product candidates noncompetitive. In particular, our HCV product candidates may not be able to compete successfully with other products in development in multiple classes of inhibitors of HCV, including protease inhibitors, polymerase inhibitors (nucleoside and non-nucleoside), NS5A inhibitors and cyclophilin inhibitors, under development by companies such as Achillion, Astra-Zeneca, Boehringer Ingelheim, Bristol-Myers Squibb, Gilead, GlaxoSmithKline, Idenix, Johnson & Johnson, Medivir, Merck, Pfizer, Presidio, Roche and Vertex, as well as by our collaborators.

Our MRSA program faces competition from other therapeutic products that address serious Gram-positive bacterial infections, such as Cubicin®, marketed by Cubist; vancomycin, marketed generically by AbbVie, Shionogi and others; and Zyvox®, marketed by Pfizer, as well as future competition from drug candidates currently in clinical development.

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

Our competitors may succeed in developing competing products and obtaining regulatory approval before we or our collaborators do with our product candidates, or they may gain acceptance for the same markets that we are targeting. If we are not first to market with one of our product candidates in one or more 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 successfully market that product candidate as a second competitor. In addition, any new product that competes with an approved product must demonstrate compelling advantages in efficacy, convenience, tolerability and safety in order to overcome price competition and to be commercially successful.

Competitive products in the form of other treatment methods or a vaccine for HCV or MRSA may render our product candidates licensed to others, as well as any future product candidates we may develop ourselves, obsolete or noncompetitive. Even if successfully developed and subsequently approved by the FDA, our product candidates will face competition based on their safety and effectiveness, the timing and scope of the regulatory approvals, the availability and cost of supply, marketing and sales capabilities, reimbursement coverage, price, patent position and other factors. If the product candidates developed under our collaboration agreements with

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AbbVie and Novartis face competition from generic products, the collaboration agreements provide that the royalty rate applicable to such product candidates is reduced significantly by a specified percentage on a product-by-product, country-by-country basis. If we and our collaborators are not able to 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 no approved products and no clinical development experience, which makes it difficult to assess our ability to develop and commercialize our future product candidates.

To date, AbbVie has been and will continue to be responsible for all of the clinical development of our ABT-450, ABT-493 and other protease inhibitor product candidates, and Novartis is responsible for all future clinical development of our EDP-239 and other NS5A product candidates. We have not yet demonstrated an ability to successfully overcome 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 programs for cyclophilin inhibitors and nucleotide polymerase inhibitors for HCV and antibiotics for MRSA, we will need to successfully:

- execute clinical development of our future product candidates;
- obtain required regulatory approvals for the development and commercialization of our future product candidates;
- develop and maintain any future collaborations we may enter into for any of these programs;
- build and maintain robust sales, distribution and marketing capabilities, either independently or in collaboration with future collaborators;
- gain market acceptance for our future product candidates; 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 future product candidates and expand our business or continue our operations.

We may require substantial additional financing to achieve our goals if the development and commercialization of ABT-450, ABT-493 or EDP-239 is delayed or terminated. A failure to obtain this necessary capital when needed could force us to delay, limit, reduce or terminate 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 preclinical 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. In our fiscal year ending September 30, 2014, we expect to incur substantial costs associated with research and development for our internally developed programs, exclusive of costs incurred by our collaborators in developing our licensed product candidates ABT-450, ABT-493 and EDP-239.

Because the outcome of planned and anticipated clinical trials of our product candidates by our collaborators is highly uncertain, we cannot reasonably estimate the timing or amounts, if any, of future milestone payments we will receive from these collaborators. If we do not continue to receive substantial milestone payments from the continued development of our product candidates, we may require substantial additional financing.

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Our future capital requirements depend on many factors, including:

- whether our existing collaborations continue to generate substantial milestone payments, and ultimately royalties, to us;
- the number and characteristics of the future product candidates we pursue;
- the scope, progress, results and costs of researching and developing any of our future product candidates on our own, and conducting preclinical research and clinical trials;
- the timing of, and the costs involved in, obtaining regulatory approvals for any future product candidates we develop independently;
- the cost of commercialization activities, if any, of any future product candidates we develop independently that are approved for sale, including marketing, sales and distribution costs;
- the cost of manufacturing our future product candidates and any products we successfully commercialize independently, including manufacturing for clinical development;
- our ability to maintain existing collaborations and to establish new collaborations, licensing or other arrangements and the financial terms of such agreements;
- the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing patents, including litigation costs and the outcome of such litigation; and
- the timing, receipt and amount of sales of, or royalties on, ABT-450, ABT-493, EDP-239 and our future product candidates, if any.

Additional funds may not be available 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.

If we are not successful in discovering further product candidates in addition to ABT-450, ABT-493 and EDP-239, our ability to expand our business and achieve our strategic objectives may be impaired.

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 future product candidates obsolete;
- a future product candidate may, on further study, be shown 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 future product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all.

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.

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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, and Yat Sun Or, Ph.D., our Senior Vice President, Research and Development and Chief Scientific Officer, as well as other employees and consultants. Although none of these individuals has informed us to date that he intends to retire or resign in the near future, the loss of 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 future 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 field 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 have incurred a substantial cumulative net loss since our inception and anticipate that we may incur substantial operating losses in one or more years in the future. To date, our principal sources of revenue have been our collaboration agreements, including our current agreements with AbbVie and Novartis, and future payments under these agreements are uncertain. We have had no products approved for commercial sale. As a result, our ability to achieve sustained profitability is unproven.

We have incurred cumulative net losses since our inception, and as of September 30, 2013, we had an accumulated deficit of $107.5 million. Our net income in the fiscal year ended September 30, 2010 resulted primarily from the conclusion of a previous collaboration which accelerated $16.2 million of deferred revenue into fiscal 2010 that was related to cash received and spent in prior years, and our net income in the fiscal year ended September 30, 2011 resulted primarily from a substantial milestone payment from AbbVie. In the fiscal year ended September 30, 2012, our net income resulted primarily from a substantial upfront license payment from Novartis. In the fiscal year ended September 30, 2013, our net income resulted primarily from milestone payments we earned from AbbVie and Novartis of $15.0 million and $11.0 million, respectively. There is no assurance, however, that we will recognize any collaboration revenue during fiscal 2014 or report net income in fiscal 2014 or subsequent years. To date, we have not commercialized any products or generated any revenue from product sales, directly or through any collaborator.

Our principal source of revenue has been our collaboration agreements, including our current agreements with AbbVie and Novartis. Future milestone payments are uncertain because our collaborators may choose not to continue research or development activities for one or more potential product candidates. For example, under a prior collaboration for the development of an antibiotic product candidate in Japan, our collaborator decided in 2010 not to pursue further development of the licensed product candidate due to its limited potency against *Haemophilus influenzae* in clinical trials of community-acquired pneumonia, which then resulted in our collaboration being terminated. In addition, we may not achieve the specified milestones, our product candidates may not be approved by the FDA or other regulatory authorities or, if they are approved, may not be accepted in the market. If we are unable to develop and commercialize one or more of our product candidates, either alone or with our collaborators, or if any such product candidate does not achieve market acceptance, we may never generate sufficient product royalties or product sales. Even if we do generate product royalties or product sales, we may never achieve or sustain profitability on a quarterly or annual basis. Our failure to sustain profitability would depress the market price of our common stock and 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.

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

As we 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.

Our government funded contract for our antibiotic program is subject to termination and uncertain future funding and there is no certainty that we will be able to enter into new agreements to provide these funds.

Under our agreement 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, NIAID has the option to make future payments to fund our early clinical development of EDP-788. If NIAID exercises each remaining option under the agreement, the aggregate funding commitment will be $42.7 million. On August 29, 2013, NIAID exercised the first two options under the agreement, pursuant to which is has agreed to provide an additional $9.2 million in funding for preclinical and early clinical development of EDP-788. To date only $23.5 million has been committed for our work under the agreement. NIAID has several remaining options to decide whether it wants to continue the program in its sole discretion. In addition, the ability of government agencies such as NIAID to perform under these types of agreements is dependent upon adequate continued funding of the agencies and their programs. We have no control over the resources and funding NIAID may devote to our agreement, which may be subject to periodic renewal and which generally may be terminated by NIAID at any time. For example, in accordance with the spending cuts, known as sequestration, to implement the Budget Control Act of 2011, NIAID notified us on March 4, 2013 of the possibility that NIAID may not exercise the options on our contract or may negotiate lower prices or other terms via a bilateral modification. And again in September 2013 NIAID notified of possible reductions in funding due to the budget impasse and government shut-down at the commencement of the U.S. government s fiscal year beginning October 1, 2013. Any significant reductions in the funding of U.S. government agencies or in the funding areas targeted by our business could materially and adversely affect our antibiotic program and our results of operations and financial condition. If we fail to satisfy our contractual obligations under the agreement, the applicable Federal Acquisition Regulations allow the government to terminate the agreement in whole or in part, and we may be required to perform corrective actions, including but not limited to delivering to the government any incomplete work. If NIAID does not exercise future funding options under the agreement, terminates the agreement or fails to perform its responsibilities under the agreement, it could materially impact our antibiotic program and our financial results.

In addition, 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 revenue. In addition, U.S. government contracts are conditioned upon the continuing availability of Congressional appropriations. Congress usually appropriates funds on a fiscal year basis even though contract performance may take several years. Consequently, at the outset of a major program, the contract is usually incrementally funded and additional funds are normally committed to the contract by the procuring agency as appropriations are made by Congress for future fiscal years. Any failure of NIAID to continue

to fund our contract could have a material adverse effect on our business, results of operations and financial condition.

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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 are prolonged or delayed, we or our collaborators 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. The first top-line results of a Phase 3 trial of ABT-450 in combination therapy was reported in November 2013, but none of the other product candidates in our pipeline has yet advanced beyond Phase 2 clinical trials. The ABT-450 Phase 3 trials or any future Phase 3 trials may fail to demonstrate sufficient safety and efficacy. Moreover, regulatory and administrative delays may adversely affect our or our collaborators clinical development plans and jeopardize our or our collaborators ability to attain product approval, commence product sales, compete successfully against other HCV therapies and generate revenues.

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; or

varying interpretations of data by the FDA and similar foreign regulatory agencies.

The results of any Phase 3 clinical trial may not be adequate to support marketing approval. These clinical trials are lengthy and usually involve from many hundreds to thousands of patients. In addition, if the FDA disagrees with our or our collaborator s choice of the key testing criteria, or primary endpoint, or the results for the primary endpoint are not robust or significant relative to the control group of patients not receiving the experimental therapy or are subject to confounding factors, or are not adequately supported by other study endpoints, the FDA may refuse to approve our or our collaborator s product candidate. The FDA also may require additional clinical trials as a condition for approving any of these product candidates. We estimate that it will likely be 2015 before an NDA for one of our collaborator s product candidates could be approved by the FDA.

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 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

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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. For example, Novartis drug candidate that is a cyclophilin inhibitor had been placed on clinical hold by the FDA based on a small number of cases of pancreatitis in clinical trial patients, one of which resulted in a patient s death. While Novartis obtained a revision of the clinical hold and has initiated a Phase 2 drug-drug interaction study, the clinical hold has the potential to result in delays in development of other similar inhibitors, including delays due to additional preclinical or clinical testing protocols for all similar inhibitors. If we or our 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 or our collaborators 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.

If we or our collaborators are required to suspend or discontinue clinical trials due to side effects or other safety risks associated with our product candidates, or if we or our collaborators are required to conduct studies on the long-term effects associated with the use of our product candidates, efforts to commercialize our 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 or our collaborators 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, our collaborators or regulatory authorities, such as the FDA, to interrupt, delay or halt clinical trials of our product candidates and could result in the FDA 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 or our collaborators from commercializing our product candidates.

Our Bicyclolide product candidates are in a novel class of antibiotics. Regulatory authorities may require more extensive studies of the long-term effects for regulatory approval, which could delay development of EDP-788 or our other future antibiotic product candidates. These studies could also be required at any time after regulatory approval of any of our product candidates. Some or all of our product candidates may prove to be unsafe for human use.

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, whether ABT-450, ABT-493, EDP-239, EDP-788 or any of our future product candidates, may not be predictive of the results of later-stage clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy results despite having progressed through preclinical studies and initial clinical trials. Many companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to adverse safety profiles or lack of efficacy, notwithstanding promising results in earlier studies. Similarly, future clinical trial results may not

be successful for these or other reasons.

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This 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 to early to late stage clinical trials towards approval and commercialization, it is customary that various aspects of the development program, such as manufacturing and 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 or our collaborators 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 or our collaborators ability to commence product sales and generate revenues.

The regulatory approval processes of the FDA and comparable foreign authorities are lengthy, time-consuming and inherently unpredictable, and if we or our collaborators 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 approval is unpredictable and depends 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 or our collaborators 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 other regulatory authority. Neither we nor our collaborators have obtained regulatory approval for any of our 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 or comparable foreign regulatory authorities may disagree with the design or implementation of our or our collaborators clinical trials;
- we or our collaborators may be unable to demonstrate to the satisfaction of the FDA or 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 or comparable foreign regulatory authorities for approval;
- we or our collaborators may be unable to demonstrate that a product candidate s clinical and other benefits outweigh its safety risks;

the FDA or comparable foreign regulatory authorities may disagree with our or our collaborators 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 submission 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 or our collaborators contract for clinical and commercial supplies; and

the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our or our collaborators clinical data insufficient for approval.

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We and our collaborators cannot be assured that after spending substantial time and resources, we or our collaborators will obtain regulatory approval. Even if we or our collaborators 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 or our collaborators do or could shorten the patent protection period during which we may have the exclusive right to commercialize our product candidates. In addition, we or our collaborators may not be able to ultimately achieve the prices intended for our products. In many foreign countries, 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.

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, 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 our collaborators, 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 our collaborators are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we or our collaborators are not able to maintain regulatory compliance, our product candidate 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 or our collaborators may delay or terminate the development of a product candidate at any time if we or our collaborators believe the perceived market or commercial opportunity does not justify further investment, which could materially harm our business.

Even though the results of preclinical studies and clinical trials that we or our collaborators have conducted or may conduct in the future may support further development of one or more of our product candidates, we, or our

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collaborator in the case of our partnered product candidates, 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 FDA approval, 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, our collaborators may have the right to make decisions regarding the development and commercialization of product candidates without consulting us, and may make decisions with which we do not agree.

Our internal computer systems, or those of our collaborators, 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. 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 or those of our collaborators. 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. 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, we or our collaborators could incur liability and the further development of our product candidates could be delayed.

Risks Related to Commercialization of 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 future 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 future products if and when they are approved, or enter into licensing or collaboration agreements where our collaborator is responsible for commercialization, like in the case of our collaborations with Novartis and AbbVie, or where we have the right to assist in the future development and commercialization of such products. For example, we have a co-detail option with respect to any product that may be developed under our Novartis collaboration, which would allow us to establish a limited sales force in the United States for a portion of the product s sales.

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 future 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.

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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 our product candidates licensed to AbbVie and Novartis, if approved, as well as of any future product candidates we plan to develop independently or in collaboration with others.

Even if ABT-450, ABT-493 or EDP-239 or any other product candidate that we may develop in the future obtains regulatory approval, whether as part of a combination therapy or as a monotherapy, the product may not gain market acceptance among physicians, healthcare payors, patients and the medical community. For example, the standard of care in HCV is likely to evolve rapidly as many new product candidates are being developed and tested. The degree of market acceptance of any products for which we receive approval for commercial sale depends on a number of factors, including:

- the efficacy and safety of our partnered product candidates, as demonstrated in clinical trials, and the degree to which these product candidates represent a clinically meaningful improvement in care as compared with other available therapies;
- the clinical indications for which any product candidates become approved;
- acceptance among physicians, major operators of clinics and patients of any of our product candidates as safe, effective and preferred treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the potential and perceived advantages of our product candidates over alternative treatments;
- the cost of treatment in relation to alternative treatments;
- the availability of adequate reimbursement and pricing by third parties and government authorities;

the continued projected growth of the HCV drug market;

the relative convenience and ease of administration of any combination therapies including our product candidates;

the prevalence and severity of adverse side effects, whether involving the use of our products candidates or similar, competitive products; and

the effectiveness of our or our collaborators sales and marketing efforts.

If our product candidates are approved and then fail to achieve market acceptance, we would not be able to generate significant revenue. Further, if new, more favorably received therapies are introduced after our product candidates achieve market acceptance, then we may not be able to maintain that market acceptance over time.

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Even if we or our collaborators are able to commercialize any product candidates, the resulting products 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, may significantly change the way healthcare is financed by both governmental and private insurers. While we cannot predict what impact on federal reimbursement policies this law will have in general or specifically on any product that we or any of our collaborators commercializes, the ACA 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, some states have indicated that they intend not to implement certain sections of the ACA, and some members of the U.S. Congress are still working to repeal the ACA. We cannot predict whether these challenges will continue or other proposals will be made or adopted, or what impact these efforts may have on us or any of our collaborators. Our or any collaborators ability to commercialize any products successfully also will 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. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that reimbursement will be available for any product that we or any collaborator commercialize and, if reimbursement is available, the level of reimbursement. In addition, reimbursement may impact the demand for, or the price of, any product candidate for which we or a collaborator obtains marketing approval. If reimbursement is not available or is available only to limited levels, we or our collaborators may not be able to successfully commercialize any product candidate for which marketing approval is obtained.

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. 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. Our or any collaborator s inability to promptly obtain coverage and profitable payment rates from both government-funded and private payors for any approved products that we or our collaborators 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.

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 or our collaborators may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies.

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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 our collaborators 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 our products 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 profitability 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 our future 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 our future 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 future 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 either of our existing collaboration agreements 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 current or future product candidates may be terminated or delayed;
- our cash expenditures related to development of certain of our future 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 clinical product candidate supplies and any commercial supplies of any approved future 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 work with third-party contract manufacturers to produce sufficient quantities of any future product candidates for preclinical testing, clinical trials and commercialization. If we are unable to arrange for such a third-party manufacturing source, or fail to do so on commercially reasonable terms, we may not be able to successfully produce, develop and market our future product candidates, or we may be delayed in doing so.

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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 future 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.

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

Although manufacturing for each of our lead product candidates, namely ABT-450, ABT-493 and EDP-239, is being conducted by our collaborators, 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 research product candidates, and we expect to continue to use such third party manufacturers for such intermediates for any future product candidates we develop independently, including EDP-788. 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 future product candidates. Furthermore, since these 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

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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 future 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 future 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 academic institutions, CROs, hospitals, clinics and other third-party collaborators who are outside our control to monitor, support, conduct and/or oversee preclinical and clinical studies of our future product candidates. We will also rely on third parties to perform clinical trials on our future 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 future 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 future 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 future product candidates, our dependence on such relationships may adversely affect our business.

Our commercialization strategy for some of our future product candidates may depend on our ability to enter into collaboration agreements with other companies to obtain assistance and funding for the development and potential commercialization of these product candidates, similar to what we have done with AbbVie and Novartis. 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

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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 EDP-788, our lead candidate for the treatment of MRSA, is currently funded under a contract with the 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

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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 future 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.

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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 or any future collaborators 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 and Novartis license or otherwise acquire 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 or, in the case of the Novartis agreement, where it is decided that it would be useful to acquire such third-party right to develop or commercialize the product, they are entitled under our collaboration agreements 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.

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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 and anti-infectives. 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.

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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 or our collaborators own or have exclusively licensed;

- we or our collaborators or any future collaborators 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 or our collaborators or any future collaborators 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;

- the ownership of the intellectual property arising out of our collaborations is subject to complex legal and factual issues, and in certain circumstances our collaborators may own or jointly own important intellectual property relating to our product candidates. Although we have rights to such intellectual property under our collaboration agreements, such rights could potentially be lost or diminished if the applicable collaboration agreement is terminated, which could affect our ability to commercialize our product candidates;

- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;

- it is possible that our pending patent applications will not lead to issued patents;

- issued patents that we own or have exclusively licensed may not provide us with any competitive advantages, or may be held invalid or unenforceable, as a result of legal challenges by our competitors; 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, obtaining, maintaining and enforcing

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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 Industry

Unstable market and economic conditions may have serious adverse consequences on our business, financial condition and stock price.

As has been widely reported, global credit and financial markets have been experiencing extreme disruptions over the past several years, including severely diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, increases in unemployment rates and uncertainty about economic stability. There can be no assurance that further deterioration in credit and financial markets and confidence in economic conditions will not occur. Our general business strategy may be adversely affected by the recent economic downturn and volatile business environment and continued unpredictable and unstable market conditions, particularly for securities of biotechnology companies such as our common stock. There is a possibility that our stock price may decline, due in part to the volatility of the stock market and any general economic downturn. If the current equity and credit markets become more volatile, deteriorate or do not improve, it may make any necessary debt or equity financing more difficult to secure, more costly and/or more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our growth strategy, financial performance and stock price and could require us to delay or abandon our business and clinical development plans. In addition, there is a risk that one or more of our current service providers, manufacturers and other partners may not survive these difficult economic times, which could directly affect our ability to attain our operating goals on schedule and on budget.

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;

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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 $5.0 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 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;

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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.

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.

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Risks Related to Our Common Stock

Our executive officers, directors and principal stockholders own a significant percentage of our stock and will be able to exercise control over all matters submitted to stockholders for approval.

Our executive officers and directors, together with our stockholders that hold more than 5% of our outstanding common stock, beneficially owned, in the aggregate, outstanding shares representing approximately two-thirds of our outstanding common stock after giving effect to the shares allocated in our IPO on March 20, 2013. Although some of our 5% stockholders have sold shares since the IPO, we believe that our officers and directors and their affiliates and our 5% stockholders beneficially owned, in the aggregate, approximately 55.54% of our outstanding common stock as of December 1, 2013. As a result, if these stockholders were to choose to act together, they would be able to control all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would control the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. The interests of this group of stockholders may not always coincide with our corporate interests or the interests of other stockholders, and they may act in a way in which you may not agree with or in a way that may not be in the best interests of other stockholders. This concentration of voting power could delay or prevent an acquisition of our company on terms that other stockholders may desire.

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 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

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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 named 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 named executive officers are parties to employment agreements that provide for aggregate cash payments of up to approximately $2.1 million for severance and other benefits and acceleration of vesting of stock options in the event of a termination of employment in connection with a change of control of our company. Based on the September 30, 2013 closing price of our common stock at $22.92 per share, the aggregate intrinsic value of unvested stock options subject to accelerated vesting upon these events was $2.0 million as of September 30, 2013. The accelerated vesting of 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 financial condition and results. In addition, these potential severance payments may discourage or prevent third parties from seeking a business combination with us.

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

As a public company, we incur significant legal, accounting and other expenses that we did not incur as a private company. We are subject to the reporting and other requirements of the Securities Exchange Act of 1934, as amended, known as the Exchange Act, portions of the Sarbanes-Oxley Act of 2002, as well as rules subsequently adopted by the Securities and Exchange Commission, or SEC, and The NASDAQ Stock Market, or NASDAQ. These rules and regulations require, among other things, that we file annual, quarterly and current reports with respect to our business and financial condition and establish and maintain effective disclosure and financial controls and corporate governance practices. These rules and regulations substantially increase our legal and financial compliance costs and make some activities more time-consuming and costly, and we expect that this our legal and financial compliance costs will further increase after we are no longer an emerging growth company as defined in the recently enacted Jumpstart Our Business Startups Act of 2012, or the JOBS Act. Our management and other personnel devote a substantial amount of time to these compliance initiatives. We estimate that incremental annual compliance costs associated with these reporting obligations will be at least $1.0 million.

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. We could be an emerging growth company for up to five years, although circumstances could cause us to lose that status earlier, including if the market value of our common stock held

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by non-affiliates exceeds $700 million as of any March 31 (the end of our second fiscal quarter) before that time, in which case we would no longer be an emerging growth company as of the following September 30 (our fiscal year end). 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.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption to delay the adoption of new or revised accounting standards and, therefore, will be subject to adopting new or revised accounting standards at the same time as other public companies that are not emerging growth companies.

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 will be required to disclose changes made in our internal controls and procedures on a quarterly basis and our management will be 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 could be an emerging growth company for up to five years. 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.

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

Our stock price is likely to be volatile. 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 any of our collaborators regarding our product candidates they are developing, including announcements regarding clinical or regulatory developments or our collaboration;

results from or delays of clinical trials of our 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;

our or our collaborators decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;

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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 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 future 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;

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;

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.

We do not anticipate paying any cash dividends on our capital stock in the foreseeable future.

We do not currently intend to pay any cash dividends on our common stock in the foreseeable future. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

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, 2013, we had outstanding 17,929,781 shares of common stock. In addition, 2,114,683 shares of common stock that are either subject to outstanding options or reserved for future issuance under our equity plans 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 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

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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 25,000 square feet of office and laboratory space. The term of our current lease expires on September 30, 2018, with an option to extend the term for an additional five years.

We believe that our existing facilities are adequate for our current needs, as the facility has sufficient laboratory space to house additional scientists to be hired as we expand. When our lease expires, we may exercise our option to extend the term of the lease or we may look for additional or alternate space for our operations and we believe that suitable additional or alternative space will be available in the future on commercially reasonable terms.

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 each quarter in the year ended September 30, 2013:

	High	Low
Second Quarter (beginning March 21, 2013)	$ 18.20	$16.81
Third Quarter	$21.69	$16.49
Fourth Quarter	$25.00	$16.98

As of November 30, 2013, there were approximately 88 stockholders of record of our common stock.

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

In March 2013, we completed our initial public offering of 4,600,000 shares of our common stock at a public offering price of $14.00 per share. The offer and sale of the shares in the offering were registered pursuant to a registration statement on Form S-1 (File No. 333-184779), which was declared effective by the Securities and Exchange Commission on March 20, 2013.

As of September 30, 2013, we have used approximately $6.0 million of the net proceeds from the initial public offering to fund our programs for the development of a cyclophilin inhibitor candidate and the development of a

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nucleotide polymerase inhibitor candidate and to fund new research and development activities. None of the net proceeds have been paid directly or indirectly to any of our directors or officers (or their associates) or persons owning ten percent or more of any class of our equity securities or to any other affiliates. The balance of the net proceeds from the offering has been invested in cash and cash equivalents and in short-term and long-term marketable securities, consisting of investment grade, interest bearing instruments and U.S. government securities, with maturities of no longer than 24 months. These investments are reflected in cash and cash equivalents, short-term marketable securities and long-term marketable securities on our balance sheet at September 30, 2013. There has been no material change in the expected use of the net proceeds from our IPO as described in our registration statement on Form S-1.

Performance Graph[(1)]

The following graph shows a comparison from March 21, 2013 through September 30, 2013 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 SIX 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 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.

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ITEM 6. SELECTED CONSOLIDATED FINANCIAL DATA

We have derived the consolidated statement of operations data for the years ended September 30, 2013, 2012 and 2011 and the consolidated balance sheet data as of September 30, 2013 and 2012 from our audited financial statements included elsewhere in this Annual Report on Form 10-K. The selected statement of operations data for the year ended September 30, 2010 and the balance sheet data as of September 30, 2011 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.

	Year Ended September 30,			
	2013	2012	2011	2010
	(in thousands, except per share data)			
Consolidated Statement of Operations Data:				
Revenue	$ 32,053	$ 41,706	$ 41,882	$ 22,763
Operating expenses:				
Research and development	16,841	15,115	11,547	9,716
General and administrative	6,183	5,302	5,036	6,105
Total operating expenses	23,024	20,417	16,583	15,821
Income from operations	9,029	21,289	25,299	6,942
Other income (expense):				
Interest income	248	118	83	14
Interest expense	(31)		(3,161)	
Change in fair value of warrant liability	381	(8)	(686)	482
Therapeutic tax credit			750	
Gain on embedded derivative			670	
Other income (expense), net			355	309
Total other income (expense), net	598	110	(1,989)	805
Net income before income tax	9,627	21,399	23,310	7,747
Income tax benefit				157
Net income	9,627	21,399	23,310	7,904
Accretion of redeemable convertible preferred stock to redemption value	(2,526)	(5,367)	(5,454)	(5,452)
Net income attributable to participating securities	(13,670)	(14,663)	(16,291)	(2,236)
Net income (loss) attributable to common stockholders	$ (6,569)	$ 1,369	$ 1,565	$ 216
Net income (loss) per share attributable to common stockholders[(1)]:				
Basic	$ (0.67)	$ 1.26	$ 1.40	$ 0.19
Diluted	$ (0.67)	$ 1.13	$ 1.32	$ 0.18
Weighted average common shares outstanding[(1)]:				
Basic	9,788	1,089	1,119	1,131

Diluted	9,788	2,475	1,857	1,565

	As of September 30,		
	2013	2012	2011
		(in thousands)	
Consolidated Balance Sheet Data:			
Cash, cash equivalents and short- and long-term marketable securities	$ 112,183	$ 45,418	$ 23,329
Working capital[(2)]	100,187	41,574	22,950
Total assets	116,973	52,162	26,096
Warrant liability	1,620	2,001	1,993
Redeemable convertible preferred stock		158,955	153,588
Convertible preferred stock		327	327
Total stockholders equity (deficit)	110,468	(115,353)	(131,961)

(1) See Note 14 to our financial statements for further details on the calculation of basic and diluted net income per share attributable to common stockholders.
(2) We define working capital as current assets less current liabilities.

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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 Financial Data and our financial statements and related notes included elsewhere in this Annual Report on Form 10-K. This discussion and other parts of this prospectus 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 research and development-focused biotechnology company that uses its robust chemistry-driven approach and drug discovery capabilities to create small molecule drugs in the infectious disease field. We are discovering and developing inhibitors designed for use against the hepatitis C virus, referred to as HCV. We believe that a successful approach to a complete cure for HCV in most patients will likely require treatment with a combination of drugs that attack different mechanisms necessary for replication and survival of HCV. Further, as there are many variants of HCV, we are developing inhibitors that may be used in multiple combination therapies, each designed and tested for effectiveness against one or more of those variants. Our development of inhibitors for validated HCV target mechanisms, as well as our collaborations with AbbVie (which name refers to Abbott Laboratories for all periods before January 1, 2013) and Novartis, should allow us to participate in multiple unique drug combinations as we and our collaboration partners seek the best combination therapies for HCV in its various forms. We estimate that total worldwide sales of HCV therapies were over $4 billion in 2012. We believe that annual worldwide sales of these therapies and future approved therapies could increase sales in this market to $10 to $20 billion within the next ten years. In addition to our HCV programs, we have used our internal research capabilities to discover a new class of antibiotics which we are developing for the treatment of multi-drug resistant bacteria, including methicillin-resistant *Staphylococcus aureus* bacteria, also referred to as MRSA. We have utilized our internal chemistry and drug discovery capabilities and our collaborations to generate all of our development-stage programs, and we have active research efforts to broaden our infectious disease drug pipeline.

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The following table summarizes our product development pipeline in HCV antivirals as well as MRSA antibiotics:

Our HCV portfolio includes five compounds, each of which is an inhibitor of one of four fundamental, validated HCV targets:

NS3 Protease Inhibitors: ABT-450 and ABT-493. Our lead product candidate, ABT-450, is a protease inhibitor being developed by AbbVie in several combination regimens; AbbVie is completing several Phase 3 trials for the most advanced regimen, the first of which reported top-line results in November 2013, with the results of the other trials expected later in 2013 or the first quarter of calendar 2014. ABT-493, our next-generation protease inhibitor, is being developed also through our collaboration with AbbVie.

NS5A Inhibitor: EDP-239. Our lead NS5A product candidate, EDP-239, is being developed through our collaboration with Novartis.

Cyclophilin Inhibitors. Our independent research activities are focused on our lead cyclophilin inhibitor candidates, which are in preclinical development.

Nucleotide Polymerase Inhibitor. We also have a small-molecule drug discovery effort underway for nucleotide polymerase inhibitors.

In our HCV programs, AbbVie has funded all research and development of our protease inhibitors since we entered into the collaboration in November 2006, and is responsible for obtaining regulatory approvals and

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commercializing ABT-450, ABT-493 and any follow-on products worldwide. We received $57.2 million from AbbVie upon signing the collaboration agreement and its simultaneous purchase of preferred stock from us in 2006. We also received a $40.0 million milestone payment in December 2010 following AbbVie s successful completion of a Phase 2a clinical trial and a $15.0 million milestone payment in December 2012 following AbbVie s initiation of dosing in a Phase 3 clinical trial that includes ABT-450. Assuming AbbVie s successful development of the first protease inhibitor product, we will also be eligible to receive $195.0 million of additional pre-commercialization milestone payments. We are also eligible to receive additional milestone payments totaling up to $80.0 million upon AbbVie s achievement of similar commercial regulatory approval milestones for each additional collaboration product containing a protease inhibitor, as well as tiered royalties per product 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, on net sales, if any, by AbbVie allocable to the collaboration s protease inhibitors.

Under our collaboration with Novartis, which we entered into in February 2012, Novartis is responsible for all further development of our NS5A inhibitors. Novartis was also responsible for funding further research that we conducted through August 2013 to discover additional NS5A compounds. We received an upfront payment of $34.4 million in March 2012 and an $11.0 million milestone payment in January 2013 based on Novartis initiation of dosing in a Phase 1 clinical trial that included EDP 239. We are eligible to receive up to $395 million of additional milestone payments for the first NS5A inhibitor product for which the specified milestones are achieved.

Our independent research activities are focused on our lead cyclophilin candidates, which are in preclinical development. We also have a small-molecule drug discovery effort underway for nucleotide polymerase inhibitors. We are currently funding all research and development for these two programs, and we expect to incur substantially greater expenses as we seek to advance these programs into clinical development.

In addition to our HCV programs, we have used our internal research capabilities to discover a new class of antibiotics called Bicyclolides, which we are developing to overcome multi-drug resistant bacteria, including methicillin-resistant *Staphulococcus aureus* bacteria, also referred to as MRSA. Up to $23.5 million of the preclinical development of our lead antibiotic candidate, EDP-788, is funded under a September 2011 contract with the National Institute of Allergy and Infectious Diseases, a division of the National Institutes of Health, an agency of the United States Department of Health and Human Services, or NIAID, and there is potential for further NIAID funding of early clinical development. In August 2013 NIAID agreed to provide additional funding of $9.2 million under our contract, increasing total funding from NIAID to approximately $23.5 million.

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 infectious diseases. We have historically funded our operations primarily through the sale of convertible preferred stock and payments received under our collaborations and a government contract. On March 26, 2013, we completed our IPO of 4,600,000 shares of our common stock at an offering price of $14.00 per share, which included the exercise in full by the underwriters of their option to purchase up to 600,000 additional shares of our common stock. We received net proceeds of approximately $59.9 million, after deducting underwriting discounts and commissions. As of September 30, 2013, we had $112.2 million in cash and investments. We are eligible to receive over the next several years an aggregate of $430 million based on potential future pre-commercialization milestones under our AbbVie and Novartis collaborations, assuming successful clinical trial results for the respective collaboration programs and our collaborators continued development of our product candidates through successful regulatory and reimbursement approvals in the United States and other major markets. In addition, we are eligible to receive tiered royalties ranging on a blended basis from the low double digits up to the high teens on worldwide net sales of any products containing protease inhibitors or NS5A inhibitors developed pursuant to the collaborations, as well as up to $160 million of commercialization sales milestones under our Novartis collaboration.

Our revenue from our collaboration agreements has resulted in our reporting net income in fiscal 2013, 2012 and 2011. However, we had an accumulated deficit of $107.5 million as of September 30, 2013 and we have

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generated no royalties or other revenue from product sales. We expect that our revenue in the near term will continue to be substantially dependent on our collaborations with AbbVie and Novartis and their continued advancement of the related development programs. Given the schedule of potential milestone payments and the uncertain nature and timing of clinical development, we cannot predict when or whether we will receive further milestone payments under these collaborations or whether we will continue to report either revenue or net income in future years.

Financial Operations Overview

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 not generated any revenue from product sales. We have entered into three significant collaboration agreements. In November 2006, we entered into a collaboration agreement with AbbVie and in February 2012 we entered into a collaboration agreement with Novartis. In September 2011, we entered into a contract with NIAID, which will fund us for the preclinical development of our lead product candidate in our new class of Bicyclolide antibiotics.

The following table is a summary of revenue recognized from our collaboration agreements and our government contract for the years ended September 30, 2013, 2012 and 2011:

	Year Ended September 30,		
	2013	2012	2011
	(in thousands)		
AbbVie agreement:			
Upfront license payment and research funding	$	$	$ 1,882
Milestone payments	15,000		40,000
Novartis agreement:			
Upfront license payment and research funding	1,675	35,567	
Milestone payments	11,000		
NIAID contract	4,378	6,139	
Total revenue	$ 32,053	$ 41,706	$ 41,882

AbbVie Agreement

Under the terms of the AbbVie agreement, as amended, we received an upfront license payment of $44.7 million and a commitment for research funding through December 15, 2010, and we granted AbbVie an option to enter into a six-month evaluation period. We received a total of $8.1 million of research funding and expense reimbursement from AbbVie through June 15, 2011, the conclusion of the evaluation period. In December 2010, we received a $40.0 million milestone payment from AbbVie related to AbbVie s successful completion of a Phase 2a clinical study of an ABT-450-containing regimen. We recognized revenue from these payments, as well as from a $1.6 million premium above fair value paid for Series G-1 redeemable convertible preferred stock that AbbVie purchased concurrently with the execution of the original agreement, over the period from the date of the original agreement through the end of the evaluation period using the proportional performance model. Under this revenue recognition model, the revenue we recognized was limited to the amount of nonrefundable payments received or receivable to date. Related to these payments by AbbVie, we recognized revenue of $41.9 million during the year ended September 30, 2011. Since all of

our research obligations under the agreement were concluded by June 30, 2011, any future milestone payments received will be recognized as revenue when each milestone is achieved by AbbVie. During the year ended September 30, 2013, we earned and recognized as revenue a $15.0 million milestone payment based on AbbVie s initiation of dosing in a Phase 3 clinical trial that included ABT-450. Under the terms of the AbbVie agreement, we are eligible to receive future milestone payments totaling up to $195 million related to the successful development of the first HCV treatment regimen

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by AbbVie incorporating one of our collaboration s protease inhibitors. We are also eligible to receive additional milestone payments totaling up to $80.0 million upon AbbVie s achievement of similar commercial regulatory approval milestones for each additional collaboration product containing a protease inhibitor. We are also eligible to receive royalties on AbbVie s net sales, if any, allocable to any one of our collaboration s protease inhibitors.

Novartis Agreement

Under the terms of the Novartis agreement, we received an upfront payment of $34.4 million and a commitment to fund research at an agreed amount for one year. We recognized the upfront license payment upon receipt as we determined that the license to which the payment related and the research services were separable elements under the agreement that could be accounted for as each was delivered or provided. During the year ended September 30, 2012, revenue recognized under this agreement was $35.6 million, which consisted of the upfront license payment and research funding earned during that period. Our agreement with Novartis initially provided that we would receive up to $1.8 million in research funding during the first year of the agreement, which ended in February 2013, and was amended to extend the funding period for an additional six months through August 2013 at the same reimbursement rate. Additionally, our collaboration with Novartis provides for future milestone payments totaling of up to $406 million if certain goals related to drug development and net product sales are achieved by Novartis. In January 2013, we received an $11.0 million milestone payment based on Novartis November 2012 initiation of dosing in a Phase 1 clinical trial that included EDP-239. During the year ended September 30, 2013, we recognized $12.7 million of revenue under the Novartis agreement, of which $11.0 million was attributed to license fees and $1.7 million was attributed to the performance of research services. An additional milestone payment of $15.0 million will be due upon Novartis initiation of a subsequent Phase 2 trial using a combination treatment regimen containing an NS5A inhibitor. We are also eligible to receive royalties on Novartis net sales, if any, allocable to our collaboration s NS5A inhibitors.

NIAID Contract

Under the terms of the NIAID contract, NIAID will pay us research and development funding of up to $14.3 million over an initial period of 30 months. The award also contains six option periods, which in aggregate could extend the contract at the option of NIAID up to an additional 30 months and provide us additional funding of up to $28.4 million. In August 2013 NIAID agreed to provide additional funding of $9.2 million under our contract, which will bring total funding from NIAID to approximately $23.5 million. We recognize revenue under this contract as the research and development services are performed. We recognized revenue of $4.4 million and $6.1 million under this agreement during the years ended September 30, 2013 and 2012, respectively.

As our internal product candidates are currently in preclinical development, we have not generated any revenue from product sales and do not expect to generate any revenue from product sales for at least the next several years. We expect that our revenue for the next several years will be derived primarily from payments under our current collaboration agreements with AbbVie and Novartis, payments under our NIAID contract, and any additional collaborations or government contracts that we may enter into in the future. We cannot provide assurance as to the timing of future milestone or royalty payments or that we will receive any of these payments at all.

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Operating Expenses

The following table summarizes our operating expenses for the years ended September 30, 2013, 2012 and 2011:

	Year Ended September 30,		
	2013	2012	2011
	(in thousands)		
Research and development	$ 16,841	$ 15,115	$ 11,547
General and administrative	6,183	5,302	5,036
Total operating expenses	$ 23,024	$ 20,417	$ 16,583

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. 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 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 maintain 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 increase in the future as we advance our two independent HCV programs and our antibiotic program for MRSA into clinical development.

Our research and drug discovery programs are at an early stage; 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 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, consisting of salaries, related benefits and stock-based compensation, of our executive, finance, business and corporate development and other

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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, including legal expenses to pursue patent protection of our intellectual property.

We expect that general and administrative expenses will increase in the future as we expand our operating activities and incur additional costs associated with operating as a public company. These public company related increases will likely include costs of additional personnel; additional legal fees, accounting and audit fees and directors and officers liability insurance premiums; and costs related to investor relations.

Other Income (Expense)

Interest income. Interest income consists of interest earned on our cash and investment balances. In the past our interest income has not been significant due to nominal cash and investment balances and low interest earned on invested balances. We anticipate that our interest income will increase in the future due to our higher cash and investment balances now existing as a result of the $34.4 million upfront payment we received from Novartis in March 2012, the $15.0 million milestone payment we received from AbbVie in December 2012 and the $11.0 million milestone payment we received from Novartis in January 2013, as well as our receipt of $59.9 million of cash proceeds, net of underwriting discounts and commissions, from our IPO in March 2013.

Interest expense. Interest expense consisted of cash interest paid on our bridge notes and non-cash interest expense related to the accretion of debt issuance costs and debt discounts associated with our issuance of bridge notes in the first quarter of fiscal 2011. We anticipate that we will have little or no interest expense in the future related to debt as our outstanding bridge notes were fully repaid in the first quarter of fiscal 2011 and we no longer have any debt outstanding. Presently interest expense consists of non-cash interest expense which is being accreted to the value of accrued third-party license fees over the term of the obligation.

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

Therapeutic tax credit. We recorded other income for the year ended September 30, 2011 related to the Qualifying Therapeutic Discovery Project, or QTDP, reimbursement program of the United States government, which provided for reimbursement in calendar year 2010 of certain costs paid or incurred during calendar years 2009 and 2010 that were directly related to the conduct of a QTDP. We do not anticipate any further income related to the QTDP program.

Gain on embedded derivative. In connection with the repayment of our bridge financing that we entered into and fully repaid in the first quarter of fiscal 2011, we settled an embedded derivative at no cost to us and recorded a gain on settlement consisting of the value of the embedded derivative.

Other income (expense), net. Other income (expense), net consisted primarily of miscellaneous service income unrelated to our core operations. We do not expect to generate this income in the future as we do not anticipate providing these services in the future.

Critical Accounting Policies

Our financial statements are prepared in accordance with accounting principles generally accepted in the United States of America. The preparation of our 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

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related disclosures. We believe that the estimates and assumptions involved in the accounting policies described below may have the greatest potential impact on our financial statements and, therefore, consider these to be our critical accounting policies. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions and conditions. See also Note 2 of our 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.

JOBS Act

On April 5, 2012, the JOBS Act was signed into law. The JOBS Act 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 disclosure 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. These exemptions will apply for a period of five years following the completion of our initial public offering in March 2013 or until we no longer meet the requirements of being an emerging growth company, whichever is earlier.

Revenue Recognition

Our revenue is 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 we have fulfilled our performance obligations under the contract.

We apply Accounting Standards Update No. 2009-13, *Multiple-Deliverable Revenue Arrangements*, or ASU 2009-13 for multiple element arrangements entered into or materially modified on or after October 1, 2011. The selling prices of deliverables under the arrangement may be derived using third-party evidence, or TPE, or a best estimate of selling price, or BESP, if vendor-specific objective evidence of fair value, or 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 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. 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 the arrangement are separate units of accounting if (i) the delivered item has value to the customer on a standalone

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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. 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. We may exercise significant judgment in determining whether a deliverable is a separate unit of accounting.

In February 2012, we entered into a license and collaboration agreement with Novartis for the development, manufacture, and commercialization of our lead development candidate, EDP-239, from our NS5A inhibitor program for HCV. Under the terms of the Novartis agreement, Novartis agreed to pay us a nonrefundable upfront fee and reimbursement of manufacturing and quality assurance expenses related to EDP-239 totaling $34.4 million. In addition, Novartis agreed to fund up to $1.8 million of our NS5A research activities through February 2013. In March 2013 the agreement was amended to extend the funding period for an additional six months through August 2013 at the same reimbursement rate. Under the agreement, we are eligible to receive aggregate milestone payments of up to $406 million for the first NS5A inhibitor product for which applicable milestones relating to clinical trials, regulatory approvals, and net sales are achieved by Novartis. We are also eligible to receive tiered royalties ranging on a blended basis from the low double digits up to the high teens on net product sales by Novartis, if any, allocable to our collaboration s NS5A inhibitors.

We determined that the deliverables under the Novartis agreement include the exclusive, royalty-bearing, sublicensable license to EDP-239 and the research services. We concluded that the EDP-239 license had standalone value to Novartis and was separable from the research services because the license is sublicensable, there are no restrictions as to Novartis use of the license, and Novartis has the requisite scientific expertise in the HCV NS5A field. We also concluded that participation on a joint steering committee, as provided for by the agreement, is protective in nature as we have no decision making authority, there are no penalties or recourse if we choose not to participate, and the purpose of the steering committee is to keep us apprised of the status of the development and commercialization efforts. Therefore, no arrangement consideration was allocated to the joint steering committee participation. We were not able to establish VSOE or TPE for either the license or the research services and instead allocated the arrangement consideration between the license and research services based on their relative selling prices using BESP. We developed our estimate of BESP of the license using a discounted cash flow analysis, taking into consideration assumptions including the development and commercialization timeline, discount rate, probability of success, and probable treatment combination and associated peak sales figures which generate royalty amounts. The funding rate for the research services is consistent with the rate received in our prior collaboration arrangement with AbbVie and is consistent with its fully burdened cost of service. Therefore, our determination of BESP for the research services is consistent with the reimbursement rate stated in the contract.

In determining our best estimate of selling price, we considered discounted cash flow models. Our key assumptions in these models included the following market conditions and entity-specific factors: (a) the specific rights provided under the license to develop and commercialize EDP-239 worldwide, (b) the stage of development of EDP-239 and estimated remaining development and commercialization timelines, (c) the probability of successfully developing and commercializing EDP-239, (d) the probable treatment combination, (e) the market size for EDP-239 including the associated sales figures which generate royalty revenue, (f) the expected product life of EDP-239 assuming commercialization, and (g) the competitive environment. We assumed that royalties from sales of EDP-239 would be based on a drug compound that will be part of a triple combination drug therapy. The time to commercialization was based on our estimates, which projected the first sales of EDP-239 in 2018. We utilized a discount rate of 15% in our analysis, representing the weighted average cost of capital derived from returns on equity for comparable companies.

These assumptions involve judgment and inherent uncertainty; however, significant changes in key assumptions used to determine the BESP would not have a significant effect on the revenue recognized.

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Stock-Based Compensation

We measure stock options granted to employees and directors at fair value on the date of grant and recognize the corresponding compensation expense of those awards, net of estimated forfeitures, over the requisite service period, which is generally the vesting period of the respective award. Generally, we issue stock options with only service-based vesting conditions and record the expense for these options using the straight-line method. We have historically granted stock options with exercise prices equivalent to the fair value of our common stock as of 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. Prior to March 2013 we were a privately-held company and lacked company-specific historical and implied volatility information. Therefore, we estimated our expected volatility based on the historical volatility of our publicly traded peer companies and expect to continue to do so 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. 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. Expected dividend yield 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. The assumptions we used to determine the fair value of stock options granted in each period were as follows, presented on a weighted average basis:

	Year Ended September 30,		
	2013	2012	2011
Risk-free interest rate	1.05%	0.93%	2.73%
Expected term (in years)	6.09	6.00	6.25
Expected volatility	73%	78%	87%
Expected dividends	0%	0%	0%

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. We recognize 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. 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.

Valuation of Warrants to Purchase Series 1 Preferred Stock

We classify warrants to purchase 1,999,989 shares of our Series 1 nonconvertible preferred stock as liabilities on our balance sheets as these warrants are free-standing financial instruments that may require us to transfer assets upon exercise. The warrants were initially recorded at fair value and are subsequently remeasured to fair value at each balance sheet date using probability-weighted discounted cash flow. Changes in fair value of the warrants are recognized as a component of other income (expense) in our statement of operations. We will continue to adjust the liability for changes in fair value until the earlier of the exercise or expiration of the warrants.

These warrants issued in October 2010 in connection with a bridge note financing entitled the note holders to purchase shares of Series 1 nonconvertible preferred at an exercise price of $0.01 per share. Upon issuance of the warrants, the number of Series 1 nonconvertible preferred shares issuable upon exercise of these warrants was not fixed. The

number of Series 1 nonconvertible preferred shares was one share for each dollar of the original principal amount of the term note plus, if the milestone payment from the AbbVie agreement was not received on or before March 31, 2011, an additional share for each dollar of the original principal amount of the note. Additionally, if a liquidation event occurred, thereby requiring repayment of the term notes, these warrants would

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automatically expire and would therefore have no value. Upon our repayment of the term notes in December 2010, the number of shares issuable upon exercise of the Series 1 nonconvertible preferred stock warrants became fixed at one share for each warrant, and the possibility that the term notes could be redeemed and the warrants would have no value was eliminated.

As of September 30, 2013, the warrant liability consists solely of the warrants for the purchase of Series 1 nonconvertible preferred stock. As of September 30, 2013, the Company utilized a probability weighted valuation model which takes into consideration various outcomes that may require the Company to transfer assets upon exercise. The fair value of these outstanding warrants to purchase our Series 1 nonconvertible preferred stock as recorded in our balance sheet was $1.6 million and $2.0 million at September 30, 2013 and 2012, respectively. The Series E warrants expired during the year ended September 30, 2013.

Income Taxes

Income taxes are provided for tax effects of transactions reported in the 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 purposes and for income tax reporting purposes. Deferred taxes are determined based on the difference between the financial statement value and tax basis of assets and liabilities using enacted tax rates in effect in the years in which the differences are expected to reverse. 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. Based on our analysis of both positive and negative factors, we have determined that it is more likely than not that we will not be able to realize our deferred tax assets, and therefore we have recorded a full valuation allowance against our deferred tax assets. Our analysis included an assessment of our past profitability and our future projections of forecasted revenue and expense levels. More specifically, we considered the following factors in determining that it is more likely than not that we will not be able to realize our deferred tax assets:

- We have incurred cumulative net losses since our inception, and as of September 30, 2013 we had an accumulated deficit of $107.5 million. We expect that we may incur substantial operating losses in the future. Our net income for the year ended September 30, 2013 resulted primarily from milestone payments we received from AbbVie and Novartis of $15.0 million and $11.0 million, respectively. Our net income in the fiscal year ended September 30, 2012 resulted primarily from an upfront payment of $34.4 million from our collaborator Novartis. Our net income in the fiscal year ended September 30, 2011 resulted primarily from a milestone payment from our collaborator AbbVie;

- We are a clinical-stage biopharmaceutical company and we have not yet demonstrated an ability to successfully overcome many of the risks and uncertainties associated with late stage clinical development, regulatory approval and commercialization of therapeutic products;

- To date, we have not commercialized any products or generated any revenue from product sales, directly or through any collaborator;

- Our financial prospects and ability to generate revenue for the next several years are substantially dependent upon the development and marketing efforts of AbbVie and Novartis for our drug product candidates, and

we have limited control over the resources, time and effort that our collaborators may devote to our drug product candidates;

Because the outcome of planned and anticipated clinical trials of our product candidates by our collaborators is highly uncertain, we cannot reasonably estimate the timing or amounts, if any, of future milestone payments we will receive from these collaborators;

Our own independent HCV development programs and antibiotic program are in preclinical development and we will be required to invest significant capital and incur significant additional research and development expenses to develop and commercialize these compounds;

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Since our inception, we have not paid a material amount of U.S. federal income taxes; and

We do not have any taxable income in prior carryback periods or any taxable temporary differences which could represent a source of taxable income upon future reversal.

We account for uncertainty in income taxes recognized in the 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. 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 recognize 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.

Results of Operations

Comparison of Years Ended September 30, 2013, 2012 and 2011

	Year Ended September 30,		
	2013	2012	2011
	(in thousands)		
Revenue	$ 32,053	$ 41,706	$ 41,882
Research and development expenses	16,841	15,115	11,547
General and administrative expenses	6,183	5,302	5,036
Other income (expense):			
Interest income	248	118	83
Interest expense	(31)		(3,161)
Change in fair value of warrant liability	381	(8)	(686)
Therapeutic tax credit			750
Gain on embedded derivative			670
Other income			355

Revenue. We recognized revenue of $32.1 million in the year ended September 30, 2013, as compared to $41.7 million in the year ended September 30, 2012. During fiscal 2013, we received and recognized as revenue a $15.0 million milestone payment under our collaboration with AbbVie based on AbbVie s initiation of dosing in a Phase 3 clinical trial that includes ABT-450. We did not have any revenue related to the AbbVie collaboration during fiscal 2012. During the year ended September 30, 2013 we also recognized revenue of $12.7 million under our collaboration with Novartis, as a result of an $11.0 million milestone payment we received in January 2013 based on Novartis initiation of dosing in a Phase 1 clinical trial that includes EDP-239, and $1.7 million related to research services we provided. During the comparable period in 2012, Novartis paid us an upfront payment of $34.4 million and $1.1 million related to research services we provided, which we recognized as revenue during the period. Government contract revenue was $4.4 million and $6.1 million during the years ended September 30, 2013 and 2012, respectively, related to our contract with NIAID for the EDP-788 program.

We recognized revenue of $41.7 million during the year ended September 30, 2012, as compared to $41.9 million during the year ended September 30, 2011. In fiscal 2012, Novartis paid us an upfront payment of $34.4 million from Novartis, which we recognized as revenue during the year ended September 30, 2012. We also recognized $1.1 million related to research funding from Novartis. Government contract revenue was $6.1 million in the year ended September 30, 2012 for the EDP-788 program related to the contract with NIAID. We did not have any government

contract revenue in fiscal 2011. In fiscal 2011, upon AbbVie s successful completion of a Phase 2a clinical study, we received a milestone payment of $40.0 million which we recognized as revenue during the year based on our completion of our deliverables under the AbbVie Agreement. We also recorded $1.9 million of revenue related to research funding received from AbbVie during the year ended September 30, 2011.

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Research and development expenses.

	Year Ended September 30,		
	2013	2012	2011
	(in thousands)		
Development programs:			
Protease inhibitor	$	$	$ 1,109
NS5A inhibitor	1,717	2,993	6,097
Antibiotic	3,186	4,127	
Research and drug discovery	11,938	7,995	4,341
Total research and development expenses	$ 16,841	$ 15,115	$ 11,547

Research and development expenses were $16.8 million in the year ended September 30, 2013, as compared to $15.1 million for the same period in 2012. The increase year over year was due primarily to an increase of $3.9 million in preclinical expenses for our early stage drug discovery programs. This increase was partially offset by a decrease of $1.3 million in expenses for our NS5A inhibitor program and a decrease of $0.9 million in our expenses for our antibiotic program. In fiscal 2013 we incurred higher research expenses in our early stage drug discovery programs due to an increase in both the number of preclinical studies and the related costs. Our research costs related to our NS5A inhibitor program decreased as a result of our signing a collaboration agreement with Novartis in February 2012, at which time Novartis became responsible for further clinical trial costs. Prior to entering into the collaboration agreement with Novartis, we had been incurring costs for preclinical and clinical development of EDP-239. We continued to incur research expense for NS5A research to identify additional compounds through August 2013, for which we had been receiving funding from Novartis through August 2013. We incurred preclinical expense for the development of EDP-788 as a result of the contract we entered into in September 2011 with NIAID, which is funding our research program for EDP-788.

Research and development expenses were $15.1 million for the year ended September 30, 2012 as compared to $11.5 million for the year ended September 30, 2011. The increase year over year was due primarily to an increase of $4.1 million of preclinical expenses for our antibiotic program, specifically the development of EDP-788, for which we had no costs in 2011 as well as an increase in our early stage drug discovery programs of $3.7 million. These increases were partially offset by a decrease of $1.1 million in our expenses for our protease inhibitor program and a decrease of $3.1 million in expenses for our NS5A inhibitor program. We incurred preclinical expense for the development of EDP-788 as a result of the contract we entered into in September 2011 with NIAID, which is funding our research program for EDP-788. We incurred increased research expenses in our early stage drug discovery programs due to an increase in both the number of preclinical studies and the related costs as well as a $0.9 million expense for the cost of a third-party license for intellectual property used in our research activities. Our research costs related to our NS5A inhibitor program decreased as a result of our signing a collaboration agreement with Novartis in February 2012, at which time Novartis became responsible for further clinical trial costs. Prior to entering into the collaboration agreement with Novartis, we had been incurring costs for preclinical and clinical development of EDP-239. We continue to incur research expense for NS5A research to identify additional compounds, for which we were receiving funding from Novartis through August 2013. Our research costs related to our protease inhibitor program decreased as a result of the conclusion of our research program with AbbVie in June 2011.

From inception of each development program through September 30, 2013, we incurred cumulative expenses of $18.7 million for our protease inhibitor program, $13.2 million for our NS5A inhibitor program, and $7.3 million for our

EDP-788 antibiotic program.

General and administrative expenses. General and administrative expenses increased by $0.9 million from $5.3 million in fiscal 2012 to $6.2 million in fiscal 2013. The increase was primarily due to increased stock-based compensation expense of $0.6 million related to additional employee stock options and a higher value of our common stock, as well as additional expenses incurred as a result of operating as a publicly traded company.

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General and administrative expenses increased by $0.3 million from $5.0 million in fiscal 2011 to $5.3 million in fiscal 2012. The increase was primarily due to increased stock-based compensation expense, as a result of additional stock option grants to employees and a higher value of our common stock, as well as higher accounting and audit fees, partially offset by lower facility costs as a result of our move to a new office location on October 1, 2011.

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

Interest income. The increase in interest income for the year ended September 30, 2013, as compared to the year ended September 30, 2012, was due to higher average cash and investment balances following the receipt of the $15.0 million milestone payment received from AbbVie and the $11.0 million milestone payment received from Novartis in fiscal 2013, as well as our receipt of $59.9 million of cash proceeds, net of underwriting discounts and commissions, from our IPO in March 2013.

The increase in interest income for the year ended September 30, 2012, as compared to the year ended September 30, 2011, was due to higher average cash and investment balances primarily due to the receipt of the $34.4 million upfront payment from Novartis in the second quarter of fiscal 2012.

Interest expense. In fiscal 2013, interest expense consisted of non-cash interest expense which is being accreted to the value of accrued third-party license fees over the term of the obligation.

Interest expense of $3.2 million for the year ended September 30, 2011 related entirely to our bridge financing in October and November 2010, under which we borrowed $2.0 million from existing investors. In connection with the convertible note agreement for this financing, we issued warrants for the purchase of our Series 1 nonconvertible preferred stock, which were initially valued at $1.3 million and recorded as a debt discount. The convertible note agreement included call and put options that constituted an embedded derivative valued at $0.7 million, which was also recorded as a debt discount. We incurred issuance costs of $0.2 million, which were recorded as deferred financing costs. In December 2010, we repaid the $2.0 million of principal outstanding plus interest and an applicable premium of $1.0 million to the note holders upon receipt of a $40.0 million milestone payment from AbbVie. Upon repayment, we accreted the debt discounts and deferred financing costs to interest expense and recorded the premium as interest expense. We had no outstanding debt and therefore no related interest expense for the year ended September 30, 2013 or 2012.

Change in fair value of warrant liability. We account for any outstanding warrants for our Series E redeemable convertible preferred stock and our Series 1 nonconvertible preferred stock as liabilities held at fair value, with changes in fair value recorded as a component of other income (expense).

During the year ended September 30, 2013, we recorded a small amount of income due to the expiration of our series E warrants during the year, as well as a $0.4 million decrease in the fair value of our warrant liability due to the remeasurement of the fair value of the outstanding warrants for Series 1 nonconvertible preferred stock.

During the year ended September 30, 2012, we recorded a small amount of expense related to the increase in the fair value of our warrant liability as of September 30, 2012 primarily as a result of the remeasurement of our Series E warrants at the reporting date utilizing the Black-Scholes option-pricing model.

We recorded expense related to the increase in the fair value of our warrant liability for the year ended September 30, 2011 of $0.7 million due primarily to the remeasurement of the fair value of our Series 1 nonconvertible preferred stock warrants, which increased in value primarily due to the resolution of certain contingencies under the terms of the warrants.

Therapeutic tax credit. We recognized income from a therapeutic tax credit of $0.8 million for the year ended September 30, 2011 under the QTDP program, which provided for reimbursement in calendar year 2010 of

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certain costs we paid or incurred during calendar years 2009 and 2010 directly related to the conduct of a QTDP. We did not receive any such reimbursements during fiscal 2013 or 2012.

Gain on embedded derivative. We recorded a gain of $0.7 million on an embedded derivative for the year ended September 30, 2011 related to a derivative liability embedded in our convertible note agreement that was settled upon repayment of the notes in December 2010. We had no comparable item for either of the years ended September 30, 2013 or 2012.

Other income (expense), net. Other income in fiscal 2011 consisted primarily of miscellaneous service income unrelated to our core operations. Commencing in fiscal 2012, we no longer provided these services.

Liquidity and Capital Resources

From our inception through September 30, 2013, we have financed our operations primarily through contract payments under our collaborations, private placements of our equity, government research and development contracts and grants and our IPO that was completed in March 2013 for which we received $59.9 million, net of underwriting discounts and commissions. As of September 30, 2013, we had $112.2 million in cash, cash equivalents and short- and long-term marketable securities.

The following table shows a summary of our cash flows for each of the years ended September 30, 2013, 2012 and 2011.

	Year Ended September 30,		
	2013	2012	2011
	(in thousands)		
Cash provided by (used in):			
Operating activities	$ 10,653	$ 22,623	$ 24,019
Investing activities	$ (69,216)	$ (18,040)	$ (17,682)
Financing activities	$ 56,911	$ (909)	$ 34

Net cash provided by (used in) operating activities

During the year ended September 30, 2013, operating activities provided $10.7 million of cash. The cash flow provided by operating activities primarily resulted from our net income of $9.6 million, as well as from net non-cash charges of $0.3 million and $0.7 million from changes in our operating assets and liabilities, respectively. Our net income in fiscal 2013 was primarily due to $32.1 million of revenue recognized upon receipt of a $15.0 million milestone payment earned under our collaboration agreement with AbbVie and an $11.0 million milestone payment earned under our collaboration with Novartis, and $4.4 million recognized under our government contract with NIAID, net of our operating expenses. Our net non-cash charges in the period primarily consisted of $1.1 million of stock-based compensation expense and $1.3 million related to amortization of the premium on our marketable securities, offset in part by $1.8 million of purchased premium on marketable securities. Net sources of cash from changes in our operating assets and liabilities during the year ended September 30, 2013 consisted primarily of a $1.4 million decrease in accounts receivable and unbilled receivables and $0.1 increase in accounts payable, which were offset in part by $0.3 million increase in prepaid expenses and other current assets and $0.3 decrease in accrued expenses. Cash provided by the $1.4 million decrease in accounts receivable was primarily due to the timing of our billings and collections under the NIAID contract. The $0.2 million net use of cash from changes in accounts payable and accrued expenses was primarily due to the timing of payments made by us to vendors.

During the year ended September 30, 2012, operating activities provided $22.6 million of cash. The cash flow provided by operating activities primarily resulted from our net income of $21.4 million and net non-cash charges of $1.1 million, together partially offset by net uses of cash of $0.1 million from changes in our operating assets and liabilities. Our net income in the period was primarily due to $35.6 million of revenue recognized

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related to the upfront payment and research funding we received under our collaboration arrangement with Novartis as well as $6.1 million of revenue recognized from the NIAID contract, both offset by our operating expenses. Our net non-cash charges in the period primarily consisted of $0.6 million of amortization of the premium on our marketable securities and $0.4 million of stock-based compensation expense. Net uses of cash from changes in our operating assets and liabilities during the year ended September 30, 2012 consisted primarily of a $0.8 million and $1.9 million increase in our accounts receivable and unbilled receivables, respectively, principally related to our collaboration agreements with NIAID and Novartis, as well as an increase of $0.2 million in our prepaid expenses and other current assets. These amounts were partially offset by a $2.5 million increase in accounts payable and accrued expenses and a $0.5 million increase in other long-term liabilities. Our accounts payable, accrued expense and other long-term liabilities balances were affected by the timing of payments made by us to our vendors and additionally reflected a $1.0 million payable for the cost of a third-party license used in our research activities.

During the year ended September 30, 2011, operating activities provided $24.0 million of cash. The cash flow provided by operating activities primarily resulted from our net income of $23.3 million and net non-cash charges of $3.1 million, together partially offset by net uses of cash of $2.4 million from changes in our operating assets and liabilities. Our net income was primarily due to $41.9 million of revenue recognized related to the milestone payment we received under our collaboration agreement with AbbVie during fiscal 2011, offset by our operating expenses. Our net non-cash charges in the year primarily consisted of $2.1 million of non-cash interest expense, a $0.7 million expense from the increase in the fair value of warrants and $0.5 million of depreciation and amortization expense, offset by a $0.7 million non-cash gain from settlement of an embedded derivative. Non-cash interest expense was primarily due to the accretion of debt discounts and deferred financing costs recorded upon repayment of our bridge financing notes in October and November 2010. Net uses of cash from changes in our operating assets and liabilities during the year ended September 30, 2011 consisted primarily of a $1.1 million decrease in accrued expenses, a $0.4 million decrease in deferred revenue, a $0.4 million decrease in accrued rent and a $0.3 million increase in prepaid expenses and other assets. The aggregate $1.8 million net use of cash from changes in accrued expenses, accrued rent, and prepaid expenses and other assets was primarily due to the timing of payments made by us to vendors. The decrease of $0.4 million in deferred revenue was the result of revenue we had deferred as of the end of fiscal 2010 that we recognized upon the completion of our obligations under our collaboration agreement with AbbVie during fiscal 2011.

Net cash provided by (used in) investing activities

During the year ended September 30, 2013, net cash used by investing activities was $69.2 million. Net cash used by investing activities during this period consisted primarily of cash paid for the purchases of marketable securities of $115.9 million and purchases of laboratory equipment of $0.6 million, which was partially offset by cash received from the sales and maturities of marketable securities of $47.3 million.

During the year ended September 30, 2012, net cash used in investing activities was $18.0 million. Net cash used in investing activities during this period consisted primarily of purchases of marketable securities, which used cash of $47.7 million, partially offset by cash received from the sale and maturities of marketable securities of $28.7 million and an increase in cash of $1.1 million due to a release of a letter of credit in December 2011 related to the previous lease of our Watertown facility.

During the year ended September 30, 2011, net cash used in investing activities was $17.7 million. Net cash used in investing activities in the year consisted primarily of purchases of marketable securities, which used cash of $33.6 million, and purchases of $0.4 million of laboratory equipment, partially offset by cash received from the sale of marketable securities of $16.8 million.

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Net cash provided by (used in) financing activities

Net cash provided by financing activities during the year ended September 30, 2013 was $56.9 million and consisted of $59.9 million of proceeds from our IPO, net of underwriting discounts and commissions, and proceeds of $0.6 million from the exercise of stock options, offset by $3.5 million of expenses related to our IPO and paid during the period.

Net cash used in financing activities for fiscal 2012 consisted of payments of deferred offering costs in anticipation of our initial public offering, partially offset by proceeds received from the exercise of stock options.

Net cash provided by financing activities for fiscal 2011 primarily related to the exercise of stock options. In addition, during the first quarter of fiscal 2011, we entered into a bridge note financing arrangement with existing investors, under which we borrowed $2.0 million. We repaid that amount in full within the same quarter.

As of September 30, 2013, we had $112.2 million in cash, cash equivalents and investments. We believe that our existing cash, cash equivalents and investments as of September 30, 2013, will be sufficient to meet our anticipated cash requirements for at least the next 24 months. However, our forecast of the period of time through which 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 collaborations continue to generate substantial milestone payments, and ultimately royalties, to us;
- the number and characteristics of the future product candidates we pursue;
- the scope, progress, results and costs of researching and developing any of our future product candidates on our own, including conducting preclinical research and clinical trials;
- the timing of, and the costs involved in, obtaining regulatory approvals for any future product candidates we develop independently;
- the cost of commercialization activities, if any, of any future product candidates we develop independently that are approved for sale, including marketing, sales and distribution costs;
- the cost of manufacturing our future product candidates and any products we successfully commercialize independently, including manufacturing for clinical development;

our ability to maintain existing collaborations and to establish new collaborations, licensing or other arrangements and the financial terms of such agreements;

the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing patents, including litigation costs and the outcome of such litigation; and

the timing, receipt and amount of sales of, or royalties on, ABT-450, EDP-239 and our future product candidates, if any.

We do not believe that inflation had a material effect on our business, financial condition or results of operations in the last two fiscal years. If our costs were to become subject to significant inflationary pressures, we could not offset such higher costs through revenue increases. 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.

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Recently Issued Accounting Pronouncements

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 our financial statements upon adoption.

Contractual Obligations and Commitments

We lease office space in Watertown, Massachusetts under a seven-year lease that commenced on October 1, 2011. In fiscal 2012, we entered into an intellectual property license agreement that will require us to make certain non-cancelable payments over the next three years. The following table summarizes our contractual obligations at September 30, 2013 and the effect such obligations are expected to have on our liquidity and cash flow in future periods:

	Payments Due By Fiscal Year				
	Total	Less Than 1 Year	1-3 Years	3-5 Years	More Than 5 years
			(in thousands)		
Operating lease commitments	$ 4,865	$ 921	$ 1,918	$ 2,026	$
Intellectual property license	450	250	200		
Total[(1)]	$ 5,315	$ 1,171	$ 2,118	$ 2,026	$

(1) As of September 30, 2013, we had outstanding warrants for the purchase of 1,999,989 shares of our Series 1 nonconvertible preferred stock that we classified as a long-term liability on our balance sheet, recorded at fair value of $1.6 million. If those warrants are exercised, the Series 1 nonconvertible preferred stock issued upon exercise would require the payment of $2.0 million upon a qualifying merger or sale of this 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 DISCLOSURE ABOUT MARKET RISK

Interest Rate Sensitivity

We had cash, cash equivalents and marketable securities of $112.2 million at September 30, 2013, which consisted of cash, government and agency securities, corporate bonds and commercial paper and certificates of deposit. Interest income is sensitive to changes in the general level of interest rates; however, due to the nature of these investments, we do not believe that we have any material exposure to changes in the fair value of our investment portfolio as a result of changes in interest rates. We had no debt outstanding as of September 30, 2013.

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-37 of this Annual Report on Form 10-K.

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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 Company s 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, 2013, 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, 2013. These conclusions were communicated to the Audit Committee.

Management s Report on Internal Control Over Financial Reporting

This Annual Report on Form 10-K does not include a report of management s assessment regarding internal control over financial reporting or an attestation report of the Company s independent registered public accounting firm due to a transition period established by rules of the Securities and Exchange Commission for newly public companies. In addition, 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 a 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 fiscal quarter that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

ITEM 9B. OTHER INFORMATION

None.

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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 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 Definitive Proxy Statement relating to the 2014 Annual Meeting of Stockholders (the 2014 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 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 2014 Proxy Statement: Executive Compensation and Corporate Governance Compensation Committee Interlocks and Insider Participation.

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 2014 Proxy Statement.

The following table provides information about the securities authorized for issuance under the Company s equity compensation plans as of September 30, 2013:

Equity Compensation Plan 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)
	1,583,031[2]	$ 5.15	531,652[3]

Equity compensation plans approved by security holders[1]			
Equity compensation plans not approved by security holders			
Totals	1,583,031[2]	$ 5.15	531,652[3]

(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.

(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. Does not include 538,102 shares that were automatically added to the Company s 2012 Equity Incentive Plan by its terms as of October 1, 2013.

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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 2014 Proxy Statement.

ITEM 14. PRINCIPAL ACCOUNTING 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 2014 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

		Incorporated by Reference				
Exhibit 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.	8-K	03/28/2013	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	
10.1#		S-1/A	02/05/2013	10.7	333-184779	

	Form of Indemnification Agreement for directors and officers.				
10.2#	Amended and Restated Employment Agreement between the Company and Jay R. Luly, Ph.D., dated as of March 4, 2013.	S-1/A	03/05/2013	10.5	333-184779
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

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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).	S-1/A	02/05/2013	10.1	333-184779	
10.5	Collaboration and License Agreement between the Company and Novartis Institutes for BioMedical Research, Inc., dated February 16, 2012.	S-1/A	03/05/2013	10.2	333-184779	
10.6	Amendment No. 1, dated March 28, 2013, to that certain Collaboration and License Agreement between Enanta Pharmaceuticals, Inc. and Novartis Institutes for BioMedical Research, Inc.	10-Q	05/15/2013	10.2	001-35839	
10.7	Agreement between the Company and the National Institute of Allergy and Infectious Diseases, dated September 30, 2011.	S-1	11/06/2012	10.3	333-184779	
10.8	Modification No. 1, dated August 28, 2013, to that certain Agreement between the Company and the National Institute of Allergy and Infectious Diseases.					X
10.9	Modification No. 2, dated August 29, 2013, to that certain Agreement between the Company and the National Institute of Allergy and Infectious Diseases.					X
10.10	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	
10.11	Third Amended and Restated Registration Rights Agreement, dated as of August 23, 2012.	S-1/A	11/06/2012	10.4	333-184779	
10.12#	Amended and Restated 1995 Equity Incentive Plan.	S-1/A	03/05/2013	10.8	333-184779	
10.13#	Form of Incentive Stock Option Certificate under Amended and Restated 1995 Equity Incentive Plan.	S-1/A	03/05/2013	10.9	333-184779	
10.14#	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	
10.15#	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	

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10.16#	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).					X
10.17#	Form of Incentive Stock Option Agreement under 2012 Equity Incentive Plan.	S-1/A	03/05/2013	10.13	333-184779	
10.18#	Form of Non-Statutory Stock Option Agreement under 2012 Equity Incentive Plan.	S-1/A	03/05/2013	10.14	333-184779	
10.19#	Form of Non-Statutory Stock Option Certificate for directors under 2012 Equity Incentive Plan.	S-1/A	03/05/2013	10.15	333-184779	
10.20#	Employee Stock Purchase Plan.	S-1/A	02/05/2013	10.16	333-184779	
21.1	Subsidiaries of the Company.					X
23.1	Consent of PricewaterhouseCoopers LLP, Independent Registered Public Accounting Firm.					X
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, 2013, formatted in XBRL (eXtensible Business Reporting Language): (i) Balance Sheets as of September 30, 2013 and September 30, 2012 of Enanta Pharmaceuticals, Inc., (ii) Statements of Operations for the years ended September 30, 2013 and 2012 of Enanta Pharmaceuticals, Inc., (iii) Statements of Cash Flows for the years ended September 30, 2013 and 2012 of Enanta Pharmaceuticals, Inc., and (iv) Notes to Financial Statements of Enanta Pharmaceuticals, Inc.[1]					

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.

[1] Pursuant to Rule 406T of Regulation S-T, the Interactive Data Files in Exhibit 101 hereto are deemed not filed or part of a registration statement or prospectus for purposes of Sections 11 or 12 of the Securities Act of 1933, as amended, are deemed not filed for purposes of Section 18 of the Securities and Exchange Act of 1934, as amended, and otherwise are not subject to liability under those sections.

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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 18th day of December, 2013.

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. Jay R. Luly, Ph.D.	President and Chief Executive Officer and Director (Principal Executive Officer)	December 18, 2013
/s/ Paul J. Mellett Paul J. Mellett	Chief Financial Officer (Principal Financial and Accounting Officer)	December 18, 2013
/s/ Ernst-Günter Afting, M.D., Ph.D. Ernst-Günter Afting, M.D., Ph.D.	Director	December 18, 2013
/s/ Stephen Buckley, Jr. Stephen Buckley, Jr.	Director	December 18, 2013
/s/ Bruce L.A. Carter, Ph.D. Bruce L.A. Carter, Ph.D.	Director	December 18, 2013
/s/ Marc E. Goldberg Marc E. Goldberg	Director	December 18, 2013
/s/ David Poorvin, Ph.D. David Poorvin, Ph.D.	Director	December 18, 2013
/s/ Terry Vance Terry Vance	Director	December 18, 2013

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ENANTA PHARMACEUTICALS, INC. AND SUBSIDIARY

INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

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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 redeemable and convertible preferred stock and stockholders equity (deficit) and cash flows present fairly, in all material respects, the financial position of Enanta Pharmaceuticals, Inc. and its subsidiary at September 30, 2013 and 2012 and the results of their operations and their cash flows for each of the three years in the period ended September 30, 2013 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 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 18, 2013

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ENANTA PHARMACEUTICALS, INC. AND SUBSIDIARY

CONSOLIDATED BALANCE SHEETS

(in thousands, except share and per share amounts)

	September 30, 2013	2012
Assets		
Current assets:		
Cash and cash equivalents	$ 8,859	$ 10,511
Short-term marketable securities	92,621	33,251
Accounts receivable	808	1,049
Unbilled receivables	784	1,893
Prepaid expenses and other current assets	1,641	604
Total current assets	104,713	47,308
Property and equipment, net	1,121	611
Long-term marketable securities	10,703	1,656
Restricted cash	436	436
Other assets		2,151
Total assets	$ 116,973	$ 52,162
Liabilities, Redeemable and Convertible Preferred Stock and Stockholders Equity (Deficit)		
Current liabilities:		
Accounts payable	$ 1,481	$ 1,851
Accrued expenses	3,035	3,866
Deferred revenue	10	17
Total current liabilities	4,526	5,734
Warrant liability	1,620	2,001
Other long-term liabilities	359	498
Total liabilities	6,505	8,233
Commitments and contingencies (Note 15)		
Redeemable convertible preferred stock (Series C, D, E, F, G-1 and G-2); $0.01 par value; no shares authorized, issued or outstanding at September 30, 2013; 45,421,288 shares authorized; 43,115,343 shares issued and outstanding at September 30, 2012; aggregate liquidation preference of $159,079 at September 30, 2012		158,955
Convertible preferred stock (Series A and B); $0.01 par value; no shares authorized, issued or outstanding at September 30, 2013; 566,450 shares authorized, issued and outstanding at September 30, 2012; aggregate liquidation		327

preference of $327 at September 30, 2012		
Stockholders equity (deficit):		
Common stock; $0.01 par value; 100,000,000 shares authorized; 18,138,597 and 1,343,147 shares issued and 17,929,781 and 1,134,331 shares outstanding at September 30, 2013 and 2012, respectively	181	13
Additional paid-in capital	217,741	
Treasury stock, at par value; 208,816 shares at September 30, 2013 and 2012	(2)	(2)
Accumulated other comprehensive income (loss)	(2)	10
Accumulated deficit	(107,450)	(115,374)
Total stockholders equity (deficit)	110,468	(115,353)
Total liabilities, redeemable and convertible preferred stock and stockholders equity (deficit)	$ 116,973	$ 52,162

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

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ENANTA PHARMACEUTICALS, INC. AND SUBSIDIARY

CONSOLIDATED STATEMENTS OF OPERATIONS

(in thousands, except share and per share amounts)

	Year Ended September 30,		
	2013	**2012**	**2011**
Revenue	$ 32,053	$ 41,706	$ 41,882
Operating expenses:			
Research and development	16,841	15,115	11,547
General and administrative	6,183	5,302	5,036
Total operating expenses	23,024	20,417	16,583
Income from operations	9,029	21,289	25,299
Other income (expense):			
Interest income	248	118	83
Interest expense	(31)		(3,161)
Change in fair value of warrant liability	381	(8)	(686)
Therapeutic tax credit			750
Gain on embedded derivative			670
Other income (expense), net			355
Total other income (expense), net	598	110	(1,989)
Net income	9,627	21,399	23,310
Accretion of redeemable convertible preferred stock to redemption value	(2,526)	(5,367)	(5,454)
Net income attributable to participating securities	(13,670)	(14,663)	(16,291)
Net income (loss) attributable to common stockholders	$ (6,569)	$ 1,369	$ 1,565
Net income (loss) per share attributable to common stockholders:			
Basic	$ (0.67)	$ 1.26	$ 1.40
Diluted	$ (0.67)	$ 1.13	$ 1.32
Weighted average common shares outstanding:			
Basic	9,788,039	1,088,784	1,119,250
Diluted	9,788,039	2,474,823	1,857,273

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

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ENANTA PHARMACEUTICALS, INC. AND SUBSIDIARY

CONSOLIDATED STATEMENTS OF COMPREHENSIVE INCOME

(in thousands)

	Year Ended September 30,		
	2013	**2012**	**2011**
Net income	$ 9,627	$ 21,399	$ 23,310
Other comprehensive income (loss):			
Net unrealized gains (losses) on marketable securities, net of tax of $0	(12)	11	(1)
Total other comprehensive income (loss)	(12)	11	(1)
Comprehensive income	$ 9,615	$ 21,410	$ 23,309

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

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ENANTA PHARMACEUTICALS, INC. AND SUBSIDIARY

CONSOLIDATED STATEMENTS OF CHANGES IN REDEEMABLE AND CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS EQUITY (DEFICIT)

(in thousands, except share amounts)

, D, E, F and G ble Convertible erred Stock		Series A and B Convertible Preferred Stock		Common Stock		Additional Paid-in	Treasury Stock		Accumulated Other Compre-hensive	Accumulated	Total Stockholders Equity
	Amount	Shares	Amount	Shares	Amount	Capital	Shares	Amount	Income (Loss)	Deficit	(Deficit)
343	$ 148,134	566,450	$ 327	1,209,464	$ 12	$	(71,926)	$ (1)	$	$ (150,086)	$ (150,075)
				16,435		34					34
						1	(136,890)	(1)			
						225					225
	5,454					(260)				(5,194)	(5,454)
									(1)		(1)
										23,310	23,310
343	153,588	566,450	327	1,225,899	12		(208,816)	(2)	(1)	(131,970)	(131,961)
				117,248	1	140					141
						424					424
	5,367					(564)				(4,803)	(5,367)
									11		11
										21,399	21,399
343	158,955	566,450	327	1,343,147	13		(208,816)	(2)	10	(115,374)	(115,353)
				538,575	5	554					559
						1,063					1,063
	2,526					(823)				(1,703)	(2,526)
343)	(161,481)	(566,450)	(327)	11,656,875	117	161,691					161,808
				4,600,000	46	59,846					59,892

				(4,590)					(4,590)
							(12)		(12)
								9,627	9,627
$	$	18,138,597	$ 181	$ 217,741	(208,816)	$ (2)	$ (2)	$ (107,450)	$ 110,468

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

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ENANTA PHARMACEUTICALS, INC. AND SUBSIDIARY

CONSOLIDATED STATEMENTS OF CASH FLOWS

(In thousands)

	Year Ended September 30,		
	2013	**2012**	**2011**
Cash flows from operating activities			
Net income	$ 9,627	$ 21,399	$ 23,310
Adjustments to reconcile net income to net cash provided by operating activities:			
Depreciation and amortization expense	221	172	499
Non-cash interest expense	31		2,059
Change in fair value of warrant liability	(381)	8	686
Gain on embedded derivative			(670)
Stock-based compensation expense	1,063	424	225
Gain on disposal of property and equipment	(100)	(63)	(7)
Premium on marketable securities	(1,811)		
Amortization of premium on marketable securities	1,262	590	317
Change in operating assets and liabilities:			
Accounts receivable	241	(788)	(251)
Unbilled receivables	1,109	(1,893)	
Prepaid expenses and other current assets	(307)	(235)	(273)
Other assets	22	5	(26)
Accounts payable	115	763	58
Accrued expenses	(285)	1,726	(1,120)
Deferred revenue	(7)	17	(432)
Other long-term liabilities	(147)	498	(356)
Net cash provided by operating activities	10,653	22,623	24,019
Cash flows from investing activities			
Purchases of property and equipment	(606)	(252)	(445)
Proceeds from sales of property and equipment		66	9
Purchases of marketable securities	(115,888)	(47,694)	(33,574)
Sales of marketable securities	5,453	15,750	16,764
Maturities of marketable securities	41,825	12,950	
Change in restricted cash		1,140	(436)
Net cash used in investing activities	(69,216)	(18,040)	(17,682)
Cash flows from financing activities			
Proceeds from issuance of convertible notes			2,000
Repayment of convertible notes			(2,000)
	59,892		

Proceeds from initial public offering, net of underwriting discounts and commissions			
Proceeds from exercise of stock options	559	141	34
Payments of initial public offering costs	(3,540)	(1,050)	
Net cash provided by (used in) financing activities	56,911	(909)	34
Net increase (decrease) in cash and cash equivalents	(1,652)	3,674	6,371
Cash and cash equivalents at beginning of period	10,511	6,837	466
Cash and cash equivalents at end of period	$ 8,859	$ 10,511	$ 6,837
Supplemental disclosure of cash flow information:			
Cash paid for interest	$	$	$ 1,056
Supplemental disclosure of noncash financing activities:			
Accretion of redeemable convertible preferred stock to redemption value	$ 2,526	$ 5,367	$ 5,454
Deferred initial public offering costs included in accounts payable and accrued expenses	$	$ 1,079	$
Conversion of preferred stock into common stock	$ 161,808	$	$

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

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ENANTA PHARMACEUTICALS, INC AND SUBSIDIARY

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

(Amounts in thousands, except share and per share data)

1. Nature of the Business

Enanta Pharmaceuticals, Inc. (the Company), incorporated in Delaware in 1995, is a research and development-focused biotechnology company that uses its chemistry-driven approach and drug discovery capabilities to create small molecule drugs in the infectious disease field. The Company is developing novel protease, NS5A, cyclophilin and nucleotide polymerase inhibitors targeted against the hepatitis C virus (HCV). Additionally, the Company has created a new class of macrolide antibiotics known as Bicyclolides that overcomes bacterial resistance. Antibacterial focus areas include superbugs, respiratory tract infections, and intravenous and oral treatments for hospital and community Methicillin-resistant *Staphylococcus aureus* (MRSA).

The Company is subject to risks common to companies in the biotechnology industry including, but not limited to, new technological innovations, dependence on collaborative arrangements, protection of proprietary technology, dependence on key personnel, 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 approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel infrastructure, and extensive 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 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 financial statements, and the reported amounts of revenues and expenses during the reporting period. Significant estimates and assumptions reflected in these 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; the valuation of outstanding warrants and stock-based awards; the useful lives of property and equipment; 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 three months 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.

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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 and losses as a component of stockholders equity (deficit). The cost of securities sold is determined on a specific identification basis, and realized gains and losses are included in other income (expense) within the statement 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 statement of operations. There were no such adjustments necessary during the years ended September 30, 2013, 2012 or 2011.

Restricted Cash

As of September 30, 2013 and 2012, the Company had outstanding a letter of credit collateralized by a money market account of $436 to the benefit of the landlord of the Company s current office lease. This amount was classified as long-term restricted cash as of September 30, 2013 and 2012.

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, 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 and a U.S. government contract (see Note 8). As of September 30, 2013 and 2012, accounts receivable and unbilled receivables consisted of amounts due from the Company s collaborators and under a U.S. government contract (see Note 8).

The Company is completely dependent on third-party manufacturers for product supply for preclinical research activities in its non-partnered programs. In particular, the Company relies and expects to continue to rely exclusively on one manufacturer 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 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 based on three levels of inputs, of which the first two are considered observable and the last is considered unobservable, is used to measure fair value:

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.

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The Company s cash equivalents and marketable securities assets and its warrant liabilities are carried at fair value determined according to the fair value hierarchy described above (see Note 3). 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. The carrying value of accrued third-party license fees included in other long-term liabilities has been recorded at its present value, which approximates fair value.

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	3 5 years
Leasehold improvements	Shorter of life of lease or estimated useful life
Purchased software	3 5 years
Computer equipment	3 5 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 of are removed from the accounts and any resulting gain or loss is included in income (loss) from 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 is 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.

For agreements entered into prior to October 1, 2011, the Company evaluated license agreements with multiple deliverables to determine if the deliverable elements could be recognized separately by considering (i) if the delivered elements (typically the license) had standalone value to the customer, (ii) if the fair value of any undelivered elements (typically the research and development services and the steering committee activities)

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could be determined based on vendor-specific objective evidence (VSOE) or vendor objective evidence (VOE), and (iii) if the arrangement included a general right of return relative to the delivered item, the delivery or performance of the undelivered item was considered probable and substantially within the control of the Company. VSOE of fair value was based on the consistent price of a deliverable when the Company regularly sold it on a standalone basis. Alternatively, VOE was based upon third-party objective evidence of fair value. If the delivered elements had value on a standalone basis and the fair value of the undelivered elements could be determined based on VSOE or VOE, revenues of such elements were then accounted for separately as delivered with arrangement consideration allocated to the delivered elements based on the residual value method. If either (i) the delivered elements were considered to not have standalone value or (ii) VSOE or VOE of fair value for any of the undelivered elements could not be determined, the arrangement was accounted for as a single unit of accounting and all payments received were recognized as revenue over the estimated period of performance of the entire arrangement.

On October 1, 2011, the Company adopted Accounting Standards Update (ASU) 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 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. In validating the Company s BESP, the Company considers 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 the arrangement are separate units of accounting 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. 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. The Company elected to adopt ASU 2009-13 prospectively as of October 1, 2011.

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.

Under a collaborative research and license agreement, a steering committee is typically responsible for overseeing the general working relationships, determining the protocols to be followed in the research and development performed, and evaluating the results from the continued development of the product in order to determine the clinical studies to be performed. The Company evaluates whether its participation in joint research and development steering committees is a substantive obligation or whether the services are considered inconsequential or perfunctory. The Company s participation on a steering committee is considered participatory and therefore accounted for as a separate

element when the collaborator requires the participation of the Company to ensure all elements of an arrangement are maximized. Steering committee services that are

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considered participatory are combined with other research services or performance obligations required under an arrangement, if any, in determining the level of effort required in an arrangement and the period over which the Company expects to complete its aggregate performance obligations. Alternatively, the Company s participation on a steering committee is considered protective and therefore not accounted for as a separate element in a case where the Company can exercise or control when to be involved at its own discretion. Factors the Company considers in determining if its participation in a joint steering committee is participating or protective include: (i) which party negotiated or requested the steering committee, (ii) how frequently the steering committee meets, (iii) whether or not there are any penalties or other recourse if the Company does not attend the steering committee meetings, (iv) which party has decision making authority on the steering committee, and (v) whether or not the collaborator has the requisite experience and expertise associated with the research and development of the licensed intellectual property.

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 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, if any, is recognized based on contractual terms when reported sales are reliably measurable and collectibility is reasonably assured, provided that there are no performance obligations then remaining. To date, none of the Company s products have been approved, and therefore the Company has not earned any royalty revenue from product sales.

During the years ended September 30, 2013 and 2012, the Company also generated revenue from a government contract under which the Company is reimbursed for certain allowable costs incurred for the funded project. Revenue from the government contract is recognized when the related service is performed. The related costs incurred by the Company under the government contract are included in research and development expense in the statements of operations.

Amounts received prior to satisfying all revenue recognition criteria are recorded as deferred revenue in the accompanying balance sheets. Amounts not expected to be recognized as revenue within the next twelve months of the 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 portion of the upfront payment that had not previously been recorded as revenue but was classified as deferred revenue at the date of such termination.

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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. 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.

Deferred Offering Costs

The Company capitalized certain legal, accounting and other third-party fees that were directly associated with the equity financing that resulted in the Company s initial public offering of its common stock (IPO) in March 2013. Those fees were included in other assets in the accompanying balance sheet until the IPO was consummated. The Company recorded deferred financing costs of $2,129 at September 30, 2012 in other assets in the accompanying balance sheet. After consummation of the IPO, these costs were recorded in stockholders equity as a reduction of additional paid-in capital generated as a result of the offering.

Accounting for Stock-Based Compensation

The Company measures all stock options and other stock-based awards granted to employees at the fair value on the date of the grant using the Black-Scholes option-pricing model. The fair value of the awards is recognized as 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-only conditions. The Company classifies stock-based compensation expense in the 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.

The Company accounts for uncertainty in income taxes recognized in the 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

recognize 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.

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Net Income (Loss) Per Share

The Company follows the two-class method when computing net income (loss) per share during periods before the IPO when the Company had issued shares that met the definition of participating securities. The two-class method is used to determine net income (loss) per share for each class of common and participating securities according to dividends declared or accumulated and participation rights in undistributed earnings. The two-class method requires income available to common stockholders for the period to be allocated between common and participating securities based upon their respective rights to receive dividends as if all income for the period had been distributed.

The Company s redeemable convertible preferred shares and convertible preferred shares contractually entitled the holders of such shares to participate in dividends, but did not contractually require the holders of such shares to participate in losses of the Company. Accordingly, in periods in which the Company had participating securities and reported a net loss or a net loss attributable to common stockholders resulting from preferred stock dividends or accretion, net losses were not allocated to participating securities.

Basic net income (loss) per share attributable to common stockholders is computed by dividing the net income (loss) attributable to common stockholders by the weighted average number of shares of common stock outstanding for the period. Diluted net income (loss) attributable to common stockholders is computed by adjusting net income (loss) attributable to common stockholders to reallocate undistributed earnings based on the potential impact of dilutive securities, including outstanding stock options and outstanding warrants. Diluted net income (loss) per share attributable to common stockholders is computed by dividing the diluted net income (loss) attributable to common stockholders 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 outstanding warrants. For periods in which the Company has reported net losses, diluted net loss per share attributable to common stockholders is the same as basic net loss per share attributable to common stockholders, since dilutive common shares are not assumed to have been issued if their effect is anti-dilutive. Diluted net loss per share attributable to common stockholders is the same as basic net loss per share attributable to common stockholders for the year ended September 30, 2013.

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 in the infectious disease field. Revenue is generated exclusively from transactions occurring in the United States, and all assets are held in the United States.

Comprehensive Income (Loss)

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

Recently Issued and Adopted Accounting Pronouncements

In May 2011, the Financial Accounting Standards Board (the FASB) issued ASU No. 2011-04, *Fair Value Measurement: Amendments to Achieve Common Fair Value Measurement and Disclosure Requirements in U.S. GAAP and IFRS*s (ASU 2011-04). This accounting standard clarifies the application of certain existing fair value measurement guidance and expands the disclosures for fair value measurements that are estimated using significant

unobservable (Level 3) inputs. This ASU is effective on a prospective basis for annual and interim reporting periods beginning on or after December 15, 2011. The Company s adoption of this standard on January 1, 2012 did not have a material effect on its financial position, results of operations or cash flows.

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In February 2013, the FASB issued ASU No. 2013-02, *Comprehensive Income (Topic 220): Reporting of Amounts Reclassified Out of Accumulated Other Comprehensive Income* (ASU 2013-02). This newly issued accounting standard requires an entity to provide information about the amounts reclassified out of accumulated other comprehensive income by component. In addition, an entity is required to present, either on the face of the statement where net income is presented or in the notes, significant amounts reclassified out of accumulated other comprehensive income by the respective line items of net income but only if the amount reclassified is required under U.S. GAAP to be reclassified to net income in its entirety in the same reporting period. For other amounts that are not required under U.S. GAAP to be reclassified in their entirety to net income, an entity is required to cross-reference to other disclosures required under U.S. GAAP that provide additional detail about those amounts. ASU 2013-02 is effective for fiscal years and interim periods within those years beginning after December 15, 2012 and will become effective for the Company on October 1, 2013. The Company does not believe the adoption of this standard will have an impact on the Company s financial position, results of operations or cash flows.

In July 2013, The FASB issued ASU No. 2013-11, *Income Taxes (Topic 740): Presentation of an Unrecognized Tax Benefit When a Net Operating Loss Carryforward, a Similar Tax Loss, or a Tax Credit Carryforward Exists* (ASU 2013-11). This newly issued accounting standard requires that an unrecognized tax benefit, or a portion of an unrecognized tax benefit, should be presented in the financial statements as a reduction to a deferred tax asset for a net operating loss carryforward, a similar tax loss, or a tax credit carryforward, except as follows. To the extent a net operating loss carryforward, a similar tax loss, or a tax credit carryforward is not available at the reporting date under the tax law of the applicable jurisdiction to settle any additional income taxes that would result from the disallowance of a tax position or the tax law of the applicable jurisdiction does not require the entity to use, and the entity does not intend to use, the deferred tax asset for such purpose, the unrecognized tax benefit should be presented in the financial statements as a liability and should not be combined with deferred tax assets. The assessment of whether a deferred tax asset is available is based on the unrecognized tax benefit and deferred tax asset that exist at the reporting date and should be made presuming disallowance of the tax position at the reporting date. For example, an entity should not evaluate whether the deferred tax asset expires before the statute of limitations on the tax position or whether the deferred tax asset may be used prior to the unrecognized tax benefit being settled. ASU 2013-11 is effective for fiscal years and interim periods within those years beginning after December 15, 2013 and will become effective for the Company on October 1, 2014. The Company does not believe the adoption of this standard will have an impact on its financial position, results of operations or cash flows.

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 financial statements upon adoption.

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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, 2013 and 2012 and indicate the fair value hierarchy of the valuation inputs utilized to determine such fair value:

	Fair Value Measurements as of September 30, 2013 Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents	$ 7,517	$	$	$ 7,517
U.S. Treasury notes	1,005			1,005
Commercial paper		10,596		10,596
Corporate bonds		84,755		84,755
U.S. Agency bonds		3,518		3,518
Certificate of deposit		3,450		3,450
	$ 8,522	$ 102,319	$	$ 110,841
Liabilities:				
Warrant liability	$	$	$ 1,620	$ 1,620
	$	$	$ 1,620	$ 1,620

	Fair Value Measurements as of September 30, 2012 Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents	$ 6,471	$	$	$ 6,471
U.S. Treasury notes	1,015			1,015
Commercial paper		8,143		8,143
Corporate bonds		25,749		25,749
	$ 7,486	$ 33,892	$	$ 41,378
Liabilities:				
Warrant liability	$	$	$ 2,001	$ 2,001
	$	$	$ 2,001	$ 2,001

During the years ended September 30, 2013, 2012 and 2011, there were no transfers between Level 1, Level 2 and Level 3.

Cash equivalents at September 30, 2013 and 2012 consist of money market funds.

As of September 30, 2013, the warrant liability consists solely of the warrants for the purchase of Series 1 nonconvertible preferred stock. As of September 30, 2013, the Company utilized a probability weighted valuation model which takes into consideration various outcomes that may require the Company to transfer assets upon exercise. The fair value of these outstanding warrants to purchase our Series 1 nonconvertible preferred stock as recorded in the balance sheet was $1,620 and $1,981 at September 30, 2013 and 2012, respectively. The Series E warrants expired during the year ended September 30, 2013.

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The recurring Level 3 fair value measurements of the Company s warrant liability using probability-weighted discounted cash flow include the following significant unobservable inputs:

	Unobservable Input	Range (Weighted Average)
Warrant Liability	Probabilities of payout	25% - 90%
	Periods in which payout is expected to occur	2014 - 2018
	Discount rate	4.25%

The fair value of warrants for the purchase of Series E redeemable convertible preferred stock (see Note 12) was $20, as of September 30, 2012. These warrants expired unexercised in fiscal 2013.

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

Balance, September 30, 2010	$ 26
Warrants issued	1,280
Increase in fair value	705
Decrease in fair value	(18)
Balance, September 30, 2011	1,993
Warrants expired	(8)
Increase in fair value	19
Decrease in fair value	(3)
Balance, September 30, 2012	2,001
Warrants expired	(20)
Decrease in fair value	(361)
Balance, September 30, 2013	$ 1,620

4. Marketable Securities

As of September 30, 2013 and 2012, the fair value of available-for-sale marketable securities by type of security was as follows:

	September 30, 2013			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 10,596	$	$	$ 10,596

Corporate bonds	84,757	23	(25)	84,755
U.S. Agency bonds	3,519		(1)	3,518
Certificate of deposit	3,450			3,450
U.S. Treasury notes	1,004	1		1,005
	$ 103,326	$ 24	$ (26)	$ 103,324

	September 30, 2012			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Commercial paper	$ 8,143	$	$	$ 8,143
Corporate bonds	25,741	9	(1)	25,749
U.S. Treasury notes	1,013	2		1,015
	$ 34,897	$ 11	$ (1)	$ 34,907

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At September 30, 2013, marketable securities consisted of investments that mature within one year, with the exception of certain corporate bonds, which have maturities within two years and an aggregate fair value of $10,703. At September 30, 2012, marketable securities consisted of investments that mature within one year, with the exception of one U.S. Treasury note and one corporate bond, which have maturities within two years and an aggregate fair value of $1,656.

5. Property and Equipment

Property and equipment consisted of the following as of September 30, 2013 and 2012:

	September 30,	
	2013	2012
Laboratory and office equipment	$ 4,395	$ 4,142
Leasehold improvements	272	268
Purchased software	438	402
Computer equipment	90	76
Furniture	263	259
	5,458	5,147
Less: Accumulated depreciation and amortization	(4,337)	(4,536)
	$ 1,121	$ 611

Depreciation and amortization expense was $221, $172 and $499 for the years ended September 30, 2013, 2012 and 2011, respectively. During the years ended September 30, 2013, 2012 and 2011, assets with a cost of $420, $273 and $3,810, respectively, were sold or disposed of, resulting in a gain of $100, $63 and $7, respectively.

6. Accrued Expenses and Other Long-Term Liabilities

Accrued expenses (current) and other long-term liabilities consisted of the following as of September 30, 2013 and 2012:

	September 30,	
	2013	2012
Accrued expenses:		
Accrued payroll and related expenses	$ 1,041	$ 1,305
Accrued vendor manufacturing	989	1,330
Accrued professional fees	378	718
Accrued third-party license fee	240	222
Accrued other	387	291
	$ 3,035	$ 3,866

Other long-term liabilities:

Accrued rent expense	$ 127	$ 75
Present value of accrued third-party license fee	184	423
Asset retirement obligation	48	
	$ 359	$ 498

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7. Convertible Notes

2011 Bridge Financing

In October 2010, the Company entered into a convertible note and warrant purchase agreement with existing investors to sell, in one or more closings, term notes in the aggregate principal amount of up to $6,500. The term notes had an interest rate of 5% per annum, with principal and interest payable at the stated maturity date of October 4, 2011 or earlier if put to the Company by the noteholders or if called by the Company. The noteholders could put the term notes to the Company for early repayment upon receipt by the Company of the next milestone payment under the Company s collaborative development and license agreement with Abbott Laboratories (subsequently AbbVie, as explained in Note 8 below) or upon a merger, sale or liquidity event as specified by the note agreement. The Company could call the term notes for early repayment at any time. If called by the Company prior to maturity, repaid at maturity, or put by the noteholders upon receipt by the Company of the milestone payment, the term notes required payment of a prepayment premium equal to 51.8% of the principal amount of the notes in addition to the principal and interest payable. If put by the noteholders due to a liquidity event, the term notes were not subject to the premium of 51.8% but were to be repaid at the following multiples:

two times the principal amount if the liquidity event occurred on or before March 31, 2011, and

three times the principal amount if the liquidity event occurred after March 31, 2011.

During the first closing of the term notes in October and November 2010, the Company borrowed a total of $2,000. The remaining balance of $4,500 under the agreement was not drawn down.

The call and put options within the notes agreement constituted an embedded derivative, which was required to be separately recognized and measured at fair value, resulting in the Company recognizing a debt discount and derivative liability of $670 at the date of issuance of the notes.

In addition, each purchaser of a note received warrants to purchase shares of Series 1 nonconvertible preferred stock (the Nonconvertible Preferred) at the rate of one warrant for each dollar of the original purchase amount of the note. The warrants have an exercise price of $0.01 per share. At issuance, the number of shares issuable upon exercise of the warrants was not yet fixed. The number of shares for which the warrants could be exercisable was (i) one share for each dollar of the original principal amount of the Note, plus (ii) if the milestone payment was not received on or before March 31, 2011, an additional share for each dollar of the original principal amount of the notes. Additionally, if a liquidation event occurred, thereby requiring repayment of the term notes, these warrants would automatically expire and would therefore have no value. At the date of issuance, the warrants were valued using the Black-Scholes option-pricing model for each of the two scenarios described above. A decision tree was used to estimate the probability of how many shares the warrants would ultimately be exercised into or if the warrants would have no value under the third scenario. This resulted in a total fair value of $1,280 at date of issuance. Upon repayment of the notes in December 2010, the number of shares issuable upon exercise of the warrants became fixed at 1,999,989 Nonconvertible Preferred shares. Additionally, the possibility that the notes would not be redeemed and that the warrants would therefore have no value was eliminated. Accordingly, at the time of the note repayment in December 2010, the Company revalued the warrants, which resulted in a total fair value of $1,983. The change in the fair value of the warrants was recorded within other income (expense) in the statement of operations. Additionally, as these warrants are financial instruments that may require a future transfer of assets, they are classified as liabilities on the balance sheet. Upon issuance of the notes and warrants, the fair value of the warrants was recorded as a warrant

liability and a corresponding debt discount was recognized. The warrants are remeasured at each balance sheet date (see Note 3), and the change in fair value is recorded within other income (expense).

Following the receipt by the Company of a $40,000 milestone payment from Abbott Laboratories in December 2010 (see Note 8), the Company repaid the $2,000 in principal plus the accrued interest of $20 and the applicable premium of $1,036 (51.8% of the principal amount). Upon repayment of the notes, the unamortized debt discount derived from both the embedded derivatives and the warrants was accreted as a charge to interest expense and the

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derivative liability (recorded at fair value) was removed, resulting in a gain recorded in the statement of operations. The warrants, however, remained outstanding as of September 30, 2013. The note agreement was canceled upon the December 2010 repayment and no further warrants were issued under the agreement.

In connection with this financing, the Company incurred issuance costs of $41 during the year ended September 30, 2011. These costs were initially recorded as deferred financing costs in other assets on the balance sheet. In December 2010, upon repayment of the notes, the total deferred financing costs of $150 were charged to interest expense.

8. Collaboration Agreements

AbbVie Collaboration

On November 27, 2006, the Company entered into a Collaborative Development and License Agreement with Abbott Laboratories (Abbott) to identify, develop and commercialize HCV NS3 and NS3/4A protease inhibitor compounds, including ABT-450. On January 1, 2013, Abbott announced that it had completed the spin-off of its research-based pharmaceuticals business into a new, independent biopharmaceutical company named AbbVie Inc. (AbbVie). AbbVie was formed to hold Abbott s research-based pharmaceuticals business and, as a result of the spin-off, became an independent public company. In connection with the spin-off, Abbott assigned to AbbVie the above Collaborative Development and License Agreement (AbbVie Agreement). Under the terms of the AbbVie Agreement, as amended, through September 30, 2009, AbbVie paid $48,300 to the Company for 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 tiered royalties per product ranging from the low double digits up to twenty percent, or up to the high teens on a blended basis, on net sales by AbbVie allocable to the collaboration s protease inhibitors.

Also on November 27, 2006, the Company entered into a Series G preferred stock purchase agreement with AbbVie. In connection with that agreement, the Company sold to AbbVie 4,620,764 shares of Series G-1 redeemable convertible preferred stock at a price of $2.70518 per share, resulting in gross proceeds of $12,500. Due to the simultaneous issuance of Series G-2 redeemable convertible preferred stock to a third party unrelated to AbbVie which had similar terms to Series G-1 but a lower price per share, the Company determined that the Series G-1 redeemable convertible preferred stock sold to AbbVie was issued at a premium of $1,617. The Company aggregated this premium with the upfront payments received and recorded it as deferred revenue to be recognized over the period of performance under the AbbVie Agreement.

In January 2009, the Company and AbbVie amended the AbbVie Agreement to include an option for AbbVie to opt into an evaluation period that would commence upon the termination or expiration of the research program term and continue for a period of six months. During the evaluation period, AbbVie would have the right to analyze certain compounds for the purpose of identifying any suitable for further development. In December 2009, the Company and AbbVie further amended the AbbVie Agreement to extend funding of the research activities for another year through December 2010. In December 2010, AbbVie opted into the evaluation period and additional research activities for the six-month evaluation period ended June 15, 2011. In connection with these amendments, AbbVie paid the Company an additional $4,150 for the research services performed from December 15, 2009 through June 15, 2011.

On December 17, 2010, the Company received a milestone payment of $40,000 for the successful completion of AbbVie s Phase 2a clinical study. During the year ended September 30, 2013, the Company received an additional $15,000 milestone payment under the AbbVie Agreement as a result of AbbVie s initiation of dosing in a Phase 3 clinical trial of a regimen that included ABT-450. Through September 30, 2013, the Company had received upfront license payments, research funding, and milestone payments totaling $107,450 under the AbbVie Agreement.

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As of September 30, 2013, the Company is eligible to receive additional future milestone payments totaling up to $40,000 upon AbbVie s achievement of regulatory filing milestones for the first protease inhibitor product resulting from its collaboration and additional milestone payments totaling up to $155,000 upon AbbVie s achievement of commercial regulatory approval milestones in selected world markets. The Company is also eligible to receive additional milestone payments totaling up to $80,000 upon AbbVie s achievement of similar commercial regulatory approval milestones for each additional product containing a protease inhibitor.

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.

Under this model, the revenue recognized was 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. At each reporting period, the Company updated its estimates of effort remaining to complete its obligations under the AbbVie Agreement, the expected term over which the obligations would be performed, and the total expected revenue based on all known information. Based on the obligations and responsibilities of the joint steering committee, the Company determined that the obligation to participate in the joint steering committee was participatory only through the research and evaluation period, which ended June 15, 2011. Subsequent to the research and evaluation period, all decisions related to the development, commercialization and marketing are to be 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 fiscal 2010 and 2011, revenue related to the AbbVie Agreement was recognized in the amounts of $6,518 and $41,882, respectively. In fiscal 2011, the Company completed all remaining obligations under the agreement. As a result no revenue was recognized related to this agreement during the year ended September 30, 2012. Since all obligations under the AbbVie Agreement were concluded by the end of fiscal 2011, any future milestone payments received will be recognized as revenue when each milestone is achieved by AbbVie. During fiscal 2013 the Company earned and recognized as revenue a $15,000 milestone payment based on AbbVie s initiation of dosing in a Phase 3 clinical trial that included ABT-450.

The Company has the option, but not the obligation, to co-develop and share in the profit of any product in the United States that is developed as a result of the AbbVie Agreement. This option for the first compound (ABT-450) expired in fiscal 2011. The Company has no further obligations in regard to the AbbVie Agreement and will evaluate future options as they arise.

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 a product in a country.

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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 last date upon which the manufacture, use or sale of a product would infringe one of the licensed patents, and (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 continues 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 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.

Novartis Collaboration

On February 16, 2012, the Company entered into a license and collaboration agreement with Novartis (the Novartis Agreement) for the development, manufacture and commercialization of its lead development candidate, EDP-239, from its NS5A HCV inhibitor program. Under the terms of the Novartis Agreement, Novartis agreed to pay a nonrefundable upfront fee to the Company and reimbursement of manufacturing and quality assurance expenses related to EDP-239 totaling $34,442. In November 2012, a clinical milestone payment of $11,000 was earned and recognized as revenue upon Novartis initiation of dosing in the first Phase 1 clinical trial involving EDP-239. Under the Novartis Agreement, the Company is eligible to receive additional milestone payments totaling up to $395,000 upon Novartis initiation of clinical trials, achievement of regulatory approvals, and/or net sales of products containing the Company s NS5A inhibitors, including a milestone payment of $15,000 that will be due upon Novartis initiation of the first Phase 2 clinical trial using a combination containing an NS5A inhibitor from the collaboration. The Company is also eligible to receive tiered royalties ranging on a blended basis from the low double digits up to the high teens on net product sales by Novartis allocable to the collaboration s NS5A inhibitors. In addition, Novartis agreed to fund research activities for one year commencing February 2012, up to a total of $1,800. In March 2013, the Novartis Agreement was amended to extend research funding for an additional six months at the same reimbursement rate.

The Company determined that the deliverables under the Novartis Agreement include (i) the exclusive, royalty-bearing, sublicensable license to EDP-239 and (ii) the research services. The Company concluded that the

EDP-239 license had standalone value to Novartis and was separable from the research services as the license is

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sublicensable, there are no restrictions as to Novartis use of the license and Novartis has the requisite scientific expertise in the HCV NS5A field. The Company also concluded that its participation on the joint steering committee, as provided by the agreement, is protective in nature as the Company has no decision making authority, there are no penalties or recourse if the Company chooses not to participate, and the purpose of the steering committee is to keep the Company apprised as to the status of the development and commercialization efforts. Therefore, no arrangement consideration was allocated to the joint steering committee participation. The Company was not able to establish VSOE or TPE for either the license or the research services and instead allocated the arrangement consideration between the license and research services based on their relative selling prices using BESP. The Company developed its estimate of BESP of the license using a discounted cash flow analysis, taking into consideration assumptions including the development and commercialization timeline, discount rate, probability of success, and probable treatment combination and associated peak sales figures that generate royalty amounts. The funding rate for the research services is consistent with the rate received in the Company s prior collaboration arrangement with AbbVie and is consistent with its fully burdened cost of service. Therefore, the Company s determination of BESP for the research services is consistent with the reimbursement rate stated in the contract. These assumptions involve judgment and inherent uncertainty; however, significant changes in key assumptions used to determine the BESP would not have a significant effect on the total amount of revenue recognized.

The Company received an upfront cash payment of $34,442 in March 2012 related to the Novartis Agreement. The Company recognized the entire upfront cash receipt as revenue because the allocated selling price of the license deliverable exceeded the upfront noncontingent cash payments received. During the year ended September 30, 2012, the Company recognized total revenue of $35,567 related to the delivery of the license and the performance of the research services.

During the year ended September 30, 2013, the Company recognized total revenue of $12,675 under the Novartis Agreement, of which $10,894 was attributable to license fees and $1,781 was attributable to the performance of research services.

Subject to certain exceptions, on a country-by-country and product-by-product basis, a party s rights and obligations under the agreement continue until the later of (i) the expiration of the last to expire patent rights of a covered product in the applicable country or (ii) ten years from the first commercial sale of a covered product in the applicable country. As a result, the final expiration date of the Novartis license is indeterminable at this time. Upon expiration of the agreement with respect to a particular product and country, the licenses granted to Novartis in the agreement with respect to such product and country will remain in effect and convert to a nonexclusive, perpetual, unrestricted, fully paid, royalty-free, worldwide license.

The Company may terminate the agreement (i) in the event of a material breach by Novartis, subject to prior notice and the opportunity to cure, (ii) in the event Novartis fails to use commercially reasonable efforts to develop and commercialize covered products in its territory, or (iii) in the event Novartis is subject to an insolvency event. Novartis may terminate the agreement (i) in the event of a material breach by the Company, subject to prior notice and the opportunity to cure, (ii) in the event the Company is subject to an insolvency event, or (iii) for any reason upon 120 days prior written notice. In the case of a termination for cause by the Company or a termination without cause by Novartis, any licenses and other rights granted by either party to the other will terminate and revert back to the granting party and the Company will regain control of the prosecution of the patents relating to the Company s intellectual property. If such termination occurs prior to the second anniversary of the end of the research term, the Company obtains a worldwide, exclusive, fully paid, perpetual license, with the right to sublicense, under all collaboration intellectual property owned in whole or in part by Novartis, to research, develop and commercialize compounds and products contemplated by the collaboration. If such termination occurs after the second anniversary of the end of the research term, then Novartis agrees to negotiate with the Company to grant it a worldwide, exclusive,

field-limited, royalty-bearing license, with right to sublicense, under all collaboration intellectual property owned in whole or in part by Novartis.

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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), which contract could provide up to $42,700 in development funding to the Company over a five-year period. The contract will fund the preclinical and clinical development of a new class of bridged bicyclic antibiotics known as Bicyclolides to be used as medical countermeasures against multiple biodefense Category A and B bacteria.

The contract has an initial term of 30 months ending on March 30, 2014. NIAID has the option to extend the contract up to six times. If each extension option is exercised, the contract would be extended until September 30, 2016. The initial award under the initial term was $14,300, with the possibility of up to a total of $42,700 if each option period is exercised by NIAID.

In August 2013 NIAID exercised the first two options under this agreement which obligate it to provide an additional $9,200 in funding to the Company for preclinical and early clinical development of EDP-788, bringing total funding paid or committed to date by NIAID to approximately $23,500 through February 2015.

The Company recognizes revenue under this agreement as development services are performed in accordance with the funding agreement. During the year ended September 30, 2012, $6,139 of revenue was recognized under this agreement, of which $1,049 was invoiced but unpaid and included in accounts receivable and $1,668 was unpaid and included in unbilled receivables on the Company s balance sheet as of September 30, 2012. During the year ended September 30, 2013, the Company recognized revenue of $4,378, under this agreement, of which $258 was invoiced but unpaid and included in accounts receivable and $784 was unpaid and included in unbilled receivables at September 30, 2013.

NIAID may terminate performance of work under this contract if it determines that a termination is in the government s interest or if the Company defaults in performing the contract. After termination, the Company would submit a final termination settlement proposal in order to settle all outstanding liabilities, including those arising from the termination of subcontracts, the cost of which would be reimbursable in whole or in part, under this contract, contingent on approval by NIAID. If the Company and NIAID fail to agree in whole or in part on the amount of costs to be paid because of the termination of work, NIAID shall determine, on the basis of information available, the amount to be repaid.

9. Stockholders Equity

On March 1, 2013, the Company effected an increase in the number of authorized shares of its common stock from 70,000,000 to 100,000,000 shares. On March 1, 2013, the Company effected a 1-for-4.31 reverse stock split of its issued and outstanding shares of common stock and a proportional adjustment to the existing conversion ratio for each series of redeemable convertible preferred stock and convertible preferred stock. Accordingly, all share and per share amounts for all periods presented in these financial statements and notes thereto have been adjusted retroactively, where applicable, to reflect the reverse stock split and adjustment of the preferred share conversion ratios.

On March 26, 2013 the Company completed an initial public offering of its common stock, which resulted in the sale of 4,600,000 shares. The Company received net proceeds from the IPO of $59,892 based upon the price of $14.00 per share and after deducting underwriting discounts and commissions paid by the Company.

Upon the closing of the initial public offering, all outstanding shares of the Company s redeemable convertible preferred stock and convertible preferred stock were converted into 11,656,875 shares of common stock.

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.

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During the year ended September 30, 2011, the Company reacquired 208,816 shares of restricted common stock that were forfeited by former employees. The Company recorded these shares as treasury stock at their par value.

10. Preferred Stock

Redeemable convertible preferred stock consisted of the following as of September 30, 2012:

	Preferred Shares Authorized	Preferred Shares Issued and Outstanding	Liquidation Preference	Carrying Value	Common Stock Issuable Upon Conversion
Series C redeemable convertible preferred stock	2,543,603	2,543,603	$ 8,551	$ 8,551	590,159
Series D redeemable convertible preferred stock	5,968,334	5,968,334	37,922	37,922	1,853,953
Series E redeemable convertible preferred stock	16,132,546	14,368,037	66,136	66,136	4,386,373
Series F redeemable convertible preferred stock	6,894,966	6,894,966	15,000	15,000	1,599,760
Series G-1 redeemable convertible preferred stock	4,729,543	4,620,764	12,500	12,376	1,072,103
Series G-2 redeemable convertible preferred stock	9,152,296	8,719,639	18,970	18,970	2,023,110
	45,421,288	43,115,343	$ 159,079	$ 158,955	11,525,458

Upon the closing of the initial public offering, all outstanding shares of the Company s redeemable convertible preferred stock were converted into 11,525,458 shares of common stock.

Prior to the conversion, the rights and preferences of the Company s outstanding redeemable convertible preferred stock were as follows:

Voting Rights

Holders of all redeemable convertible preferred stock had the right to vote the number of shares equal to the number of shares of common stock into which such redeemable convertible preferred stock could be converted into on the date for determination of stockholders entitled to vote at a meeting or on the date of any written consent.

Dividends

Holders of the Series C redeemable convertible preferred stock, Series D redeemable convertible preferred stock and Series E redeemable convertible preferred stock were entitled to receive, out of funds legally available, cumulative dividends at an annual rate of 7%, 9% and 9%, respectively, when and if declared by the board of directors. Holders of the Series F redeemable convertible preferred stock and Series G-1 and G-2 redeemable convertible preferred stock were entitled to receive, out of funds legally available, noncumulative dividends at an annual rate of 7%, if declared by the board of directors. No dividends were declared or paid through conversion date.

The Company recorded cumulative dividends and accretion to redemption value through charges to stockholders deficit of $2,526 and $5,367 in fiscal 2013 and 2012, respectively, in connection with these dividend and redemption rights.

Liquidation Preference

In the event of any liquidation, dissolution or winding up of the Company, inclusive of a sale of the Company, a sale of the capital stock representing a majority of the voting power or a merger or consolidation of the Company into or with another corporation in which the existing Company held less than 80% of the voting power of the surviving or resulting corporation, the Series E, Series F and Series G-1 and G-2 redeemable convertible

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preferred stockholders were entitled to receive, in preference to all other stockholders, except for holders of Series 1 Nonconvertible Preferred stock, and to the extent available, an amount equal to the original offering price per share ($2.51, $2.1755, $2.70518 and $2.1755, respectively, adjusted for any stock dividends, stock splits or reclassifications) plus all dividends declared but unpaid.

Holders of Series E redeemable convertible preferred stock were to receive with their preference all cumulative dividends, whether or not declared. In the event that proceeds were not sufficient to permit payment in full to these holders, the proceeds were to be ratably distributed among Series E, F, G-1 and G-2 holders in proportion to the full preferential amount each such holder was otherwise entitled to receive.

After the Series E, Series F and Series G-1 and G-2 redeemable convertible preferred stockholders were paid, and to the extent available, the Series D redeemable convertible preferred stockholders were entitled to receive, in preference to all other stockholders, an amount equal to the original offering price per share ($3.00, adjusted for any stock dividends, stock splits or reclassifications) plus all cumulative Series D dividends, whether or not declared.

After the Series D redeemable convertible preferred stockholders were paid, and to the extent available, the Series C redeemable convertible preferred stockholders were entitled to receive an amount equal to the original offering price per share ($1.72, adjusted for any stock dividends, stock splits or reclassifications) plus all cumulative Series C dividends, whether or not declared.

After payments were made in full to the Series 1 Nonconvertible Preferred stockholders, if any, to the holders of the Series G-1, Series G-2, Series F, Series E, Series D and Series C redeemable convertible preferred stock and to the holders of the Series B and Series A convertible preferred stock, to the extent available, holders of the common stock and holders of the Series C, Series D and Series E redeemable convertible preferred stock were to receive the remaining amounts available for distribution ratably in proportion to the number of common shares held by them or issuable to them upon conversion of their preferred stock into common stock. The distributions were subject to an overall distribution limit of two times the original purchase price per share of the Series C, Series D and Series E redeemable convertible preferred stock.

Conversion

Each share of redeemable convertible preferred stock was convertible into common stock at the option of the stockholder at any time after the date of issuance. Each share of the preferred stock automatically converted into shares of common stock at the applicable Series C, Series D, Series E, Series F and Series G-1 and G-2 redeemable convertible preferred stock conversion ratio upon the closing of the Company s initial public offering on March 26, 2013. The conversion ratio of the Series C, Series D, Series E, Series F and Series G-1 and G-2 redeemable convertible preferred stock as defined was $1.72, $3.00, $2.51, $2.1755, $2.70518 and $2.1755, respectively. The conversion prices of Series C, Series D, Series E, Series F, Series G-1 and G-2 redeemable convertible preferred stock were $7.41320, $9.65772, $8.22176, $9.37641, $11.65933 and $9.37641, respectively. As a result, all outstanding shares of Series D redeemable convertible preferred stock converted into common stock on a 0.31063-for-1 basis, all outstanding shares of Series E redeemable convertible preferred stock converted into common stock on a 0.30529-for-1 basis, and all outstanding shares of Series C, Series F, Series G-1 and G-2 redeemable convertible preferred stock converted into common stock on a 0.23202-for-1 basis.

Redemption Rights

At the written election of the majority of the holders of the Series C, Series D, Series E, Series F or Series G-1 and G-2 redeemable convertible preferred stock, the shares of Series C, Series D, Series E, Series F and Series G-1 and

G-2 redeemable convertible preferred stock outstanding prior to conversion were redeemable in installments commencing December 31, 2013. The carrying values of the Series C, Series D, Series E, Series F and Series G-1 and G-2 redeemable convertible preferred stock were being accreted to their redemption values through their respective redemption dates.

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Reissuance

Shares of any Series C, Series D, Series E, Series F or Series G-1 or G-2 redeemable convertible preferred stock that were redeemed or converted were retired or canceled and are not reissuable by the Company.

Convertible preferred stock, which was not redeemable, consisted of the following as of September 30, 2012:

	Preferred Shares Authorized	Preferred Shares Issued and Outstanding	Liquidation Preference	Carrying Value	Common Stock Issuable Upon Conversion
Series A convertible preferred stock	379,450	379,450	$ 140	$ 140	88,033
Series B convertible preferred stock	187,000	187,000	187	187	43,384
	566,450	566,450	$ 327	$ 327	131,417

Upon the closing of the initial public offering, all outstanding shares of the Company s convertible preferred stock were converted into 131,417 shares of common stock.

Prior to the conversion, the holders of the Convertible Preferred Stock had the following rights and preferences:

Voting Rights

Holders of all convertible preferred stock had the right to vote the number of shares equal to the number of shares of common stock into which such preferred shares could be converted into on the date for determination of stockholders entitled to vote at a meeting or on the date of any written consent.

Dividends

If dividends were declared and paid on the shares of Series A or Series B convertible preferred stock, the Company was required to declare at the same time a dividend payable with respect to the Series C, Series D, Series E, Series F, Series G-1 and G-2 redeemable convertible preferred stock equivalent to the dividend amount they would receive if all convertible preferred shares were converted into common stock. The Company could not pay dividends to the holders or the Series A and Series B convertible preferred stock until all cumulative dividends accrued but unpaid on Series C, Series D, Series E, Series F, Series G-1 and G-2 redeemable convertible preferred stock had been paid in full. Holders of the Series A and B convertible preferred stock were not entitled to receive cumulative dividends.

Liquidation Preference

In the event of any liquidation, dissolution or winding up of the Company, inclusive of a deemed liquidation (defined as a sale of the Company, a sale of the capital stock representing a majority of the voting power, or a merger or consolidation of the Company into or with another corporation of the Company in which the existing Company holds less than 80% of the voting power of the surviving or resulting corporation), after the holders of the Series 1 nonconvertible preferred stock, if any, and redeemable preferred stock had been paid, and to the extent available, holders of Series A and Series B convertible preferred stock were entitled to receive an amount equal to the original offering price per share ($0.37 for Series A convertible preferred stock and $1.00 for Series B convertible preferred

stock, adjusted for any stock dividends, stock splits or reclassifications) plus all dividends declared but unpaid.

Conversion

Each share of Convertible Preferred Stock was convertible into common stock at the option of the holder at any time after the date of issuance. Each share of the convertible preferred stock automatically converted into shares

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of common stock at the applicable conversion ratio upon the closing of the Company s initial public offering on March 26, 2013. All outstanding shares of Series A and Series B convertible preferred stock converted into common stock on a 0.23202-for-1 basis.

Redemption Rights

There were no redemption rights afforded the Series A and Series B convertible preferred stock. The holders of Series A and Series B convertible preferred stock had liquidation rights in the event of a deemed liquidation that, in certain situations, were not solely within the control of the Company. Therefore, the Series A and Series B convertible preferred stock was classified outside of stockholders deficit.

Reissuance

Shares of any Series A or Series B convertible preferred stock that were converted into common stock were retired or canceled and are not reissuable by the Company.

Series 1 Nonconvertible Preferred Stock

The Company s Certificate of Incorporation authorizes the issuance of up to 1,999,989 shares of Series 1 Nonconvertible Preferred Stock (the Series 1 Nonconvertible Preferred) at a par value of $0.01 per share. As of September 30, 2013 and 2012, there were no shares of Series 1 Nonconvertible Preferred issued or outstanding. 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 holders 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 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.

If all outstanding warrants to purchase the Series 1 Nonconvertible Preferred stock are exercised, the resulting outstanding shares of Series 1 Nonconvertible Preferred stock will carry an aggregate $2,000 liquidation preference that is superior to the common stock and any other classes of preferred stock then outstanding.

11. Common Stock Warrants

As of September 30, 2010, warrants to purchase 345,952 shares of common stock were outstanding. The value of these warrants was classified as equity as these warrants were exercisable into common stock only and, as such, would not require a transfer of assets. In October 2010, these warrants expired unexercised. As a result, as of September 30, 2013 and 2012, no common stock warrants were outstanding.

12. Preferred Stock Warrants

Warrants to Purchase Series E Preferred Stock

Warrants for the purchase of Series E redeemable convertible preferred stock (Series E preferred stock) were issued by the Company in fiscal 2002 and fiscal 2004 during various financings. As these warrants were financial

instruments that would require a transfer of assets because of the redemption feature at the option of the holders of the Series E preferred stock, these warrants were classified as liabilities on the Company s balance sheet at September 30, 2012.

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As of September 30, 2011, warrants for the purchase of 341,556 shares of Series E preferred stock were outstanding. During the year ended September 30, 2012, warrants for the purchase of 329,056 shares expired. As of September 30, 2012, warrants for the purchase of 12,500 shares of Series E preferred stock remained outstanding. These warrants expired during the year ended September 30, 2013.

The Company is required to remeasure the fair value of these preferred stock warrants at each reporting date, with any adjustments recorded as other income (expense). The warrants outstanding at each reporting date were remeasured using the Black-Scholes option-pricing model, and the resulting change in fair value was recorded in other income (expense) in the statement of operations. As a result, the Company recorded income of $18 for the year ended September 30, 2011, and expense of $19 for the year ended September 30, 2012, related to the change in fair value of the warrants. The Company also recorded income of $8 for the year ended September 30, 2012 related to the expiration of 329,056 warrants. As of September 30, 2012, the total fair value of the outstanding Series E preferred stock warrants was $20. During the year ended September 30, 2013, the Company recorded income of $20 related to the expiration of the Series E preferred stock warrants.

Warrants to Purchase Series 1 Preferred Stock

In October and November 2010, a total of 1,999,989 warrants to purchase Series 1 nonconvertible preferred stock were issued. These warrants expire on October 4, 2017. As these warrants are free-standing financial instruments that may require the Company to transfer assets upon exercise, these warrants are classified as liabilities. These warrants had a fair value upon issuance of $1,280. The Company is required to remeasure the fair value of these preferred stock warrants at each reporting date, with any adjustments recorded within the change in fair value of warrant liability included in other income (expense) in the statement of operations. The warrants were remeasured at each reporting period, resulting in net income of $361 and $3 for the years ended September 30, 2013 and 2012, respectively. As of September 30, 2013 and 2012, the total fair value of the Series 1 nonconvertible preferred stock warrants was $1,620 and $1,981, respectively.

The following table summarizes the Company s warrant activity since October 1, 2010:

	Common Stock Exercisable	Weighted Average Exercise Price	Series E Preferred Stock Exercisable	Weighted Average Exercise Price	Series 1 Preferred Stock Exercisable	Weighted Average Exercise Price
Outstanding, as of September 30, 2010	345,952	$ 8.40	341,556	$ 2.51		
Granted					1,999,989	
Expired	(345,952)					
Exercised						
Outstanding, as of September 30, 2011			341,556	$ 2.51	1,999,989	$ 0.01
Granted						
Expired			(329,056)			
Exercised						
			12,500	$ 2.51	1,999,989	$ 0.01

Outstanding, as of September 30, 2012			
Granted			
Expired	(12,500)		
Exercised			
Outstanding, as of September 30, 2013	1,999,989	$	0.01

13. Stock-Based Awards

In 2012 the Company s Board adopted the 2012 Equity Incentive Plan (the 2012 Plan), which was subsequently approved by the stockholders and became effective immediately prior to the closing of the Company s initial public offering on March 26, 2013. The 2012 Plan permits the Company to sell or issue

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common stock or restricted common stock, and to grant incentive stock options or nonqualified stock options for the purchase of common stock, restricted stock units, stock appreciation rights or other cash incentive awards, to employees, members of the board of directors and consultants of the Company. The total number of shares of common stock that could be issued under the plan initially was 321,613 shares, plus additional shares from the 1995 Equity Incentive Plan that had not been issued or reserved for future issuance upon exercise of options. The number of shares of common stock that may be issued under the 2012 Plan is also subject to increase on the first day of each fiscal year by the lowest of (i) 3% of the Company s outstanding shares of common stock as of that date, (ii) 2,088,167 shares of common stock, or (iii) an amount determined by the board of directors.

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 common stock or restricted common stock, or to grant 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. The 1995 Plan is administered by the board of directors and exercise prices, vesting and other restrictions are all determined at their discretion.

Options granted under the 1995 Plan to non-executive employees generally vest 25% per year and expire after ten years; options granted to executive employees generally vest over one to four years; and options granted to the board of directors vest over two to three years. The total number of shares of common stock that could be issued under the 1995 Plan was 2,540,759 shares as of September 30, 2012. On January 17, 2013, the Company s stockholders approved an amendment to the 1995 Plan to increase the number of shares of common stock reserved for issuance under the 1995 Plan to 2,772,777 shares. 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, of which 346,038 shares remained available for future grant as of September 30, 2013, and no further awards will be made under the 1995 Plan.

On January 3, 2013, the Company s stockholders approved an Employee Stock Purchase Plan (ESPP). A total of 185,614 shares of common stock are reserved for issuance under this plan, which became effective immediately prior the closing of the Company s initial public offering. As of September 30, 2013, the Company had not commenced any offering under the ESPP and no 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 awards is generally four years, with restrictions lapsing evenly over the period.

In March 2013, the Company granted to certain executives 167,052 options that vest upon the achievement of certain performance-based targets. The fair value of these options at the grant date was $1,487. In April 2013, the terms of the options were modified resulting in an immaterial change in the fair value of the options. Through September 30, 2013, the Company recorded no compensation expense related to these options as none of the performance-based targets was deemed to be probable of being achieved.

Stock Option Valuation

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model. Prior to March 2013 the Company had been a private company and lacked company-specific historical and implied volatility information. Therefore, the Company estimated its expected stock volatility based on the historical volatility of its publicly traded peer companies inclusive of the Company, commencing March 2013 and expects to

continue to do so until such time as it has adequate historical data regarding the volatility of its own traded stock price. The expected term of the Company s stock options has been determined utilizing the simplified method for awards that qualify as plain-vanilla options. The risk-free interest rate is determined

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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. Expected dividend yield is based on the fact that the Company has never paid cash dividends and does not expect to pay any cash dividends in the foreseeable future. The relevant data used to determine the value of the stock option grants is as follows, presented on a weighted average basis:

	Year Ended September 30,		
	2013	2012	2011
Risk-free interest rate	1.05%	0.93%	2.73%
Expected term (in years)	6.09	6.00	6.25
Expected volatility	73%	78%	87%
Expected dividend yield	0%	0%	0%

The Company recognizes 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 stock-based compensation expense in future periods.

As required by the 1995 Plan and 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. For the periods prior to the March 2013 IPO, the Company valued its common stock by taking into consideration its most recently available valuation of common shares performed by management and the board of directors as well as additional factors which may have changed since the date of the most recent contemporaneous valuation through the date of grant. For the periods after the IPO, the Company based fair value of its common stock on the quoted market price.

The following table summarizes stock option activity for the year ended September 30, 2013:

	Shares Issuable Under Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (in years)	Aggregate Intrinsic Value
Outstanding as of September 30, 2012	1,771,471	$ 2.08	4.1	$ 19,911
Granted	364,318	14.21		
Exercised	(538,575)	1.04		
Forfeited	(11,319)	12.54		
Expired	(2,864)	2.59		
Outstanding as of September 30, 2013	1,583,031	$ 5.15	5.0	$ 28,133
Options vested and expected to vest as of September 30, 2013	1,408,344	$ 4.06	4.5	$ 26,568

Options exercisable as of September 30, 2013	1,161,570	$ 2.33	3.5	$ 23,919

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 for those shares that had exercise prices lower than the fair value of the Company s common stock. The aggregate intrinsic value of stock options exercised was $7,574, $606 and $10 during the years ended September 30, 2013, 2012 and 2011, respectively.

The Company received cash proceeds from the exercise of stock options of $559, $141 and $34 during the years ended September 30, 2013, 2012 and 2011, respectively. The weighted average grant date fair value of options granted during the years ended September 30, 2013, 2012 and 2011 was $9.16, $7.79 and $1.95, respectively.

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The Company recorded stock-based compensation expense for the years ended September 30, 2013, 2012 and 2011 in the following expense categories:

	Year Ended September 30,		
	2013	2012	2011
Research and development	$ 393	$ 126	$ 66
General and administrative	670	298	159
	$ 1,063	$ 424	$ 225

As of September 30, 2013, the Company had an aggregate of $1,416 of unrecognized stock-based compensation cost, which is expected to be recognized over a weighted average period of 2.8 years.

14. Net Income (Loss) Per Share

Basic and diluted net income (loss) per share attributable to common stockholders was calculated as follows for the years ended September 30, 2013, 2012 and 2011:

	Year Ended September 30,		
	2013	2012	2011
Basic net income (loss) per share attributable to common stockholders:			
Numerator:			
Net income	$ 9,627	$ 21,399	$ 23,310
Accretion of redeemable convertible preferred stock to redemption value	(2,526)	(5,367)	(5,454)
Net income attributable to participating securities	(13,670)	(14,663)	(16,291)
Net income (loss) attributable to common stockholders	$ (6,569)	$ 1,369	$ 1,565
Denominator:			
Weighted average common shares outstanding basic	9,788,039	1,088,784	1,119,250
Net income (loss) per share attributable to common stockholders basic	$ (0.67)	$ 1.26	$ 1.40

	Year Ended September 30,		
	2013	2012	2011

Diluted net income (loss) per share attributable to common stockholders:			
Numerator:			
Net income	$ 9,627	$ 21,399	$ 23,310
Accretion of redeemable convertible preferred stock to redemption value	(2,526)	(5,367)	(5,454)
Net income attributable to participating securities	(13,670)	(13,225)	(15,401)
Net income (loss) attributable to common stockholders diluted	$ (6,569)	$ 2,807	$ 2,455
Denominator:			
Weighted average common shares outstanding basic	9,788,039	1,088,784	1,119,250
Dilutive effect of common stock equivalents		1,386,039	738,023
Weighted average common shares outstanding diluted	9,788,039	2,474,823	1,857,273
Net income (loss) per share attributable to common stockholders diluted	$ (0.67)	$ 1.13	$ 1.32

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Stock options for the purchase of 1,713,313 weighted average shares were excluded from the computation of diluted net loss per share attributable to common stockholders for the year ended September 30, 2013 because those options had an anti-dilutive impact due to the net loss attributable to common stockholders.

Stock options for the purchase of 33,359 and 594,453 weighted average shares were excluded from the computation of diluted net income per share attributable to common stockholders for the years ended September 30, 2012 and 2011, respectively because those options had an anti-dilutive impact due to the assumed proceeds per share using the treasury stock method being greater than the average fair value of the Company s common shares for those periods.

Warrants outstanding as of September 30, 2011 for the purchase of 341,556 shares of Series E preferred stock were excluded from the calculations of diluted net income per share attributable to common stockholders for the year ended September 30, 2011 because those warrants had an anti-dilutive impact due to the assumed proceeds per share using the treasury stock method being greater than the average fair value of the Company s common shares for that year.

15. Commitments and Contingencies

Leases

During the year ended September 30, 2011, the Company signed a seven-year lease for new office space that began on October 1, 2011. The lease provides for payment escalations during the term of the lease. Payment escalations as specified in the lease agreements are accrued such that rent expense is recognized on a straight-line basis over the terms of occupancy. The Company incurred rent expense of $948, $948 and $1,995, respectively, for the years ended September 30, 2013, 2012 and 2011.

Future minimum lease payments for operating leases as of September 30, 2013 are as follows:

Year ending September 30,	
2014	921
2015	946
2016	972
2017	999
2018	1,027
Total	$ 4,865

The Company previously leased office space in Watertown, Massachusetts under a ten-year lease that expired at the end of September 2011. In connection with the prior office lease, the Company issued a $1,140 letter of credit through November 2011 to the benefit of the landlord, collateralized by a certificate of deposit. On December 2, 2011, the landlord released the letter of credit to the Company. In connection with the new lease, the Company issued a $436 letter of credit, collateralized by a money market account. As of September 30, 2013 and 2012, the Company classified amounts of $436 in each year, as restricted cash relating to this lease.

Intellectual Property Licenses

During the year ended September 30, 2012, in response to correspondence the Company received from a third party related to assets the Company used or may have used in prior periods, the Company entered into a non-exclusive

intellectual property license agreement with the third party. Under the agreement, the Company is required to pay the third party licensor an upfront license fee of $350 and additional license fees of $250 on the first anniversary, $250 on the second anniversary and $200 on the third anniversary of the agreement. In addition, the Company is required to pay (1) annual maintenance fees of $105 for each year that the agreement remains in effect, commencing on the first anniversary of the agreement, in order to maintain the right to use the license, and

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(2) a one-time fee of $50 in each circumstance in which the Company provides the licensed intellectual property to one of its collaborators with the prior consent of the licensor. As a result, during fiscal 2012, the Company recorded in research and development expense a total of $895 of license expense, which represented the fair value of the payments that the Company believed related to its use of the assets in fiscal 2012 and prior periods. As of September 30, 2012, the Company had recorded liabilities totaling $995 related to the agreement. Of that amount, $350 was included in accounts payable, $222 was included in accrued expenses and $423 was included in other long-term liabilities on the balance sheet at September 30, 2012. During the year ended September 30, 2013, the Company paid $350 due under the agreement. As of September 30, 2013, the Company had recorded liabilities totaling $674 in connection with the agreement, of which $250 was included in accounts payable, $240 was included in accrued expenses and $184 was included in other long-term liabilities on the balance sheet.

Future non-cancelable minimum payments under the agreement are as follows:

Year ending September 30,	
2014	250
2015	200
Total	$ 450

As of September 30, 2012, the Company was aware of a potential license it may need to acquire with respect to patents it used, or may have used, in its HCV research during prior years or periods. As of September 30, 2012, the Company believed that license costs were probable, but a range of the loss could not be reasonably estimated; therefore, no accrual for this matter was recorded as of September 30, 2012. During the year ended September 30, 2013, the Company obtained an amendment to an existing license agreement to extend rights to patents previously licensed for one of its programs for use in its other HCV research. Under the amended agreement, the Company is obligated to pay milestones totaling up to $5,000, plus low single digit royalties, for each HCV product it develops and obtains regulatory approval outside of its collaboration with AbbVie or any other collaboration it may enter into in the future with a partner that has already licensed these patents. In the same period, the Company paid a milestone payment of $500 under this amended agreement.

Litigation and Contingencies Related to Use of Intellectual Property

From time to time, the Company may become subject to legal proceedings, claims and litigation arising in the ordinary course of business. The Company currently is not a party to any threatened or pending litigation. However, third parties might allege that the Company or its collaborators are infringing their patent rights or that the Company is otherwise violating their intellectual property rights. Such third parties may resort to litigation against the Company or its collaborators, which the Company has agreed to indemnify. With respect to some of these patents, the Company expects that it will be required to obtain licenses and could be required to pay license fees or royalties, or both. These licenses may not be available on acceptable terms, or at all. A costly license, or inability to obtain a necessary license, could have a material adverse effect on the Company s financial condition, results of operations or cash flows. The Company accrues contingent liabilities when it is probable that future expenditures will be made and such expenditures can be reasonably estimated.

Indemnification Agreements

In the ordinary course of business, the Company may provide indemnifications of varying scope and terms to customers, vendors, lessors, business partners, and other parties with respect to certain matters including, but not limited to, losses arising out of breach of such agreements, from services to be provided by the Company, or from intellectual property infringement claims made by third parties. In addition, the Company has entered into indemnification agreements with members of its board of directors that will require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as directors or officers. The maximum potential amount of future payments the Company could be required to make under

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these indemnification agreements is, in many cases, unlimited. To date, the Company has not incurred any material costs as a result of such indemnifications. In addition, the Company maintains officers and directors insurance coverage. The Company does not believe that the outcome of any claims under indemnification arrangements will have a material effect on its financial position, results of operations or cash flows, and it has not accrued any liabilities related to such obligations in its financial statements as of September 30, 2013 or 2012.

16. Income Taxes

During the years ended September 30, 2013, 2012 and 2011, no provision for income taxes was recorded due primarily to the Company s use of net operating loss carryforwards to fully offset the income before income taxes of $9,627, $21,399 and $21,310, respectively, generated in those periods. As the deferred tax assets for those utilized net operating loss carryforwards had previously been recorded with a full valuation allowance, the use of the net operating loss carryforwards in each of those periods resulted in an income tax benefit being recognized, which substantially offset the tax provision recorded as a result of the income before income taxes generated.

In all periods presented, all income before income taxes was sourced from the United States.

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

	Year Ended September 30,		
	2013	2012	2011
Federal statutory income tax rate	34.0%	34.0%	34.0%
State taxes, net of federal benefit	5.5	5.4	5.8
Federal research and development tax credit	(2.5)	(0.2)	(2.0)
Change in deferred tax asset valuation allowance	(51.0)	(40.1)	(40.5)
Other	14.0	0.9	2.7
Effective income tax rate	0.0%	0.0%	0.0%

Net deferred tax assets as of September 30, 2013 and 2012 consisted of the following:

	September 30,	
	2013	2012
Net operating loss carryforwards	$ 8,495	$ 10,961
Tax credit carryforwards	6,111	5,731
Capitalized research and development expenses	6,953	9,750
Other temporary differences	1,333	1,357
Gross deferred tax assets	22,892	27,799
Valuation allowance	(22,892)	(27,799)
Net deferred tax assets	$	$

As of September 30, 2013, the Company had net operating loss carryforwards for federal and state income tax purposes of $24,107 and $5,653, respectively, which begin to expire in fiscal 2018 and 2015, respectively. The Company also has available research and development tax credit and other credit carryforwards for federal and state income tax purposes of $4,375 and $2,631, respectively, which begin to expire in fiscal 2021 and 2016, respectively. Utilization of the net operating loss carryforwards and research and development tax credit carryforwards may be subject to a substantial annual limitation under Section 382 of the Internal Revenue Code of 1986 due to ownership changes that could occur in the future. These ownership changes may limit the amount of carryforwards that can be utilized annually to offset future taxable income.

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During the years ended September 30, 2013, 2012 and 2011, gross deferred tax assets related to net operating loss carryforwards decreased due to their utilization as a result of income before income taxes generated during those periods.

The Company has concluded, based on the weight of available evidence, that its net deferred tax assets are not more likely than not to be realized in the future. Management has considered the Company s history of cumulative net losses incurred since inception, its lack of commercialization of any products or generation of any revenue from product sales since inception and its inability to reasonably estimate the timing or amounts, if any, of future milestone payments it will receive from its collaborators, and concluded that it is more likely than not that the Company will not realize the benefits of the deferred tax assets. Accordingly, a full valuation allowance has been established against the deferred tax assets as of September 30, 2013 and 2012. Management reevaluates the positive and negative evidence at each reporting period.

Changes in the valuation allowance for deferred tax assets during the years ended September 30, 2013, 2012 and 2011 were as follows:

	Year Ended September 30,		
	2013	2012	2011
Valuation allowance as of beginning of year	$ 27,799	$ 36,369	$ 45,812
Decreases recorded as benefit to income tax provision	(4,907)	(8,570)	(9,443)
Increases recorded to income tax provision			
Valuation allowance as of end of year	$ 22,892	$ 27,799	$ 36,369

The Company has not recorded any amounts for unrecognized tax benefits as of September 30, 2013 or 2012.

The Company files tax returns as prescribed by the tax laws of the jurisdictions in which it operates. In the normal course of business, the Company is subject to examination by federal and state jurisdictions, where applicable. There are currently no pending income tax examinations. The Company s tax years are still open under statute from 2009 to the present. Earlier years may be examined to the extent that tax credit or net operating loss carryforwards are used in future periods. The Company s policy is to record interest and penalties related to income taxes as part of its income tax provision.

17. 401(k) Plan

In December 1999, the Company established a 401(k) plan. This plan covers substantially all employees who meet minimum age and service requirements. Under the terms of the plan, the Company contributes on an annual basis up to 2% of an employee s base salary up to a maximum of $4 per employee.

During the years ended September 30, 2013, 2012 and 2011, the Company recognized $90, $84 and $95, respectively, of expense related to its contributions to this plan.

18. Related Party Transactions

The Company has entered into consulting agreements for research and development and business management activities with certain members of the Company s board of directors. Consulting fees expensed and paid for each of the

years ended September 30, 2013, 2012 and 2011 were $10, $75 and $75.

As further described in Note 7, the Company had entered into a note and warrant purchase agreement with stockholders in fiscal 2011.

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19. Qualifying Therapeutic Discovery Project Program

In November 2010, the Company received notification from the Internal Revenue Service that it had been awarded grants for four research projects under the Qualifying Therapeutic Discovery Project Credit program, covering 50% of qualifying expenses incurred, up to a maximum of $244 for each of the four projects. During the year ended September 30, 2011, the Company recorded the proceeds of $750 in other income (expense) in the statement of operations, upon approval of the qualifying expenses.

A payment of $489 was received in fiscal 2011 for work on two of the projects already carried out during fiscal 2010. For the two projects carried out during fiscal 2011, a total amount receivable of $261 was recorded as of September 30, 2011, and was received in full in October 2011.

20. Selected Quarterly Financial Data (unaudited)

Quarterly financial information for fiscal 2013 and 2012 is presented in the following table, in thousands, except per share data:

	2013 Quarter Ended			
	December 31, 2012	March 31, 2013	June 30, 2013	September 30, 2013
Revenue	$ 27,859	$ 1,196	$ 1,649	$ 1,349
Operating expenses	5,950	5,197	5,827	6,050
Other income (expense), net	48	252	40	258
Net income (loss)	21,957	(3,749)	(4,138)	(4,443)
Net income (loss) per share attributable to common shareholders basic	$ 1.61	$ (2.28)	$ (0.23)	$ (0.25)
Net income (loss) per share attributable to common shareholders diluted	$ 1.45	$ (2.28)	$ (0.23)	$ (0.25)

	2012 Quarter Ended			
	December 31, 2011	March 31, 2012	June 30, 2012	September 30, 2012
Revenue	$ 741	$ 36,565	$ 2,542	$ 1,858
Operating expenses	3,923	4,470	6,070	5,954
Other income (expense), net	23	16	27	44
Net income (loss)	(3,159)	32,111	(3,501)	(4,052)
Net income (loss) per share attributable to common shareholders basic	$ (4.44)	$ 2.41	$ (4.31)	$ (4.78)
Net income (loss) per share attributable to common shareholders diluted	$ (4.44)	$ 2.17	$ (4.31)	$ (4.78)

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EXHIBIT INDEX

Exhibit Number	Exhibit Description	Incorporated by Reference				Filed Herewith
		Form	Date	Exhibit Number	File Number	
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.	8-K	03/28/2013	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	
10.1#	Form of Indemnification Agreement for directors and officers.	S-1/A	02/05/2013	10.7	333-184779	
10.2#	Amended and Restated Employment Agreement between the Company and Jay R. Luly, Ph.D., dated as of March 4, 2013.	S-1/A	03/05/2013	10.5	333-184779	
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	
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).	S-1/A	02/05/2013	10.1	333-184779	
10.5	Collaboration and License Agreement between the Company and Novartis Institutes for BioMedical Research, Inc., dated February 16, 2012.	S-1/A	03/05/2013	10.2	333-184779	
10.6	Amendment No. 1, dated March 28, 2013, to that certain Collaboration and License Agreement between Enanta Pharmaceuticals, Inc. and Novartis Institutes for BioMedical Research, Inc.	10-Q	05/15/2013	10.2	001-35839	
10.7	Agreement between the Company and the National Institute of Allergy and	S-1	11/06/2012	10.3	333-184779	

	Infectious Diseases, dated September 30, 2011.	
10.8	Modification No. 1, dated August 28, 2013, to that certain Agreement between the Company and the National Institute of Allergy and Infectious Diseases.	X

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Exhibit Number	Exhibit Description	Incorporated by Reference				Filed Herewith
		Form	Date	Exhibit Number	File Number	
10.9	Modification No. 2, dated August 29, 2013, to that certain Agreement between the Company and the National Institute of Allergy and Infectious Diseases.					X
10.10	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	
10.11	Third Amended and Restated Registration Rights Agreement, dated as of August 23, 2012.	S-1/A	11/06/2012	10.4	333-184779	
10.12#	Amended and Restated 1995 Equity Incentive Plan.	S-1/A	03/05/2013	10.8	333-184779	
10.13#	Form of Incentive Stock Option Certificate under Amended and Restated 1995 Equity Incentive Plan.	S-1/A	03/05/2013	10.9	333-184779	
10.14#	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	
10.15#	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	
10.16#	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).					X
10.17#	Form of Incentive Stock Option Agreement under 2012 Equity Incentive Plan.	S-1/A	03/05/2013	10.13	333-184779	
10.18#	Form of Non-Statutory Stock Option Agreement under 2012 Equity Incentive Plan.	S-1/A	03/05/2013	10.14	333-184779	
10.19#	Form of Non-Statutory Stock Option Certificate for directors under 2012 Equity Incentive Plan.	S-1/A	03/05/2013	10.15	333-184779	
10.20#	Employee Stock Purchase Plan.	S-1/A	02/05/2013	10.16	333-184779	
21.1	Subsidiaries of the Company.					X
23.1	Consent of PricewaterhouseCoopers LLP, Independent Registered Public Accounting Firm.					X
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

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Exhibit Number	Exhibit Description	Incorporated by Reference				Filed Herewith
		Form	Date	Exhibit Number	File Number	
101	The following materials from the Annual Report of Enanta Pharmaceuticals, Inc. on Form 10-K for the year ended September 30, 2013, formatted in XBRL (eXtensible Business Reporting Language): (i) Balance Sheets as of September 30, 2013 and September 30, 2012 of Enanta Pharmaceuticals, Inc., (ii) Statements of Operations for the years ended September 30, 2013 and 2012 of Enanta Pharmaceuticals, Inc., (iii) Statements of Cash Flows for the years ended September 30, 2013 and 2012 of Enanta Pharmaceuticals, Inc., and (iv) Notes to Financial Statements of Enanta Pharmaceuticals, Inc.[1]					

[#] 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.

[1] Pursuant to Rule 406T of Regulation S-T, the Interactive Data Files in Exhibit 101 hereto are deemed not filed or part of a registration statement or prospectus for purposes of Sections 11 or 12 of the Securities Act of 1933, as amended, are deemed not filed for purposes of Section 18 of the Securities and Exchange Act of 1934, as amended, and otherwise are not subject to liability under those sections.