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 December 31, 2017

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

Commission File Number: 001-33004

ACER THERAPEUTICS INC.

(Exact Name of Registrant as Specified in Its Charter)

Texas	**76-0333165**
(State or Other Jurisdiction of Incorporation or Organization)	**(IRS Employer Identification No.)**
One Gateway Center, Ste. 351, 300 Washington St., Newton MA	**02458**
(Address of Principal Executive Offices)	**(Zip Code)**

Registrant's Telephone Number, Including Area Code: (844) 902-6100

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

Title of Each Class	**Name of Each Exchange on Which Registered**
Common Stock, $.01 par value per share	The Nasdaq Stock Market LLC
Warrants to purchase common stock	The Nasdaq Stock Market LLC

Securities registered pursuant to Section 12(g) of the Act: None

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

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

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

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

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

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

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

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

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

The aggregate market value of the voting and non-voting common equity held by non-affiliates of the registrant as of June 30, 2017, based upon the closing price as of such date was $4,951,912.

As of March 1, 2018, 7,497,433 shares of the registrant's common stock, par value $0.01 per share, were outstanding.

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Unless otherwise indicated, references in this report to "Acer," "the Company," "we," "us" and "our" refer to the business of Acer Therapeutics Inc. "ACER THERAPEUTICS," "EDSIVO" and the Acer logo are trademarks of Acer Therapeutics Inc. We do not intend our use or display of other companies' trade names, trademarks or service marks to imply relationships with, or endorsements or sponsorship of us by, these other companies.

Forward-Looking Statements

This Annual Report on Form 10-K contains forward-looking statements which are made pursuant to the safe harbor provisions of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. Statements contained in this report, other than statements of historical fact, constitute "forward-looking statements." The words "expects," "believes," "hopes," "anticipates," "estimates," "may," "could," "intends," "exploring," "evaluating," "progressing," "proceeding" and similar expressions are intended to identify forward-looking statements.

These forward-looking statements do not constitute guarantees of future performance. Investors are cautioned that statements which are not strictly historical statements, including, without limitation, statements regarding current or future financial payments, costs, returns, royalties, performance and position, plans and objectives for future operations, plans and objectives for product development, plans and objectives for present and future clinical trials and results of such trials, plans and objectives for regulatory approval, litigation, intellectual property, product development, manufacturing plans and performance, management's initiatives and strategies, and the development of our product candidates, including EDSIVO™ (celiprolol) and ACER-001, constitute forward-looking statements. Such forward-looking statements are subject to a number of risks and uncertainties that could cause actual results to differ materially from those anticipated. These risks and uncertainties include, but are not limited to, those risks discussed in "Risk Factors," as well as, without limitation, risks associated with:

- the strategies, prospects, plans, expectations and objectives of management for future operations, including the anticipated timing of filings;
- the progress, scope or duration of the development of product candidates or programs;
- the benefits that may be derived from product candidates or the commercial or market opportunity in any target indication;
- our ability to protect our intellectual property rights;
- our anticipated operations, financial position, costs or expenses;
- statements regarding future economic conditions or performance;
- statements concerning proposed new products, services or developments;
- the expected benefits of and potential value created by the September 19, 2017 merger with Acer Therapeutics Inc., a Delaware corporation (the "Merger"), for our shareholders; and
- statements of belief and any statement of assumptions underlying any of the foregoing.

These forward-looking statements speak only as of the date made. We assume no obligation or undertaking to update any forward-looking statements to reflect any changes in expectations with regard thereto or any change in events, conditions or circumstances on which any such statement is based. You should, however, review additional disclosures we make in the reports we file with the Securities and Exchange Commission, or SEC.

PART I

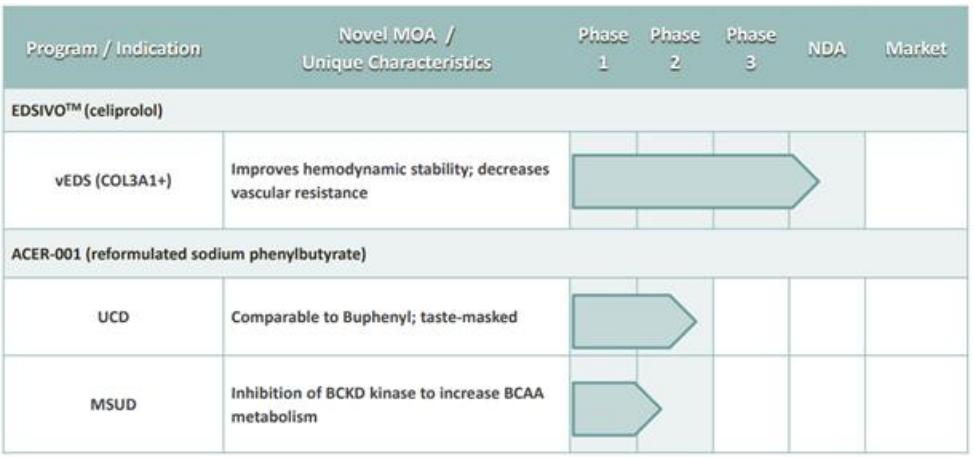
Item 1. Business.

Unless otherwise indicated, we use "Acer," "the Company," "we," "our" and "us" to refer to the business of Acer Therapeutics Inc.

Overview

We are a pharmaceutical company focused on the acquisition, development, and commercialization of therapies for patients with serious rare and ultra-rare diseases with critical unmet medical need. Our late-stage clinical pipeline includes two candidates for severe genetic disorders: EDSIVO™ (celiprolol) for vascular Ehlers-Danlos syndrome, or vEDS, and ACER-001 (a fully taste-masked, immediate release formulation of sodium phenylbutyrate) for urea cycle disorders, or UCD, and Maple Syrup Urine Disease, or MSUD. There are no FDA-approved drugs for vEDS and MSUD and limited options for UCD, which collectively impact approximately 7,000 patients in the United States. The therapies we are developing have clinical proof-of-concept and mechanistic differentiation, and we intend to seek approval for them in the United States by using the regulatory pathway established under section 505(b)(2) of the Federal Food, Drug and Cosmetic Act, or FFDCA, that allows an applicant to rely at least in part on third-party data for approval, which may expedite the preparation, submission, and approval of a marketing application.

Our current product candidate pipeline is summarized in the graphic below:



- NDA submission for EDSIVO™ expected by the end of the first half of 2018.
- NDA submissions for ACER-001 anticipated in 2019 and 2021, subject to our ability to obtain sufficient capital resources.

Our Strategy

Our goal is to become a leading pharmaceutical company that acquires, develops and commercializes therapies for the treatment of rare and ultra-rare diseases with significant unmet medical need. The key elements of our strategy include:

- focus on orphan and ultra-orphan diseases with significant unmet need;
- accelerate development timelines and lower costs, while reducing risk;

- provide differentiated products that create value;
- protect our assets via intellectual property protections and regulatory and market exclusivities; and
- commercialize our products in geographies that make strategic sense.

We plan to continue evaluating external opportunities to acquire or license product candidates in order to enhance our pipeline and leverage our business development, clinical development, regulatory and commercial expertise. We believe our management team has the capability and experience to continue to execute this business model.

Product Candidates

EDSIVO™

Background

Our most advanced product candidate is EDSIVO™ (celiprolol) for the treatment of vEDS. EDSIVO™ is a selective adrenergic modulator and a New Chemical Entity ("NCE") in the United States. Celiprolol is currently approved in the European Union for the treatment of hypertension and angina. Ehlers-Danlos syndrome, or EDS, is an inherited disorder caused by mutations in the genes responsible for the structure, production, or processing of collagen, an important component of the connective tissues in the human body, or proteins that interact with collagen. EDS is a spectrum disorder where patients present with various forms, the most serious of which is vEDS, also known as EDS type IV, which is generally caused by a mutation in the COL3A1 gene. vEDS causes abnormal fragility in blood vessels, which can give rise to aneurysms, abnormal connections between blood vessels known as arteriovenous fistulas, arterial dissections, and spontaneous vascular ruptures, all of which can be potentially life-threatening. Gastrointestinal and uterine fragility or rupture also commonly occur in vEDS patients. Spontaneous arterial rupture has a peak incidence in the third or fourth decade of life in vEDS patients, but may occur earlier and is the most common cause of sudden death in vEDS patients. Arterial rupture or dissection events occur in 25% of patients before the age of 20 but increase to 90% of patients by the age of 40. The median survival age of vEDS patients in the United States is 51 years, with arterial rupture being the most common cause of sudden death.

Pregnancy-related complications also occur in women with vEDS and include arterial dissection or rupture, uterine rupture, hemorrhage, premature rupture of membranes, lacerations, and complications during and after surgery.

Diagnosis and Incidence

vEDS is diagnosed through clinical observation, which is usually confirmed by mutational analysis of the COL3A1 gene. In the absence of a family history of the disorder, however, most vEDS patients are not diagnosed until the occurrence of an arterial aneurysm or dissection, bowel perforation, or organ rupture. As a result, it has been difficult to precisely measure the incidence or prevalence of vEDS in any population. Studies estimate the prevalence of vEDS as ranging from approximately 1 in 90,000 to 1 in 250,000. In 2017, we commissioned a patient-finder study that phenotypically identified 4,169 vEDS patients in the United States from an analysis of a commercially available patient claims database, with data of over 180 million unique patient lives. Based on that information, we estimate the prevalence of phenotypically-defined vEDS in the United States could be greater than 1 in 45,000.

Current Treatment Options for vEDS

Currently, there are no approved pharmacologic therapies in the United States or the European Union for the treatment of vEDS. Medical intervention for vEDS focuses on surgery, symptomatic treatment, genetic counseling and prophylactic measures, such as avoiding intense physical activity, scuba diving, and violent sports. Arterial, digestive or uterine complications in vEDS patients typically require immediate hospitalization, observation in an intensive care unit, and sometimes surgery. Pregnant women with vEDS are considered to be at risk and receive special care.

While vEDS patients are encouraged to take steps to minimize the chances of an arterial rupture or dissection, there are no pharmacologic options to reduce the likelihood of such an event, and accordingly all current treatments for vEDS focus on the repair of arterial ruptures or dissection. Therefore, patients must adopt a "watch and wait" approach following any confirmed diagnosis. Unfortunately, many of these arterial events have high mortality associated with them, and thus, a pharmacologic intervention that reduces the rate of events would be clinically meaningful.

EDSIVO™ for Treatment of vEDS

Rationale for EDSIVO™ Treatment in vEDS

In 2004, researchers at Assistance Publique—Hôpitaux de Paris, Hôpital Européen Georges Pompidou, or AP-HP, in Paris, France, published data on vEDS patients, observing that an abnormally low intima-media thickness generates a higher wall stress than in control subjects at the site of an elastic artery, which may increase the risk of arterial dissection and rupture. Based on this observation, the investigators aimed to assess the preventive effect of celiprolol for major cardiovascular events in patients with vEDS via a multicenter, prospective, randomized, open trial with blinded evaluation of clinical events, which is referred to herein as the Ong trial, and was published on October 30, 2010 in The Lancet. The Ong trial was funded by the French Ministry of Health, and the principal investigator for the study was Professor Pierre Boutouyrie.

Fifty-three participants were enrolled in the Ong trial and randomized at eight centers in France and one center in Belgium. Patient ages ranged from 15 to 65 (with a mean age of 35), with a female-to-male ratio of 2-to-1. Patients were randomly assigned to a five-year intervention, receiving either celiprolol or no treatment, with important phenotype characteristics equally balanced between the celiprolol group and the control group. Celiprolol was administered twice daily to patients in the celiprolol group and the dosage was up-titrated every six months by 100 milligrams per day to a maximum of 400 milligrams per day. Patients assigned to the control group received the same attention as those assigned to the celiprolol group but did not receive celiprolol or any beta blocker. Thirty-three of the 53 patients participating in the study had proven mutations in the COL3A1 gene. Of those patients with proven mutations, demographic and arterial characteristics did not differ from those of the study population as a whole. The duration of follow-up was five years or until the first qualifying cardiac or arterial event. The primary endpoint was a composite of cardiac or arterial events (rupture or dissection, fatal or not) during follow-up. Secondary endpoints were gastrointestinal or uterine rupture. The study was ended early after a consensus decision of the safety monitoring board, the methodologist of AP-HP, and the principal investigator because significant differences were recorded between the treatment group and the control group after 64 months. Mean duration of follow-up was 47 months prior to trial halt. As described in the tables below, in 5 of 25 patients on celiprolol a primary endpoint was recorded, compared with 14 of 28 patients in the control group. The hazard ratio, or HR, for event-free survival, was 0.36, (95% CI 0.15—0.88; p=0.040), meaning that with celiprolol the risk of having a cardiac or arterial event was reduced by 64% compared to control. Combined primary and secondary endpoints affected 6 patients on celiprolol and 17 patients in the control group, (HR 0.31; 95% CI 0.14—0.71; p=0.010):

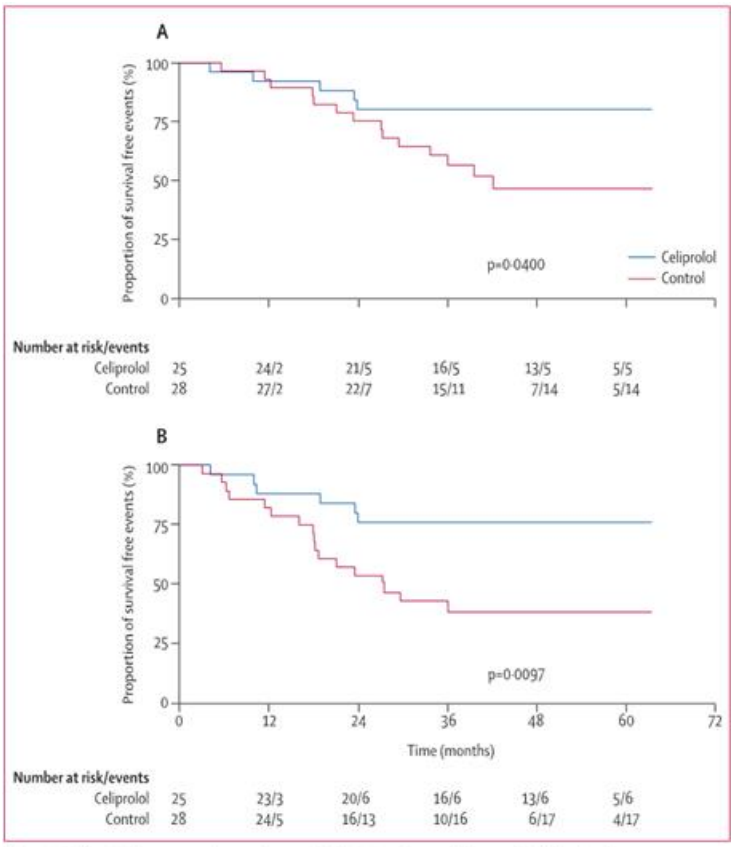


Figure 2: **Kaplan-Meier curves of event-free survival in 53 patients with vascular Ehlers-Danlos**
Primary endpoint (A). Primary and secondary endpoints (B).

As described in the table below, in the 33 patients with COL3A1 mutations, the primary endpoint was noted in 2 of the 13 patients in the treatment group, compared with 11 of the 20 patients in the control group, (HR 0.24; 95% CI 0.08—0.71; p=0.041). Combined primary and secondary endpoints were recorded in 3 of 13 patients on celiprolol and 14 of the patients in the control group, (HR 0.25; 95% CI 0.10—0.64; p=0.017), correlating to a three times reduction in arterial events among treated patients compared to non-treated patients. The results in the trial did not vary significantly between those patients who had a confirmed mutation in the COL3A1 gene versus the overall 53-patient population:

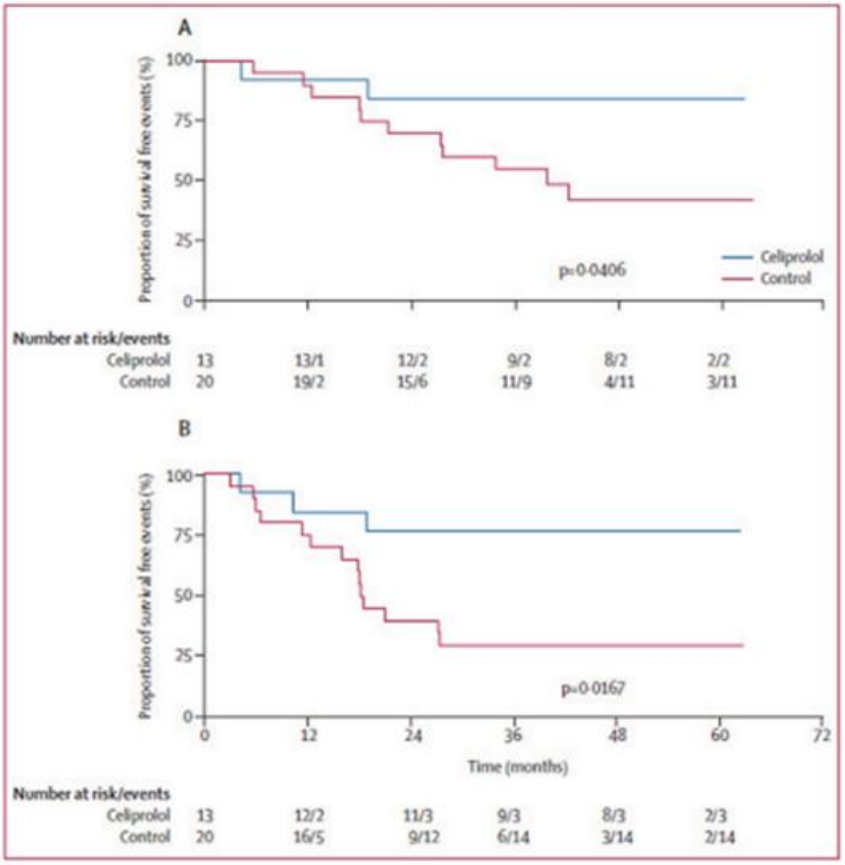


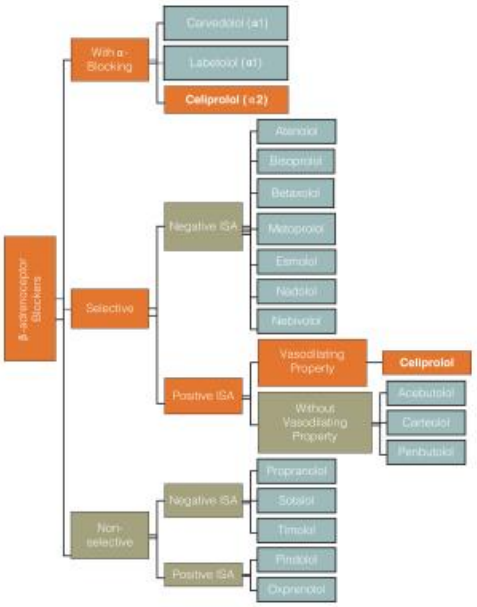
Figure 3: **Kaplan-Meier curves of event-free survival in 33 patients with positive COL3A1 mutation**

Primary endpoint (A). Primary and secondary endpoints (B).

AP-HP granted us an exclusive right to access and use the data generated by the Ong trial. We have conducted a retrospective, source-verified analysis of that data, including the primary and secondary endpoints, which confirmed the published results of the Ong trial.

The precise mechanism of action of celiprolol in vEDS patients is not known. Celiprolol is a cardioselective beta1 antagonist, with beta2 agonist vasodilatory properties, meaning it demonstrates antihypertensive and antianginal activity; however, it lacks the typical side effects of beta1 antagonists, such as bronchoconstriction, depression of left ventricular function, and peripheral vasoconstriction, likely a result of its beta2 agonist activity. Accordingly, investigators initially surmised that celiprolol would reduce central blood pressure and thus mechanical load on collagen fibers within the arterial wall, thereby reducing the risk of arterial dissection and rupture. However, celiprolol did not decrease brachial blood pressure or heart rate. Moreover, systolic and pulse pressures increased after treatment, which is consistent with findings of celiprolol treatment in normotensive individuals, or individuals with normal blood pressure. Celiprolol's lack of blood pressure lowering in normotensive people was explained by its beta2-adrenoceptor agonist properties. The impact of the beta1 antagonist and beta2 agonist properties of celiprolol are known to vary among individuals with high blood pressure and those with normal blood pressure. In individuals with high blood pressure, the beta1 antagonist properties predominate resulting in a reduction in blood pressure; however, in mildly hypertensive or healthy individuals, the beta2 agonist properties predominate, which does not induce a reduction in blood pressure. Most of the subjects enrolled in the Ong trial had normal blood pressure at inclusion, and thus, the protective effect of celiprolol was found unlikely to be through blood pressure lowering. Researchers have hypothesized that another possible mechanism to explain the benefit patients experienced while treated with celiprolol in the Ong trial is the strong associations between beta-adrenergic receptors and transforming growth factor, or TGF, beta pathways. Chronic stimulation of beta2 receptors might enhance collagen synthesis through increased expression of TGF-beta. Thus, in response to celiprolol, an increase of collagen synthesis might have strengthened the arterial, gastrointestinal, and/or uterine walls, thereby reducing its susceptibility to rupture.

We do not believe that there are any other drugs approved or in development in the United States or Europe that have a similar mechanism of action to celiprolol:



Registration Plan

Celiprolol has not been approved for any indication in the United States. Celiprolol has been approved for the treatment of hypertension in the European Union since 1984. An NDA for celiprolol for the treatment for hypertension was submitted to the FDA by Rorer (subsequently acquired by Aventis Pharma SA, or Aventis) in June 1987 but was withdrawn prior to FDA review and therefore never approved. We have obtained from Aventis the exclusive right to reference the celiprolol data included in the marketing authorization dossier filed with and approved by the U.K. Medicines and Healthcare Products Regulatory Agency, or MHRA. We have also licensed from AP-HP exclusive worldwide rights to the data from the Ong trial.

We intend to seek FDA approval for celiprolol for the treatment of vEDS by submitting an NDA under Section 505(b)(2) of the FFDCA, referred to as a 505(b)(2) NDA, where at least some of the information required for approval comes from studies not conducted by or for the applicant and for which the applicant has not obtained a right of reference or use. The FDA interprets this to mean that an applicant may rely for approval on data in published literature or on the FDA's finding of safety or effectiveness of a previously approved drug product owned by a third party. We anticipate the use of third-party data would minimize the amount of original data we would be required to generate and shorten the time needed for preparation, submission, and review of the marketing applications.

In September 2015, we met with the FDA to discuss the existing clinical data for EDSIVO™. At that meeting, the FDA agreed that an additional clinical trial is not likely needed and stated that we may submit a 505(b)(2) NDA for EDSIVO™ for the treatment of vEDS. The FDA indicated to us at that time that it expected that the 505(b)(2) NDA for EDSIVO™ is likely to qualify for priority review. Priority review provides an expedited six-month review cycle after acceptance of the NDA for filing, instead of the traditional ten-month review cycle, for drugs that treat a serious condition and demonstrate the potential to be a significant improvement in safety or effectiveness of the treatment, prevention, or diagnosis of the condition. The FDA determines whether an application will receive priority review at the time the application is accepted for filing.

In May 2017, we held a Type C meeting with the FDA to discuss non-clinical and manufacturing data, and proactively identify whether there were any gaps for us to address in advance of a pre-NDA meeting. In our non-clinical data package, we are addressing a potential preclinical gap by conducting in vitro drug-drug interaction studies, which were missing from the Aventis MHRA dossier. We also reached agreement with the FDA regarding Chemistry, Manufacturing and Controls (CMC) specifications. Furthermore, the FDA provided us with additional guidance on the expected presentation of the existing clinical data for EDSIVO™ to support the NDA filing.

We plan to have a pre-NDA meeting, which may consist of one or more consults, with the FDA in the second quarter of 2018. Subsequently, we expect to submit the 505(b)(2) NDA for EDSIVO™ for the treatment of vEDS at the end of the first half of 2018.

ACER-001

Background

Sodium phenylbutyrate, or NaPB, is currently approved in the United States and the European Union to treat patients with UCD. Our product candidate ACER-001 is a fully taste-masked, immediate release formulation of NaPB, designed to treat urea cycle disorders, or UCD, and Maple Syrup Urine Disease, or MSUD.

Urea Cycle Disorders (UCD) Background

The urea cycle is a series of biochemical reactions that occur primarily in the liver, which converts toxic ammonia produced by the breakdown of protein and other nitrogen-containing molecules in the human body into urea for excretion. UCD are a group of disorders caused by genetic mutations that result in a deficiency in one of the six enzymes that catalyze the urea cycle, which can lead to an excess accumulation of ammonia in the bloodstream, a condition known as hyperammonemia. Acute hyperammonemia can cause lethargy, somnolence, coma, and multi-organ failure, while chronic hyperammonemia can lead to headaches, confusion, lethargy, self-chosen vegetarian diet, failure to thrive, behavioral changes, and learning and cognitive deficits. Common symptoms of both acute and chronic hyperammonemia also include seizures and psychiatric symptoms.

Diagnosis and Incidence

The diagnosis of UCD is based on clinical observations, confirmed by biochemical and molecular genetic testing. A plasma ammonia concentration of 150 lmol/L or higher associated with a normal anion gap and a normal plasma glucose concentration is an indication for the presence of UCD. Plasma quantitative amino acid analysis and measurement of urinary orotic acid can distinguish between the various types of UCD. A definitive diagnosis of UCD depends on either molecular genetic testing or measurement of enzyme activity. Molecular genetic testing is possible for all urea cycle defects. Studies suggest that the incidence of UCD in the United States ranges between 1 in 35,000 live births to 1 in 8,200 live births. Approximately 2,000 patients suffer from UCD in the United States.

Current Treatment Options for UCD

The current treatment of UCD consists of dietary management to limit ammonia production in conjunction with medications that provide alternative pathways for the removal of ammonia from the bloodstream. Dietary protein must be carefully monitored and some restriction is necessary; too much dietary protein causes excessive ammonia production. However, if protein intake is too restrictive or insufficient calories are provided, the body will break down lean muscle mass to obtain the amino acids or energy it requires, which can also lead to excessive ammonia in the bloodstream. Dietary management may also include supplementation with special amino acid formulas developed specifically for UCD, which can be prescribed to provide approximately 50% of the daily dietary protein allowance. Some patients may also require individual branched-chain amino acid supplementation.

Medications for UCD primarily comprise nitrogen scavenger drugs, which are substances that provide alternative excretion pathways for nitrogen by bypassing the urea cycle. The use of these alternative pathways for nitrogen removal is important for the management of acute episodes of hyperammonemia and are also included as part of a long-term treatment regime for UCD patients. Current nitrogen scavenger treatments for UCD are based on sodium benzoate or phenylbutyrate, which conjugate with glycine and glutamine, respectively, allowing for urinary excretion of nitrogen as hippurate and phenylacetylglutamine, respectively.

According to a 2016 study by Shchelochkov et al., published in Molecular Genetics and Metabolism Reports, while nitrogen scavenging medications are effective in helping to manage UCD, non-compliance with treatment is common. Reasons given for non-compliance include the unpleasant taste associated with available medications, the frequency with which medication must be taken and the high cost of the medication.

Phenylbutyrate is available as both NaPB, which is marketed as Buphenyl®, and glycerol phenylbutyrate, which is marketed as Ravicti®. While a study provided by Horizon Therapeutics, Inc. in the Ravicti package insert involving 46 adults with UCD demonstrated that Buphenyl and Ravicti were similarly effective in controlling the blood level of ammonia over a 24-hour period, many patients who take their medicine orally prefer Ravicti, as it is significantly more palatable than Buphenyl. However, the cost of Ravicti—approaching $600,000 to $800,000 per patient per year (based on patient weight)—is often prohibitive.

In cases where dietary management or medication is not effective, patients with UCD may require a liver transplant.

ACER-001 for Treatment of UCD

Rationale for ACER-001 Treatment in UCD

ACER-001 is a proprietary, immediate release, taste-masked suspension formulation of NaPB. Buphenyl, a non-taste-masked formulation of NaPB, has been approved by the FDA for UCD with demonstrated efficacy and safety in UCD patients of all ages. We believe that if it is approved, ACER-001's taste-masked properties will make it a compelling alternative to existing phenylbutyrate-based treatments, as the unpleasant taste associated with NaPB is cited as a major impediment to patient compliance with those treatments.

Registration Plan

We intend to initially seek FDA approval to market ACER-001 in the United States using a 505(b)(2) NDA through which we may reference data from an application previously approved by the FDA. We also intend to seek approval in the European Union and potentially other territories outside the United States, after the 505(b)(2) NDA for treatment of UCD is filed. Because the FDA has approved an NDA for NaPB, which is referred to as the reference listed drug, or RLD, we intend to rely on the RLD's pre-clinical and clinical safety data, while supplementing the data with a bridging study that demonstrates bioequivalence of ACER-001 to NaPB. We anticipate submitting to the FDA the 505(b)(2) NDA for ACER-001 for the treatment of UCD in 2019, subject to our ability to generate sufficient capital resources to fund development.

Maple Syrup Urine Disease (MSUD)

Background

Maple Syrup Urine Disease, or MSUD, is a rare inherited disorder caused by defects in the mitochondrial branched-chain ketoacid dehydrogenase complex, which results in elevated blood levels of the branched-chain amino acids, or BCAA, leucine, valine, and isoleucine, as well as the associated branded-chain ketoacids, or BCKA, in a patient's blood. Left untreated, this can result in neurological damage, mental disability, coma or death. There are currently no approved pharmacologic therapies in the United States or the European Union for MSUD. Treatment of MSUD consists primarily of a severely restricted diet to limit the intake of BCAA, with aggressive medical interventions when blood-levels of BCAA or BCKA become elevated. The most severe presentation of MSUD, known as "classic" MSUD, accounts for 80% of cases and can result in neonatal onset with encephalopathy and coma. Although metabolic management of the disease is possible via a highly restrictive diet, the outcome is unpredictable and a significant portion of affected individuals are mentally impaired or experience neurological complications.

Studies indicate that MSUD affects an estimated 1 in 185,000 infants worldwide. The disorder occurs more frequently in the Old Order Mennonite population, with an estimated incidence of about 1 in 380 newborns, and the Ashkenazi Jewish population, with an estimated incidence of 1 in 26,000. Approximately 3,000 patients suffer from MSUD worldwide, of whom approximately 800 are located in the United States.

ACER-001 for Treatment of MSUD

Rationale for ACER-001 Treatment in MSUD

Therapy with NaPB in UCD patients has been associated with a selective reduction in BCAA despite adequate dietary protein intake.

Based on this clinical observation, investigators at Baylor College of Medicine, or BCM, explored the potential of NaPB treatment to lower BCAA and their corresponding BCKA in patients with MSUD. The investigators found that BCAA and BCKA were both significantly reduced following NaPB therapy in control subjects and in patients with MSUD, although there was no simple correlation between the patients' levels of residual enzymatic activity with the response of plasma BCAA and their BCKA to NaPB. NaPB demonstrated a statistically significant reduction of leucine in all three healthy subjects and in three out of the five MSUD patients who participated in the trial. The reduction in leucine, the most toxic of the BCAAs, in the three responsive MSUD patients ranged between 28-34%, which is considered by clinicians to be a meaningful response.

Registration Plan

We intend to seek FDA approval to market ACER-001 for the treatment of MSUD in the United States by submitting a 505(b)(2) NDA through which we may be able to rely on the preclinical and clinical safety data from the RLD's NDA while supplementing the data with additional pharmokinetic, pharmodynamic, efficacy and safety data specifically in the MSUD population. We anticipate submitting to the FDA the 505(b)(2) NDA for ACER-001 for the treatment of MSUD in 2021, subject to our ability to generate sufficient capital resources to fund development. We also intend to seek approval in the European Union and other territories outside the United States, after the supplemental NDA for treatment of MSUD is filed.

ACER-001 Planned Clinical Development in MSUD Patients

Subject to our ability to generate sufficient capital resources, we intend to support a 505(b)(2) NDA for ACER-001 for the treatment of MSUD by submitting an Investigational New Drug Application, or IND, in 2019. The following four clinical trials are planned:

Study One

This multicenter, open-label, uncontrolled clinical trial will enroll approximately 60 subjects with MSUD ages 8 to 48 years, who have baseline blood leucine levels >150 µmol/L, while achieving steady-state leucine intake via a restricted diet. All subjects will receive ACER-001 for 7 days. Response will be defined as a greater than or equal to 30% decrease in blood leucine from baseline. We anticipate that at day seven, 40 subjects will be identified as responders.

Study Two

This multicenter, double-blind, placebo-controlled study will enroll approximately 40 subjects with MSUD who responded to sodium phenylbutyrate in Study 1. After a washout period from Study 1, subjects will be randomized equally to either ACER-001 or placebo for 4 weeks. Efficacy will be assessed by the mean change in blood leucine level from baseline to week four in the ACER-001-treated group as compared to the mean change in the placebo group.

Study Three

This multicenter, open-label, uncontrolled clinical trial will enroll approximately 20 subjects with MSUD who did not respond to sodium phenylbutyrate in Study 1. After a washout period from Study 1, subjects will undergo six weeks of forced dose-titration with three different doses of ACER-001. Treatments will consist of three consecutive two-week courses of ACER-001 at increasing doses above the top dose studied in Study 1. Blood leucine levels will be monitored after two weeks of treatment at each dose level.

Study Four

This multicenter, open-label, extension study will enroll up to 60 subjects who respond to ACER-001 treatment in Study 1 and complete Study 2, and any subjects from Study 3 who are identified as responders following dose titration. Blood leucine levels will be monitored every four weeks, and additional safety information will be collected.

Commercialization Strategy

Assuming the FDA approves EDSIVO™ and ACER-001, we expect that the majority of vEDS, UCD and MSUD patients will be treated at tertiary care centers, and therefore can be addressed with a targeted sales force. vEDS patients will primarily be treated by vascular medicine or cardiology specialists, while the UCD and MSUD patients will primarily be managed by metabolic geneticists and dietitians. We intend to build our own commercial infrastructure in the United States to target these centers and will evaluate whether to commercialize in other geographies ourselves or with an experienced partner.

Competition

We are not aware of any other companies that are pursuing a treatment for vEDS. However, the pharmaceutical industry is highly competitive. We face competition from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. Given the significant unmet medical need for novel therapies to treat UCD and MSUD, many companies, public and private universities and research organizations are actively engaged in the discovery, research and development of product candidates to treat these conditions. As a result, there are and will likely continue to be extensive resources invested in the discovery and development of new products to treat these unmet medical needs. We anticipate facing intense and increasing competition as new products enter the market and advanced technologies become available.

Our potential competitors and the related stage of development for their product candidates in our other target indications include the following:

- UCD: Horizon Pharma plc / SOBI, Inc. (Marketed); Promethera Biosciences S.A./N.V. (Phase 2); Aeglea BioTherapeutics Inc. (Phase 1/2); Dimension Therapeutics Inc. (Phase 1/2); Synlogic, Inc. (preclinical)
- MSUD: Synlogic, Inc. (preclinical)

Many of our competitors, either alone or with strategic partners, have or will have substantially greater financial, technical and human resources. Accordingly, our competitors may be more successful than us in developing or marketing products and technologies that are more effective, safer or less costly. Additionally, our competitors may obtain regulatory approval for their products more rapidly and may achieve more widespread market acceptance. These companies also compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical study sites and patient registration for clinical studies and acquiring technologies complementary to, or necessary for, our programs. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

There are other non-pharmaceutical therapeutic approaches that are used or may be used for our targeted indications. For example, liver transplantation may be used in some cases to treat UCD or MSUD in pediatric patients who have developed acute liver failure. Other products in clinical development or marketed for other indications may be used in conjunction with our product candidates if we are able to identify potential market opportunities of interest. For example, a study that is currently enrolling vEDS patients at AP-HP includes adding irbesartan, an angiotensin II receptor blocker, with celiprolol, to provide supplemental vascular protection and thus reduce recurrence of arterial events in vEDS patients.

We believe that the key competitive factors that will affect the development and commercial success of its product candidates are efficacy, safety and tolerability profile, convenience in dosing, product labeling, price, and the availability of reimbursement.

Licenses and Royalties

Baylor College of Medicine ("BCM") License

In April 2014, we obtained exclusive rights to patents and certain other intellectual property relating to ACER-001 and preclinical and clinical data, through an exclusive license agreement with BCM. Under the terms of the agreement, as amended, we have worldwide exclusive rights to develop, manufacture, use, sell and import products incorporating the licensed intellectual property. The license agreement requires us to make upfront and annual payments to BCM, reimburse certain of BCM's legal costs, make payments upon achievement of defined milestones, and pay royalties on net sales of any developed product over the royalty term.

Aventis Pharma SA

In June 2016, and as amended in November 2017, we entered into an agreement with Aventis Pharma SA granting us the exclusive access and exclusive right to use the data included in the marketing authorization application dossier filed with and approved by the MHRA in 1986 for the treatment of mild to moderate hypertension pursuant to the UK regulatory approval procedure, for the sole purpose of allowing us to further develop, manufacture, register and commercialize celiprolol in North America and South America for the treatment of EDS, Marfan syndrome and Loeys-Dietz syndrome. We have paid in full for the exclusive access and right to use the data.

Assistance Publique—Hôpitaux de Paris (AP-HP)

In August 2016, we entered into an agreement with AP-HP granting us the exclusive worldwide rights to access and use data from the Ong trial. We intend to use this pivotal clinical data to support an NDA filing for EDSIVO™ for the treatment of vEDS. The agreement requires us to make certain upfront payments to AP-HP, reimburse certain of AP-HP's costs, make payments upon achievement of defined milestones and pay royalties on net sales of celiprolol over the royalty term.

Manufacturing

We contract with third parties for the manufacture, testing, and storage of our product candidates and intend to continue to do so in the future. We do not own and have no plans to build our own manufacturing capabilities for clinical or commercial supply. Because we rely on contract manufacturers, we have hired consultants with extensive technical, manufacturing, analytical, regulatory and quality assurance and control experience to oversee contract manufacturing and testing activities, and to compile manufacturing and quality information for our regulatory submissions.

Intellectual Property

EDSIVO™

We intend to protect our commercial rights in the United States via multiple pathways. We believe that we will be eligible for NCE exclusivity, which is a five-year period of marketing exclusivity during which time the FDA will not approve another drug with the same active ingredient, regardless of the indication for use, in the United States. In January 2015, the FDA granted EDSIVO™ orphan drug designation, which provides upon the approval of a drug intended to treat a rare condition seven years of marketing exclusivity during which time the FDA will not approve the same drug for the same indication, unless it demonstrates clinical superiority. Orphan drug exclusivity does not prevent the FDA from approving the same drug for a different indication, or a different drug for the same indication. NCE exclusivity and orphan drug exclusivity run concurrently. Furthermore, EDSIVO™ may qualify for an additional six months of Pediatric Exclusivity in the United States, which requires the submission of one or more studies that meet requirements to be specified by the FDA in a Written Request for pediatric studies. Pediatric Exclusivity can be obtained either before or after NDA approval. Pediatric Exclusivity is attached to the end of an existing exclusivity and runs consecutively. We may also consider making modifications to the formulation to obtain additional intellectual property. While unapproved drugs may be imported into the United States under specified circumstances, such as for use in clinical studies under a valid and effective IND or for further manufacture into an IND drug or an approved drug, we intend to aggressively assert our rights, via regulatory and legal means, to limit the importation of non-FDA approved versions of celiprolol. We intend to provide a robust patient assistance program, or PAP, to minimize the out-of-pocket costs associated with treatment for vEDS patients in the United States who might otherwise seek to obtain celiprolol elsewhere.

ACER-001

We obtained exclusive rights to certain patents and other intellectual property from BCM for the use of NaPB for the treatment of inborn errors of BCAA metabolism, including MSUD.

The licensed patent covers methods and compositions for treating humans (and animals) with various formulations and prodrugs of NaPB for inborn errors of BCAA metabolism, including MSUD, and does not expire until 2030. We made filings in the geographic regions that represent the largest incidence and prevalence of MSUD: the United States, selected countries in Europe (including Turkey) and Brazil. BCM has been issued one patent in each of the United States and the European Union with respect to ACER-001, each of which was exclusively licensed to us pursuant to our agreement with BCM.

We filed a formulation patent application with respect to ACER-001 in January 2016 and plan to seek further patent protection in major markets, including the United States and the European Union.

We also expect to benefit from potential commercial exclusivity afforded to the first drug approved after obtaining orphan drug designation for the treatment of MSUD. We were granted orphan drug designation for ACER-001 for the treatment of MSUD by the FDA in August 2014. Orphan drug exclusivity provides upon the approval of a drug intended to treat a rare condition seven years of marketing exclusivity during which time the FDA will not approve the same drug for the same indication, unless it demonstrates clinical superiority, in the U.S. and ten years in the European Union post-approval. Orphan drug exclusivity does not prevent the FDA from approving the same drug for a different indication, or a different drug for the same indication. NCE exclusivity and orphan drug exclusivity run concurrently.

Furthermore, we may qualify to receive an additional six months of Pediatric Exclusivity in the U.S., which runs consecutively to an existing exclusivity, and an additional two years in the European Union.

Government Regulation and Product Approval

Government authorities in the United States at the federal, state and local level and in other countries extensively regulate, among other things, the use of unapproved drugs, pre-clinical and clinical studies, development, testing, quality control, manufacture, packaging, storage, recordkeeping, labeling, advertising, promotion, distribution, import, and export of pharmaceutical products such as those we are developing. The process for obtaining approvals or authorizations to market a drug product in the United States and in foreign countries and jurisdictions, along with pre- and post-approval compliance with applicable statutes and regulations, require the expenditure of substantial time and financial resources. Our product candidates must be approved by the FDA before they may be legally marketed in the United States and by the appropriate foreign regulatory agencies before they may be legally marketed in foreign countries. Generally, our activities in other countries will be subject to regulation that is similar in nature and scope as that imposed in the United States, although there can be important differences. Additionally, some significant aspects of approval requirements within the European Union are addressed uniformly, while country-specific requirements must also be met.

U.S. Drug Approval Process. In the United States, the FDA regulates drugs under the FFDCA and the FDA's implementing regulations. Drugs are also subject to other federal, state and local statutes and regulations. The process of obtaining marketing approvals and pre- and post-approval compliance with applicable federal, state, and local statutes and regulations require the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time before or after approval, may subject an applicant to a variety of administrative or judicial sanctions, such as the FDA's refusal to approve a pending NDA, withdrawal of an approval, imposition of a clinical hold on a clinical study or studies, issuance of a warning letter or untitled letter, product recall, product seizure, total or partial suspension of production or distribution, injunction, fines, refusals or cancellation of government contracts, restitution, disgorgement, or civil or criminal penalties.

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 in compliance with the FDA's current good laboratory practice, or cGLP, regulations;
- submission to the FDA of an IND to which the FDA has no objections and which must become effective before clinical trials in the United States may begin;
- approval by an institutional review board, or IRB, for each clinical site before each trial may be initiated;
- performance of adequate and well-controlled human clinical trials to establish the safety and efficacy of the drug candidate for each proposed indication in accordance with the FDA's current Good Clinical Practice, or cGCP, regulations, IND regulations, and human subject protection regulations;
- submission to the FDA of an NDA or a biologics license application, or BLA;
- satisfactory review by an FDA advisory committee, if applicable;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the product is produced to assess compliance with the FDA's current Good Manufacturing Practice, cGMP, regulation and to assure that the methods used in, and the facilities and controls used for, manufacture, processing, and packing are adequate to preserve the drug's identity, strength, quality and purity; and
- FDA review and approval of the NDA or BLA.

Preclinical Studies. Preclinical studies include laboratory evaluation of product chemistry, toxicity and formulation, as well as animal studies to assess the characteristics and potential safety and efficacy of the product. An IND 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 trial protocol, among other things, to the FDA as part of an IND. Some preclinical testing may continue even after the IND is submitted. An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises questions or concerns, including concerns that human research subjects will be exposed to unreasonable health risks, related to one or more proposed clinical trials and places the trial on a partial or full clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. As a result, submission of an IND may not result in the FDA allowing clinical trials to commence.

Clinical Trials. Clinical trials involve the administration of the investigational new drug to patients under the supervision of qualified investigators in accordance with IND regulations and human subject protection regulations as well as cGCP standards, which include the requirement that all research patients provide their informed consent for their participation in any clinical trial and that an IRB approve each study before it begins. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. A protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND. In addition, an IRB for each institution participating in the clinical trial must review and approve each protocol and protocol amendment for any clinical trial before it commences at that institution. Information about certain clinical trials must be submitted within specific timeframes to the National Institutes of Health, or NIH, for public dissemination on their ClinicalTrials.gov website.

Human clinical trials are typically conducted in three or four sequential phases, which may overlap or be combined:

Phase 1: The drug is initially introduced into a small number of healthy human subjects and tested for safety, dosage tolerance, absorption, metabolism, distribution, and excretion or, on occasion, in patients with severe problems or life-threatening disease to gain an early indication of its effectiveness.

Phase 2: The drug is administered to a limited patient population to preliminarily evaluate the efficacy of the product for a specific targeted disease, gather additional safety information and to determine dosage tolerance, optimal dosage, and method of delivery.

Phase 3: The drug is administered to a larger patient population, generally at geographically dispersed clinical trial sites, in well-controlled clinical trials to generate enough data to statistically evaluate the efficacy and safety of the product to determine effectiveness, to establish the overall risk-benefit profile of the product, and to provide adequate information for the labeling of the product and ultimately to support approval.

Phase 4: In some cases, the FDA may condition approval of an NDA for a product candidate on the sponsor's agreement to conduct additional clinical trials after NDA approval. In other cases, a sponsor may voluntarily conduct additional clinical trials post-approval to gain more information about the drug. Such post-approval trials are typically referred to as Phase 4 clinical trials.

Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and more frequently if serious and unexpected adverse reactions occur. Trial sponsors must monitor other information including published as well as unpublished scientific papers, reports from foreign regulatory authorities and reports of foreign commercial marketing experience for the investigational drug and notify the FDA and clinical trial investigators of certain information. Phase 1, Phase 2 and Phase 3 clinical trials may fail to be completed successfully within a specified period, or at all. Furthermore, the FDA may impose a clinical hold on one or more or all of the clinical studies or the sponsor may suspend or terminate a clinical trial or development of an investigational product at any time for a variety of reasons, including a finding that the research patients are being exposed to an unacceptable health risk. Development, or the aspects of development, that are affected by the clinical hold may not continue unless and until the sponsor addresses all of the FDA's concerns and has been notified that the hold is removed. Similarly, an IRB can suspend or terminate its approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the protocol or the IRB's requirements or if the drug has been associated with unexpected serious harm to patients. Nearly all Phase 3 trials and some other trials are overseen by a data and safety monitoring board ("DSMB"), which is composed of physicians, statisticians, and other experts who are independent of the clinical trial sponsor. Similar to IRBs, DSMBs review the progress of a clinical trial and participant safety, but they may also review data on the effectiveness of the drug being studied. DSMB members can stop a trial early if safety concerns arise or if they determine that the trial should be stopped due to "futility" meaning that the trial will not be able to answer the question or questions it set out to explore.

Concurrent with clinical trials, companies may need to complete additional animal trials and must develop information about the chemistry and physical characteristics of the drug and finalize a process for manufacturing the drug in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the drug candidate and, among other things, the manufacturer must develop methods for testing the identity, strength, quality and purity of the final drug product. Additionally, appropriate packaging must be selected and tested and stability studies must be completed to establish an expiration date and demonstrate that the drug candidate does not undergo unacceptable deterioration prior to the expiration date.

The NDA Approval Process. Assuming successful completion of the required clinical testing, the results of the preclinical studies and clinical trials, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product's chemistry, manufacture, controls and proposed labeling, among other things, are submitted to the FDA as part of an NDA to support approval to market the product for one or more indications. In most cases, the submission of an NDA is subject to a substantial application user fee.

The FDA is required to conduct a preliminary review of an NDA within the first 60 days after submission, before accepting it for filing, to determine whether it is sufficiently complete to permit a substantive review. The FDA may accept the NDA for filing, potentially refuse to file the NDA due to deficiencies but work with the applicant to rectify the deficiencies (in which case the NDA is filed upon resolution of the deficiencies) or refuse to file the NDA. The FDA must notify the applicant of a refusal to file a decision within 60 days after the original receipt date of the application. If the FDA refuses to file the NDA the applicant may resubmit the NDA with the deficiencies addressed. The resubmitted NDA is considered a new application subject to a new review goal, as described below. If the NDA is resubmitted for the same product (by the same person) a new application fee will not be required. The resubmitted application is also subject to review before the FDA accepts it for filing. Once an NDA is accepted for filing, the FDA begins an in-depth substantive review. Under the Prescription Drug User Fee Act, or PDUFA, and the FDA's commitments under the current PDUFA Reauthorization Act, the FDA has a goal of reviewing and acting on 90% of standard non-priority NDA applications within ten months from the filing date of the NDA.

The FDA reviews an NDA to determine, among other things, whether the drug is safe and effective for its intended use and whether the facility in which it is manufactured, processed, packaged or held meets standards designed to assure the product's continued safety, quality and purity. The FDA is required to refer an application for a novel drug to an advisory committee or explain why such referral was not made. An advisory committee is a panel of independent experts, including clinicians and other scientific experts, that reviews, evaluates and provides a recommendation in response to specific questions raised by the FDA, which may include 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.

Before approving an NDA, the FDA may inspect the facility or facilities where the product is manufactured. The FDA will not approve an application 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 investigational sites to evaluate the integrity of the data and confirm compliance with cGCP.

After the FDA evaluates the NDA and conducts its inspections, it may issue an approval letter or a Complete Response Letter. An approval letter authorizes the commercial marketing of the drug subject to specific prescribing information for specific indications and, if applicable, specific post-approval requirements. A Complete Response Letter indicates that the review cycle of the application is complete and the application is not ready for approval. After receiving a Complete Response Letter, the applicant must decide within twelve months (subject to extension), if it wants to resubmit the NDA addressing the deficiencies identified by the FDA in the Complete Response Letter, withdraw the NDA, or request an opportunity for a hearing to challenge the FDA's determination. A Complete Response Letter may require additional clinical data and/or an additional pivotal Phase 3 clinical trial(s), and/or other significant, expensive and time-consuming requirements related to clinical trials, nonclinical studies or manufacturing. Even if such data are submitted, the FDA may ultimately decide that the NDA does not satisfy the criteria for approval. Data from clinical trials are not always conclusive and the FDA may interpret data differently than we interpret data.

The FDA also may require implementation of a Risk Evaluation and Mitigation Strategy, or REMS, to mitigate any identified or suspected serious risks. The REMS plan could include medication guides, physician communication plans, assessment plans and elements to assure safe use, such as restricted distribution methods, patient registries or other risk minimization tools.

The drug testing and approval process requires substantial time, effort and financial resources, and may take several years to complete. Data obtained from clinical activities are not always conclusive and may be susceptible to varying interpretations, which could delay, limit or prevent marketing approval. The FDA may not grant marketing approval on a timely basis, or at all.

Even if the FDA approves a product, it may limit the approved indications for use for the product. The FDA requires that the approved product labeling include information regarding contraindications, warnings or precautions. It may also require that post-approval studies, including a long-term registry, be conducted to further assess a drug's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution restrictions or other risk management mechanisms, which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-marketing studies or surveillance programs. After approval, some types of changes to the approved product, such as adding new indications or labeling claims or manufacturing changes may be subject to further testing requirements and FDA review and approval. Also after approval, the FDA may require labeling changes as new information becomes known, particularly if new risks are identified, such as unexpected adverse events. The FDA has the authority to prevent or limit further marketing of a drug based on the results of these post-marketing studies and programs or other information that may become known after approval.

Hatch-Waxman Exclusivity. The Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments, amended the FFDCA and established abbreviated pathways to market, as well as incentives for the development of new drug products. The Hatch-Waxman Amendments established section 505(b)(2) of the FFDCA that provides an alternative pathway for submission of an NDA, referred to as the 505(b)(2) application, when some or all of the safety and efficacy investigations relied on for approval were not conducted by or for the applicant and for which the applicant has not obtained a right of reference. The Hatch-Waxman Amendments also established the Abbreviated New Drug Application, or ANDA, approval pathway, which provides an expedient route for generic drugs that have the same active ingredient as a previously approved drug. At the same time, to incentivize continued pharmaceutical innovation, the Hatch-Waxman Amendments authorized periods of market exclusivity to protect certain approved new drugs from competition for five or three year periods.

Under the Hatch-Waxman Amendments, a new drug containing an active ingredient that had never before been approved in any other NDA, ANDA, or 505(b)(2) NDA is provided five years of market exclusivity upon approval. The FDA refers to this exclusivity as NCE exclusivity. During the NCE exclusivity period, the FDA cannot approve an ANDA or a 505(b)(2) application for a drug containing the same active ingredient. For NCE exclusivity, the FDA regulations interpret "active ingredient" to mean "active moiety," which is defined as "the molecule or ion, excluding those appended portions of the molecule that cause the drug to be an ester, salt . . . , or other noncovalent derivative . . . of the molecule, responsible for the physiological or pharmacological action of the drug substance." Although the FDA may not approve an ANDA or 505(b)(2) NDA with the same active ingredient during the five-year NCE exclusivity period, an ANDA or 505(b)(2) NDA may be submitted to the FDA after four years if it contains a certification of patent invalidity or non-infringement.

The Hatch-Waxman Amendments also provide three years of market exclusivity for an NDA, a 505(b)(2) NDA, or a supplement to either of these applications for a drug product containing an active moiety that has been previously approved, 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. During this three-year exclusivity period, the FDA will not make effective the approval of any ANDA or 505(b)(2) NDA for the same active moiety for the same conditions of use. Five-year and three-year exclusivity will not delay the submission or approval of a new drug containing the same active moiety if it is the subject of a full NDA for which the applicant conducted, sponsored, or obtained a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

Other Regulatory Requirements. Drugs manufactured or distributed pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, annual establishment registration and product listing and associated user fees, compliance with the cGMP, recordkeeping, periodic reporting, product sampling and distribution, advertising and promotion, and adverse drug experience monitoring and reporting with the product. After approval, most changes to the approved product labeling, such as adding new indications are subject to prior FDA review and approval. Also, any post-approval changes in the drug substance, drug product, production process, quality controls, equipment, or facilities that have a substantial potential to have an adverse effect on the identity, strength, quality, purity, or potency of the drug product is subject to FDA review and approval. Any such changes that have a moderate potential to have an adverse effect on the identity, strength, quality, purity, or potency of the drug product must be submitted to the FDA for review 30 days prior to implementation. All manufacturing facilities, as well as records required to be maintained under FDA regulations, are subject to inspection or audit by the FDA. In addition, manufacturers are required to pay annual user fees for establishment registration and user fees for the submission of each new or supplemental application with clinical data.

The FDA may impose a number of post-approval requirements as a condition of approval of an NDA. For example, the FDA may require post-approval testing, including Phase 4 clinical trials, and surveillance to further assess and monitor the product's safety and effectiveness after commercialization. The Food and Drug Administration Amendments Act of 2007 gave the FDA the authority to require a REMS from drug manufacturers to manage a known or potential serious risk associated with the drug and to ensure that the benefits of a drug outweigh its risks. Examples of a REMS include, but are not limited to, a Medication Guide, a patient package insert to help mitigate a serious risk of the drug, and a communication plan to healthcare providers to support the implementation of an element of the REMS.

In addition, drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and register or obtain permits or licenses in states where they do business, and are subject to periodic unannounced inspections by the FDA and state regulatory authorities with jurisdiction over their activities to determine compliance with regulatory requirements. A drug manufacturer is responsible for ensuring that its third-party contractors operate in compliance with applicable laws and regulations including the cGMP regulation. The failure of a drug manufacturer or any of its third-party contractors to comply with federal or state laws or regulations may subject the drug manufacturer to possible legal or regulatory action, such as an untitled letter, warning letter, recall, suspension of manufacturing or distribution or both, suspension of state permit or license, seizure of product, import detention, injunctive action, and civil and criminal penalties.

Changes to the manufacturing process are strictly regulated and often require prior FDA approval before being implemented. FDA regulations also require a drug manufacturer to conduct investigations and implement appropriate corrective actions to address any deviations from cGMP requirements and impose reporting and documentation requirements upon the manufacturer and any third-party contractors (including contract manufacturers and laboratories) involved in the manufacture of a drug product. Accordingly, manufacturers must continue to expend significant time, money and effort to maintain and ensure ongoing cGMP compliance and to confirm and ensure ongoing cGMP compliance of their third-party contractors.

Once an approval is granted, the FDA may withdraw the approval if there is new information or evidence that the drug is unsafe or not shown to be safe for use under the conditions of its approval, or that new information shows there is a lack of substantial evidence of effectiveness, or that the approved application contained an untrue statement of material fact, or that the required patient information was not submitted within 30 days after receiving notice from the FDA of the failure to submit such information. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety and risk information; imposition of a post-market study requirement to assess new safety risks; or implementation of a REMS that may include distribution or other restrictions.

The FDA closely regulates drug advertising and promotional activities, including promotion of an unapproved drug, direct-to-consumer advertising, dissemination of scientific information about a drug not on the approved labeling, off-label promotion, communications with payors and formulary committees, industry-sponsored scientific and educational activities, and promotional activities involving the internet and social media. A company's product claims must be true and not misleading, provide fair balance, provide adequate risk information, and be consistent with the product label approved by the FDA. Failure to comply with these requirements can lead to regulatory actions including, among other things, warning letters, corrective advertising, injunction, violation and related penalties under the False Claims Act and result in reputational and economic harm.

Physicians may prescribe FDA-approved drugs for uses that are not described in the product's labeling and that differ from those uses tested by the manufacturer. Such off-label uses occur across medical specialties. Physicians may believe that such off-label uses are the best treatment for many patients in varied circumstances. The FDA does not regulate the behavior of physicians in their choice of treatments for their individual patients. The FDA does, however, regulate manufacturers' communications about their drug products and interprets the FFDCA to prohibit pharmaceutical companies from promoting their FDA-approved drug products for uses that are not specified in the FDA-approved labeling. Companies that market drugs for off-label uses have been subject to warning letters, related costly litigation, criminal prosecution, and civil liability under the FFDCA and the False Claims Act.

In addition, the distribution of prescription pharmaceutical products is subject to the Prescription Drug Marketing Act, or PDMA, which regulates the distribution of drug and drug samples at the federal level, and sets minimum standards for the registration and regulation of wholesale drug distributors by the states.

Orphan Designation. The Orphan Drug Act of 1983 provides incentives, including marketing exclusivity, user fee waivers and tax benefits, to companies that undertake development and marketing of products to treat rare diseases, which are defined as diseases for which there is a patient population of fewer than 200,000 persons in the U.S. or a patient population greater than 200,000 in the U.S. where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the U.S. A drug that receives orphan drug designation may receive up to seven years of exclusive marketing in the U.S. for that indication, which means the FDA may not approve any other application to market the same drug for the same indication for a period of seven years, except in limited circumstances, such as a showing of clinical superiority over the product with orphan exclusivity or where the manufacturer is unable to assure sufficient product quantity. A drug may be entitled to an additional six months of exclusive marketing if it satisfies the requirements for pediatric exclusivity.

The European Medicines Agency (EMA) Committee for Orphan Medicinal Products, or COMP, grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than five in 10,000 persons in the European Union Community and for which no satisfactory method of diagnosis, prevention, or treatment has been authorized (or the product would be a significant benefit to those affected). Additionally, the designation is granted for products intended for the diagnosis, prevention, or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the medicinal product. In the European Union, orphan drug designation entitles a party to financial incentives such as reduction of fees or fee waivers and ten years of market exclusivity is granted following medicinal product approval. This period may be reduced to six years if the orphan drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity.

New Legislation and Regulations. From time to time, legislation is drafted, introduced and passed in Congress that could significantly change the statutory provisions governing the testing, approval, manufacturing, and marketing of products regulated by the FDA. In addition to new legislation, FDA regulations and policies are often revised or interpreted by the agency in ways that may significantly affect our business and products. It is impossible to predict whether further legislative changes will be enacted or FDA regulations, guidance, policies or interpretations will be changed, or the impact of such changes, if any.

Pharmaceutical Coverage, Pricing, and Reimbursement. Significant uncertainty exists as to the coverage and reimbursement status of any drug products for which we may obtain marketing approval. Sales of any of our product candidates, if approved, will depend, in part, on the extent to which the costs of the products will be covered by third-party payors, including government health programs such as Medicare and Medicaid, commercial health insurers and managed care organizations. The process for determining whether a third-party payor will provide coverage for a drug product typically is separate from the process for establishing the reimbursement rate that a payor will pay for the drug product once coverage is approved. Some third-party payors may limit coverage to specific drug products on an approved list, also known as a formulary, which might not include all of the approved drugs for a particular indication.

In order to obtain coverage and reimbursement for any product that might be approved for sale, we may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of the product, in addition to the costs required to obtain FDA or other comparable regulatory approvals. Whether or not we conduct such studies, our product candidates may not be considered medically necessary or cost-effective. A third-party payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Further, one payor's determination to provide coverage for a product does not assure that other payors will also provide coverage, and adequate reimbursement, for the product. Third-party reimbursement may not be sufficient to enable us to maintain price levels high enough to realize an appropriate return on our investment in product development.

The containment of healthcare costs has become a priority of federal, state and foreign governments, and the prices of drugs have been a focus in this effort. Third-party payors are increasingly challenging the prices charged for medical products and services, examining the medical necessity and reviewing the cost-effectiveness of drug products and medical services and questioning safety and efficacy. Emphasis on managed care in the United States has increased and we expect will continue to increase the pressure on drug pricing. If third-party payors do not consider our products to be cost-effective compared to other available therapies, they may not cover the products for which we receive FDA approval or, if they do, the level of payment may not be sufficient to allow us to sell our products at a profit.

The U.S. government, state legislatures, and foreign governments have shown significant interest in implementing cost-containment programs to limit the growth of government-paid healthcare costs and drug prices in general, including for therapies for rare diseases. These measures include price controls, transparency requirements triggered by the introduction of new high-cost drugs into the market, drug re-importation, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs. Some laws and regulations have already been enacted in these areas, and additional measures have been introduced or are under consideration at both the federal and state levels. Additionally, at the request of U.S. Senators, the Government Accountability Office is currently investigating abuses of the Orphan Drug Act, which could potentially lead to legislation that affects reimbursement for drugs with small patient populations. Adoption of such controls and measures and tightening of restrictive policies in jurisdictions with existing controls and measures could limit payments for pharmaceuticals such as our drug product candidates and could adversely affect our net revenue and results.

In addition, in the United States, the Affordable Care Act contains provisions that have the potential to substantially change healthcare delivery and financing, including impacting the profitability of drugs. For example, the Affordable Care Act revised the methodology by which rebates owed by manufacturers for covered outpatient drugs are calculated under the Medicaid Drug Rebate Program, extended the Medicaid Drug Rebate Program to utilization of covered drugs dispensed to individuals enrolled in Medicaid managed care organizations and subjected manufacturers to new annual fees for certain branded prescription drugs.

Pricing and reimbursement methodologies vary widely from country to country. Some countries require that drug products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost-effectiveness of a particular product candidate to currently available therapies. For example, the European Union provides options for its member states to restrict the range of drug products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. European Union member states may approve a specific price for a drug product or they may instead adopt a system of direct or indirect controls on our profitability in placing the drug product on the market. Other member states allow companies to fix their own prices for drug products, but monitor and control company profits. The downward pressure on healthcare costs in general, particularly prescription drugs, has become 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 competitive pressure that may reduce pricing within a country. Any country that has price controls or reimbursement limitations for drug products may not allow favorable reimbursement and pricing arrangements for any of our products.

Coverage policies, third-party reimbursement rates, and drug pricing regulation may change at any time, and there is the potential for significant movement in these areas in the foreseeable future. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive marketing approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

Healthcare Law and Regulation. Healthcare providers, physicians and third-party payors play a primary role in the recommendation and prescribing of any product candidates for which we may obtain marketing approval. Our business operations and arrangements with investigators, healthcare professionals, consultants, third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws. These laws may constrain the business or financial arrangements and relationships through which we research, manufacture, market, promote, sell and distribute our products that obtain marketing approval. Restrictions under applicable federal and state healthcare laws include, but are not limited to, the following:

- the federal healthcare Anti-Kickback Statute prohibits, among other things, persons or entities from knowingly and willfully soliciting, offering, receiving or paying any remuneration (including any kickback, bribe or rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, lease, order or recommendation of, any good, facility, item or service, for which payment may be made, in whole or in part, under a federal healthcare program such as Medicare and Medicaid;
- the federal False Claims Act and civil monetary penalties law impose penalties and provide for civil whistleblower or qui tam actions against individuals or entities for, among other things, knowingly presenting, or causing to be presented, to the federal government, claims for payment or approval that are false or fraudulent or making a false record or statement to avoid, decrease or conceal an obligation to pay money to the federal government;
- the civil monetary penalties statute, which imposes penalties against any person or entity who, among other things, is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, among other things, imposes criminal liability for knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or 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;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, also imposes certain obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information without proper written authorization;
- the federal transparency requirements under the Affordable Care Act requires manufacturers of drugs, devices, biologicals and medical supplies to annually report to the Centers for Medicare & Medicaid Services, or CMS, an agency within the U.S. Department of Health and Human Services, or HHS, information related to payments and other transfers of value provided to physicians and teaching hospitals and certain ownership and investment interests held by physicians and their immediate family members; and
- analogous state and foreign laws, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; state and foreign laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government or otherwise restrict payments that may be made to healthcare providers; state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; and state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Further, the Affordable Care Act, among other things, amended the intent requirement of the federal Anti-Kickback Statute and certain criminal statutes governing healthcare fraud. A person or entity no longer needs to have actual knowledge of the statute or specific intent to violate it. In addition, the Affordable Care Act provided that the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws 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. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to our business, we may be subject to significant administrative, civil, and/or criminal penalties, damages, fines, disgorgement, individual imprisonment, exclusion from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. If any of the physicians or other providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to administrative, civil, and/or criminal sanctions, including exclusions from government funded healthcare programs.

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

The U.S. Foreign Corrupt Practices Act and Other Anti-Corruption Laws

We may be subject to a variety of domestic and foreign anti-corruption laws with respect to our regulatory compliance efforts and operations. The U.S. Foreign Corrupt Practices Act, commonly known as the FCPA, is a criminal statute that prohibits an individual or business from paying, offering, promising or authorizing the provision of money (such as a bribe or kickback) or anything else of value (such as an improper gift, hospitality, or favor), directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision in order to assist the individual or business in obtaining, retaining, or directing business or other advantages (such as favorable regulatory rulings). The FCPA also obligates companies with securities listed in the United States to comply with certain accounting provisions. Those provisions require a company such as ours to (i) maintain books and records that accurately and fairly reflect all transactions, expenses, and asset dispositions, and (ii) devise and maintain an adequate system of internal accounting controls sufficient to provide reasonable assurances that transactions are properly authorized, executed and recorded. The FCPA is subject to broad interpretation by the U.S. government. The past decade has seen a significant increase in enforcement activity. In addition to the FCPA, there are a number of other federal and state anti-corruption laws to which we may be subject, including, the U.S. domestic bribery statute contained in 18 USC § 201 (which prohibits bribing U.S. government officials) and the U.S. Travel Act (which in some instances addresses private-sector or commercial bribery both within and outside the United States). Also, a number of the countries in which we may conduct activities have their own domestic and international anti-corruption laws, such as the UK Bribery Act 2010. There have been cases where companies have faced multi-jurisdictional liability under the FCPA and the anti-corruption laws of other countries for the same illegal act.

We can be held liable under the FCPA and other anti-corruption laws for the illegal activities of our employees, representatives, contractors, collaborators, agents, subsidiaries, or affiliates, even if we did not explicitly authorize such activity. Although we will seek to comply with anti-corruption laws, there can be no assurance that all of our employees, representatives, contractors, collaborators, agents, subsidiaries or affiliates will comply with these laws at all times. Noncompliance with these laws could subject us to whistleblower complaints, investigations, sanctions, settlements, prosecution, other enforcement actions, disgorgement of profits, significant fines, damages, other civil and criminal penalties or injunctions, suspension and/or debarment from contracting with certain governments or other persons, the loss of export privileges, reputational harm, adverse media coverage, and other collateral consequences. In addition, our directors, officers, employees, and other representatives who engage in violations of the FCPA and certain other anti-corruption statutes may face imprisonment, fines, and penalties. If any subpoenas or investigations are launched, or governmental or other sanctions are imposed, or if we do not prevail in any possible civil or criminal litigation, our business, results of operations and financial condition could be materially harmed. In addition, responding to any action will likely result in a materially significant diversion of our management's attention and resources and significant defense costs and other professional fees. Enforcement actions and sanctions could further harm our business, results of operations, and financial condition.

Employees

As of December 31, 2017, we had six full-time employees, no part-time employees, and seven consultants or independent contractors working for us. None of our employees are represented by a labor union or subject to a collective bargaining agreement. We have not experienced a work stoppage and consider our relations with our employees to be good.

Organizational History

On September 19, 2017, Acer Therapeutics Inc., a Texas corporation, formerly known as Opexa Therapeutics, Inc. (the "Registrant"), completed its business combination with Acer Therapeutics Inc., a Delaware corporation ("Private Acer"), in accordance with the terms of the Agreement and Plan of Merger and Reorganization, dated as of June 30, 2017, by and among the Registrant, Opexa Merger Sub, Inc. ("Merger Sub") and Private Acer (the "Merger Agreement"), pursuant to which Merger Sub merged with and into Private Acer, with Private Acer surviving as a wholly-owned subsidiary of the Registrant (the "Merger"). This transaction was approved by the Registrant's shareholders at a special meeting of its shareholders on September 19, 2017. Also on September 19, 2017, in connection with, and prior to the completion of, the Merger, the Registrant effected a 1-for-10.355527 reverse stock split of its then outstanding common stock (the "Reverse Split") and immediately following the Merger, the Registrant changed its name to "Acer Therapeutics Inc." pursuant to amendments to its certificate of formation filed with the Texas Secretary of State on September 19, 2017.

Following the completion of the Merger, the business conducted by the Registrant became primarily the business conducted by Private Acer, which is a pharmaceutical company that acquires, develops and intends to commercialize therapies for patients with serious rare diseases with critical unmet medical need. Under the terms of the Merger Agreement, the Registrant issued shares of its common stock to Private Acer's stockholders, at an exchange rate of one share of common stock (after giving effect to the Reverse Split and the conversion of Private Acer's Series A and Series B preferred stock and convertible debt) in exchange for each share of Private Acer common stock outstanding immediately prior to the Merger. The exchange rate was determined through arm's length negotiations between the Registrant and Private Acer. The Registrant also assumed all issued and outstanding stock options under the Acer Therapeutics Inc. 2013 Stock Incentive Plan, with such stock options henceforth representing the right to purchase a number of shares of the Registrant's common stock equal to the number of shares of Private Acer's common stock previously represented by such stock options.

Immediately after the Merger: (i) there were approximately 6.5 million shares of the Registrant's common stock outstanding; (ii) the former Private Acer stockholders, including investors in the Concurrent Financing (as defined below), owned approximately 89% of the outstanding common stock of the Registrant; and (iii) the Registrant's shareholders immediately prior to the Merger, whose shares of the Registrant's common stock remained outstanding after the Merger, owned approximately 11% of the outstanding common stock of the Registrant.

The issuance of the shares of the Registrant's common stock to the former stockholders of Private Acer was registered with the SEC on a Registration Statement on Form S-4 (Reg. No. 333-219358). Immediately prior to the Merger, Private Acer issued and sold an aggregate of approximately $15.7 million (inclusive of the conversion of approximately $5.7 million of principal and accrued interest on outstanding convertible promissory notes issued by Private Acer) of shares of Private Acer's common stock (the "Concurrent Financing") to certain current stockholders of Private Acer and certain new investors at a per share price of $9.47.

The Registrant's common stock traded on a pre-split basis through the close of business on Wednesday, September 20, 2017 on the Nasdaq Capital Market under the ticker symbol "OPXA." Commencing with the open of trading on Thursday, September 21, 2017, the post-split shares began trading on the Nasdaq Capital Market under the ticker symbol "ACER." On September 21, 2017, the Registrant's Series M Warrants, previously trading through the close of business on Wednesday, September 20, 2017 under the ticker symbol "OPXAW," commenced trading on the Nasdaq Capital Market, under the ticker symbol "ACERW." The Registrant's common stock and Series M Warrants have new CUSIP numbers of 00444P 108 and 00444P 116, respectively.

For accounting and financial reporting purposes, Private Acer was considered to have acquired the Registrant in the Merger. Private Acer was incorporated on December 26, 2013, as part of a reorganization whereby Acer Therapeutics, LLC was converted into a corporation organized under the laws of the State of Delaware. On March 20, 2015, Private Acer acquired Anchor Therapeutics, Inc. ("Anchor"), with Anchor becoming a wholly-owned subsidiary of Private Acer. On August 19, 2016, Anchor's pepducin business reverted back to the pre-acquisition holders of Anchor's equity.

Company Information

Our principal executive offices are located at One Gateway Center, Suite 351 (300 Washington St.), Newton, Massachusetts 02458, and our telephone number is (844) 902-6100. Our website address is www.acertx.com. The information found on our website, or that may be accessed by links on our website, is not part of this report.

Available Information

We are subject to the information and reporting requirements of the Securities Exchange Act of 1934, or the Exchange Act, under which we file periodic reports, proxy and information statements and other information with the United States Securities and Exchange Commission, or SEC. Copies of the reports, proxy statements and other information may be examined without charge at the Public Reference Room of the SEC, 100 F Street, N.E., Room 1580, Washington, D.C. 20549, or on the Internet at http://www.sec.gov. Copies of all or a portion of such materials can be obtained from the Public Reference Room of the SEC upon payment of prescribed fees. Please call the SEC at 1-800-SEC-0330 for further information about the Public Reference Room.

Financial and other information about us is available on our website (**www.acertx.com**). Information on our website is not incorporated by reference into this report. We make available on our website, free of charge, copies of our annual report on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K, and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act as soon as reasonably practicable after filing such material electronically or otherwise furnishing it to the SEC. Copies are available in print to any Acer shareholder upon request in writing to Attention: Investor Relations, Acer Therapeutics, One Gateway Center, Suite 351 (300 Washington St.), Newton, MA 02458.

Item 1A. Risk Factors.

Investing in our securities involves a high degree of risk. You should consider the following risk factors, as well as other information contained or incorporated by reference in this report, before deciding to invest in our securities. The following factors affect our business, our intellectual property, the industry in which we operate and our securities. The risks and uncertainties described below are not the only ones we face. Additional risks and uncertainties not presently known or which we consider immaterial as of the date hereof may also have an adverse effect on our business. If any of the matters discussed in the following risk factors were to occur, our business, financial condition, results of operations, cash flows or prospects could be materially adversely affected, the market price of our securities could decline and you could lose all or part of your investment in our securities.

Risks Related to Our Business and Financial Condition

We have a limited operating history and have incurred significant losses since our inception and anticipate that we will continue to incur losses for the foreseeable future and may never achieve or maintain profitability. The absence of any commercial sales and our limited operating history make it difficult to assess our future viability.

We are a development-stage pharmaceutical company with a limited operating history. On September 19, 2017, we completed the reverse merger, or the Merger, with Acer Therapeutics Inc., a Delaware corporation, or Private Acer, in accordance with the terms of the Agreement and Plan of Merger and Reorganization, dated as of June 30, 2017, by and among Private Acer, ourselves and Opexa Merger Sub, Inc. Also on September 19, 2017, in connection with, and prior to the completion of, the Merger, we effected a 1-for-10.355527 reverse stock split of our common stock and changed our name to "Acer Therapeutics Inc."

Pharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk. We are focused principally on repurposing and/or reformulating existing drugs for (ultra) orphan diseases with significant unmet medical need. We are not profitable and Private Acer had incurred losses in each year since its inception in 2013. We have only a limited operating history upon which you can evaluate our business and prospects. In addition, we have limited experience and have not yet demonstrated an ability to successfully overcome many of the risks and uncertainties frequently encountered by companies in new and rapidly evolving fields, particularly in the specialty pharmaceutical industry. We have not generated any revenue to date. We continue to incur significant research and development and other expenses related to our ongoing operations. Our net loss for the year ended December 31, 2016, as a private company was $6.7 million. As of December 31, 2017, we had an accumulated deficit of $25.6 million. We expect to continue to incur losses for the foreseeable future as we continue our development of, and seek marketing approvals for, our product candidates.

We have devoted substantially all of our financial resources to identify, acquire, and develop our product candidates, including providing general and administrative support for our operations. To date, we have financed our operations primarily through the sale of equity securities and convertible promissory notes. The amount of our future net losses will depend, in part, on the rate of our future expenditures and our ability to obtain funding through equity or debt financings, strategic collaborations, or grants. Pharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk. We expect losses to increase as we conduct clinical trials and continue to develop our lead product candidates. We expect to invest significant funds into the research and development of our current product candidates to determine the potential to advance these product candidates to regulatory approval.

If we obtain regulatory approval to market a product candidate, our future revenue will depend upon the size of any markets in which our product candidates may receive approval and our ability to achieve sufficient market acceptance, pricing, reimbursement from third-party payors, and adequate market share for our product candidates in those markets. Even if we obtain adequate market share for our product candidates, because the potential markets in which our product candidates may ultimately receive regulatory approval could be very small, we may never become profitable despite obtaining such market share and acceptance of our products.

We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future, and our expenses will increase substantially if and as we:

- continue the clinical development of our product candidates;
- continue efforts to discover new product candidates;
- undertake the manufacturing of our product candidates or increase volumes manufactured by third parties;
- advance our programs into larger, more expensive clinical trials;
- initiate additional pre-clinical, clinical, or other trials or studies for our product candidates;
- seek regulatory and marketing approvals and reimbursement for our product candidates;
- establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval and market for ourselves;
- seek to identify, assess, acquire and/or develop other product candidates;
- make milestone, royalty or other payments under third-party license agreements;
- seek to maintain, protect and expand our intellectual property portfolio;
- seek to attract and retain skilled personnel; and
- experience any delays or encounter issues with the development and potential for regulatory approval of our clinical candidates such as safety issues, clinical trial accrual delays, longer follow-up for planned studies, additional major studies or supportive studies necessary to support marketing approval.

Further, the net losses we incur may fluctuate significantly from quarter to quarter and year to year, such that a period-to-period comparison of our results of operations may not be a good indication of our future performance.

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

We have not generated any revenues from commercial sales of any of our current product candidates, EDSIVO™ (for vascular Ehlers-Danlos syndrome, or vEDS) and ACER-001 (for urea cycle disorders, or UCD, and Maple Syrup Urine Disease, or MSUD). Our ability to generate product revenue depends upon our ability to successfully commercialize these product candidates or other product candidates that we may develop, in-license or acquire in the future. Our ability to generate future product revenue from our current or future product candidates also depends on a number of additional factors, including our ability to:

- successfully complete research and clinical development of current and future product candidates;
- establish and maintain supply and manufacturing relationships with third parties, and ensure adequate and legally compliant manufacturing of product candidates;
- obtain regulatory approval from relevant regulatory authorities in jurisdictions where we intend to market our product candidates;
- launch and commercialize future product candidates for which we obtain marketing approval, if any, and if launched independently, successfully establish a sales force and marketing and distribution infrastructure;

- obtain coverage and adequate product reimbursement from third-party payors, including government payors;
- achieve market acceptance for our products, if any;
- establish, maintain and protect our intellectual property rights; and
- attract, hire and retain qualified personnel.

In addition, because of the numerous risks and uncertainties associated with clinical product development, including that our product candidates may not advance through development or achieve regulatory approval, we are unable to predict the timing or amount of any potential future product sales revenues. Our expenses also could increase beyond expectations if we decide to or are required by the United States Food and Drug Administration, or FDA, or comparable foreign regulatory authorities, to perform studies or trials in addition to those that we currently anticipate. Even if we complete the development and regulatory processes described above, we anticipate incurring significant costs associated with launching and commercializing these products.

We will require substantial additional financing to obtain marketing approval of our product candidates and commercialize our product candidates, and a failure to obtain this necessary capital when needed on acceptable terms, or at all, could force us to delay, limit, reduce or terminate our product development, other operations or commercialization efforts.

Since our inception, substantially all of our resources have been dedicated to the clinical development of our product candidates. As of December 31, 2017, we had an accumulated deficit of $25.6 million, cash and cash equivalents of $15.6 million and current liabilities aggregating $2.0 million. Based on available resources, we believe that our cash and cash equivalents currently on hand are sufficient to fund our anticipated operating and capital requirements through the end of 2018. However, our current capital resources are not sufficient to fund our planned operations for the next 12 months from the date of the financial statements included in this report and thus there is substantial doubt about the Company's ability to continue as a going concern within one year after such date.

We believe that we will continue to expend substantial resources for the foreseeable future on the completion of clinical development and regulatory preparedness of our product candidates, preparations for a commercial launch of our product candidates, if approved, and development of any other current or future product candidates we may choose to further develop. These expenditures will include costs associated with research and development, conducting preclinical studies and clinical trials, obtaining marketing approvals, and manufacturing and supply as well as marketing and selling any products approved for sale. In addition, other unanticipated costs may arise. Because the outcome of any drug development process is highly uncertain, we cannot reasonably estimate the actual amounts necessary to successfully complete the development and commercialization of our current product candidates, if approved, or future product candidates, if any.

Our operating plan may change as a result of factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings or other sources, such as strategic collaborations. Such financing may result in dilution to shareholders, imposition of debt covenants and repayment obligations, or other restrictions that may adversely affect our business. In addition, we may seek additional capital due to favorable market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans.

Our future capital requirements depend on many factors, including:

- the scope, progress, results, and costs of researching and developing our current product candidates, future product candidates and conducting preclinical and clinical trials;
- the cost of seeking regulatory and marketing approvals and reimbursement for our product candidates;
- the cost of commercialization activities if our current product candidates and future product candidates are approved for sale, including marketing, sales and distribution costs, and preparedness of our corporate infrastructure;

- the cost of manufacturing current product candidates and future product candidates that we obtain approval for and successfully commercialize;
- our ability to establish and maintain strategic collaborations, licensing or other arrangements and the financial terms of such agreements;
- the number and characteristics of any additional product candidates we may develop or acquire;
- any product liability or other lawsuits related to our products or commenced against us;
- the expenses needed to attract and retain skilled personnel;
- the costs associated with being a public company;
- the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing our intellectual property rights, including litigation costs and the outcome of such litigation; and
- the timing, receipt and amount of sales of, or royalties on, any future approved products, if any.

Additional funds may not be available when we need them, on terms that are acceptable to us, or at all. 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 development activities for our current product candidates or future product candidates, if any;
- delay, limit, reduce or terminate our research and development activities; or
- delay, limit, reduce or terminate our establishment of sales and marketing capabilities or other activities that may be necessary to commercialize our future product candidates.

Our auditors have expressed doubt in their audited financial report in respect of our 2017 fiscal year about our ability to continue as a going concern.

In their audited financial report in respect of our 2017 fiscal year, our independent registered public accounting firm included in its report an emphasis-of-a-matter indicating that the recurring losses from our operations raised a substantial doubt as to our ability to continue as a going concern. As of December 31, 2017, we had an accumulated deficit of $25.6 million, cash and cash equivalents of $15.6 million and current liabilities aggregating $2.0 million. Based on available resources, we believe that our cash and cash equivalents currently on hand are sufficient to fund our anticipated operating and capital requirements through the end of 2018. However, our current capital resources are not sufficient to fund our planned operations for the next 12 months from the date of the financial statements included in this report. Moreover, we expect to continue to incur losses for the foreseeable future as we continue our development of and seek marketing approvals for, our product candidates. These matters raise substantial doubt about our ability to continue as a going concern. Because we have been issued an opinion by our independent registered public accounting firm that substantial doubt exists as to whether we can continue as a going concern, it may be more difficult for us to attract investors. Unless we are able to raise additional capital, it is possible that the opinion of our independent registered public accounting firm on our audited financial report in respect of future years may include a similar going concern qualification.

Funding from our ATM facility may be limited or be insufficient to fund our operations or to implement our strategy.

We will need to keep current our shelf registration statement and an offering prospectus relating to the ATM facility with Brinson Patrick (now a division of IFS Securities, Inc.) in order to use the program to sell shares of our common stock, as well as provide certain periodic deliverables required by the sales agreement for the facility. The number of shares and price at which we may be able to sell shares under our ATM facility may be limited due to market conditions and other factors beyond our control.

We have incurred, and expect to continue to incur, increased costs and risks as a result of being a public company.

As a public company, we are required to comply with the Sarbanes-Oxley Act of 2002, or SOX, as well as rules and regulations implemented by the Securities and Exchange Commission, or SEC, and The NASDAQ Stock Market, or NASDAQ. Changes in the laws and regulations affecting public companies, including the provisions of SOX and rules adopted by the SEC and by NASDAQ, have resulted in, and will continue to result in, increased costs as we respond to their requirements. Given the risks inherent in the design and operation of internal controls over financial reporting, the effectiveness of our internal controls over financial reporting is uncertain. If our internal controls are not designed or operating effectively, we may not be able to conclude an evaluation of our internal control over financial reporting as required or we or our independent registered public accounting firm may determine that our internal control over financial reporting was not effective. We currently have a very limited workforce, and it may be difficult to adhere to appropriate internal controls over financial reporting or disclosure controls with such limited staffing. In addition, our independent registered public accounting firm may either disclaim an opinion as it relates to management's assessment of the effectiveness of our internal controls or may issue an adverse opinion on the effectiveness of our internal controls over financial reporting, especially in light of the fact that we currently have a very limited workforce. Investors may lose confidence in the reliability of our financial statements, which could cause the market price of our common stock to decline and which could affect our ability to run our business as we otherwise would like to. New rules could also make it more difficult or more costly for us to obtain certain types of insurance, including directors' and officers' liability insurance, and we may be forced to accept reduced policy limits and coverage or incur substantially higher costs to obtain the coverage that is the same or similar to our current coverage. The impact of these events could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees and as executive officers. We cannot predict or estimate the total amount of the costs we may incur or the timing of such costs to comply with these rules and regulations.

Under the corporate governance standards of NASDAQ, a majority of our board of directors and each member of our Audit and Compensation Committees must be an independent director. If any vacancies on our Board or Audit or Compensation Committees occur that need to be filled by independent directors, we may encounter difficulty in attracting qualified persons to serve on our Board and, in particular, our Audit Committee. If we fail to attract and retain the required number of independent directors, we may be subject to SEC enforcement proceedings and delisting of our common stock from the NASDAQ Capital Market.

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

We are subject to the reporting requirements of the Exchange Act, SOX and NASDAQ rules and regulations. SOX requires, among other things, that we maintain effective disclosure controls and procedures and internal control over financial reporting. We must perform system and process evaluation and testing of our internal control over financial reporting to allow management to report on the effectiveness of our internal controls over financial reporting in our Annual Report on Form 10-K filing for that year, as required by Section 404 of SOX. As a private company, Private Acer had never been required to test its internal controls within a specified period. This will require that we incur substantial internal costs to continue to expand our accounting and finance functions and that we expend significant management efforts. We may experience difficulty in meeting these reporting requirements in a timely manner.

Although we are committed to continuing to improve our internal control processes, and although we will continue to diligently and vigorously review our internal control over financial reporting, we cannot be certain that, in the future, a material weakness or significant deficiency will not exist or otherwise be discovered. We may discover weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our financial statements. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

If we are not able to comply with the requirements of Section 404 of SOX, or if we are unable to maintain proper and effective internal controls, we may not be able to produce timely and accurate financial statements. If that were to happen, the market price of our common stock could decline and we could be subject to sanctions or investigations by NASDAQ, the SEC, or other regulatory authorities.

Any acquisitions that we make could disrupt our business and harm our financial condition.

We expect to evaluate potential strategic acquisitions of complementary businesses, products or technologies on a global geographic footprint. We may also consider joint ventures, licensing and other collaborative projects. We may not be able to identify appropriate acquisition candidates or strategic partners, or successfully negotiate, finance or integrate acquisitions of any businesses, products or technologies. Furthermore, the integration of any acquisition and management of any collaborative project may divert our management's time and resources from our core business and disrupt our operations. We do not have any experience with acquiring companies, or with acquiring products outside of the United States. Any cash acquisition we pursue would potentially divert the cash we have on our balance sheet from our present clinical development programs. Any stock acquisitions would dilute our shareholders' ownership. While we from time to time evaluate potential collaborative projects and acquisitions of businesses, products, and technologies, and anticipate continuing to make these evaluations, we have no present agreements with respect to any acquisitions or collaborative projects.

Risks Related to the Clinical Development and Marketing Approval of Our Product Candidates

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

None of our current product candidates have gained marketing approval for sale in the United States or any other country, and we cannot guarantee that we will ever have marketable products. Our business is substantially dependent on our ability to complete the development of, obtain marketing approval for, and successfully commercialize our product candidates in a timely manner. We cannot commercialize our product candidates in the United States without first obtaining approval from the FDA to market each product candidate. Similarly, we cannot commercialize our product candidates outside of the United States without obtaining regulatory approval from comparable foreign regulatory authorities. Our product candidates could fail to receive marketing approval for many reasons, including the following:

- the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- the FDA or comparable foreign regulatory authorities may find the human subject protections for our clinical trials inadequate and place a clinical hold on an Investigational New Drug Application, or IND, at the time of its submission precluding commencement of any trials or a clinical hold on one or more clinical trials at any time during the conduct of our clinical trials;
- the FDA could determine that we cannot rely on Section 505(b)(2) of the Federal Food, Drug and Cosmetic Act, or FFDCA, for any or all of our product candidates;
- we 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 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 interpretation of data from preclinical studies or clinical trials;

- the FDA could determine that we have identified the wrong reference listed drug or drugs or that approval of our 505(b)(2) application for any of our product candidates is blocked by patent or non-patent exclusivity of the reference listed drug or drugs;
- the data collected from clinical trials of our product candidates may not be sufficient to support the submission of an application to obtain marketing approval in the United States or elsewhere;
- the FDA or comparable foreign regulatory authorities may find inadequate the manufacturing processes or facilities of third-party manufacturers with which we 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 that would delay marketing approval.

Before obtaining marketing approval for the commercial sale of any drug product for a target indication, we must demonstrate in preclinical studies and well-controlled clinical trials and, with respect to approval in the United States, to the satisfaction of the FDA, that the product is safe and effective for its intended use and that the manufacturing facilities, processes, and controls are adequate to preserve the drug's identity, strength, quality and purity. In the United States, it is necessary to submit and obtain approval of a New Drug Application, or NDA, from the FDA. An NDA must include extensive preclinical and clinical data and supporting information to establish the product safety and efficacy for each desired indication. The NDA must also include significant information regarding the chemistry, manufacturing, and controls for the product. After the submission of an NDA, but before approval of the NDA, the manufacturing facilities used to manufacture a product candidate must be inspected by the FDA to ensure compliance with the applicable Current Good Manufacturing Practice, or cGMP, requirements. The FDA and the Competent Authorities of the Member States of the European Economic Area, or EEA, and comparable foreign regulatory authorities, may also inspect our clinical trial sites and audit clinical study data to ensure that our studies are properly conducted in accordance with the IND regulations, human subject protection regulations, and good clinical practice, or cGCP.

Obtaining approval of an NDA is a lengthy, expensive and uncertain process, and approval may not be obtained. Upon submission of an NDA, the FDA must make an initial determination that the application is sufficiently complete to accept the submission for filing. We cannot be certain that any submissions will be accepted for filing and reviewed by the FDA, or ultimately be approved. If the application is not accepted for review, the FDA may require that we conduct additional clinical studies or preclinical testing, or take other actions before it will reconsider our application. If the FDA requires additional studies or data, we would incur increased costs and delays in the marketing approval process, which may require us to expend more resources than we have available. In addition, the FDA may not consider any additional information to be complete or sufficient to support the filing or approval of the NDA.

Regulatory authorities outside of the United States, such as in Europe and Japan and in emerging markets, also have requirements for approval of drugs for commercial sale with which we must comply prior to marketing in those areas. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our product candidates. Clinical trials conducted in one country may not be accepted or the results may not be found adequate by regulatory authorities in other countries, and obtaining regulatory approval in one country does not mean that regulatory approval will be obtained in any other country. However, the failure to obtain regulatory approval in one jurisdiction could have a negative impact on our ability to obtain approval in a different jurisdiction. Approval processes vary among countries and can involve additional product candidate testing and validation and additional administrative review periods. Seeking foreign regulatory approval could require additional non-clinical studies or clinical trials, which could be costly and time-consuming. Foreign regulatory approval may include all of the risks associated with obtaining FDA approval. For all of these reasons, we may not obtain foreign regulatory approvals on a timely basis, if at all.

The process to develop, obtain marketing approval for, and commercialize product candidates is long, complex and costly, both inside and outside of the United States, and approval is never guaranteed. The time required to obtain approval by the FDA and comparable foreign authorities is unpredictable but typically takes many years following the commencement of clinical trials and depends upon numerous factors, including the substantial discretion of the regulatory authorities. In addition, approval policies, regulations, or the type and amount of clinical

data necessary to gain approval may change during the course of a product candidate's clinical development and may vary among jurisdictions. Even if our product candidates were to successfully obtain approval from regulatory authorities, any such approval might significantly limit the approved indications for use, including more limited patient populations, require that precautions, warnings or contraindications be included on the product labeling, including black box warnings, require expensive and time-consuming post-approval clinical studies, risk evaluation and mitigation strategies or surveillance as conditions of approval, or, through the product label, the approval may limit the claims that we may make, which may impede the successful commercialization of our product candidates. Following any approval for commercial sale of our product candidates, certain changes to the product, such as changes in manufacturing processes and additional labeling claims, as well as new safety information, may require new studies and will be subject to additional FDA notification, or review and approval. Also, marketing approval for any of our product candidates may be withdrawn. If we are unable to obtain marketing approval for our product candidates in one or more jurisdictions, or any approval contains significant limitations, our ability to market to our full target market will be reduced and our ability to realize the full market potential of our product candidates will be impaired. Furthermore, we may not be able to obtain sufficient funding or generate sufficient revenue and cash flows to continue or complete the development of any of our current or future product candidates.

If we are unable to submit an application for approval under Section 505(b)(2) of the FFDCA or if we are required to generate additional data related to safety and efficacy in order to obtain approval under Section 505(b)(2), we may be unable to meet our anticipated development and commercialization timelines.

Our current strategy for seeking marketing authorization in the United States for our product candidates relies primarily on Section 505(b)(2) of the FFDCA, which permits use of a marketing application, referred to as a 505(b)(2) application, where at least some of the information required for approval comes from studies not conducted by or for the applicant and for which the applicant has not obtained a right of reference or use. The FDA interprets this to mean that an applicant may rely for approval on such data as that found in published literature or the FDA's finding of safety or effectiveness, or both, of a previously approved drug product owned by a third party. There is no assurance that the FDA would find third-party data relied upon by us in a 505(b)(2) application sufficient or adequate to support approval, and the FDA may require us to generate additional data to support the safety and efficacy of our product candidates. Consequently, we may need to conduct substantial new research and development activities beyond those we currently plan to conduct. Such additional new research and development activities would be costly and time-consuming and there is no assurance that such data generated from such additional activities would be sufficient to obtain approval.

If the data to be relied upon in a 505(b)(2) application are related to drug products previously approved by the FDA and covered by patents that are listed in the FDA's Orange Book, we would be required to submit with our 505(b)(2) application a Paragraph IV Certification in which we must certify that we do not infringe the listed patents or that such patents are invalid or unenforceable, and provide notice to the patent owner or the holder of the approved NDA. The patent owner or NDA holder would have 45 days from receipt of the notification of our Paragraph IV Certification to initiate a patent infringement action against us. If an infringement action is initiated, the approval of our NDA would be subject to a stay of up to 30 months or more while we defend against such a suit. Approval of our product candidates under Section 505(b)(2) may, therefore, be delayed until patent exclusivity expires or until we successfully challenge the applicability of those patents to our product candidates. Alternatively, we may elect to generate sufficient clinical data so that we would no longer need to rely on third-party data, which would be costly and time-consuming and there would be no assurance that such data generated from such additional activities would be sufficient to obtain approval.

We may not be able to obtain shortened review of our applications, and the FDA may not agree that our product candidates qualify for marketing approval. If we are required to generate additional data to support approval, we may be unable to meet anticipated or reasonable development and commercialization timelines, may be unable to generate the additional data at a reasonable cost, or at all, and may be unable to obtain marketing approval of our product candidates. If the FDA changes its interpretation of Section 505(b)(2) allowing reliance on data in a previously approved drug application owned by a third party, or there is a change in the law affecting Section 505(b)(2), this could delay or even prevent the FDA from approving any Section 505(b)(2) application that we submit.

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

Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. The FDA and comparable foreign regulatory authorities have substantial discretion in the approval process and determining when or whether marketing approval will be obtained for our current product candidates. Even if we believe the data collected from clinical trials of our current product candidates are promising, such data may not be sufficient to support approval by the FDA or comparable foreign authorities. Our future clinical trial results may not be successful.

It is impossible to predict the extent to which the clinical trial process may be affected by legislative and regulatory developments. Due to these and other factors, our current product candidates or future product candidates could take a significantly longer time to gain marketing approval than expected or may never gain marketing approval. This could delay or eliminate any potential product revenue by delaying or terminating the potential commercialization of our current product candidates.

Preclinical trials must also be conducted in accordance with FDA and comparable foreign authorities' legal requirements, regulations or guidelines, including Good Laboratory Practice, or GLP, an international standard meant to harmonize the conduct and quality of nonclinical studies and the archiving and reporting of findings. Preclinical studies including long-term toxicity studies and carcinogenicity studies in experimental animals may result in findings that may require further evaluation, which could affect the risk-benefit evaluation of clinical development, or which may even lead the regulatory agencies to delay, prohibit the initiation of or halt clinical trials or delay or deny marketing authorization applications. Failure to adhere to the applicable GLP standards or misconduct during the course of preclinical trials may invalidate the data and require one or more studies to be repeated or additional testing to be conducted.

Clinical trials must also be conducted in accordance with FDA and comparable foreign authorities' legal requirements, regulations or guidelines, including human subject protection requirements and GCP. Clinical trials are subject to further oversight by these governmental agencies and IRBs at the medical institutions where the clinical trials are conducted. In addition, clinical trials must be conducted with supplies of our current product candidates produced under cGMP and other requirements. Our clinical trials are conducted at multiple sites, including some sites in countries outside the United States and the European Union, which may subject us to further delays and expenses as a result of increased shipment costs, additional regulatory requirements and the engagement of foreign and non-EU clinical research organizations, as well as expose us to risks associated with clinical investigators who are unknown to the FDA or the European regulatory authorities, and with different standards of diagnosis, screening and medical care.

The commencement and completion of clinical trials for our current product candidates may be delayed, suspended or terminated as a result of many factors, including but not limited to:

- the delay or refusal of regulators or IRBs to authorize us to commence a clinical trial at a prospective trial site and changes in regulatory requirements, policies and guidelines;
- the FDA or comparable foreign regulatory authorities disagreeing as to the design or implementation of our clinical trials;
- failure to reach 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;
- delays in patient enrollment and variability in the number and types of patients available for clinical trials;
- the inability to enroll a sufficient number of patients in trials to ensure adequate statistical power to detect statistically significant treatment effects;
- lower than anticipated retention rates of patients and volunteers in clinical trials;
- clinical sites deviating from trial protocol or dropping out of a trial;

- adding new clinical trial sites;
- negative or inconclusive results, which may require us to conduct additional preclinical or clinical trials or to abandon projects that we expect to be promising;
- safety or tolerability concerns could cause us to suspend or terminate a trial if we find that the participants are being exposed to unacceptable health risks;
- regulators or IRBs requiring that we or our investigators suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements;
- our third-party research and manufacturing contractors failing to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- difficulty in maintaining contact with patients after treatment, resulting in incomplete data;
- delays in establishing the appropriate dosage levels;
- the quality or stability of our current product candidates falling below acceptable standards;
- the inability to produce or obtain sufficient quantities of our current product candidates to complete clinical trials; and
- exceeding budgeted costs due to difficulty in predicting accurately the costs associated with clinical trials.

Patient enrollment is a significant factor in the timing of clinical trials and is affected by many factors, including the size and nature of the patient population, the proximity of patients to clinical sites, the eligibility criteria for the trial, the design of the clinical trial, competing clinical trials and clinicians' and patients' perceptions as to the potential advantages of the drug being studied in relation to other available therapies, including any new drugs or treatments that may be approved for the indications we are investigating.

There are significant requirements imposed on us and on clinical investigators who conduct clinical trials that we sponsor. Although we are responsible for selecting qualified clinical investigators, providing them with the information they need to conduct the clinical trial properly, ensuring proper monitoring of the clinical trial, and ensuring that the clinical trial is conducted in accordance with the general investigational plan and protocols contained in the IND, we cannot ensure the clinical investigators will maintain compliance with all regulatory requirements at all times. The pharmaceutical industry has experienced cases where clinical investigators have been found to incorrectly record data, omit data, or even falsify data. We cannot ensure that the clinical investigators in our trials will not make mistakes or otherwise compromise the integrity or validity of data, any of which would have a significant negative effect on our ability to obtain marketing approval, our business, and our financial condition.

We could 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 the data safety monitoring board, or DSMB, for such trial, or by the FDA or comparable foreign regulatory authorities. We or such authorities may impose 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 comparable foreign regulatory authorities resulting in the imposition of a clinical hold, safety issues or adverse side effects, failure to demonstrate a benefit from using the drug, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial. If we experience delays in the completion or termination of any clinical trial of our current product candidates, the commercial prospects of our current product candidates will be harmed, and our ability to generate product revenues from our product candidates will be delayed. In addition, any delays in completing our clinical trials will increase our costs, slow our development and approval process and jeopardize our ability to commence product sales and generate revenues. 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 marketing approval of our product candidates.

Any of these occurrences could materially adversely affect our business, financial condition, results of operations, and prospects. 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 marketing approval of our current product candidates. Significant clinical trial delays could also allow our competitors to bring products to market before we are able to do so, shorten any periods during which we have the exclusive right to commercialize our current product candidates and impair our ability to commercialize our current product candidates, which may harm our business, financial condition, results of operations, and prospects.

Clinical failure can occur at any stage of clinical development. Because the results of earlier clinical trials are not necessarily predictive of future results, any product candidate we advance through clinical trials may not have favorable results in later clinical trials or receive marketing approval.

Clinical failure can occur at any stage of our clinical development. The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical trials. A number of companies in the pharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials. Clinical trials may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical or preclinical testing. Data obtained from tests are susceptible to varying interpretations, and regulators may not interpret our data as favorably as we do, which may delay, limit or prevent marketing approval. In addition, the design of a clinical trial can determine whether our results will support approval of a product or approval of a product for desired indications, and flaws or shortcomings in the design of a clinical trial may not become apparent until the clinical trial is well advanced. We have limited experience in designing clinical trials and may be unable to design and execute a clinical trial to support marketing approval for our desired indications. Further, clinical trials of potential products often reveal that it is not practical or feasible to continue development efforts. If one of our product candidates is found to be unsafe or lack efficacy, we will not be able to obtain marketing approval for it and our business would be harmed. For example, if the results of our clinical trials of our product candidates do not achieve pre-specified endpoints or we are unable to provide primary or secondary endpoint measurements deemed acceptable by the FDA or comparable foreign regulators or if we are unable to demonstrate an acceptable level of safety relative to the efficacy associated with our proposed indications, the prospects for approval of our product candidates would be materially and adversely affected. A number of companies in the pharmaceutical industry, including those with greater resources and experience than us, have suffered significant setbacks in Phase 2 and Phase 3 clinical trials, even after seeing promising results in earlier clinical trials.

In some instances, there can be significant variability in safety and/or efficacy results between different trials of the same product candidate due to numerous factors, including differences in trial protocols and design, the size and type of the patient population, adherence to the dosing regimen and the rate of dropout among clinical trial participants. We do not know whether any clinical trials we may conduct will demonstrate consistent and/or adequate efficacy and safety to obtain marketing approval for our product candidates.

As an organization, we have never completed any clinical trial before and may be unable to do so efficiently or at all for our current product candidates or any product candidate we develop.

We intend to conduct clinical trials of our product candidates. The conduct of clinical trials and the submission of a successful NDA is a complicated process. As an organization, we have not completed a clinical trial before, and we have limited experience in preparing and submitting regulatory filings. Consequently, we may be unable to successfully and efficiently execute and complete necessary clinical trials in a way that leads to NDA submission and approval of our current product candidates or for any other product candidate we develop. We may require more time and incur greater costs than anticipated and may not succeed in obtaining marketing approval of the product candidates we develop. Failure to commence or complete, or delays in, our planned clinical trials would prevent us from or delay us in commercializing our current product candidates or any other product candidate we develop.

Marketing approval may be substantially delayed or may not be obtained for one or all of our product candidates if regulatory authorities require additional or more time-consuming studies to assess the safety and efficacy of our product candidates.

We may be unable to initiate or complete development of our product candidates on schedule, if at all. The completion of the studies for our product candidates will require us to obtain substantial additional funding beyond our current resources. In addition, regulatory authorities may require additional or more time-consuming studies to assess the safety or efficacy of our product candidates than we are currently planning. We may not be able to obtain adequate funding to complete the necessary steps for approval for any or all of our product candidates. Additional delays may result if the FDA, an FDA Advisory Committee (if one is convened to review our NDA) or other regulatory authority indicates that a product candidate should not be approved or there should be restrictions on approval, such as the requirement for a REMS, to ensure the safe use of the drug. Delays in marketing approval or rejections of applications for marketing approval in the United States or other markets may result from many factors, including:

- the FDA's or comparable foreign regulatory authorities' disagreement with the design or implementation of our clinical trials;
- regulatory requests for additional analyses, reports, data, non-clinical and preclinical studies and clinical trials;
- regulatory questions or disagreement by the FDA or comparable regulatory authorities regarding interpretations of data and results and the emergence of new information regarding our current or future product candidates or the field of research;
- unfavorable or inconclusive results of clinical trials and supportive non-clinical studies, including unfavorable results regarding safety or efficacy of our product candidates during clinical trials;
- failure to meet the level of statistical significance required for approval;
- inability to demonstrate that a product candidate's clinical and other benefits outweigh its safety risks;
- lack of adequate funding to commence or continue our clinical trials due to unforeseen costs or other business decisions;
- regulatory authorities may find inadequate the manufacturing processes or facilities of the third-party manufacturers with which we contract for clinical and commercial supplies;
- we may have insufficient funds to pay the significant user fees required by the FDA upon the filing of an NDA; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner that would delay marketing approval.

The lengthy and unpredictable approval process, as well as the unpredictability of future clinical trial results, may result in our failure to obtain marketing approval to market our other product candidates, which would significantly harm our business, results of operations and prospects.

Our product candidates may cause undesirable adverse effects or have other properties that could delay or prevent their marketing approval, limit the commercial profile of an approved label, or result in significant negative consequences following marketing approval, if obtained.

Undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of marketing approval by the FDA or other comparable foreign authorities. If any of our current product candidates or any other product candidate we develop is associated with serious adverse, undesirable or unacceptable side effects, we may need to abandon such candidate's development or limit development to certain uses or subpopulations in which such side effects are less prevalent, less severe or more acceptable from a risk-benefit perspective. Many compounds that initially showed promise in early-stage or clinical testing have later been found to cause side effects that prevented further development of the compound. Results of our trials could reveal a high and unacceptable prevalence of these

or other side effects. In such an event, our trials could be suspended or terminated and the FDA or comparable foreign regulatory authorities could order us to cease further development of or deny approval of our product candidates for any or all targeted indications. The drug-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims.

If our product candidates receive marketing approval, and we or others later identify undesirable side effects caused by such products, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw approvals of such product;
- we may be required to recall a product or change the way such product is administered to patients;
- additional restrictions may be imposed on the marketing of the particular product or the manufacturing process for the product or any component thereof;
- regulatory authorities may require the addition of labeling statements, such as a precaution, "black box" warning or other warnings or a contraindication;
- we or our collaborators may be required to implement a REMS or create a medication guide outlining the risks of such side effect for distribution to patients;
- we or our collaborators could be sued and held liable for harm caused to patients;
- the product may become less competitive; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of our product candidates, if approved, and could materially adversely affect our business, financial condition, results of operations and prospects.

Even if we receive marketing approval for our product candidates, such approved products will be subject to ongoing 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 we may be subject to penalties and legal sanctions if we fail to comply with regulatory requirements or experience unanticipated problems with our approved products.

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, registration, as well as continued compliance with cGMP regulations and GCP for any clinical trials that we conduct post-approval. Any marketing approvals that we receive for our product candidates may also be subject to limitations on the approved indicated uses for which the product may be marketed or to conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor safety and efficacy.

Later discovery of previously unknown problems with an approved product, including adverse events of unanticipated severity or frequency, or with manufacturing operations or processes, or failure to comply with regulatory requirements, or evidence of acts that raise questions about the integrity of data supporting the product approval, 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 clinical trials;
- refusal by the FDA to approve pending applications or supplements to approved applications filed by us, or suspension or revocation of product 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.

The FDA's policies may change and additional government regulations may be enacted that could prevent, limit or delay marketing approval, manufacturing or commercialization of our product candidates. 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 are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or we are not able to maintain regulatory compliance, we may lose any marketing approval that may have been obtained and we may not achieve or sustain profitability, which would adversely affect our business.

Agencies like the FDA and national competition regulators in European countries regulate the promotion and uses of drugs not consistent with approved product labeling requirements. If we are found to have improperly promoted our current product candidates for uses beyond those that are approved, we may become subject to significant liability.

Regulatory authorities like the FDA and national competition laws in Europe strictly regulate the promotional claims that may be made about prescription products, such as EDSIVOTM or ACER-001, if approved. In particular, a product may not be promoted for uses that are not approved by the FDA or comparable foreign regulatory authorities as reflected in the product's approved labeling, known as "off-label" use, nor may it be promoted prior to obtaining marketing approval. If we receive marketing approval for our product candidates for our proposed indications, physicians may nevertheless use our products for their patients in a manner that is inconsistent with the approved label if the physicians personally believe in their professional medical judgment it could be used in such manner. Although physicians may prescribe legally available drugs for off-label uses, manufacturers may not market or promote such off-label uses.

In addition, the FDA requires that promotional claims not be "false or misleading" as such terms are defined in the FDA's regulations. For example, the FDA requires substantial evidence, which generally consists of two adequate and well-controlled head-to-head clinical trials, for a company to make a claim that its product is superior to another product in terms of safety or effectiveness. Generally, unless we perform clinical trials meeting that standard comparing our product candidates to competitive products and these claims are approved in our product labeling, we will not be able promote our current product candidates as superior to other products. If we are found to have made such claims we may become subject to significant liability. In the United States, the federal government has levied large civil and criminal fines against companies for alleged improper promotion and has enjoined several companies from engaging in improper promotion. The FDA has also requested that companies enter into consent decrees or corporate integrity agreements. The FDA could also seek permanent injunctions under which specified promotional conduct is monitored, changed or curtailed.

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

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

- the federal Anti-Kickback Statute, which 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, or in return for, either the referral of an individual for, or the purchase, lease, order or recommendation of, any good, facility, item or service, for which payment may be made, in whole or in part, under federal and state healthcare programs such as Medicare and Medicaid;
- federal civil and criminal false claims laws and civil monetary penalty laws, including the federal False Claims Act, which impose criminal and civil penalties, including civil whistleblower or qui tam actions, against individuals or entities for, among other things, knowingly presenting, or causing to be presented, to the federal government, including the Medicare and Medicaid programs, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;
- the civil monetary penalties statute, which imposes penalties against any person or entity who, among other things, is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created new federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private), knowingly and willfully embezzling or stealing from a healthcare benefit program, willfully obstructing a criminal investigation of a healthcare offense and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and its implementing regulations, which impose obligations on covered entities, including healthcare providers, health plans, and healthcare clearinghouses, as well as their respective business associates that create, receive, maintain or transmit individually identifiable health information for or on behalf of a covered entity, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- the federal Open Payments program, created under Section 6002 of the Patient Protection and Affordable Care Act, or the Affordable Care Act, and its implementing regulations, which imposed annual reporting requirements for manufacturers of drugs, devices, biologicals and medical supplies for certain payments and "transfers of value" provided to physicians and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members, where failure to submit timely, accurately and completely the required information for all covered payments, transfers of value and ownership or investment interests may result in civil monetary penalties; and

- analogous state and foreign laws, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government or otherwise restrict payments that may be made to healthcare providers; state and foreign laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; and state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Further, the Affordable Care Act, among other things, amended the intent requirement of the federal Anti-Kickback Statute and certain criminal statutes governing healthcare fraud. A person or entity no longer needs to have actual knowledge of the statute or specific intent to violate it. In addition, the Affordable Care Act provided that the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act.

Efforts to ensure that our future business arrangements with third parties will comply with applicable healthcare laws and regulations may involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, including, without limitation, damages, fines, imprisonment, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations, which could significantly harm our business. If any of the physicians or other healthcare providers or entities with whom we expect to do business, including our current and future collaborators, if any, are found not to be in compliance with applicable laws, those persons or entities may be subject to criminal, civil or administrative sanctions, including exclusion from participation in government healthcare programs, which could also affect our business.

The impact of recent healthcare reform legislation and other changes in the healthcare industry and healthcare spending on us is currently unknown, and may adversely affect our business model.

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

Our revenue prospects could be affected by changes in healthcare spending and policy in the United States and abroad. We operate in a highly regulated industry and new laws and judicial decisions, or new interpretations of existing laws or decisions, related to healthcare availability, the method of delivery or payment for healthcare products and services could negatively impact our business, financial condition, results of operations and prospects. There is significant interest in promoting healthcare reform, as evidenced by the enactment in the United States of the Affordable Care Act. Among other things, the Affordable Care Act contains provisions that may reduce the profitability of drug products, including, for example, revising the methodology by which rebates owed by manufacturers for covered outpatient drugs under the Medicaid Drug Rebate Program are calculated, extending the Medicaid Drug Rebate Program to utilization of prescriptions of individuals enrolled in Medicaid managed care plans, imposing mandatory discounts for certain Medicare Part D beneficiaries, and subjecting drug manufacturers to payment of an annual fee.

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

It is likely that federal and state legislatures within the United States and foreign governments will continue to consider changes to existing healthcare legislation. We cannot predict the reform initiatives that may be adopted in the future or whether initiatives that have been adopted will be repealed or modified. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare may adversely affect:

- the demand for any drug products for which we may obtain marketing approval;
- our ability to set a price that we believe is fair for our products;
- our ability to obtain coverage and reimbursement approval for a product;
- our ability to generate revenues and achieve or maintain profitability; and
- the level of taxes that we are required to pay.

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 our business, financial condition or results of operations.

Our research and development activities and our third-party manufacturers' and suppliers' activities involve the controlled storage, use, and disposal of hazardous materials, including the components of our product candidates and other hazardous compounds. We and our manufacturers and suppliers are subject to laws and regulations governing the use, manufacture, storage, handling, and disposal of these hazardous materials. In some cases, these hazardous materials and various wastes resulting from their use are stored at our and our manufacturers' facilities pending their use and disposal. We cannot eliminate the risk of contamination, which could cause an interruption of our commercialization efforts, research and development efforts and business operations, environmental damage resulting in costly clean-up and liabilities under applicable laws and regulations governing the use, storage, handling, and disposal of these materials and specified waste products. Although we believe that the safety procedures utilized by us and our third-party manufacturers for handling and disposing of these materials generally comply with the standards prescribed by these laws and regulations, we cannot guarantee that this is the case or eliminate the risk of accidental contamination or injury from these materials. In such an event, we may be held liable for any resulting damages and such liability could exceed our resources and state or federal or other applicable authorities may curtail our use of specified materials and/or interrupt our business operations. Furthermore, environmental laws and regulations are complex, change frequently, and have tended to become more stringent. We cannot predict the impact of such changes and cannot be certain of our future compliance. We do not currently carry biological or hazardous waste insurance coverage.

Other Risks Related to Our Business

We may not be able to win government, academic institution or non-profit contracts or grants.

From time to time, we may apply for contracts or grants from government agencies, non-profit entities and academic institutions. Such contracts or grants can be highly attractive because they provide capital to fund the ongoing development of our product candidates without diluting our shareholders. However, there is often significant competition for these contracts or grants. Entities offering contracts or grants may have requirements to apply for or to otherwise be eligible for certain contracts or grants that our competitors may be able to satisfy that we cannot. In addition, such entities may make arbitrary decisions as to whether to offer contracts or make grants, to whom the contracts or grants may or will be awarded and the size of the contracts or grants to each awardee. Even if we are able to satisfy the award requirements, there is no guarantee that we will be a successful awardee. Therefore, we may not be able to win any contracts or grants in a timely manner, if at all.

If we fail to attract and retain key management and scientific personnel, we may be unable to successfully develop or commercialize our product candidates.

Our success as a specialty pharmaceutical company depends on our continued ability to attract, retain and motivate highly qualified management and scientific and clinical personnel. The loss of the services of any of our senior management could delay or prevent obtaining marketing approval or commercialization of our product candidates.

We may not be able to attract or retain qualified management and scientific personnel in the future due to the intense competition for a limited number of qualified personnel among specialty pharmaceutical businesses, and other pharmaceutical, biotechnology and other businesses. Our failure to attract, hire, integrate and retain qualified personnel could impair our ability to achieve our business objectives.

If a successful product liability claim or series of claims is brought against us for uninsured liabilities or in excess of insured liabilities, we could be forced to pay substantial damage awards.

The use of any of our product candidates in clinical trials, and the sale of any approved products, may expose us to product liability claims. We currently maintain product liability insurance coverage for up to $5.0 million per occurrence, up to an aggregate limit of $5.0 million. We intend to monitor the amount of coverage we maintain as the size and design of our clinical trials evolve, and if we are successful in obtaining approval to commercialize any of our product candidates, and adjust the amount of coverage we maintain accordingly. However, there is no assurance that such insurance coverage will fully protect us against some or all of the claims to which we might become subject. We might not be able to maintain adequate insurance coverage at a reasonable cost or in sufficient amounts or scope to protect us against potential losses. In the event a claim is brought against us, we might be required to pay legal and other expenses to defend the claim, as well as uncovered damages awards resulting from a claim brought successfully against us.

Furthermore, whether or not we are ultimately successful in defending any such claims, we might be required to direct financial and managerial resources to such defense and adverse publicity could result, all of which could harm our business.

Our employees, independent contractors, investigators, contract research organizations, consultants, collaborators and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements and insider trading.

We are exposed to the risk that our employees and other third parties may engage in fraudulent conduct or other illegal activity. Misconduct by employees and other third parties could include intentional, reckless and/or negligent conduct or disclosure of unauthorized activities to us that violate FDA regulations, including those laws requiring the reporting of true, complete and accurate information to the FDA, manufacturing standards, federal and state healthcare fraud and abuse laws and regulations, or laws that require the reporting of financial information or data accurately. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws intended to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Activities subject to these laws also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter employee and other third-party misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant civil, criminal and administrative penalties, damages, monetary fines, disgorgement, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings and curtailment or restructuring of our operations, any of which could adversely affect our ability to operate.

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

As of December 31, 2017, we had six full-time employees, no part-time employees, and seven consultants or independent contractors working for us. As our development and commercialization plans and strategies develop, we expect to need additional managerial, operational, sales, marketing, financial, legal and other resources. Our management may need to divert a disproportionate amount of our attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. As we 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 such third parties, as well as additional collaborators and suppliers.

We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, operational mistakes, loss of business opportunities, loss of employees, and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenue could be reduced and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize product candidates and compete effectively will depend, in part, on our ability to effectively manage any future growth.

Our internal computer systems, or those of our development collaborators, third-party clinical research organizations or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our product development programs.

Despite the implementation of security measures, our internal computer systems and those of our current and any future CROs and other contractors, consultants and collaborators 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 material 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 development programs and our business operations. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our marketing approval efforts and significantly increase our costs to recover or reproduce the data. Likewise, we intend to rely on third parties to manufacture our product candidates and conduct clinical trials, and similar events relating to their computer systems could also have a material adverse effect on our business. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development and commercialization of our product candidates could be delayed.

We are involved in litigation that may be expensive and time consuming, and if resolved adversely, could harm our business, financial condition, or results of operations.

As described in Note 7 to our consolidated financial statements included in this report, Piper Jaffray & Co. has filed a lawsuit against Private Acer alleging breach of contract. Defending against this lawsuit may be costly and may significantly divert the time and attention of our management from our operations. There can be no assurance that a favorable outcome will be obtained. A negative outcome, whether by final judgment or an unfavorable settlement, could result in payment of significant monetary damages and could adversely affect our financial condition and results of operations.

Risks Related to Commercialization of Our Product Candidates

Even if we obtain the required regulatory approvals in the United States and other territories, the commercial success of our product candidates will depend on market awareness and acceptance of our product candidates.

Even if we obtain marketing approval for our current product candidates or any other product candidates that we may develop or acquire in the future, the products may not gain market acceptance among physicians, key opinion leaders, healthcare payors, patients and the medical community. Market acceptance of any approved products depends on a number of factors, including:

- the timing of market introduction;
- the efficacy and safety of the product, as demonstrated in clinical trials;
- the clinical indications for which the product is approved and the label approved by regulatory authorities for use with the product, including any precautions, warnings or contraindications that may be required on the label;
- acceptance by physicians, key opinion leaders and patients of the product as a safe and effective treatment;
- the cost, safety and efficacy of treatment in relation to alternative treatments;
- the availability of coverage and adequate reimbursement and pricing by third-party payors and government authorities;
- the number and clinical profile of competing products;
- the growth of drug markets in our various indications;
- relative convenience and ease of administration;
- marketing and distribution support;
- the prevalence and severity of adverse side effects; and
- the effectiveness of our sales and marketing efforts.

Market acceptance is critical to our ability to generate revenue. Any product candidate, if approved and commercialized, may be accepted in only limited capacities or not at all. If any approved products are not accepted by the market to the extent that we expect, we may not be able to generate revenue and our business would suffer.

If the market opportunities for our product candidates are smaller than we believe they are, then our revenues may be adversely affected and our business may suffer.

The diseases that our current and future product candidates are being developed to address are rare. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, and our assumptions relating to pricing are based on estimates. Given the small number of patients who have the diseases that we are targeting, our eligible patient population and pricing estimates may differ significantly from the actual market addressable by our product candidates.

Currently, most reported estimates of the prevalence of vEDS, UCD and MSUD are based on studies of small subsets of the population of specific geographic areas, which are then extrapolated to estimate the prevalence of the diseases in the broader world population. It is difficult to precisely measure the incidence or prevalence of vEDS in any population. Studies estimate the prevalence of vEDS as ranging from approximately 1 in 90,000 to 1 in 250,000. Studies indicate that MSUD affects an estimated 1 in 185,000 infants worldwide.

Approximately 3,000 patients suffer from MSUD worldwide, of whom approximately 800 are located in the United States. It is estimated that vEDS, UCD and MSUD collectively impact approximately 7,000 patients in the United States. As new studies are performed the estimated prevalence of these diseases may change. The number of patients may turn out to be lower than expected. There can be no assurance that the prevalence of vEDS, UCD or MSUD in the study populations accurately reflect the prevalence of these diseases in the broader world population. If our estimates of the prevalence of vEDS, UCD or MSUD or of the number of patients who may benefit from treatment with EDSIVO™ or ACER-001 prove to be incorrect, the market opportunities for our product candidates may be smaller than we believe they are, our prospects for generating revenue may be adversely affected and our business may suffer. Likewise, the potentially addressable patient population for each of our product candidates may be limited or may not be amenable to treatment with our product candidates, and new patients may become increasingly difficult to identify or gain access to, which would adversely affect our business, financial condition, results of operations and prospects.

We currently have limited marketing and sales experience. If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may be unable to generate any revenue.

We have never commercialized a product candidate, and we currently have no marketing and sales organization. To the extent our product candidates are approved for marketing, if we are unable to establish marketing and sales capabilities or enter into agreements with third parties to market and sell our product candidates, we may not be able to effectively market and sell our product candidates or generate product revenue.

We have never commercialized a product candidate, and we currently do not have marketing, sales or distribution capabilities for our product candidates. In order to commercialize any of our products that receive marketing approval, we would have to build marketing, sales, distribution, managerial and other non-technical capabilities or make arrangements with third parties to perform these services, and we may not be successful in doing so. In the event of successful development of our product candidates, if we elect to build a targeted specialty sales force, such an effort would be expensive and time consuming. Any failure or delay in the development of our internal sales, marketing and distribution capabilities would adversely impact the commercialization of these products. We may choose to collaborate with third parties that have their own sales forces and established distribution systems, in lieu of or to augment any sales force and distribution systems we may create. If we are unable to enter into collaborations with third parties for the commercialization of approved products, if any, on acceptable terms or at all, or if any such collaborator does not devote sufficient resources to the commercialization of our product or otherwise fails in commercialization efforts, we may not be able to successfully commercialize our product candidates if we receive marketing approval. If we are not successful in commercializing our product candidates, either on our own or through collaborations with one or more third parties, our future revenue will be materially and adversely impacted.

If we fail to enter into strategic relationships or collaborations, our business, financial condition, commercialization prospects and results of operations may be materially adversely affected.

Our product development programs and the potential commercialization of our current product candidates will require substantial additional cash to fund expenses. Therefore, in addition to financing the development of our product candidates through additional equity financings or through debt financings, we may decide to enter into collaborations with pharmaceutical or biopharmaceutical companies for the development and potential commercialization of our product candidates.

We face significant competition in seeking appropriate collaborators. Collaborations are complex and time-consuming to negotiate and document. We may also be restricted under existing and future collaboration agreements from entering into agreements on certain terms with other potential collaborators. We may not be able to negotiate collaborations on acceptable terms, or at all. If that were to occur, we may have to curtail the development of a particular product, reduce or delay one or more of our development programs, delay our potential commercialization or reduce the scope of our sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we will not be able to bring our product candidates to market and generate product revenue. If we do enter into a collaboration agreement, it could be subject to the following risks, each of which may materially harm our business, commercialization prospects and financial condition:

- we may not be able to control the amount or timing of resources that the collaborator devotes to the product development program;
- the collaborator may experience financial difficulties and thus not commit sufficient financial resources to the product development program;
- we may be required to relinquish important rights such as marketing, distribution and intellectual property rights;
- a collaborator could move forward with a competing product developed either independently or in collaboration with third parties, including our competitors; or
- business combinations or significant changes in a collaborator's business strategy may adversely affect our willingness to complete our obligations under any arrangement.

Our product candidate EDSIVOTM has not been approved for any indication in the United States, which may result in greater research and development expenses, regulatory issues that could delay or prevent approval, or discovery of unknown or unanticipated adverse effects.

EDSIVOTM is a repurposing of celiprolol for the treatment of vEDS. An NDA for this drug in the treatment of hypertension was submitted to the FDA in 1987, however, the NDA was withdrawn prior to review. However, the drug has been approved in Europe for the treatment of hypertension since 1984. Regulatory approval of EDSIVOTM may be more expensive and take longer than for other, more well-known or extensively studied pharmaceutical product candidates due to our and regulatory agencies' lack of experience with celiprolol. The novelty of this product candidate may lengthen the regulatory review process, require us to conduct additional studies or clinical trials, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval limitations or restrictions. There is also an increased risk that we may discover previously unknown or unanticipated adverse effects during our clinical trials and beyond. Any such events could adversely impact our business prospects, financial condition and results of operations.

Coverage and reimbursement may be limited or unavailable in certain market segments for our product candidates, which could make it difficult for us to sell our products profitably.

There is significant uncertainty related to third-party coverage and reimbursement of newly approved pharmaceuticals. Market acceptance and sales of any approved product candidates will depend significantly on the availability of coverage and adequate reimbursement from third-party payors and may be affected by existing and future healthcare reform measures. Patients who are prescribed treatments for their conditions and providers performing the prescribed services generally rely on third-party payors to reimburse all or part of the associated healthcare costs. Government authorities and third-party payors, such as private health insurers, health maintenance organizations, and government payors like Medicare and Medicaid, decide which drugs they will pay for and establish reimbursement levels. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for drugs and products. Coverage and reimbursement may not be available for any product that we commercialize and, even if coverage is provided, the level of reimbursement may not be satisfactory. Inadequate reimbursement levels may adversely affect the demand for, or the price of, any drug candidate for which we obtain marketing approval.

Reimbursement by a third-party payor may depend upon a number of factors, including the third-party payor's determination that use of a product is, among other things:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

Obtaining coverage and adequate reimbursement approval for a product from a government or other third-party payor is a time consuming and costly process that could require us to conduct expensive pharmacoeconomic studies and provide supporting scientific, clinical and cost-effectiveness data for the use of our products to the payor. We may not be able to provide data sufficient to gain acceptance with respect to coverage and adequate reimbursement. In addition to examining the medical necessity and cost-effectiveness of new products, coverage may be limited 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. There may also be formulary placements that result in lower reimbursement levels and higher cost-sharing borne by patients, any of which could have an adverse effect on our revenues and profits. Moreover, a third-party 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. Additionally, coverage and reimbursement for drug products can differ significantly from payor to payor. One third-party payor's decision to cover a particular drug product does not ensure that other payors will also provide coverage for the drug product, or even if coverage is available, establish an adequate reimbursement rate.

We cannot be sure that coverage or adequate reimbursement will be available for any of our product candidates. Also, we cannot be sure that reimbursement amounts will not reduce the demand for, or the price of, our products. If reimbursement is not available or is available only to limited levels, we may not be able to commercialize certain of our products. In the United States, third-party payors are increasingly attempting to contain healthcare costs by limiting both coverage and the level of reimbursement of new drugs. Third-party payors are increasingly challenging the prices charged for medical products and services, examining the medical necessity and reviewing the cost-effectiveness of drug products and medical services and questioning safety and efficacy. As a result, significant uncertainty exists as to whether and how much third-party payors will reimburse patients for their use of newly approved drugs, which in turn will put pressure on the pricing of drugs. Additionally, emphasis on managed care in the United States has increased and we expect will continue to increase the pressure on drug pricing. If third-party payors do not consider our products to be cost-effective compared to other available therapies, they may not cover the products for which we receive FDA approval or, if they do, the level of payment may not be sufficient to allow us to sell our products at a profit.

Coverage policies, third-party reimbursement rates and drug pricing regulation may change at any time, and there is the potential for significant movement in these areas in the foreseeable future. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive marketing approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

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

The life sciences industry is highly competitive, and we face significant competition from many pharmaceutical, biopharmaceutical and biotechnology companies that are generally developing and marketing therapeutic products. Such competition may include large pharmaceutical and biotechnology companies, specialty pharmaceutical and generic companies and medical technology companies. Our future success depends on our ability to demonstrate and maintain a competitive advantage with respect to the design, development and commercialization of our product candidates for the treatment of orphan and ultraorphan diseases for which there is a small patient population in the United States. A drug designated an orphan drug may receive up to seven years of exclusive marketing in the United States for that indication. Our objective is to design, develop and commercialize product candidates by repurposing or reformulating existing drugs for orphan diseases with significant unmet medical need.

Many of our potential competitors have significantly greater financial, manufacturing, marketing, development, technical and human resources than we do. Large pharmaceutical and biotechnology companies, in particular, have extensive experience in clinical testing, obtaining regulatory approvals, recruiting patients and in manufacturing clinical products. These companies also have significantly greater research and marketing capabilities than we do and may also have products that have been approved or are in late stages of development, and have collaborative arrangements in our target markets with leading companies and research institutions. Established companies may also invest heavily to accelerate discovery and development of compounds that could make the product candidates that we develop obsolete. As a result of all of these factors, the obtaining of orphan drug designation for our product candidates is essential to our viability since our competitors may, among other things:

- have greater name and brand recognition, financial and human resources;
- develop and commercialize products that are safer, more effective, less expensive, or more convenient or easier to administer;
- obtain quicker marketing approval;
- establish superior proprietary positions;
- have access to more manufacturing capacity;
- implement more effective approaches to sales and marketing; or
- form more advantageous strategic alliances.

Should any of these events occur, our business, financial condition, results of operations, and prospects could be materially adversely affected. If we are not able to compete effectively against potential competitors, our business will not grow and our financial condition and operations will suffer.

We believe that our ability to successfully compete will depend on our ability to obtain orphan drug designation as well as:

- our ability to design and successfully execute appropriate clinical trials;
- our ability to recruit and enroll patients for our clinical trials;
- the results of our clinical trials and the efficacy and safety of our product candidates;
- the speed at which we develop our product candidates;
- achieving and maintaining compliance with regulatory requirements applicable to our business;

- the timing and scope of regulatory approvals, including labeling;
- adequate levels of reimbursement under private and governmental health insurance plans, including Medicare and Medicaid;
- our ability to protect intellectual property rights related to our product candidates;
- our ability to commercialize and market any of our product candidates that may receive marketing approval;
- our ability to manufacture and sell commercial quantities of any approved product candidates to the market;
- acceptance of our product candidates by physicians, other healthcare providers and patients; and
- the cost of treatment in relation to alternative therapies.

If our competitors are able to obtain orphan drug exclusivity for their products that are the same drug as our product candidates, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time or benefit from that exclusivity.

We have orphan drug exclusivity in the United States for EDSIVOTM for vEDS and ACER-001 for MSUD. We expect to seek orphan drug exclusivity from the European Medicines Agency, or the EMA, for ACER-001 for MSUD, however, there can be no assurance that we will be successful. If we are unable to maintain our current orphan drug exclusivity or are unable to secure orphan status in Europe for ACER-001 for MSUD, it may have a material negative effect on our business.

Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, that product is entitled to a period of marketing exclusivity, which precludes the applicable regulatory authority from approving another marketing application for the same drug for the same indication for that time period. The applicable period is seven years in the United States and ten years in the European Union. The exclusivity period in the European Union can be reduced to six years if the product no longer meets the criteria for orphan drug designation or if its commercialization is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be lost if the FDA or the EMA determines that the request for designation was materially defective or if the manufacturer is unable to ensure sufficient quantity of the product to meet the needs of patients with the rare disease or condition. Maintaining and/or obtaining orphan drug exclusivity for EDSIVOTM and ACER-001 may be important to the product candidate's success. Even if we obtain orphan drug exclusivity, we may not be able to maintain it. For example, if a competitive product that treats the same disease as our product candidate is shown to be clinically superior to our product candidate, any orphan drug exclusivity we have obtained will not block the approval of such competitive product and we may effectively lose what had previously been orphan drug exclusivity. Orphan drug exclusivity for EDSIVOTM or ACER-001 also will not bar the FDA from approving another celiprolol drug product or a sodium phenylbutyrate, or NaPB product, for another indication. In the United States, reforms to the Orphan Drug Act, if enacted, could also materially affect our ability to maintain orphan drug exclusivity for EDSIVOTM for vEDS and ACER-001 for MSUD.

Price controls may be imposed in foreign markets, which may adversely affect our future profitability.

In some countries, particularly member states of the European Union, the pricing of prescription drugs is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after receipt of marketing approval for a product. In addition, there can be considerable pressure by governments and other stakeholders on prices and reimbursement levels, including as part of cost containment measures. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various European Union member states and parallel distribution, or arbitrage between low-priced and high-priced member states, can further reduce prices. In some countries, we may be required to conduct a clinical trial or other studies that compare the cost-effectiveness of our product candidates to other available therapies in order to obtain or maintain reimbursement or pricing approval. Publication of discounts by third-party payors or authorities may lead to further pressure on the prices or reimbursement levels within the country of publication and other countries. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be adversely affected.

Rapid technological change could make our products obsolete.

Pharmaceutical technologies have undergone rapid and significant change, and we expect that they will continue to do so. As a result, there is significant risk that our product candidates may be rendered obsolete or uneconomical by new discoveries before we recover any expenses incurred in connection with their development. If our product candidates are rendered obsolete by advancements in pharmaceutical technologies, our prospects will suffer.

Government controls and healthcare reform measures could adversely affect our business.

The business and financial condition of pharmaceutical and biotechnology companies are affected by the efforts of governmental and third-party payors to contain or reduce the costs of healthcare. In the United States and in foreign jurisdictions, there have been, and we expect that there will continue to be, a number of legislative and regulatory proposals aimed at changing the healthcare system. For example, in some foreign countries, particularly in Europe, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product candidate. To obtain reimbursement or pricing approval in some countries, we may be required to conduct additional clinical trials that compare the cost-effectiveness of any product candidate to other available therapies. If reimbursement of any product candidate is unavailable or limited in scope or amount in a particular country, or if pricing is set at unsatisfactory levels, we may be unable to achieve or sustain profitability in such country. In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or MMA, changed the way Medicare covers and pays for pharmaceutical products. The legislation established Medicare Part D, which expanded Medicare coverage for outpatient prescription drug purchases by the elderly but provided authority for limiting the number of drugs that will be covered in any therapeutic class. The MMA also introduced a new reimbursement methodology based on average sales prices for physician-administered drugs. Any negotiated prices for any product candidate covered by a Part D prescription drug plan will likely be lower than the prices that might otherwise be obtained outside of the Medicare Part D prescription drug plan. Moreover, while Medicare Part D applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own payment rates. Any reduction in payment under Medicare Part D may result in a similar reduction in payments from non-governmental payors.

The United States and several other jurisdictions are considering, or have already enacted, a number of legislative and regulatory proposals to change the healthcare system in ways that could affect our ability to sell any product candidate. Among policy-makers and payors in the United States and elsewhere, there is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and/or expanding access to healthcare. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives. There have been, and likely will continue to be, legislative and regulatory proposals at the federal and state levels directed at broadening the availability of healthcare and containing or lowering the cost of healthcare. We cannot predict the initiatives that may be adopted in the future. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare may adversely affect: the demand for any product candidate; the ability to set a price that we believe is fair for any product candidate; our ability to generate revenues and achieve or maintain profitability; the level of taxes that we are required to pay; and the availability of capital.

Risks Related to Third Parties

We rely on third-party suppliers and other third parties for production of our product candidates and our dependence on these third parties may impair the advancement of our research and development programs and the development of our product candidates.

We do not currently own or operate manufacturing facilities for clinical or commercial production of our product candidates. We lack the resources and the capability to manufacture any of our product candidates on a clinical or commercial scale. Instead, we rely on, and expect to continue to rely on, third parties for the supply of raw materials and manufacture of drug supplies necessary to conduct our preclinical studies and clinical trials. Our reliance on third parties may expose us to more risk than if we were to manufacture our current product candidates or other products ourselves. Delays in production by third parties could delay our clinical trials or have an adverse

impact on any commercial activities. In addition, the fact that we are dependent on third parties for the manufacture of and formulation of our product candidates means that we are subject to the risk that the products may have manufacturing defects that we have limited ability to prevent or control. Although we oversee these activities to ensure compliance with our quality standards, budgets and timelines, we have had and will continue to have less control over the manufacturing of our product candidates than potentially would be the case if we were to manufacture our product candidates. Further, the third parties we deal with could have staffing difficulties, might undergo changes in priorities or may become financially distressed, which would adversely affect the manufacturing and production of our product candidates. In addition, a third party could be acquired by, or enter into an exclusive arrangement with, one of our competitors, which would adversely affect our ability to access the formulations we require.

The facilities used by our current contract manufacturers and any future manufacturers to manufacture our product candidates must be inspected by the FDA after we submit our NDA. We do not control the manufacturing process of, and are completely dependent on, our contract manufacturers for compliance with the regulatory requirements, known as cGMPs, for manufacture of both active drug substances and finished drug products. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or others, the FDA may refuse to approve our NDA. If the FDA or a comparable foreign regulatory authority does not approve our NDA because of concerns about the manufacture of our product candidates or if significant manufacturing issues arise in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop our product candidates, to obtain marketing approval of our NDA or to continue to market our product candidates, if approved. Although we are ultimately responsible for ensuring compliance with these regulatory requirements, we do not have day-to-day control over a contract manufacturing organization, or CMO, or other third-party manufacturer's compliance with applicable laws and regulations, including cGMPs and other laws and regulations, such as those related to environmental health and safety matters. Any failure to achieve and maintain compliance with these laws, regulations and standards could subject us to the risk that we may have to suspend the manufacturing of our product candidates or that obtained approvals could be revoked, which would adversely affect our business and reputation. In addition, third-party contractors, such as our CMOs, may elect not to continue to work with us due to factors beyond our control. They may also refuse to work with us because of their own financial difficulties, business priorities or other reasons, at a time that is costly or otherwise inconvenient for us. If we were unable to find adequate replacement or another acceptable solution in time, our clinical trials could be delayed or our commercial activities could be harmed.

Problems with the quality of the work of third parties may lead us to seek to terminate our working relationships and use alternative service providers. However, making this change may be costly and may delay clinical trials. In addition, it may be very challenging, and in some cases impossible, to find replacement service providers that can develop and manufacture our drug candidates in an acceptable manner and at an acceptable cost and on a timely basis. The sale of products containing any defects or any delays in the supply of necessary services could adversely affect our business, financial condition, results of operations, and prospects.

Growth in the costs and expenses of components or raw materials may also adversely affect our business, financial condition, results of operations, and prospects. Supply sources could be interrupted from time to time and, if interrupted, supplies may not be resumed (whether in part or in whole) within a reasonable timeframe and at an acceptable cost or at all.

We plan to rely on third parties to conduct clinical trials for our product candidates. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, it may cause delays in commencing and completing clinical trials of our product candidates or we may be unable to obtain marketing approval for or commercialize our product candidates.

Clinical trials must meet applicable FDA and foreign regulatory requirements. We do not have the ability to independently conduct Phase 2 or Phase 3 clinical trials for any of our product candidates. We expect to rely on third parties, such as CROs, medical institutions, clinical investigators and contract laboratories, to conduct all of our clinical trials of our product candidates; however, we remain responsible for ensuring that each of our clinical trials is conducted in accordance with our investigational plan and protocol. Moreover, the FDA and other foreign regulatory authorities require us to comply with IND and human subject protection regulations and current good

clinical practice standards, commonly referred to as GCPs, for conducting, monitoring, recording, and reporting the results of clinical trials to ensure that the data and results are scientifically credible and accurate and that the trial subjects are adequately informed of the potential risks of participating in clinical trials. Our reliance on third parties does not relieve us of these responsibilities and requirements. Regulatory authorities enforce these GCPs through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of our third-party contractors fail to comply with applicable GCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. There is no assurance that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials comply with GCPs. Our failure to comply with these regulations may require us to repeat clinical trials, which would delay the marketing approval process.

There are significant requirements imposed on us and on clinical investigators who conduct clinical trials that we sponsor. Although we are responsible for selecting qualified CROs or clinical investigators, providing them with the information they need to conduct the clinical trials properly, ensuring proper monitoring of the clinical trials, and ensuring that the clinical trials are conducted in accordance with the general investigational plan and protocols contained in the IND, we cannot ensure that the CROs or clinical investigators will maintain compliance with all regulatory requirements at all times. The pharmaceutical industry has experienced cases where clinical investigators have been found to incorrectly record data, omit data, or even falsify data. We cannot ensure that the CROs or clinical investigators in our trials will not make mistakes or otherwise compromise the integrity or validity of data, any of which would have a significant negative effect on our ability to obtain marketing approval, our business, and our financial condition.

We or the third parties we rely on may encounter problems in clinical trials that may cause us or the FDA or foreign regulatory agencies to delay, suspend or terminate our clinical trials at any phase. These problems could include the possibility that we may not be able to manufacture sufficient quantities of materials for use in our clinical trials, conduct clinical trials at our preferred sites, enroll a sufficient number of patients for our clinical trials at one or more sites, or begin or successfully complete clinical trials in a timely fashion, if at all. Furthermore, we, the FDA or foreign regulatory agencies may suspend clinical trials of our product candidates at any time if we or they believe the subjects participating in the trials are being exposed to unacceptable health risks, whether as a result of adverse events occurring in our trials or otherwise, or if we or they find deficiencies in the clinical trial process or conduct of the investigation.

The FDA and foreign regulatory agencies could also require additional clinical trials before or after granting of marketing approval for any products, which would result in increased costs and significant delays in the development and commercialization of such products and could result in the withdrawal of such products from the market after obtaining marketing approval. Our failure to adequately demonstrate the safety and efficacy of a product candidate in clinical development could delay or prevent obtaining marketing approval of the product candidate and, after obtaining marketing approval, data from post-approval studies could result in the product being withdrawn from the market, either of which would likely have a material adverse effect on our business.

Risks Related to Our Intellectual Property

Our proprietary rights may not adequately protect our technologies and product candidates.

Our commercial success will depend in part on our ability to obtain additional patents and protect our existing patent position as well as our ability to maintain adequate protection of other intellectual property for our technologies, product candidates, and any future products in the United States and other countries. If we do not adequately protect our intellectual property, competitors may be able to use our technologies and erode or negate any competitive advantage we may have, which could harm our business and ability to achieve profitability. The laws of some foreign countries do not protect our proprietary rights to the same extent or in the same manner as U.S. laws, and we may encounter significant problems in protecting and defending our proprietary rights in these countries. We will be able to protect our proprietary rights from unauthorized use by third parties only to the extent that our proprietary technologies, product candidates and any future products are covered by valid and enforceable patents or are effectively maintained as trade secrets.

We apply for patents covering both our technologies and product candidates, as we deem appropriate. However, we may fail to apply for patents on important technologies or product candidates in a timely fashion, or at all. Our existing patents and any future patents we obtain may not be sufficiently broad to prevent others from practicing our technologies or from developing competing products and technologies. We cannot be certain that our patent applications will be approved or that any patents issued will adequately protect our intellectual property.

While we are responsible for and have control over the filing and prosecuting of patent applications and maintaining patents which cover making, using or selling EDSIVO™ and ACER-001, we may lose such rights if we decide to allow any licensed patent to lapse. If we fail to appropriately prosecute and maintain patent protection for any of our product candidates, our ability to develop and commercialize those product candidates may be adversely affected and we may not be able to prevent competitors from making, using and selling competing products.

Moreover, the patent positions of pharmaceutical companies are highly uncertain and involve complex legal and factual questions for which important legal principles are evolving and remain unresolved. As a result, the validity and enforceability of patents cannot be predicted with certainty. In addition, we do not know whether:

- we or our licensors were the first to make the inventions covered by each of our issued patents and pending patent applications;
- we or our licensors were the first to file patent applications for these inventions;
- any of the patents that cover our product candidates will be eligible to be listed in the FDA's compendium of "Approved Drug Products with Therapeutic Equivalence Evaluation," sometimes referred to as the FDA's Orange Book;
- others will independently develop similar or alternative technologies or duplicate any of our technologies;
- any of our or our licensors' pending patent applications will result in issued patents;
- any of our or our licensors' patents will be valid or enforceable;
- any patents issued to us or our licensors and collaborators will provide us with any competitive advantages, or will be challenged by third parties;
- we will develop additional proprietary technologies that are patentable;
- the U.S. government will exercise any of its statutory rights to our intellectual property that was developed with government funding; or
- our business may infringe the patents or other proprietary rights of others.

The actual protection afforded by a patent varies based on products or processes, from country to country and depends upon many factors, including the type of patent, the scope of its coverage, the availability of regulatory related extensions, the availability of legal remedies in a particular country, the validity and enforceability of the patents and our financial ability to enforce our patents and other intellectual property. Our ability to maintain and solidify our proprietary position for our products will depend on our success in obtaining effective claims and enforcing those claims once granted. Our issued patents and those that may issue in the future, or those licensed to us, may be challenged, narrowed, invalidated or circumvented, and the rights granted under any issued patents may not provide us with proprietary protection or competitive advantages against competitors with similar products. Due to the extensive amount of time required for the development, testing and regulatory review of a potential product, it is possible that, before any of our product candidates can be commercialized, any related patent may expire or remain in force for only a short period following commercialization, thereby reducing any advantage of the patent.

We may also rely on trade secrets to protect some of our technology, especially where we do not believe patent protection is appropriate or obtainable. However, trade secrets are difficult to maintain. While we use reasonable efforts to protect our trade secrets, we or any of our collaborators' employees, consultants, contractors or scientific and other advisors may unintentionally or willfully disclose our proprietary information to competitors and we may not have adequate remedies in respect of that disclosure. Enforcement of claims that a third party has illegally obtained and is using trade secrets is expensive, time consuming and uncertain. In addition, foreign courts are sometimes less willing than U.S. courts to protect trade secrets. If our competitors independently develop equivalent knowledge, methods and know-how, we would not be able to assert our trade secrets against them and our business could be harmed.

We are a party to license agreements under which we license intellectual property and receive commercialization rights relating to EDSIVOTM and ACER-001. If we fail to comply with obligations in such agreements or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business; any termination of such agreements would adversely affect our business.

In April 2014, we entered into an agreement with Baylor College of Medicine pursuant to which we obtained an exclusive worldwide license to develop and commercialize NaPB for treatment of MSUD. In August 2016, we entered into an agreement with Assistance Publique—Hôpitaux de Paris, Hôpital Européen Georges Pompidou, or AP-HP, pursuant to which we obtained an exclusive worldwide right to access and use data from the Ong trial, which we intend to use to support an NDA filing for EDSIVOTM for the treatment of vEDS. Under each license agreement, we are subject to commercialization and development diligence obligations, royalty payments and other obligations. If we fail to comply with any of these obligations or otherwise breach any of these license agreements, the licensor may have the right to terminate the license in whole or in part or to terminate the exclusive nature of the license. The loss of the licenses granted to us under our agreements with these licensors or the rights provided therein would prevent us from developing, manufacturing or marketing products covered by the license or subject to supply commitments, and could materially harm our business, financial condition, results of operations and prospects.

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

Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. For example, many foreign countries have compulsory licensing laws under which a patent owner must grant licenses to third parties. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and further, may export otherwise infringing products to territories where we have patent protection, but enforcement rights are not as strong as those in the United States. These products may compete with our product candidates in jurisdictions where we do not have any issued patents and our patent claims or other intellectual rights may not be effective or sufficient to prevent them from so competing.

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

The patent protection for our product candidates may expire before we are able to maximize their commercial value, which may subject us to increased competition and reduce or eliminate our opportunity to generate product revenue.

The patents for our product candidates have varying expiration dates and, if these patents expire, we may be subject to increased competition and we may not be able to recover our development costs or market any of our approved products profitably. In some of the larger potential market territories, such as the United States and Europe, patent term extension or restoration may be available to compensate for time taken during aspects of the product's development and regulatory review. For example, depending on the timing, duration and specifics of FDA marketing approval of our product candidates, if any, one of the U.S. patents covering each of such approved product(s) or the use thereof may be eligible for up to five years of patent term restoration under the Hatch-Waxman Act. The Hatch-Waxman Act allows a maximum of one patent to be extended per FDA-approved product. Patent term extension also may be available in certain foreign countries upon regulatory approval of our product candidates.

Nevertheless, we may not be granted patent term extension either in the United States or in any foreign country because of, for example, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the term of extension, as well as the scope of patent protection during any such extension, afforded by the governmental authority could be less than we request. In addition, even though some regulatory authorities may provide some other exclusivity for a product under their own laws and regulations, we may not be able to qualify the product or obtain the exclusive time period. If we are unable to obtain patent term extension/restoration or some other exclusivity, we could be subject to increased competition and our opportunity to establish or maintain product revenue could be substantially reduced or eliminated. Furthermore, we may not have sufficient time to recover our development costs prior to the expiration of our U.S. and foreign patents.

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

The United States Patent and Trademark Office, or USPTO, and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent prosecution process. Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on any issued patent and/or pending patent applications are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of a patent or patent application. We employ an outside firm and rely on our outside counsel to pay these fees. While an inadvertent lapse may sometimes be cured by payment of a late fee or by other means in accordance with the applicable rules, there are many situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. If we fail to maintain the patents and patent applications directed to our product candidates, our competitors might be able to enter the market earlier than should otherwise have been the case, which would have a material adverse effect on our business.

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

Competitors may infringe our patents or other intellectual property rights. To counter infringement or unauthorized use, we may be required to file infringement claims, directly or through our licensors, which can be expensive and time consuming. In addition, in an infringement proceeding, a court may decide that a patent of our licensor is not valid or is unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of the patents we license at risk of being invalidated or interpreted narrowly and could put our licensors' patent applications at risk of not issuing.

Interference proceedings brought by the USPTO may be necessary to determine the priority of inventions with respect to our patents or the patents of our licensors. An unfavorable outcome could require us to cease using the technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if a prevailing party does not offer us a license on terms that are acceptable to us. Litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distraction of our management and other employees. We may not be able to prevent, alone or with our licensors, misappropriation of our proprietary rights, particularly in countries where the laws may not protect those rights as fully as in the United States.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential and proprietary information could be compromised by disclosure during this type of litigation. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock.

Third-party claims of intellectual property infringement or misappropriation may adversely affect our business and could prevent us from developing or commercializing our product candidates.

Our commercial success depends in part on us not infringing the patents and proprietary rights of third parties. There is a substantial amount of litigation, both within and outside the United States, involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, interferences, oppositions, ex-parte review and inter partes reexamination and post-grant review proceedings before the USPTO and corresponding foreign patent offices. Numerous U.S. and foreign issued patents and pending patent applications owned by third parties exist in the fields in which we are developing and may develop our product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may be subject to claims of infringement of the patent rights of third parties. If a third party claims that we infringe on their products or technology, we could face a number of issues, including:

- infringement and other intellectual property claims which, with or without merit, can be expensive and time-consuming to litigate and can divert management's attention from our core business;
- substantial damages for past infringement, which we may have to pay if a court decides that our product infringes on a competitor's patent;
- a court prohibiting us from selling or licensing our product unless the patent holder licenses the patent to us, which the collaborator would not be required to do;
- if a license is available from a patent holder, we may have to pay substantial royalties or grant cross licenses to our patents; and
- redesigning our processes so they do not infringe, which may not be possible or could require substantial funds and time.

Third parties may assert that we are employing their proprietary technology without authorization. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates that we failed to identify. 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 issued as patents. Except for the preceding exceptions, patent applications in the United States and elsewhere are generally published only after a waiting period of approximately 18 months after the earliest filing. Therefore, patent applications covering our product candidates could have been filed by others without the knowledge of us or our licensors. Additionally, pending patent applications which have been published can, subject to certain limitations, be later amended in a manner that could cover our product candidates or the use or manufacture of our product candidates. We may also face a claim of misappropriation if a third party believes that we inappropriately obtained and used trade secrets of such third party. If we are found to have misappropriated a third party's trade secrets, we may be prevented from further using such trade secrets, limiting our ability to develop our product candidates, and we may be required to pay damages.

If any third-party patents were held by a court of competent jurisdiction to cover aspects of our materials, formulations, methods of manufacture or methods for treatment, the holders of any such patents would be able to block our ability to develop and commercialize the applicable product candidate until such patent expired or unless we obtain a license. These licenses may not be available on acceptable terms, if at all. Even if we were able to obtain a license, the rights may be nonexclusive, which could result in our competitors gaining access to the same intellectual property.

Ultimately, we could be prevented from commercializing a product, or be forced to cease some aspect of our business operations, if, as a result of actual or threatened patent infringement claims, we are unable to enter into licenses on acceptable terms.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defending against claims of patent infringement or misappropriation of trade secrets 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. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, pay royalties, redesign our infringing products or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure. In addition, the uncertainties associated with litigation could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our research programs, license necessary technology from third parties, or enter into development collaborations that would help us bring our product candidates to market.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our product candidates.

As is the case with other pharmaceutical companies, our success is heavily dependent on intellectual property, particularly on obtaining and enforcing patents and patent rights. Obtaining and enforcing patents and patent rights in the specialty pharmaceutical industry involves both technological and legal complexity, and therefore, is costly, time-consuming and inherently uncertain. In addition, the United States has recently enacted and is currently implementing wide-ranging patent reform legislation. Further, several recent U.S. Supreme Court rulings have either narrowed the scope of patent protection available in certain circumstances or weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents and patent rights, once obtained.

For our U.S. patent applications containing a claim not entitled to priority before March 16, 2013, there is a greater level of uncertainty in the patent law. In September 2011, the Leahy-Smith America Invents Act, or the America Invents Act, or AIA, was signed into law. The AIA includes a number of significant changes to U.S. patent law, including provisions that affect the way patent applications will be prosecuted, reviewed after issuance, and may also affect patent litigation. The USPTO is currently developing regulations and procedures to govern administration of the AIA, and many of the substantive changes to patent law associated with the AIA. It is not clear what other, if any, impact the AIA will have on the operation of our business. Moreover, the AIA and its implementation could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of patent rights, all of which could have a material adverse effect on our business and financial condition.

An important change introduced by the AIA is that, as of March 16, 2013, the United States transitioned to a "first-inventor-to-file" system for deciding which party should be granted a patent when two or more patent applications are filed by different parties claiming the same invention. A third party that files a patent application in the USPTO after that date but before a licensor or us could therefore be awarded a patent covering an invention of ours even if said licensor or we had made the invention before it was made by the third party. This will require us to be cognizant going forward of the time from invention to filing of a patent application. Furthermore, our ability to obtain and maintain valid and enforceable patent rights depends on whether the differences between the licensor's or our technology and the prior art allow our technology to be patentable over the prior art. Since patent applications in the United States and most other countries are confidential for a period of time after filing, we cannot be certain that a licensor or we were the first to either (a) file any patent application related to our product candidates or (b) invent any of the inventions claimed in our patents or patent applications.

Among some of the other changes introduced by the AIA are changes that limit where a patentee may file a patent infringement suit and providing opportunities for third parties to challenge any issued patent in the USPTO. This applies to all U.S. patents, even those issued before March 16, 2013. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in United States federal court necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim invalid as unpatentable even though the same evidence may be insufficient to invalidate the claim if first presented in a district court action. Accordingly, a third party may attempt to use the USPTO procedures to invalidate patent rights that would not have been invalidated if first challenged by the third party as a defendant in a district court action.

Depending on decisions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

Intellectual property rights do not address 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 products that are similar to our product candidates but that are not covered by the claims of the patents that we license from others or may license or own in the future.
- Others may independently develop similar or alternative technologies or otherwise circumvent any of our technologies without infringing our intellectual property rights.
- Any of our collaborators might not have been the first to conceive and reduce to practice the inventions covered by the patents or patent applications that we license or will, in the future, own or license.
- Any of our collaborators might not have been the first to file patent applications covering certain of the patents or patent applications that we license or will, in the future, license.
- Issued patents that have been licensed to us may not provide us with any competitive advantage, or may be held invalid or unenforceable, as a result of legal challenges by our competitors.
- Our competitors might conduct research and development activities in countries where we do not have license rights, or in countries where research and development safe harbor laws exist, and then use the information learned from such activities to develop competitive products for sale in our major commercial markets.
- Ownership of patents or patent applications licensed to us may be challenged by third parties.
- The patents of third parties or pending or future applications of third parties, if issued, may have an adverse effect on our business.

Confidentiality agreements with employees, consultants and others may not adequately prevent disclosure of trade secrets and protect other proprietary information.

We consider proprietary trade secrets and/or confidential know-how and unpatented know-how to be important to our business. We may rely on trade secrets and/or confidential know-how to protect our technology, especially where patent protection is believed by us to be of limited value. However, trade secrets and/or confidential know-how can be difficult to maintain as confidential.

To protect this type of information against disclosure or appropriation by competitors, our policy is to require our employees, consultants, contractors and advisors to enter into confidentiality agreements with us. However, current or former employees, consultants, contractors and advisers may unintentionally or willfully disclose our confidential information to competitors, and confidentiality agreements may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. Enforcing a claim that a third party obtained illegally and is using trade secrets and/or confidential know-how is expensive, time consuming and unpredictable. The enforceability of confidentiality agreements may vary from jurisdiction to jurisdiction.

Failure to obtain or maintain trade secrets and/or confidential know-how trade protection could adversely affect our competitive position. Moreover, our competitors may independently develop substantially equivalent proprietary information and may even apply for patent protection in respect of the same. If successful in obtaining such patent protection, our competitors could limit our use of our trade secrets and/or confidential know-how.

We may need to license certain intellectual property from third parties, and such licenses may not be available or may not be available on commercially reasonable terms.

A third party may hold intellectual property, including patent rights that are important or necessary to the development or commercialization of our product candidates. It may be necessary for us to use the patented or proprietary technology of third parties to commercialize our product candidates, in which case we would be required to obtain a license from these third parties. Such a license may not be available on commercially reasonable terms or at all, which could materially harm our business.

We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties.

We have received confidential and proprietary information from third parties. In addition, we employ individuals who were previously employed at other biotechnology or pharmaceutical companies. We may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise improperly used or disclosed confidential information of these third parties or our employees' former employers.

Further, we may be subject to ownership disputes in the future arising, for example, from conflicting obligations of consultants or others who are involved in developing our product candidates. We may also be subject to claims that former employees, consultants, independent contractors, collaborators or other third parties have an ownership interest in our patents or other intellectual property. Litigation may be necessary to defend against these and other claims challenging our right to and use of confidential and proprietary information. If we fail in defending any such claims, in addition to paying monetary damages, we may lose our rights therein. Such an outcome could have a material adverse effect on our business.

Even if we are successful in defending against these claims, litigation could result in substantial cost and be a distraction to our management and employees.

We may be subject to claims challenging the inventorship or ownership of our patents and other intellectual property.

We may also be subject to claims that former employees, collaborators or other third parties have an ownership interest in our patents and other intellectual property. We may be subject to ownership disputes in the future arising, for example, from conflicting obligations of consultants or others who are involved in developing our product candidates. Litigation may be necessary to defend against these and other claims challenging inventorship or ownership. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

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

Because we rely on third parties to assist with research and development and to manufacture our product candidates, we must, at times, share trade secrets with them. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with our advisors, employees, third-party contractors and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have a material adverse effect on our business.

In addition, these agreements typically restrict the ability of our advisors, employees, third-party contractors and consultants to publish data potentially relating to our trade secrets, although our agreements may contain certain limited publication rights. For example, any academic institution that we may collaborate with in the future will usually expect to be granted rights to publish data arising out of such collaboration, provided that we are notified in advance and given the opportunity to delay publication for a limited time period in order for us to secure patent protection of intellectual property rights arising from the collaboration, in addition to the opportunity to remove confidential or trade secret information from any such publication. In the future we may also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development or similar agreements. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of our agreements with third parties, independent development or publication of information by any of our third-party collaborators. A competitor's discovery of our trade secrets would impair our competitive position and have an adverse impact on our business.

Risks Related to Our Securities

There is currently a limited market for our securities, and any trading market that exists in our securities may be highly illiquid and may not reflect the underlying value of our net assets or business prospects.

Although our common stock is traded on the NASDAQ Capital Market, there is currently a limited market for our securities and there can be no assurance that an active market will ever develop. Investors are cautioned not to rely on the possibility that an active trading market may develop.

Our stock may be delisted from NASDAQ, which could affect our market price and liquidity.

We are required to meet certain qualitative and financial tests (including a minimum bid price for our common stock of $1.00 per share and a minimum shareholders' equity of $2.5 million), as well as certain corporate governance standards, to maintain the listing of our common stock on the NASDAQ Capital Market. While we are exercising diligent efforts to maintain the listing of our common stock and warrants on NASDAQ, there can be no assurance that we will be able to do so, and our securities could be delisted.

Our share price is volatile, and you may not be able to resell your shares at a profit or at all.

The market price of our common stock could be subject to significant fluctuations. The market prices for securities of pharmaceutical and biotechnology companies, and early-stage drug discovery and development companies like ours in particular, have historically been highly volatile and may continue to be highly volatile in the future. The following factors, in addition to other risk factors described in this section, may have a significant impact on the market price of our common stock:

- announcements of significant changes in our business or operations;
- the development status of any of our drug candidates, such as EDSIVO™ or ACER-001, including clinical study results and determinations by regulatory authorities with respect thereto;

- the initiation, termination or reduction in the scope of any collaboration arrangements or any disputes or developments regarding such collaborations;
- our inability to obtain additional funding;
- announcements of technological innovations, new commercial products or other material events by our competitors or by us;
- disputes or other developments concerning our proprietary rights;
- changes in, or failure to meet, securities analysts' or investors' expectations of our financial performance;
- additions or departures of key personnel;
- discussions of our business, products, financial performance, prospects or stock price by the financial and scientific press and online investor communities;
- public concern as to, and legislative action with respect to, the pricing and availability of prescription drugs or the safety of drugs and drug delivery techniques;
- regulatory developments in the United States and in foreign countries; or
- dilutive effects of sales of shares of common stock by us or our shareholders, and sales of common stock acquired upon exercise or conversion by the holders of warrants, options or convertible notes.

Broad market and industry factors, as well as economic and political factors, also may materially adversely affect the market price of our common stock.

We may be or become the target of securities litigation, which is costly and time-consuming to defend.

In the past, following periods of market volatility in the price of a company's securities or the reporting of unfavorable news, security holders have often instituted class action litigation. This risk is especially relevant for us because pharmaceutical companies have experienced significant stock price volatility in recent years. If we become involved in this type of litigation, regardless of the outcome, we could incur substantial legal costs and our management's attention could be diverted from the operation of our business, causing our business to suffer.

Our "blank check" preferred stock could be issued to prevent a business combination not desired by management or our majority shareholders.

Our charter authorizes the issuance of "blank check" preferred stock with such designations, rights and preferences as may be determined by our board of directors without shareholder approval. Our preferred stock could be utilized as a method of discouraging, delaying, or preventing a change in control and as a method of preventing shareholders from receiving a premium for their shares in connection with a change of control.

Future sales of our securities could cause dilution, and the sale of such securities, or the perception that such sales may occur, could cause the price of our stock to fall.

Sales of additional shares of our common stock, as well as securities convertible into or exercisable for common stock, could result in substantial dilution to our shareholders and cause the market price of our common stock to decline. An aggregate of 7,497,433 shares of common stock were outstanding as of December 31, 2017. As of such date, another (i) 463,600 shares of common stock were issuable upon exercise of outstanding options and (ii) 317,630 shares of common stock were issuable upon the exercise of outstanding warrants. A substantial majority of the outstanding shares of our common stock and warrants (as well as a substantial majority of the shares of common stock issuable upon exercise of outstanding options and warrants) are freely tradable without restriction or further registration under the Securities Act of 1933.

We may sell additional shares of common stock, as well as securities convertible into or exercisable for common stock, in subsequent public or private offerings. We may also issue additional shares of common stock, as well as securities convertible into or exercisable for common stock, to finance future acquisitions. We will need to raise additional capital in order to initiate or complete additional development activities for EDSIVOTM in vEDS and for ACER-001 in UCD and MSUD, or to pursue additional disease indications for our product candidates, and this may require us to issue a substantial amount of securities (including common stock as well as securities convertible into or exercisable for common stock). There can be no assurance that our capital raising efforts will be able to attract the capital needed to execute on our business plan and sustain our operations. Moreover, we cannot predict the size of future issuances of our common stock, as well as securities convertible into or exercisable for common stock, or the effect, if any, that future issuances and sales of our securities will have on the market price of our common stock. Sales of substantial amounts of our common stock, as well as securities convertible into or exercisable for common stock, including shares issued in connection with an acquisition or securing funds to complete any clinical trial plans, or the perception that such sales could occur, may result in substantial dilution and may adversely affect prevailing market prices for our common stock.

We presently do not intend to pay cash dividends on our common stock.

We currently anticipate that no cash dividends will be paid on the common stock in the foreseeable future. While our dividend policy will be based on the operating results and capital needs of the business, it is anticipated that all earnings, if any, will be retained to finance the future expansion of our business.

Our shareholders may experience substantial dilution in the value of their investment if we issue additional shares of our capital stock.

Our charter allows us to issue up to 150,000,000 shares of common stock and to issue and designate the rights of, without shareholder approval, up to 10,000,000 shares of preferred stock. In order to raise additional capital, we may in the future offer additional shares of our common stock or other securities convertible into or exchangeable for our common stock at prices that may not be the same as the price per share paid by other investors, and dilution to our shareholders could result. We may sell shares or other securities in any other offering at a price per share that is less than the price per share paid by investors, and investors purchasing shares or other securities in the future could have rights superior to existing shareholders. The price per share at which we sell additional shares of our common stock, or securities convertible or exchangeable into common stock, in future transactions may be higher or lower than the price per share paid by other investors.

We may issue debt and equity securities or securities convertible into equity securities, any of which may be senior to our common stock as to distributions and in liquidation, which could negatively affect the value of our common stock.

In the future, we may attempt to increase our capital resources by entering into debt or debt-like financing that is unsecured or secured by up to all of our assets, or by issuing additional debt or equity securities, which could include issuances of secured or unsecured commercial paper, medium-term notes, senior notes, subordinated notes, guarantees, preferred stock, hybrid securities, or securities convertible into or exchangeable for equity securities. In the event of our liquidation, our lenders and holders of our debt and preferred securities would receive distributions of available assets before distributions to the holders of our common stock. Because our decision to incur debt and issue securities in future offerings may be influenced by market conditions and other factors beyond our control, we cannot predict or estimate the amount, timing or nature of our future offerings or debt financings. Further, market conditions could require us to accept less favorable terms for the issuance of our securities in the future.

Our management has significant flexibility in using the current available cash.

In addition to general corporate purposes (including working capital, research and development, business development and operational purposes), we currently intend to use our available cash to continue the development of our drug candidates, such as EDSIVO™ and ACER-001, to seek regulatory approval for EDSIVO™, and to invest in precommercial activities for EDSIVO™. Depending on future developments and circumstances, we may use some of our available cash for other purposes which may have the potential to decrease our cash runway. Notwithstanding our current intentions regarding use of our available cash, our management will have significant flexibility with respect to such use. The actual amounts and timing of expenditures will vary significantly depending on a number of factors, including the amount and timing of cash used in our operations and our research and development efforts. Management's failure to use these funds effectively would have an adverse effect on the value of our common stock and could make it more difficult and costly to raise funds in the future.

Because the Merger resulted in an ownership change under Section 382 of the Internal Revenue Code, our pre-Merger net operating loss carryforwards and certain other tax attributes will be subject to limitation or elimination. The net operating loss carryforwards and certain other tax attributes of Private Acer may also be subject to limitations as a result of ownership changes.

If a corporation undergoes an "ownership change" within the meaning of Section 382 of the Internal Revenue Code, or Section 382, the corporation's net operating loss carryforwards and certain other tax attributes arising from before the ownership change are subject to limitations on use after the ownership change. In general, an ownership change occurs if there is a cumulative change in the corporation's equity ownership by certain shareholders that exceeds fifty percentage points by value over a rolling three-year period. Similar rules may apply under state tax laws. The Merger resulted in an ownership change for us and, accordingly, our net operating loss carryforwards and certain other tax attributes will now be subject to limitation and possibly elimination. It is possible that Private Acer's net operating loss carryforwards and certain other tax attributes may also be subject to limitation as a result of prior shifts in equity ownership and/or the Merger. Additional ownership changes in the future could result in additional limitations on our and Private Acer's net operating loss carryforwards and certain other tax attributes. Consequently, even if we achieve profitability, we may not be able to utilize a material portion of our or Private Acer's net operating loss carryforwards and certain other tax attributes, which could have a material adverse effect on cash flow and results of operations.

Because the ownership of our common stock is highly concentrated, it may prevent shareholders from influencing significant corporate decisions and may result in conflicts of interest that could cause our stock price to decline.

Our executive officers and directors and their affiliates beneficially own or control approximately 57% of the outstanding shares of our common stock. Accordingly, these executive officers, directors and their affiliates, acting as a group, will have substantial influence over the outcome of corporate actions requiring shareholder approval, including the election of directors, any merger, consolidation or sale of all or substantially all of our assets or any other significant corporate transactions. These shareholders may also delay or prevent a change of control of our company, even if such a change of control would benefit our other shareholders. The significant concentration of stock ownership may adversely affect the trading price of our common stock due to investors' perception that conflicts of interest may exist or arise.

Anti-takeover provisions in our organizational documents and Texas law might discourage or delay acquisition attempts for our company that you might consider favorable.

Our certificate of formation and bylaws contain provisions that may delay or prevent an acquisition or change in control of our company. Among other things, these provisions:

- authorize the board of directors to issue without shareholder approval blank-check preferred stock that, if issued, could operate as a "poison pill" to dilute the stock ownership of a potential hostile acquirer to prevent an acquisition that is not approved by the board of directors;
- establish advance notice requirements for shareholder nominations of directors and for shareholder proposals that can be acted on at shareholder meetings; and

- limit who may call shareholder meetings.

Further, as a Texas corporation, we are also subject to provisions of Texas law, which may impair a takeover attempt that our shareholders may find beneficial. These anti-takeover provisions and other provisions under Texas law could discourage, delay or prevent a transaction involving a change in control of our company, including actions that our shareholders may deem advantageous, or negatively affect the trading price of our common stock. These provisions could also discourage proxy contests and make it more difficult for you and other shareholders to elect directors of your choosing and to cause us to take other corporate actions you desire.

Item 1B. Unresolved Staff Comments.

None.

Item 2. Properties.

On December 1, 2017, we subleased a 2,760 square foot facility comprised of office space located at One Gateway Center, Suite 351 (300 Washington Street), in Newton, Massachusetts. This location serves as our company headquarters and is used by our employees working in research and development, regulatory affairs, and general and administrative functions. The sublease term runs concurrently with the term of the underlying master lease which ends September 30, 2018.

Item 3. Legal Proceedings.

From time to time, we may be involved in various claims and legal proceedings relating to claims arising out of our operations.

See Note 7 to our consolidated financial statements included in this report for a description of our litigation with Piper Jaffray & Co.

We are not currently a party to any other legal proceedings that, in the opinion of our management, are likely to have a material adverse effect on our business. Regardless of outcome, litigation could have an adverse impact on our business because of defense and settlement costs, diversion of management resources and other factors.

Item 4. Mine Safety Disclosures.

Not applicable.

PART II

Item 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities.

Market Information and Holders

Our common stock is traded on the Nasdaq Capital Market under the symbol "ACER."

On September 19, 2017, in connection with, and prior to the completion of, the Merger, we effected a 1-for-10.355527 reverse stock split of our then outstanding common stock (the "Reverse Split"). Immediately following the Merger, we changed our name from Opexa Therapeutics, Inc. to Acer Therapeutics Inc. and our trading symbol from "OPXA" to "ACER." All share numbers in this report have been adjusted to reflect the Reverse Split. Our common stock has, from time to time, traded on a limited, sporadic and volatile basis.

The table below shows the high and low sales prices for our common stock for the periods indicated, as reported by Nasdaq.

	Price Ranges	
	High	Low
Fiscal Year Ended December 31, 2016		
First Quarter	$ 29.72	$ 17.50
Second Quarter	$ 44.42	$ 21.23
Third Quarter	$ 51.05	$ 30.24
Fourth Quarter	$ 45.36	$ 5.18
Fiscal Year Ended December 31, 2017		
First Quarter	$ 14.19	$ 7.59
Second Quarter	$ 8.28	$ 5.70
Third Quarter	$ 22.63	$ 6.55
Fourth Quarter	$ 19.50	$ 11.36

As of March 1, 2018, there were approximately 200 registered holders of our common stock. This number does not include shareholders for whom shares were held in "nominee" or "street name."

Dividends

We have never declared or paid any cash dividends on our common stock and we do not intend to pay cash dividends in the foreseeable future. We currently expect to retain any future earnings to fund the operation and expansion of our business.

Item 6. Selected Financial Data.

Not applicable.

Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations.

The following discussion of our financial condition and results of operations should be read in conjunction with the accompanying consolidated financial statements and the related footnotes thereto.

Overview

We are a pharmaceutical company focused on the acquisition, development, and commercialization of therapies for patients with serious rare and ultra-rare diseases with critical unmet medical need. Our late-stage clinical pipeline includes two candidates for severe genetic disorders: EDSIVO™ (celiprolol) for vascular Ehlers-Danlos syndrome, or vEDS, and ACER-001 (a fully taste-masked, immediate release formulation of sodium phenylbutyrate) for urea cycle disorders, or UCD, and Maple Syrup Urine Disease, or MSUD. There are no FDA-approved drugs for vEDS and MSUD and limited options for UCD, which collectively impact approximately 7,000 patients in the United States. Our products have clinical proof-of-concept and mechanistic differentiation, and we intend to seek approval for them in the United States by using the regulatory pathway established under section 505(b)(2) of the Federal Food, Drug and Cosmetic Act, or FFDCA, that allows an applicant to rely at least in part on third-party data for approval, which may expedite the preparation, submission, and approval of a marketing application.

Merger and Reverse Stock Split

On September 19, 2017, Acer Therapeutics Inc., a Texas corporation, formerly known as Opexa Therapeutics, Inc. (the "Registrant"), completed its business combination with Acer Therapeutics Inc., a Delaware corporation ("Private Acer"), in accordance with the terms of the Agreement and Plan of Merger and Reorganization, dated as of June 30, 2017, by and among the Registrant, Opexa Merger Sub, Inc. ("Merger Sub") and Private Acer (the "Merger Agreement"), pursuant to which Merger Sub merged with and into Private Acer, with Private Acer surviving as a wholly-owned subsidiary of the Registrant (the "Merger"). This transaction was approved by the Registrant's shareholders at a special meeting of its shareholders on September 19, 2017. Also on September 19, 2017, in connection with, and prior to the completion of, the Merger, the Registrant effected a 1-for-10.355527 reverse stock split of its then outstanding common stock (the "Reverse Split") and immediately following the Merger, the Registrant changed its name to "Acer Therapeutics Inc." pursuant to amendments to its certificate of formation filed with the Texas Secretary of State on September 19, 2017. All share numbers in this report have been adjusted to reflect the Reverse Split.

Following the completion of the Merger, the business conducted by the Registrant became primarily the business conducted by Private Acer, which is a pharmaceutical company that acquires, develops and intends to commercialize therapies for patients with serious rare diseases with critical unmet medical need.

Under the terms of the Merger Agreement, the Registrant issued shares of its common stock to Private Acer's stockholders, at an exchange rate of one share of common stock (after giving effect to the Reverse Split and the conversion of Private Acer's Series A and Series B preferred stock and convertible debt) in exchange for each share of Private Acer common stock outstanding immediately prior to the Merger. The exchange rate was determined through arm's length negotiations between the Registrant and Private Acer. The Registrant also assumed all issued and outstanding stock options under the Acer Therapeutics Inc. 2013 Stock Incentive Plan, with such stock options henceforth representing the right to purchase a number of shares of the Registrant's common stock equal to the number of shares of Private Acer's common stock previously represented by such stock options.

Immediately after the Merger: (i) there were approximately 6.5 million shares of the Registrant's common stock outstanding; (ii) the former Private Acer stockholders, including investors in the Concurrent Financing (as defined below), owned approximately 89% of the outstanding common stock of the Registrant; and (iii) the Registrant's shareholders immediately prior to the Merger, whose shares of the Registrant's common stock remained outstanding after the Merger, owned approximately 11% of the outstanding common stock of the Registrant.

The issuance of the shares of the Registrant's common stock to the former stockholders of Private Acer was registered with the U.S. Securities and Exchange Commission (the "SEC") on a Registration Statement on Form S-4 (Reg. No. 333-219358). Immediately prior to the Merger, Private Acer issued and sold an aggregate of approximately $15.7 million (inclusive of the conversion of approximately $5.7 million of principal and accrued

interest on outstanding convertible promissory notes issued by Private Acer) of shares of Private Acer's common stock (the "Concurrent Financing") to certain current stockholders of Private Acer and certain new investors at a per share price of $9.47.

The Registrant's common stock traded on a pre-split basis through the close of business on Wednesday, September 20, 2017, on the Nasdaq Capital Market under the ticker symbol "OPXA." Commencing with the open of trading on Thursday, September 21, 2017, the post-split shares began trading on the Nasdaq Capital Market under the ticker symbol "ACER." On September 21, 2017, the Registrant's Series M Warrants, previously trading through the close of business on Wednesday, September 20, 2017, under the ticker symbol "OPXAW," commenced trading on the Nasdaq Capital Market, under the ticker symbol "ACERW." The Registrant's common stock and Series M Warrants have new CUSIP numbers of 00444P 108 and 00444P 116, respectively.

For accounting and financial reporting purposes, Private Acer was considered to have acquired the Registrant in the Merger. Private Acer was incorporated on December 26, 2013 as part of a reorganization whereby Acer Therapeutics, LLC was converted into a corporation organized under the laws of the State of Delaware. On March 20, 2015, Private Acer acquired Anchor Therapeutics, Inc. ("Anchor"), with Anchor becoming a wholly-owned subsidiary of Private Acer. On August 19, 2016, Anchor's pepducin business reverted back to the pre-acquisition holders of Anchor's equity.

Revenue

We have no products approved for commercial sale and have not generated any revenue from product sales.

In the future, we may generate revenue by entering into licensing arrangements or strategic alliances. To the extent we enter into any license arrangements or strategic alliances, we expect that any revenue we generate will fluctuate from quarter-to-quarter as a result of the timing of achievement of pre-clinical, clinical, regulatory and commercialization milestones, if at all, the timing and amount of payments relating to such milestones, as well as the extent to which any products are approved and successfully commercialized.

If our product candidates are not developed in a timely manner, if regulatory approval is not obtained for them, or if such product candidates are not commercialized, our ability to generate future revenue, and our results of operations and financial position, would be adversely affected.

Research and Development Expenses

Research and development expenses consist of costs associated with the development of our product candidates. Our research and development expenses include:

- employee-related expenses, including salaries, benefits, and stock-based compensation;
- external research and development expenses incurred under arrangements with third parties, such as contract research organizations, contract manufacturing organizations, consultants, and our scientific advisors; and
- license fees.

We expense research and development costs as incurred. We account for nonrefundable advance payments for goods and services that will be used in future research and development activities as expenses when the service has been performed or when the goods have been received.

At any time, we are working on multiple programs, primarily within our therapeutic areas of focus. Our internal resources, employees, and infrastructure are not directly tied to any one research or drug discovery project and are typically deployed across multiple projects. As such, we do not generate meaningful information regarding the costs incurred for these early stage research and drug discovery programs on a specific project basis. However, we are currently spending the vast majority of our research and development resources on our two lead development programs.

Since Private Acer's inception in December 2013, we have spent a total of approximately $16.0 million in research and development expenses through December 31, 2017. Of the approximately $16.0 million in research and development expenses, approximately $13.6 million is directly related to EDSIVO™ and approximately $2.3 million is directly related to ACER-001. Other research and development costs, such as legal and travel costs, have not been identified as directly attributable to a specific research and development project.

We expect our research and development expenses to increase for the foreseeable future as we continue to conduct our ongoing regulatory activities, initiate new preclinical and clinical trials, and build upon our pipeline. The process of conducting clinical trials and pre-clinical studies necessary to obtain regulatory approval, preparing to seek regulatory approval, and preparing for commercialization in the event of regulatory approval, is costly and time-consuming. We may never succeed in achieving marketing approval for any of our product candidates.

Successful development of 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. We anticipate we will make determinations as to which programs to pursue and how much funding to direct to each program on an ongoing basis in response to our ability to enter into new strategic alliances with respect to each program or potential product candidate, the scientific and clinical success of each product candidate, the timing and ability to obtain regulatory approval for our product candidates (if any), and ongoing assessments as to each product candidate's commercial potential. We will need to raise additional capital and may seek to do so through public or private equity or debt financings, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements or a combination of these approaches. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. Our failure to raise capital or enter into such other arrangements as and when needed would have a negative impact on our financial condition and our ability to develop our product candidates and pursue regulatory approval.

General and Administrative Expenses

General and administrative expenses consist primarily of professional fees for legal, business consulting, auditing, and tax services. We expect that general and administrative expenses will increase in the future as we expand our operating activities. Additionally, as we prepare for the filing of our application with the FDA we expect to begin to incur significant expenses associated with preparing for the commercial launch of EDSIVO™, or "pre-commercial" costs.

We expect to incur significant additional costs associated with being a publicly-traded company. These increases will likely include legal fees, costs associated with Sarbanes-Oxley compliance, accounting fees, and directors' and officers' liability insurance premiums.

Other income (expense), net

Other income (expense), net consists primarily of interest income and expense, and various income or expense items of a non-recurring nature. We earn interest income from interest-bearing accounts and money market funds for cash and cash equivalents. Interest expense has historically been comprised of interest and other related non-cash charges incurred under convertible notes payable with our investors. Additionally, we record as part of other income (expense), net, transactional gains and losses on foreign currency denominated assets and liabilities when they are revalued each period due to changes in underlying exchange rates.

Critical Accounting Policies

This management's discussion and analysis of financial condition and results of operations is based on our consolidated financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles ("GAAP"). The preparation of these financial statements requires our management to make estimates and judgments that affect the reported amounts of assets, liabilities and expenses. On an ongoing basis, we evaluate these estimates and judgments. We base our estimates on historical experience and on various assumptions that we believe to be reasonable under the circumstances. These estimates and assumptions form the basis for making

judgments about the carrying values of assets and liabilities and the recording of expenses that are not readily apparent from other sources. Actual results may differ materially from these estimates. We believe that the accounting policies discussed below are critical to understanding our historical and future performance, as these policies relate to the more significant areas involving our judgments and estimates.

Business Combinations

Assets acquired and liabilities assumed as part of a business acquisition are generally recorded at their fair value at the date of acquisition. The excess of purchase price over the fair value of assets acquired and liabilities assumed is recorded as goodwill. Determining fair value of identifiable assets, particularly intangibles, and liabilities acquired also requires management to make estimates, which are based on all available information and in some cases assumptions with respect to the timing and amount of future revenues and expenses associated with an asset. Accounting for business acquisitions may require management to make judgments and estimates as to fair value of consideration transferred. This judgment and determination may affect the amount of consideration paid that is allocable to assets and liabilities acquired in the business purchase transaction.

Goodwill

Goodwill represents the excess of cost over fair value of net assets acquired in the Merger and in our prior acquisition of Anchor Therapeutics, Inc. ("Anchor"). We evaluate the recoverability of goodwill annually or more frequently, if events or changes in circumstances indicate that the carrying value of goodwill might be impaired. We first assess qualitative factors to determine whether it is more likely than not that the fair value of a reporting unit is less than its carrying value. This step serves as the basis for determining whether it is necessary to perform the two-step goodwill impairment test. If we determine it is more likely than not that the fair value of a reporting unit is less than its carrying value, then we will perform the two-step test. The two-step test first compares the fair value of the reporting unit to its carrying value. If the fair value exceeds the carrying value, no impairment exists, and the second step is not performed. If the fair value of the reporting unit is less than its carrying value, an impairment loss is recorded as part of the second step of the test, to the extent that the implied fair value of the reporting unit goodwill is less than the carrying value.

We review intangible assets annually to determine if any adverse conditions exist or a change in circumstances has occurred that would indicate impairment. If the carrying value of an asset exceeds its undiscounted cash flows, we write down the carrying value of the intangible asset to its fair value in the period identified.

In-process Research and Development

In-process research and development ("IPRD") represents the value of the three G-protein-coupled receptor targets (the "Targets") from the GPCR Target Pools of Anchor that we obtained the rights to in the March 20, 2015, acquisition of Anchor by Private Acer (see Note 3 to our consolidated financial statements). IPRD was recorded at fair value in conjunction with the Anchor acquisition during 2015 and is an indefinite-lived intangible asset. As such, it is tested at least annually for impairment.

Stock-Based Compensation

We account for stock-based compensation expense related to stock options granted to employees and members of our board of directors under our 2013 Stock Incentive Plan, as amended, and our 2010 Stock Incentive Plan, as amended and restated, by estimating the fair value of each stock option or award on the date of grant using the Black-Scholes model. We recognize stock-based compensation expense on a straight-line basis over the vesting term.

We account for stock options issued to non-employees by valuing the award using an option pricing model and re-measuring such awards to the current fair value until the awards are vested or a performance commitment has otherwise been reached.

Research and Development

Research and development costs are expensed as incurred and include compensation and related benefits, license fees and outside contracted research and manufacturing consultants. We often make nonrefundable advance payments for goods and services that will be used in future research and development activities. These payments are capitalized and recorded as an expense in the period that we receive the goods or when the services are performed.

Clinical Trial and Pre-Clinical Study Accruals

We make estimates of accrued expenses as of each balance sheet date in our consolidated financial statements based on certain facts and circumstances at that time. Our accrued expenses for pre-clinical studies and clinical trials are based on estimates of costs incurred for services provided by contract research organizations ("CRO"), manufacturing organizations, and for other trial-related activities. Payments under our agreements with external service providers depend on a number of factors such as site initiation, patient screening, enrollment, delivery of reports, and other events. In accruing for these activities, we obtain information from various sources and estimate the level of effort or expense allocated to each period. Adjustments to our research and development expenses may be necessary in future periods as our estimates change. As these activities are generally material to our overall financial statements, subsequent changes in estimates may result in a material change in our accruals. At December 31, 2017, we did not have material accruals for clinical and pre-clinical trials.

Results of Operations

Comparison of the years ended December 31, 2017 and 2016

The following table summarizes our results of operations for the years ended December 31, 2017 and 2016:

	Years Ended December 31,			
	2017	2016	$ Change	% Change
Research and development	$ 8,725,026	$ 5,308,662	$ 3,416,364	64%
General and administrative	5,223,101	1,391,182	3,831,919	275%
Other income (expense), net	(246,304)	282	(246,586)	*
Net loss	(14,194,431)	(6,699,562)	(7,494,869)	112%

* not meaningful

Research and Development Expenses

Research and development expense was approximately $8.7 million for the year ended December 31, 2017, as compared to approximately $5.3 million for the year ended December 31, 2016. This increase of approximately $3.4 million was principally due to an increase in spending for clinical development and manufacturing services relating to EDSIVO™ and performance under the clinical data license from AP-HP. Research and development expense for the year ended December 31, 2017, was comprised of approximately $8.2 million directly related to EDSIVO™ and approximately $447,000 directly related to ACER-001. Research and development expense for the year ended December 31, 2016, was comprised of approximately $2.3 million directly related to EDSIVO™ and approximately $1.2 million directly related to ACER-001. Other research and development costs such as legal and travel costs have not been identified as directly attributable to a specific research and development project.

General and Administrative Expenses

General and administrative expense was approximately $5.2 million for the year ended December 31, 2017, as compared to approximately $1.4 million for the year ended December 31, 2016. This increase of approximately $3.8 million was primarily due to an increase in pre-commercial activities of approximately $2.4 million. The remaining increases were comprised primarily of legal expenses, employee-related expenses, and accounting expenses.

Other Income (Expense), Net

Other expense, net of approximately $246,000 during the year ended December 31, 2017, was primarily attributable to interest expense related to the outstanding convertible promissory notes prior to conversion.

Liquidity and Capital Resources

We have never been profitable and have incurred operating losses in each year since inception. From inception to December 31, 2017, we have raised net cash proceeds of approximately $39.0 million, primarily from private placements of convertible preferred stock and common stock, and debt financings. As of December 31, 2017, we had approximately $15.6 million in cash and cash equivalents. Our net loss for the years ended December 31, 2017 and 2016, was $14.2 million and $6.7 million, respectively. As of December 31, 2017, we had an accumulated deficit of approximately $25.6 million. Substantially all of our operating losses resulted from expenses incurred in connection with our research and development programs and from general and administrative costs associated with our operations. The following table shows a summary of our cash flows for the years ended December 31, 2017, and 2016:

	Years Ended December 31,	
	2017	2016
Operating activities	$ (14,095,256)	$ (6,957,782)
Investing activities	967,281	(1,582)
Financing activities	26,938,312	7,994,837
Net increase (decrease) in cash and cash equivalents	$ 13,810,337	$ 1,035,473

Operating Activities

Net cash used in operating activities was approximately $14.1 million for the year ended December 31, 2017, as compared to approximately $7.0 million for the year ended December 31, 2016. The increase of approximately $7.1 million was principally the result of an increase in net loss due to increased research and development activities in advancing our product candidates and increased general and administrative activities.

Investing Activities

Net cash provided by investing activities during the year ended December 31, 2017, relates to cash acquired in the Merger. We had no significant cash generated from or used in investing activities during the year ended December 31, 2016.

Financing Activities

Net cash provided by financing activities during the year ended December 31, 2017, consisted of $21.5 million of net proceeds from the issuance of common stock and $5.5 million from the issuance of convertible notes payable (which, together with $174,452 of accrued interest, was converted into common stock). Net cash provided by financing activities during the year ended December 31, 2016, consisted of net proceeds from the issuance of Series B Convertible Redeemable Preferred stock.

Future Capital Requirements

We have not generated any revenue from product sales. We do not know when, or if, we will generate any revenue from product sales. We do not expect to generate any revenue from product sales unless and until we obtain regulatory approval for and commercialize any of our product candidates. At the same time, we expect our expenses to increase in connection with our ongoing development and manufacturing activities, particularly as we continue the research, development, manufacture and clinical trials of, and seek regulatory approval for, our product candidates. In addition, subject to obtaining regulatory approval of any of our product candidates and thereafter successfully commercializing any such product candidates, we anticipate that we will need substantial additional funding in connection with our continuing operations.

As of December 31, 2017, we had approximately $15.6 million in cash and cash equivalents. We expect our research and development expenses to substantially increase in connection with our ongoing activities, particularly as we advance our product candidates in or towards clinical development or potential regulatory approval.

Our future capital requirements are difficult to forecast and will depend on many factors, including but not limited to:

- our ability to obtain adequate levels of financing to meet our operating plan;
- the costs associated with filing, outcome, and timing of regulatory approvals;
- the terms and timing of any strategic alliance, licensing and other arrangements that we may establish;
- the cost and timing of hiring new employees to support our continued growth;
- the costs and timing of having clinical supplies of our product candidates manufactured;
- the initiation and progress of ongoing pre-clinical studies and clinical trials for our product candidates;
- the costs involved in patent filing, prosecution, and enforcement; and
- the number of programs we pursue.

Based on available resources, we believe that our cash and cash equivalents currently on hand are sufficient to fund our currently anticipated operating and capital requirements through the end of 2018. However, our current capital resources are not sufficient to fund our planned operations for the next 12 months from the date of the financial statements included in this report and thus there is substantial doubt about the Company's ability to continue as a going concern within one year after such date. We will continue to require substantial additional capital to continue our clinical development and pursuit of regulatory approval activities. Accordingly, we will need to raise substantial additional capital to continue to fund our operations. The amount and timing of our future funding requirements will depend on many factors, including the pace and results of our development, regulatory and commercialization efforts. Failure to raise capital as and when needed, on favorable terms or at all, would have a negative impact on our financial condition and our ability to develop and potentially commercialize (if approved) our product candidates.

We expect to incur significant expenses and increasing operating losses for at least the next two years as we initiate and continue the clinical development of, seek regulatory approval for, and potentially commercialize (if approved) our product candidates and add personnel necessary to operate as a public company with an advanced clinical pipeline of product candidates. In addition, operating as a publicly-traded company involves the hiring of additional financial and other personnel, upgrading financial information systems, and incurring costs associated with operating as a public company. We expect that our operating losses will fluctuate significantly from quarter-to-quarter and year-to-year due to the timing of clinical development programs, efforts to achieve regulatory approval and planning for potential commercialization (if approved) of our product candidates.

Until we can generate a sufficient amount of product revenue to finance our cash requirements, which would require us to obtain regulatory approval for and successfully commercialize one or more of our product candidates, we expect to finance our future cash needs primarily through the issuance of additional equity and potentially through borrowing and strategic alliances with partner companies. We do not maintain any external lines of credit or have any sources of debt or equity capital committed for funding, other than the legacy sales agreement entered into on March 25, 2016 between the Registrant and IFS Securities, Inc. (doing business as Brinson Patrick, a division of IFS Securities, Inc.). This agreement provides a facility for the offer and sale of shares of common stock from time to time depending upon market demand, in transactions deemed to be an "at-the-market" ("ATM") offering. We will need to keep current our shelf registration statement and the offering prospectus relating to the ATM facility, in addition to providing certain periodic deliverables under the sales agreement, in order to use such facility. To the extent that we raise additional capital through the issuance of additional equity or convertible debt securities, the ownership interest of our shareholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of existing shareholders. Debt financing, if available, may involve

agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through marketing and distribution arrangements or other collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or pursuit of regulatory approval efforts or grant rights to develop and market product candidates to third parties that we would otherwise prefer to develop and, if applicable, market ourselves.

Contractual Commitments

License Agreements

In August 2016, Private Acer entered into an agreement with AP-HP granting it the exclusive worldwide rights to access and use data from a multicenter, prospective, randomized, open trial related to the use of celiprolol for the treatment of vEDS. We intend to use this pivotal clinical data to support an NDA filing for EDSIVO™ for the treatment of vEDS. The agreement requires us to make certain upfront payments to AP-HP, reimburse certain of AP-HP's costs, make payments upon achievement of defined milestones and pay royalties on net sales of celiprolol over the royalty term.

In April 2014, we obtained exclusive rights to patents and certain other intellectual property relating to ACER-001 and preclinical and clinical data, through an exclusive license agreement with Baylor College of Medicine, or BCM. Under the terms of the agreement, as amended, we have worldwide exclusive rights to develop, manufacture, use, sell and import products incorporating the licensed intellectual property. The license agreement requires us to make upfront and annual payments to BCM, reimburse certain of BCM's legal costs, make payments upon achievement of defined milestones, and pay royalties on net sales of any developed product over the royalty term.

Off-Balance Sheet Arrangements

None.

Inflation

We believe that inflation has not had a material impact on our results of operations for the years ended December 31, 2017, and 2016, since inflation rates have generally remained at relatively low levels and our operations are not otherwise uniquely affected by inflation concerns.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk.

Not applicable.

Item 8. Financial Statements and Supplementary Data.

Index to Consolidated Financial Statements

Report of Independent Registered Public Accounting Firm

To the Board of Directors of Acer Therapeutics Inc.:

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Acer Therapeutics Inc. (the "Company") as of December 31, 2017 and 2016, and the related consolidated statements of operations, changes in redeemable preferred stock and stockholders' equity (deficit) and cash flows for years then ended, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2017 and 2016, and the results of its operations and its cash flows for the years then ended, in conformity with accounting principles generally accepted in the United States of America.

Emphasis of Matter

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

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) ("PCAOB") and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting, but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ Wolf & Company, P.C.

We have served as the Company's auditor since 2014.

Boston, Massachusetts
March 7, 2018

ACER THERAPEUTICS INC.
CONSOLIDATED BALANCE SHEETS

	December 31, 2017	December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	$ 15,644,355	$ 1,834,018
Prepaid expenses	881,887	540,053
Total current assets	16,526,242	2,374,071
Property and equipment, net	62,984	6,217
Other assets:		
Goodwill	7,647,267	272,315
In-process research and development	118,600	118,600
Other assets	13,648	1,901
Total assets	$ 24,368,741	$ 2,773,104
Liabilities, Redeemable Preferred Stock and Stockholders' Equity (Deficit)		
Current liabilities:		
Accounts payable	$ 95,873	$ 383,411
Accrued expenses	1,937,331	438,028
Total liabilities	2,033,204	821,439
Commitments		
Series B Convertible Redeemable Preferred stock, $0.0001 par value; none and 970,238 shares authorized, issued and outstanding at December 31, 2017 and 2016, respectively	—	8,022,219
Series A Convertible Redeemable Preferred stock, $0.0001 par value; none and 638,416 shares authorized, issued and outstanding at December 31, 2017 and 2016, respectively	—	4,114,221
Convertible Redeemable Preferred stock	—	12,136,440
Stockholders' equity (deficit):		
Preferred stock, no par value; authorized 10,000,000 shares; none issued and outstanding	—	—
Common stock, $0.01 par value; authorized 150,000,000 shares; 7,497,433 and 2,450,000 shares issued and outstanding at December 31, 2017 and 2016, respectively	74,974	24,500
Additional paid-in capital	47,812,215	1,147,946
Accumulated deficit	(25,551,652)	(11,357,221)
Total stockholders' equity (deficit)	22,335,537	(10,184,775)
Total liabilities, redeemable preferred stock and stockholders' equity (deficit)	$ 24,368,741	$ 2,773,104

See accompanying report of independent registered public accounting firm and notes to consolidated financial statements.

ACER THERAPEUTICS INC.
CONSOLIDATED STATEMENTS OF OPERATIONS
FOR THE YEARS ENDED DECEMBER 31, 2017 AND 2016

	2017	2016
Operating expenses:		
Research and development	$ 8,725,026	$ 5,308,662
General and administrative	5,223,101	1,391,182
Total operating expenses	13,948,127	6,699,844
Loss from operations	(13,948,127)	(6,699,844)
Other income (expense):		
Interest income	14,848	282
Interest and other expense	(245,061)	—
Foreign currency transaction loss	(16,091)	—
Total other income (expense), net	(246,304)	282
Net loss	$ (14,194,431)	$ (6,699,562)
Net loss per share - basic and diluted	$ (3.84)	$ (2.73)
Weighted average common shares outstanding - basic and diluted	3,694,388	2,450,000

See accompanying report of independent registered public accounting firm and notes to consolidated financial statements.

ACER THERAPEUTICS INC.
CONSOLIDATED STATEMENTS OF CHANGES IN REDEEMABLE PREFERRED STOCK AND STOCKHOLDERS' EQUITY (DEFICIT)
FOR THE YEARS ENDED DECEMBER 31, 2017 AND 2016

	Redeemable Preferred Stock				Stockholders' Equity (Deficit)				
	Series B Convertible Redeemable Preferred stock		Series A Convertible Redeemable Preferred stock		Common stock		Additional Paid-in Capital	Accumulated Deficit	Total Stockholders' Equity (Deficit)
	Shares	Amount	Shares	Amount	Shares	Amount			
Balance as of December 31, 2015	—	$ —	638,416	$ 4,099,380	2,450,000	$ 24,500	$ 1,126,366	$ (4,657,659)	$ (3,506,793)
Issuance of Series B Convertible Redeemable Preferred stock, net of issuance costs of $155,162	970,238	7,994,837	—	—	—	—	—	—	—
Accretion of issuance costs	—	27,382	—	14,841	—	—	(42,223)	—	(42,223)
Share-based compensation	—	—	—	—	—	—	63,803	—	63,803
Net loss	—	—	—	—	—	—	—	(6,699,562)	(6,699,562)
Balance as of December 31, 2016	970,238	8,022,219	638,416	4,114,221	2,450,000	24,500	1,147,946	(11,357,221)	(10,184,775)
Issuance of common stock in connection with merger	—	—	—	—	736,950	7,369	6,971,547	—	6,978,916
Conversion of convertible notes and accrued interest into common stock	—	—	—	—	599,201	5,992	5,668,460	—	5,674,452
Conversion of convertible redeemable preferred stock into common stock	(970,238)	(8,149,999)	(638,416)	(4,166,164)	1,608,654	16,087	12,300,076	—	12,316,163
Accretion of issuance costs	—	127,780	—	51,943	—	—	(179,723)	—	(179,723)
Issuance of common stock, net of issuance costs and underwriting discount	—	—	—	—	2,102,628	21,026	21,485,816	—	21,506,842
Share-based compensation	—	—	—	—	—	—	418,093	—	418,093
Net loss	—	—	—	—	—	—	—	(14,194,431)	(14,194,431)
Balance as of December 31, 2017	—	$ —	—	$ —	7,497,433	$ 74,974	$ 47,812,215	$ (25,551,652)	$ 22,335,537

See accompanying report of independent registered public accounting firm and notes to consolidated financial statements.

ACER THERAPEUTICS INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS
FOR THE YEARS ENDED DECEMBER 31, 2017 AND 2016

	Years Ended December 31,	
	2017	2016
Cash flows from operating activities:		
Net loss	$ (14,194,431)	$ (6,699,562)
Adjustments to reconcile net loss to net cash used in operating activities:		
Non-cash interest expense	242,982	—
Share-based compensation	418,093	63,803
Depreciation	3,997	3,532
Loss on disposal of property and equipment	2,078	—
Write-off of deferred financing costs	1,901	67,995
Changes in operating assets and liabilities		
Prepaid expenses	(336,834)	(430,571)
Accounts payable	(287,538)	140,011
Accrued expenses	68,144	(102,990)
Other noncurrent assets	(13,648)	
Net cash used in operating activities	(14,095,256)	(6,957,782)
Cash flows from investing activities:		
Cash acquired in Merger, net of payment in lieu of fractional shares	1,030,123	—
Purchase of property and equipment	(62,842)	(1,582)
Net cash provided by (used in) investing activities	967,281	(1,582)
Cash flows from financing activities:		
Proceeds from issuance of common stock	21,506,842	—
Proceeds from issuance of Series B Convertible Redeemable Preferred stock, net	—	7,994,837
Deferred financing costs	(68,530)	—
Proceeds from convertible notes payable	5,500,000	—
Net cash provided by financing activities	26,938,312	7,994,837
Net increase in cash and cash equivalents	13,810,337	1,035,473
Cash and cash equivalents, beginning of the year	1,834,018	798,545
Cash and cash equivalents, end of the year	$ 15,644,355	$ 1,834,018
Supplemental cash flow information and non-cash financing transactions:		
Cash paid during the year for interest	$ —	$ 720
Accretion of issuance costs on Series A Convertible Redeemable Preferred stock	$ 51,943	$ 14,841
Accretion of issuance costs on Series B Convertible Redeemable Preferred stock	$ 127,780	$ 27,382
Conversion of Series A Convertible Redeemable Preferred stock to common stock	$ 4,166,164	$ —
Conversion of Series B Convertible Redeemable Preferred stock to common stock	$ 8,149,999	$ —
Conversion of convertible notes payable and accrued interest to common stock	$ 5,674,452	$ —
Issuance of common stock in Merger (Note 1)	$ 6,978,916	$ —

See accompanying report of independent registered public accounting firm and notes to consolidated financial statements.

1. NATURE OF OPERATIONS AND BASIS OF PRESENTATION

Business

Acer Therapeutics Inc., a Texas corporation (the "Company"), formerly known as Opexa Therapeutics, Inc. (the "Registrant"), is a pharmaceutical company focused on the acquisition, development, and commercialization of therapies for patients with serious rare and ultra-rare diseases with critical unmet medical need. The Company's late-stage clinical pipeline includes two candidates for severe genetic disorders: EDSIVO™ (celiprolol) for vascular Ehlers-Danlos syndrome ("vEDS"), and ACER-001 (a fully taste-masked, immediate release formulation of sodium phenylbutyrate) for urea cycle disorders ("UCD") and Maple Syrup Urine Disease ("MSUD"). There are no FDA-approved drugs for vEDS and MSUD and limited options for UCD, which collectively impact approximately 7,000 patients in the United States. The Company's products have clinical proof-of-concept and mechanistic differentiation, and it intends to seek approval for them in the U.S. by using the regulatory pathway established under section 505(b)(2) of the Federal Food, Drug, and Cosmetic Act, or FFDCA, that allows an applicant to rely at least in part on third-party data for approval, which may expedite the preparation, submission, and approval of a marketing application.

Since its inception, the Company has devoted substantially all of its efforts to business planning, research and development, recruiting management and technical staff, acquiring operating assets and raising capital. The Company has not generated any product revenue to date and may never generate any product revenue in the future.

Merger and Reverse Stock Split

On September 19, 2017, the Registrant completed its business combination with Acer Therapeutics Inc., a Delaware corporation ("Private Acer"), in accordance with the terms of the Agreement and Plan of Merger and Reorganization, dated as of June 30, 2017, by and among the Registrant, Opexa Merger Sub, Inc. ("Merger Sub") and Private Acer (the "Merger Agreement"), pursuant to which Merger Sub merged with and into Private Acer, with Private Acer surviving as a wholly-owned subsidiary of the Registrant (the "Merger"). This transaction was approved by the Registrant's shareholders at a special meeting of its shareholders on September 19, 2017. Also on September 19, 2017, in connection with, and prior to the completion of, the Merger, the Registrant effected a 1-for-10.355527 reverse stock split of its then outstanding common stock (the "Reverse Split") and immediately following the Merger, the Registrant changed its name to "Acer Therapeutics Inc." pursuant to amendments to its certificate of formation filed with the Texas Secretary of State on September 19, 2017. All share numbers in this report have been adjusted to reflect the Reverse Split.

Following the completion of the Merger, the business conducted by the Registrant became primarily the business conducted by Private Acer, which is a pharmaceutical company that acquires, develops and intends to commercialize therapies for patients with serious rare diseases with critical unmet medical need.

Under the terms of the Merger Agreement, the Registrant issued shares of its common stock to Private Acer's stockholders, at an exchange rate of one share of common stock (after giving effect to the Reverse Split and the conversion of Private Acer's Series A and Series B preferred stock and convertible debt) in exchange for each share of Private Acer common stock outstanding immediately prior to the Merger. The exchange rate was determined through arm's length negotiations between the Registrant and Private Acer. The Registrant also assumed all issued and outstanding stock options under the Acer Therapeutics Inc. 2013 Stock Incentive Plan, with such stock options henceforth representing the right to purchase a number of shares of the Registrant's common stock equal to the number of shares of Private Acer's common stock previously represented by such stock options.

Immediately after the Merger, (i) there were approximately 6.5 million shares of the Registrant's common stock outstanding; (ii) the former Private Acer stockholders, including investors in the Concurrent Financing (as defined below), owned approximately 89% of the outstanding common stock of the Registrant; and (iii) the Registrant's shareholders immediately prior to the Merger, whose shares of the Registrant's common stock remained outstanding after the Merger, owned approximately 11% of the outstanding common stock of the Registrant.

The issuance of the shares of the Registrant's common stock to the former stockholders of Private Acer was registered with the U.S. Securities and Exchange Commission (the "SEC") on a Registration Statement on Form S-4 (Reg. No. 333-219358). Immediately prior to the Merger, Private Acer issued and sold an aggregate of approximately $15.7 million (inclusive of the conversion of approximately $5.7 million of principal and accrued interest on outstanding convertible promissory notes issued by Private Acer) of shares of Private Acer's common stock (the "Concurrent Financing") to certain current stockholders of Private Acer and certain new investors at a per share price of $9.47.

The Registrant's common stock continued to trade on a pre-split basis through the close of business on Wednesday, September 20, 2017, on the Nasdaq Capital Market under the ticker symbol "OPXA." Commencing with the open of trading on Thursday, September 21, 2017, the post-split shares began trading on the Nasdaq Capital Market under the ticker symbol "ACER." On September 21, 2017, the Registrant's Series M Warrants, previously trading through the close of business on Wednesday, September 20, 2017, under the ticker symbol "OPXAW," commenced trading on the Nasdaq Capital Market, under the ticker symbol "ACERW." The Registrant's common stock and Series M Warrants have new CUSIP numbers of 00444P 108 and 00444P 116, respectively.

Basis of Presentation

Accounting principles generally accepted in the United States require that a company whose security holders retain the majority voting interest in the combined business be treated as the acquirer for financial reporting purposes. Accordingly, the Merger was accounted for as a reverse acquisition whereby Private Acer was treated as the acquirer for accounting and financial reporting purposes. As such, references to the results of operations for the year ended December 31, 2017 include the historical results of Private Acer from January 1, 2017 through September 18, 2017 and include the consolidated results of the combined company from September 19, 2017 through December 31, 2017. References to the results of operations for the year ended December 31, 2016 include only the historical results of Private Acer, except as discussed below.

Private Acer was incorporated on December 26, 2013, as part of a reorganization whereby Acer Therapeutics, LLC was converted into a corporation organized under the laws of the State of Delaware. On March 20, 2015, Private Acer acquired Anchor Therapeutics, Inc. ("Anchor"), with Anchor becoming a wholly-owned subsidiary of Private Acer. On August 19, 2016, Anchor's pepducin business reverted back to the pre-acquisition holders of Anchor's equity.

The accompanying consolidated financial statements for periods prior to August 19, 2016 include the accounts of Private Acer and its wholly-owned subsidiary Anchor. References to the "Company" in these notes refer to (i) Private Acer, for any period prior to March 20, 2015, (ii) Private Acer and its wholly-owned subsidiary Anchor, for the period beginning on March 20, 2015 and ending on August 18, 2016, (iii) Private Acer, for the period beginning on August 19, 2016 and ending on September 18, 2017, and (iv) the Registrant and its wholly-owned subsidiary Private Acer, for the period beginning on September 19, 2017.

All intercompany balances and transactions have been eliminated in consolidation. These consolidated financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America (GAAP). Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification (ASC) and Accounting Standards Update (ASU) of the Financial Accounting Standards Board (FASB).

Going Concern Uncertainty

The accompanying financial statements have been prepared on a going concern basis, which contemplates the realization of assets and the satisfaction of liabilities in the normal course of business. The Company has experienced recurring losses since inception and expects to continue to incur losses for the foreseeable future as it continues its development of, and seeks marketing approvals for, its product candidates.

The Company has historically relied on raising capital to finance its operations and plans to raise additional capital through public or private equity and/or debt financings. There is no assurance, however, that the Company will be able to raise sufficient capital to fund its operations on terms that are acceptable, or that its operations will ever be profitable.

Based on available resources, the Company believes that its cash and cash equivalents currently on hand are sufficient to fund its anticipated operating and capital requirements through the end of 2018. There is substantial doubt about the Company's ability to continue as a going concern within one year after the date that the accompanying financial statements are issued. These financial statements do not include any adjustments relating to the recoverability of recorded asset amounts that might be necessary as a result of the above uncertainty.

2. SIGNIFICANT ACCOUNTING POLICIES

A summary of the significant accounting policies followed by the Company in the preparation of the accompanying consolidated financial statements follows:

Use of Estimates

The Company's accounting principles require management to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of assets and liabilities at the date of the financial statements and reported amounts of revenues and expenses during the reporting period. Estimates having relatively higher significance include the accounting for acquisitions, stock-based compensation, and income taxes. Actual results could differ from those estimates and changes in estimates may occur.

Cash and Cash Equivalents

The Company considers all highly liquid investments with maturities of three months or less at the date of purchase to be cash equivalents.

Research and Development Expenses

Costs incurred for research and development are expensed as incurred.

Business Combinations

Assets acquired and liabilities assumed as part of a business acquisition are generally recorded at their fair value at the date of acquisition. The excess of purchase price over the fair value of assets acquired and liabilities assumed is recorded as goodwill. Determining fair value of identifiable assets, particularly intangibles, and liabilities acquired also requires management to make estimates, which are based on all available information and in some cases assumptions with respect to the timing and amount of future revenues and expenses associated with an asset. Accounting for business acquisitions may require management to make judgments and estimates as to the fair value of consideration transferred. This judgment and determination may affect the amount of consideration paid that is allocable to assets and liabilities acquired in the business purchase transaction.

Share-Based Compensation

The Company records share-based payments at fair value. The measurement date for compensation expense related to employee awards is generally the date of the grant. The measurement date for compensation expense related to nonemployee awards is generally the date that the performance of the awards is completed and, until such time, the fair value of the awards is remeasured at the end of each reporting period. Accordingly, the ultimate expense is not fixed until such awards are vested. The fair value of awards, net of expected forfeitures, is recognized as an expense in the statement of operations over the requisite service period, which is generally the vesting period. The fair value of options is calculated using the Black-Scholes option pricing model. This option valuation model requires the use of assumptions including, among others, the volatility of stock price, the expected term of the option, and the risk-free interest rate.

The following assumptions were used to estimate the fair value of stock options granted using the Black-Scholes option pricing model:

	2017	2016
Risk-free interest rate	1.93%	1.73%
Expected life (years)	6	5
Volatility	60%	60%
Dividend rate	0%	0%

Due to its limited operating history and a limited trading history of its common stock, the Company estimates the volatility of its stock in consideration of a number of factors including the volatility of comparable public companies. The expected term of a stock option granted to employees and directors (including non-employee directors) is based on the average of the contractual term (generally 10 years) and the vesting period. For other non-employee options, the expected term is the contractual term. The assumed dividend yield is based upon the Company's expectation of not paying dividends in the foreseeable future. The Company recognizes forfeitures related to employee share-based payments as they occur. The risk-free rate for periods within the expected life of the option is based upon the U.S. Treasury yield curve in effect at the time of grant. Prior to the Merger, the fair value of the common stock for the purposes of determining the exercise price of stock options was determined by the Board of Directors after considering a broad range of factors, including the results of a third-party valuation. Subsequent to the Merger, option awards are granted at an exercise price equal to the closing market price of the Company's common stock on the Nasdaq Capital Market on the date of grant.

In-process Research and Development

In-process research and development ("IPRD") represents the value of the three G-protein-coupled receptors ("GPCR") targets (the "Targets") from the GPCR Target pools of Anchor to which the Company obtained the rights in its March 20, 2015, acquisition of Anchor. IPRD was recorded at fair value and is an indefinite-lived intangible asset. The Company reviews IPRD annually to determine if any adverse conditions exist or a change in circumstances has occurred that would indicate impairment or a change in the remaining useful life of the asset. There were no triggering events or circumstances that would indicate IPRD is impaired as of December 31, 2017.

Goodwill

Goodwill represents the excess of cost over fair value of net assets acquired. The Company evaluates the recoverability of goodwill annually or more frequently if events or changes in circumstances indicate that the carrying value of goodwill might be impaired. The Company first assesses qualitative factors to determine whether it is more likely than not that the fair value of a reporting unit is less than its carrying value. This step serves as the basis for determining whether it is necessary to perform the two-step goodwill impairment test. If the Company determines it is more likely than not that the fair value of a reporting unit is less than its carrying value, then it will perform the two-step test. The two-step test first compares the fair value of the reporting unit to its carrying value. If the fair value exceeds the carrying value, no impairment exists, and the second step is not performed. If the fair value of the reporting unit is less than its carrying value, an impairment loss is recorded as part of the second step of the test, to the extent that the implied fair value of the reporting unit goodwill is less than the carrying value. The Company performed a qualitative analysis of goodwill in the fourth quarter of 2017, in which management concluded that it is more likely than not that the fair value of the reporting unit is greater than its carrying amount.

Income Taxes

The Company is primarily subject to U.S. Federal and Massachusetts state income taxes. As per statute, the Company's tax returns since incorporation in 2013 are open to tax examinations by U.S. Federal and state tax authorities. For federal and state income taxes, deferred tax assets and liabilities are recognized based upon temporary differences between the financial statement and the tax basis of assets and liabilities. Deferred income taxes are based upon prescribed rates and enacted laws applicable to periods in which differences are expected to reverse. A valuation allowance is recorded when it is more likely than not that some portion or all of the deferred tax assets will not be realized. Accordingly, the Company provides a valuation allowance, if necessary, to reduce deferred tax assets to amounts that are realizable.

The tax positions taken or expected to be taken in the course of preparing the Company tax returns are required to be evaluated to determine whether the tax positions are "more-likely-than-not" of being sustained by the applicable tax authority. Tax positions not deemed to meet a more-likely-than-not threshold would be recorded as a tax expense in the current year. There were no uncertain tax positions that require accrual or disclosure in the consolidated financial statements as of December 31, 2017, and 2016. The Company's policy is to recognize interest and penalties related to income tax, if any, in income tax expense. As of December 31, 2017, and 2016, the Company has no accruals for interest or penalties related to income tax matters.

Basic and Diluted Net Loss per Common Share

Basic and diluted net loss per common share is computed by dividing net loss in each period by the weighted average number of shares of common stock outstanding during such period. For the periods presented, common stock equivalents, consisting of options, convertible redeemable preferred stock and warrants, were not included in the calculation of the diluted loss per share because to do so would be anti-dilutive.

Segment Information

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision maker, or decision-making group, in deciding how to allocate resources and in assessing performance. The Company views its operations and manages its business in one operating segment, which is the business of a pharmaceutical company focused on the acquisition, development, and commercialization of therapies for patients with serious rare and ultra-rare diseases with critical unmet medical need.

Recently Adopted Accounting Pronouncements

In March 2016, the FASB issued ASU No. 2016-09, *Improvements to Employee Share-Based Payment Accounting*, or ASU No. 2016-09, which simplifies several aspects of the accounting for share-based payment transactions, including the income tax consequences, classification of awards as either equity or liabilities and classification of cash flows. The Company adopted ASU No. 2016-09 as of January 1, 2017. Under the new standard, all excess tax benefits and tax deficiencies are recognized as income tax expense or benefit in the statement of operations. The tax effects of exercised or vested awards are treated as discrete items in the reporting period in which they occur. The Company applied the modified retrospective adoption approach upon adoption of the standard, and prior periods have not been adjusted. The Company has elected to recognize forfeitures related to employee share-based payments as they occur. There was no material impact on the Company's financial statements as a result of the adoption of this guidance.

Recently Issued Accounting Pronouncements

In January 2017, the FASB issued ASU No. 2017-04, *Intangibles – Goodwill and Other* (Topic 350). This ASU eliminates step 2 from the goodwill impairment test by comparing the fair value of a reporting unit with the carrying amount of the reporting unit. If the carrying amount exceeds the fair value, an impairment charge for the excess is recorded. The amendments of this ASU are effective for annual or any interim goodwill impairment tests in fiscal years beginning after December 15, 2019. Early adoption is permitted for interim or annual goodwill impairment tests performed on testing dates after January 1, 2017. The Company is currently evaluating the impact of the adoption of this ASU on the consolidated financial statements.

3. PURCHASE ACCOUNTING

Private Acer recorded $272,315 of goodwill in connection with its acquisition of Anchor in 2015. Under the merger agreement with Anchor, if the pepducin business was not sold within 12 months of the merger date, the pepducin business reverted to the holders of Anchor's equity immediately prior to the merger. The sale did not occur within this period. The reversion was accomplished through a Pepducin Transfer Agreement, entered into on August 19, 2016, pursuant to which the Company transferred all the outstanding stock of Anchor to Teserx, Inc., a new entity designated by the stockholders' representative for the benefit of holders of Anchor's equity immediately prior to the merger. The Company retained the exclusive rights to the two pepducin drug targets and obtained ownership to the third. No gain or loss was recorded by the Company when the pepducin business reverted back to the prior holders of Anchor's equity and the Company has no continuing involvement in the pepducin business or with Teserx, Inc.

Merger with Opexa Therapeutics, Inc.

The Merger was accounted for using the purchase method of accounting as a reverse acquisition. In a reverse acquisition, the post-acquisition net assets of the surviving combined company includes the historical cost basis of the net assets of the accounting acquirer (Private Acer) plus the fair value of the net assets of the accounting acquiree (the Registrant). Further, under the purchase method, the purchase price is allocated to the assets acquired, liabilities assumed, and identifiable intangible assets based on their estimated fair values, with the remaining excess purchase price over net assets acquired allocated to goodwill.

The fair value of the consideration transferred in the Merger was $7,007,069 and was calculated as the number of shares of common stock that Private Acer issued (adjusted for the exchange ratio) in order for the Registrant's shareholders to hold an 11% equity interest in the combined company post-acquisition, multiplied by the estimated fair value of Private Acer's common stock on the acquisition date. The estimated fair value of Private Acer's common stock was based on the offering price of the common stock sold in the Concurrent Financing that was both completed concurrently with and conditioned upon the closing of the Merger. This price was determined to be the best indication of fair value on that date since the price was based on an arm's length negotiation with a group consisting of both new and existing investors of Private Acer that had been advised of the pending Merger and assumed similar liquidity risk as those investors holding the majority of shares being valued as purchase consideration.

The following table summarizes the Company's determination of fair values of the assets acquired and the liabilities assumed as of the date of acquisition. As of December 31, 2017, the Company has determined that no impairment charges were necessary.

Consideration - issuance of securities and cash paid for fractional shares	$	7,007,069
Assets acquired and liabilities assumed:		
Cash	$	1,058,276
Other assets		5,000
Accrued liabilities		(1,431,159)
Goodwill		7,374,952
Total purchase price	$	7,007,069

The Company determined that the acquired legacy technology of the Registrant had no value as of the date of the acquisition.

Goodwill represents the excess of the purchase price (consideration paid plus net liabilities assumed) of an acquired business over the fair value of the underlying net tangible and intangible assets. Goodwill includes the value of the Registrant's standing as a public entity. None of the goodwill associated with the Merger is deductible for income tax purposes. All of the goodwill will be allocated to the Company's single reportable segment.

There were no changes in goodwill during the year ended December 31, 2017, after the initial purchase accounting.

Unaudited pro forma operating results, assuming the Merger occurred as of January 1, 2016, are as follows:

	Years Ended December 31,	
	2017	**2016**
Net loss	$ (17,733,252)	$ (15,993,790)
Net loss per share - basic and diluted	$ (2.73)	$ (2.73)

4. PROPERTY AND EQUIPMENT

Property and equipment consisted of the following at December 31, 2017 and December 31, 2016:

	December 31, 2017	December 31, 2016
Computer hardware and software	$ 16,555	$ 10,862
Leasehold improvements	7,968	—
Furniture & fixtures	42,503	—
Less accumulated depreciation	(4,042)	(4,645)
	$ 62,984	$ 6,217

Property and equipment are stated on the basis of historical cost less accumulated depreciation. Depreciation is provided using the straight-line method over the estimated useful lives of the assets. Major renewals and improvements are capitalized, while minor replacements, maintenance and repairs are charged to current operations. Impairment losses are recorded on long-lived assets used in operations when indicators of impairment are present and the undiscounted cash flows estimated to be generated by those assets are less than the assets' carrying amount. Computer hardware and software are depreciated over an estimated useful life of 3 years, tenant improvements are depreciated over 1 year through the end of the current lease arrangement, and office furniture is depreciated over an estimated useful life of 7 years.

5. ACCRUED EXPENSES

Accrued expenses consisted of the following at December 31, 2017, and December 31, 2016:

	December 31, 2017	December 31, 2016
Accrued legal	$ 174,592	$ 21,477
Accrued pre-commercial costs	341,159	—
Accrued consulting	102,156	13,105
Accrued audit and tax	111,250	39,820
Accrued license fees	817,578	205,444
Accrued contract manufacturing	218,108	126,700
Accrued contract research	99,140	16,800
Accrued miscellaneous expenses	73,348	14,682
	$ 1,937,331	$ 438,028

6. CONVERTIBLE NOTES PAYABLE

On March 22, 2017, Private Acer issued senior secured convertible notes payable (the "2017 Notes") to existing investors and a vendor in the aggregate principal amount of $3,125,000. The 2017 Notes accrued interest at 10% per annum and matured on the earlier of (i) March 22, 2018 (the "Maturity Date") or (ii) upon a Change in Control of Private Acer, as defined therein.

On May 31, 2017, Private Acer issued additional 2017 Notes to existing investors and a vendor in the aggregate principal amount of $2,375,000.

The 2017 Notes were convertible into common stock upon a Qualified Financing (as defined therein), a Change in Control, or an optional conversion by the holder. Conversion upon a Qualified Financing was at a price per share equal to the price per share paid for the shares sold in the Qualified Financing less a discount of: (i) 0%, if a Qualified Financing occurred on or before June 30, 2017; (ii) 10%, if a Qualified Financing occurred after June 30, 2017, but on or before September 1, 2017; or (iii) 20%, if a Qualified Financing occurred after September 1, 2017. Conversion upon a Change in Control was at the discretion of the holder such that Private Acer would pay each holder the outstanding balance on their respective note or the note would be converted at a price per share equal to the lesser of $16.57 and the price per share of common stock paid to the holders of the common stock in such Change in Control. Conversion under an optional conversion by the holder was at a price per share of $16.57 based on the outstanding balance of the note.

Upon the issuance of the 2017 Notes, the Company evaluated all terms of the 2017 Notes, including the Change in Control provision, to identify any embedded features that required bifurcation and recording as derivative instruments. The Company determined that there were no such features requiring separate accounting.

In connection with the 2017 Notes, the Company incurred debt issuance costs of $68,530 and recorded them as a debt discount. During the year ended December 31, 2017, the Company recognized $242,982 of interest expense, which includes $68,530 in amortization of debt discount and $174,452 of accrued interest on the 2017 Notes. The principal of $5,500,000 and accrued interest of $174,452 on the 2017 Notes converted into 599,201 fully-paid shares of common stock at the time of the Merger described in Note 1, with no discount on the conversion.

7. COMMITMENTS

License Agreements

In August 2016, Private Acer signed an agreement with Assistance Publique—Hôpitaux de Paris, Hôpital Européen Georges Pompidou, or AP-HP (via its Department of Clinical Research and Development) granting the Company exclusive worldwide rights to access and use data from a randomized controlled clinical study of celiprolol. The Company will use this pivotal clinical data to support an NDA regulatory filing for its lead product, celiprolol, for the treatment of vEDS. The agreement requires the Company to make certain upfront payments to AP-HP, as well as reimburse certain costs, and make payments upon achievement of defined milestones and payment of royalties on net sales of celiprolol over the royalty term.

In April 2014, Private Acer obtained exclusive rights to intellectual property relating to ACER-001 and preclinical and clinical data, through an exclusive license agreement with Baylor College of Medicine ("BCM"). Under the terms of the agreement, as amended, the Company has worldwide exclusive rights to develop, manufacture, use, sell and import Licensed Products as defined in the agreement. The license agreement requires the Company to make certain upfront and annual payments to BCM, as well as reimburse certain legal costs, and make payments upon achievement of defined milestones and payment of royalties on net sales of any developed product over the royalty term.

Litigation

From time to time, the Company or its subsidiaries may become involved in litigation or proceedings relating to claims arising from the ordinary course of business.

On September 27, 2017, Piper Jaffray & Co. filed a lawsuit against Private Acer, Piper Jaffray & Co. v. Acer Therapeutics Inc., Index No. 656055/2017, in the Supreme Court of the State of New York, County of New York. The complaint alleges that Private Acer breached its obligations to Piper Jaffray & Co. pursuant to an August 30, 2016 engagement letter between the parties and an April 28, 2017 addendum thereto by failing to pay Piper Jaffray & Co. (i) a fee of $1,097,207 in connection with the financing which closed on September 19, 2017 for aggregate consideration of approximately $15.7 million (including the conversion of the 2017 Notes described in Note 6) and (ii) $67,496 in reimbursement for expenses incurred by Piper Jaffray & Co. pursuant to the engagement letter. On November 10, 2017, Private Acer filed an answer and counterclaim in the lawsuit, denying Piper Jaffray & Co.'s breach of contract allegation, asserting several defenses, and bringing several counterclaims, including claims for breach of contract and breach of the duty of good faith and fair dealing. Piper Jaffray & Co. filed a reply to the counterclaims denying the essential allegations, and discovery has commenced. The Company has not recorded a liability as of December 31, 2017, because a potential loss is not probable or reasonably estimable given the preliminary nature of the proceedings.

8. STOCKHOLDERS' EQUITY

Immediately prior to the consummation of the Merger described in Note 1, (i) Private Acer's Series A Convertible Redeemable Preferred stock and Series B Convertible Redeemable Preferred stock were converted into 638,416 and 970,238 shares of common stock, respectively, (ii) Private Acer's 2017 Notes and accrued interest totaling $5,674,452 were converted into 599,201 shares of common stock, and (iii) 1,055,961 shares of common stock were sold by Private Acer for $9.47 per share generating $10,000,000 of gross proceeds. At the closing of the Merger, 736,950 shares of common stock were held by existing shareholders of the Registrant.

On December 14, 2017, the Company closed on an underwritten public offering of its common stock of 916,667 shares at a price of $12.00 per share. The gross proceeds were $11.0 million, before deducting the underwriting discount and other estimated offering expenses. Subsequently, on December 27, 2017, the Company sold an additional 130,000 shares in connection with the over-allotment option granted to the underwriters, for an additional $1.6 million of gross proceeds, before deducting the underwriting discount. The total amount of underwriting discount and other offering costs deducted from gross proceeds was $1.1 million.

2010 Stock Incentive Plan

The Company's 2010 Stock Incentive Plan, as amended and restated (the "2010 Plan"), provides for the grant of up to 470,170 shares of common stock as incentive or non-qualified stock options, stock appreciation rights, restricted stock units and/or restricted common stock to employees, officers, directors, consultants and advisers. The 2010 Plan was amended to increase the shares reserved for issuance from 113,465 to 470,170 shares and such amendment was approved by the Registrant's shareholders on September 19, 2017. The Board of Directors or the Compensation Committee, as applicable, administers the 2010 Plan and has discretion to determine the recipients, the number and types of equity awards to be granted and the terms and conditions of the equity awards, including the period of their exercisability and vesting. Subject to a limitation on repricing without shareholder approval, the Board or the Compensation Committee, as applicable, may also determine the exercise price of options granted under the 2010 Plan. Option awards are generally granted with an exercise price equal to the fair value of the common stock at the date of grant and have contractual terms of 10 years. Stock options granted to executive officers and employees vest over a four-year period, with 25% vesting on the one-year anniversary of the grant date and the remaining 75% vesting quarterly over the remaining three years, assuming continued service, and with vesting acceleration in full immediately prior to a change in control. Stock options granted to outside non-employee directors vest either (a) in full on the one-year anniversary of the grant date, assuming continued service, for awards to continuing directors, with vesting acceleration in full immediately prior to a change in control, or (b) quarterly over a three-year period, assuming continued service, for awards to new directors, with vesting acceleration in full immediately prior to a change in control. All outstanding and unexercised equity awards (representing 22,061 underlying shares) under the 2010 Plan were canceled in connection with the Merger. At December 31, 2017, 159,572 shares of common stock remained available for the grant of future awards under the 2010 Plan.

2013 Stock Incentive Plan

Private Acer's 2013 Stock Incentive Plan, as amended (the "2013 Plan"), which was assumed by the Company in connection with the Merger, provides for the issuance of up to 165,000 shares of common stock as incentive or non-qualified stock options and/or restricted common stock to employees, officers, directors, consultants and advisers. Option awards are generally granted with an exercise price equal to the fair value of the common stock at the date of grant and have contractual terms of 10 years. At December 31, 2017, all shares available under the 2013 Plan were subject to outstanding equity awards, and the Company does not intend to make any new awards under the 2013 Plan.

A summary of option activity under the 2010 Plan and the 2013 Plan for the year ended December 31, 2017, is as follows:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (Years)	Aggregate Intrinsic Value
Options outstanding at December 31, 2016	122,000	$ 2.55	8.8	
Granted	347,225	$ 14.10		
Exercised	—	$ —		
Cancelled/forfeited	(5,625)	$ 2.55		
Options outstanding at December 31, 2017	463,600	$ 11.23	9.5	$ 1,752,470
Options exercisable at December 31, 2017	139,634	$ 3.49	8.9	$ 1,448,223

At December 31, 2017, there was approximately $2.5 million of unrecognized compensation expense related to the share-based compensation arrangements granted under both plans and the average remaining vesting period is 3.3 years. The weighted average grant date fair value of options granted during the year ended December 31, 2017 and 2016, was $8.25 and $1.32, respectively. The amount of stock-based compensation expense recorded to general and administrative, and research and development was approximately $111,000 and $307,000, respectively, for the year ended December 31, 2017.

Warrants

A summary of warrant activity for the year ended December 31, 2017, is presented below:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Contract Term (# years)	Intrinsic Value
Assumed in the Merger at September 19, 2017	317,630	$ 123.61	0.54	—
Outstanding and exercisable at December 31, 2017	317,630	$ 123.61	0.29	—

9. INCOME TAXES

There was no provision for income taxes for the years ended December 31, 2017, and 2016, due to the Company's operating losses and a full valuation allowance on deferred tax assets. Deferred income taxes reflect the net tax effects of temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. Significant components of the Company's deferred tax assets and liabilities are as follows:

	December 31,	
	2017	2016
Deferred tax assets:		
Net operating loss carry forwards	$ 1,843,000	$ 537,000
Capitalized research and development costs	12,177,000	2,532,000
Accrued liabilities	25,000	15,000
Tax credit carryforwards	3,286,000	1,683,000
Stock-based compensation	116,000	38,000
Total deferred tax assets	17,447,000	4,805,000
Valuation allowance	(17,444,000)	(4,805,000)
Net deferred tax assets	3,000	—
Deferred tax liabilities	(3,000)	—
	$ —	$ —

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

	December 31,	
	2017	2016
Federal statutory rate	34.0%	34.0%
R&D and Orphan drug credits	7.6%	15.8%
State income tax, net of federal tax benefit	-0.2%	-17.7%
Valuation allowance	18.2%	137.1%
Reduction in tax attributed related to deconsolidation of subsidiary	0.0%	-169.0%
Tax reform impact	-57.8%	0.0%
Other, net	-1.8%	-0.2%
Effective tax rate	0.0%	0.0%

The increase in deferred tax assets in 2017 is a result of the Merger (see Note 3). Management currently believes that it is more likely than not that the deferred tax assets relating to the loss carryforwards and other temporary differences will not be realized in the future. Through December 31, 2017, for income tax reporting purposes, the Company had U.S. Federal and state net operating loss carryforwards of approximately $8.4 million (corresponding to $1.8 million on a tax effected basis in the table above), that can be carried forward and offset against taxable income. Federal and Massachusetts net operating losses can be carried forward for 20 years and begin to expire in 2024. Utilization of net operating losses may be subject to substantial annual limitations due to the "change in ownership" provisions of the Internal Revenue Code of 1986, and similar state provisions. The annual limitations may result in the expiration of net operating losses before utilization.

On December 22, 2017, The Tax Cuts and Jobs Act (the "Act") was enacted. The Act significantly revised the U.S. corporate income tax law by lowering the corporate Federal income tax rate from 35% to 21%. As of December 31, 2017, the Company has assessed the effects of the corporate rate reduction on its existing deferred tax balances which resulted in a $8.2 million reduction in the deferred tax assets. Since the Company maintains a full valuation allowance on its deferred tax assets, a corresponding reduction in the valuation allowance equal to the effect of the rate reduction on the ending deferred tax asset was also reflected. In addition to the rate reduction, the Act also requires companies with foreign subsidiaries to pay a one-time transition tax on earnings that were previously tax deferred. As of December 31, 2017, the Company does not maintain any foreign subsidiaries and does not have previously deferred foreign earnings subject to the transition tax.

10. NET LOSS PER SHARE

Basic net loss per share is computed by dividing the net loss by the weighted-average number of common shares outstanding. Diluted net loss per share is computed similarly to basic net loss per share except that the denominator is increased to include the number of additional common shares that would have been outstanding if the potential common shares had been issued and if the additional common shares were dilutive. Diluted net loss per share is the same as basic net loss per common share, since the effects of potentially dilutive securities are antidilutive.

As of December 31, 2017, and 2016, the number of shares of common stock underlying potentially dilutive securities include:

	December 31,	
	2017	2016
Convertible redeemable preferred stock	—	638,416
Warrants	317,630	—
Options to purchase common stock	463,600	122,000
Total	781,230	760,416

Item 9. Changes in and Disagreements With Accountants on Accounting and Financial Disclosure.

None.

Item 9A. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

In accordance with Exchange Act Rules 13a-15 and 15d-15, we carried out an evaluation, under the supervision and with the participation of management, including our Chief Executive Officer and Chief Financial Officer, of the effectiveness of our disclosure controls and procedures as of the end of the period covered by this report. Based on that evaluation, our Chief Executive Officer and Chief Financial Officer concluded that our disclosure controls and procedures were effective as of December 31, 2017 in enabling us to record, process, summarize and report information required to be included in our periodic SEC filings within the required time period.

Management's Report on Internal Control over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Exchange Act Rules 13a-15(f) and 15d-15(f). Under the supervision and with the participation of our management, including our Chief Executive Officer and Chief Financial Officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting based on the framework in *Internal Control—Integrated Framework* issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO).

Based on our evaluation under the framework in *Internal Control—Integrated Framework* issued by COSO, our management concluded that our internal control over financial reporting was effective as of December 31, 2017 in providing reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles.

This annual report does not include an attestation report of our registered public accounting firm regarding internal control over financial reporting. Management's report was not subject to attestation by our registered public accounting firm pursuant to rules of the SEC that permit us to provide only management's report in this annual report.

Changes in Internal Control over Financial Reporting

There was no change in internal control over financial reporting (as such term is defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) during our fourth fiscal quarter that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information.

None.

PART III

Item 10. Directors, Executive Officers and Corporate Governance.

Executive Officers

Our executive officers are elected by the Board of Directors and serve at the discretion of the Board. Our current executive officers are as follows:

Name	Age	Position
Chris Schelling	42	President, Chief Executive Officer and Director
William T. Andrews, M.D.	53	Chief Medical Officer
Harry S. Palmin	48	Chief Financial Officer

Biographical information for our executive officers is set forth below:

Chris Schelling has served as a Director and as our President and Chief Executive Officer since the completion of the merger in September 2017. Mr. Schelling founded Private Acer in December 2013 and has served as a director since that time. From December 2013 to February 2016, he served as Private Acer's chief operating officer, and since February 2016 has served as Private Acer's president and chief executive officer. Mr. Schelling also founded Apanii Consulting, LLC, a pharmaceutical and biotechnology consulting company, in December 2012 and currently serves as Apanii's chief executive officer. Prior to founding Apanii Consulting, he served as executive director of strategic marketing at BioMarin Pharmaceutical Inc., or BioMarin, a Nasdaq-listed biotechnology company, from May 2006 to October 2012. He has also held roles at Abgenix, Inc., Cell Therapeutics, Inc., Stanford Research Institute Consulting and Organon. Mr. Schelling earned a B.A. in biology from Carroll College.

William T. Andrews, M.D., FACP has served as our Chief Medical Officer since October 2017. Prior to joining the Company, Dr. Andrews provided strategic consulting services to rare disease companies from April 2016 to September 2017. Prior to that, he served Aegerion Pharmaceuticals, Inc., a biopharmaceutical company, as senior vice president, business development, from May 2014 to January 2016, and as vice president of medical affairs, from April 2012 to May 2014. He has also held roles at Santhera Pharmaceuticals, Sepracor, Inc. and ClinQuest, Inc. Prior to joining the biopharmaceutical industry over 15 years ago, Dr. Andrews practiced medicine for seven years full-time and 11 years part-time in the Boston area as a board certified internist and an attending physician at Brigham and Women's Hospital, and was on the clinical faculty at Harvard Medical School. Dr. Andrews earned a B.A. in biology from Harvard University and a Ph.D. from Yale University School of Medicine.

Harry S. Palmin has served as our Chief Financial Officer since the completion of the merger in September 2017. From December 2013 to February 2016, Mr. Palmin served as the president, chief executive officer and a director of Private Acer, from February 2016 to September 2017 he served as Private Acer's acting chief financial officer. Prior to joining Private Acer, he served in a variety of roles at Novelos Therapeutics, Inc., a pharmaceutical company, including as president and director from 1998 to October 2013, chief executive officer from January 2005 to October 2013 and acting chief financial officer from 1998 to September 2005. He has also held roles at Lehman Brothers and Morgan Stanley. Mr. Palmin earned a B.A. in economics from Brandeis University and a M.A. in international economics and finance from the Brandeis University International Business School.

Directors

All of the current directors serve until the next annual shareholders' meeting or until their successors have been duly elected and qualified. Our current Board of Directors is as follows:

Name	Age	Position
Stephen J. Aselage	66	Chairman of the Board of Directors
Jason Amello	49	Director
Hubert Birner, Ph.D., MBA	51	Director
John M. Dunn	66	Director
Michelle Griffin	52	Director
Luc Marengere, Ph.D.	52	Director
Chris Schelling	42	Director, President and Chief Executive Officer

Biographical information for our directors is set forth below:

Stephen J. Aselage has served as Chairman of the Board since the completion of the merger in September 2017. Since October 2015, Mr. Aselage has served as the chairman of Private Acer's board of directors. He has served as president and chief executive officer of Retrophin, Inc., a Nasdaq-listed, fully integrated biopharmaceutical company, since November 2014 and as a member of its board of directors since October 2012. From May 2014 to November 2014, Mr. Aselage served as the chief operations officer and interim chief executive officer of Retrophin. Prior to joining Retrophin, he held a variety of roles at BioMarin, as executive vice president and chief business officer from December 2009 to September 2012 and senior vice president of global commercial development from July 2005 to December 2009. He has also held leadership roles at Cell Therapeutics, Inc., Sangstat Medical Corporation, Advanced Tissue Sciences, Inc. and Genentech, Inc. Mr. Aselage earned a B.S. in biology from the University of Notre Dame.

Jason Amello has served as a Director since the completion of the merger in September 2017. Since September 2013, Mr. Amello has served as senior vice president, chief financial officer and treasurer of Akebia Therapeutics, Inc., a biopharmaceutical company focused on the development and commercialization of novel therapeutics for patients with kidney disease. From May 2012 to May 2013, he served as executive vice president, chief financial officer and treasurer of ZIOPHARM Oncology, Inc., a biopharmaceutical company focused on the discovery and development of new cancer therapies. From April 2000 to June 2011, Mr. Amello held various positions at Genzyme Corporation, a biotechnology company focused on development of therapeutics for multiple diseases and disorders, most recently as senior vice president, corporate controller, and chief accounting officer. Earlier in his career, Mr. Amello spent 10 years in the business advisory and assurance practice of Deloitte, serving in various roles of increasing responsibility through senior manager. He currently serves on the board of directors of the New England Baptist Hospital, an orthopedic specialty hospital. Mr. Amello earned a B.A. in accounting from Boston College and is a Certified Public Accountant in the Commonwealth of Massachusetts.

Hubert Birner, Ph.D., MBA has served as a Director since the completion of the merger in September 2017. Since April 2017, Dr. Birner has served as a member of Private Acer's board of directors. Since 2000, he has served in a variety of roles for TVM Capital, an independent affiliation of international private equity and venture capital firms, where he currently serves as the managing partner of TVM Capital and TVM Life Science Management. Dr. Birner currently serves as the chairman of the boards of directors of Argos Therapeutics, Inc., a Nasdaq-listed immuno-oncology company, and NOXXON Pharma N.V., a EuroNext Growth Paris-listed biopharmaceutical company, and as a member of the board of directors of Proteon Therapeutics, Inc., a Nasdaq-listed biopharmaceutical company, as well as a number of privately held life science companies. Prior to his tenure at TVM Capital, Dr. Birner held roles at Zeneca Group PLC and McKinsey & Company. He served as the vice chairman of Evotec AG, a Frankfurt Stock Exchange-listed company focused on the discovery and development of small molecule drugs, from 2005 to 2013, and as a director of Probiodrug AG, a Euronext Amsterdam-listed biopharmaceutical company, from 2014 to 2015. Dr. Birner earned a Ph.D. in biochemistry from Ludwig-Maximilian University of Munich and an MBA from Harvard Business School.

John M. Dunn has served as a Director since the completion of the merger in September 2017. Since October 2015, Mr. Dunn has served as a member of Private Acer's board of directors. Since November 2014, he has served as general counsel of Vital Therapies, Inc., a biotherapeutic company. Prior to joining Vital Therapies, Mr. Dunn was a consultant from February 2012 to November 2014, an executive vice president of Biogen Idec, Inc., now Biogen, Inc., a biotechnology company, from November 2003 to January 2012, where he was the head of that firm's corporate venture group, and general counsel of IDEC Pharmaceuticals from 2002 until its merger with Biogen in November 2003. Mr. Dunn earned a B.S. in finance and a J.D. from the University of Wyoming.

Michelle Griffin has served as a Director since the completion of the merger in September 2017. Since April 2013, Ms. Griffin has served as the principal of Pacific Biotechnology Consulting Group, a firm providing consulting services to biotechnology companies and their boards of directors. Prior to her time with Pacific Biotechnology Consulting Group, Ms. Griffin served from January 2011 to March 2013 as executive vice president, operations and chief financial officer of OncoGenex Pharmaceuticals, Inc. Ms. Griffin served as a member of the board of directors and as chair of the audit committee for PhaseRx, Inc. from 2016 until its acquisition by Roivant Sciences GmbH in 2018, OncoGenex Pharmaceuticals, Inc. from 2008 to 2011, and Sonus Pharmaceuticals, Inc. (subsequently acquired by OncoGenex) from 2004 to 2008, and during various periods from 1997 to 2011 served in the capacity of Chief Financial Officer for Trubion Pharmaceuticals, Inc., Dendreon Corporation and Corixa Corporation. Ms. Griffin earned her B.S. in marketing from George Mason University and her M.B.A. with a specialization in finance and international business from Seattle University.

Luc Marengere, Ph.D. has served as a Director since the completion of the merger in September 2017. Since April 2016, Dr. Marengere has served as a member of Private Acer's board of directors. He serves as managing partner of TVM Life Science Venture VII, L.P, a venture capital fund, which he joined in March 2012. From October 2001 to March 2012, Dr. Marengere was a managing general partner with VG Partners, a merchant bank. He serves on the boards of directors of a number of privately held life science companies. From January 2015 to March 2017, Dr. Marengere also served on the board of directors of CoLucid Pharmaceuticals, Inc., a Nasdaq-listed biopharmaceutical company. He has also held roles at CDP Capital – Technology Ventures and MDS Capital Corp. Dr. Marengere earned a Ph.D. from the University of Toronto, an M.S. in endocrinology from Queen's University and a B.S. in biochemistry from the University of Ottowa.

Chris Schelling. Refer to "Executive Officers" section above for Mr. Schelling's biographical information.

Audit Committee

The Audit Committee of the Board currently consists of Ms. Griffin (chair), and Messrs. Amello and Dunn, each of whom is an independent, non-employee director. The Audit Committee selects, on behalf of our Board, an independent public accounting firm to audit our financial statements, discusses with the independent auditors their independence, reviews and discusses the audited financial statements with the independent auditors and management, recommends to our Board whether the audited financials should be included in our annual reports to be filed with the SEC, and oversees management's identification, evaluation, and mitigation of major risks to the Company. The Audit Committee operates pursuant to a written charter. During the last fiscal year, the Audit Committee held four meetings.

All of the members of the Audit Committee are non-employee directors who: (1) met the criteria for independence as required by Nasdaq listing standards and as set forth in Rule 10A-3(b)(1) under the Securities Exchange Act of 1934, as amended (the "Exchange Act"); (2) did not participate in preparation of our financial statements during the past three years; and (3) are able to read and understand fundamental financial statements, including a balance sheet, income statement, and cash flow statement. The Board has determined that Ms. Griffin and Messrs. Amello and Dunn each, individually, qualifies as an "audit committee financial expert" as defined in SEC rules and regulations and also possesses the financial sophistication and requisite experience as required under Nasdaq listing standards.

Code of Ethics

In accordance with SEC rules, the Audit Committee and the Board of Directors has adopted a Policy on Whistleblower Protection and Code of Ethics which is applicable to our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions, which we sometimes refer to as our senior financial officers. The Board of Directors believes that these individuals must set an exemplary standard of conduct, particularly in the areas of accounting, internal accounting control, auditing and finance. This Code of Ethics sets forth ethical standards to which the designated officers must adhere and other aspects of accounting, auditing and financial compliance. The Code of Ethics is available on our website at **www.acertx.com**. Please note that the information contained on our website is not incorporated by reference in, or considered to be a part of, this report.

Section 16(a) Beneficial Ownership Reporting Compliance

Section 16(a) of the Exchange Act requires our directors and executive officers, and persons who beneficially own more than 10% of a registered class of our equity securities, to file with the SEC initial reports of ownership and reports of changes in ownership. These reporting persons are required by SEC regulations to furnish us with copies of all such reports they file. To our knowledge, based solely on our review of the copies of such reports furnished to us and written representations from certain insiders that no other reports were required, we believe all of the reporting persons complied with all applicable Section 16(a) filing requirements applicable to them with respect to transactions during the fiscal year ended December 31, 2017, except that one group Form 4 was filed late by funds affiliated with TVM Capital Life Science and Drs. Birner and Marengere to report the purchase of common stock by TVM Capital Life Science funds in our December 2017 underwritten public offering.

Item 11. Executive Compensation

Executive Officer Compensation

The following table sets forth certain information concerning compensation earned by or paid to certain persons who we refer to as our "Named Executive Officers" for services provided for the fiscal year ended December 31, 2017. Our Named Executive Officers include persons who (i) served as our principal executive officer or acted in a similar capacity during 2017, (ii) were serving at fiscal year-end as our two most highly compensated executive officers, other than the principal executive officer, whose total compensation exceeded $100,000, and (iii) if applicable, up to two additional individuals for whom disclosure would have been provided as a most highly compensated executive officer, but for the fact that the individual was not serving as an executive officer at fiscal year-end.

2017 Summary Compensation Table

Name and Principal Position	Year	Salary	Bonus	Option Awards(1)		All Other Compensation		Total
Chris Schelling(2)	2017	$ 116,667	$ —	$ 468,791	(3)	$ —		$ 585,458
President and Chief Executive Officer								
William T. Andrews, M.D., FACP(4)	2017	$ 100,000	$ 40,000	$ 878,921	(3)	$ —		$ 1,018,921
Chief Medical Officer								
Harry Palmin(5)	2017	$ 99,167	$ —	$ 594,151	(3)	$ —		$ 693,318
Chief Financial Officer								
Neil K. Warma(6)	2017	$ 301,147	$ —	$ —		$ 889,307	(7)	$ 1,190,454
Former President, Chief Executive Officer and Acting Chief Financial Officer (Opexa Therapeutics, Inc.)	2016	$ 416,625	$ —	$ 201,846		$ —		$ 618,471

(1) Amounts shown in this column represent the aggregate grant date fair value of stock option awards made during 2017, calculated in accordance with ASC Topic 718. See Note 2 to our financial statements appearing elsewhere in this report for a discussion of the relevant assumptions used in calculating these amounts.

(2) Mr. Schelling was appointed as an executive officer on September 19, 2017, the effective date of the merger. Mr. Schelling's current base annual salary is $400,000.

(3) Option awards were granted to Messrs. Schelling, Andrews and Palmin on October 4, 2017 with an exercise price equal to the closing market price of our common stock on the Nasdaq Capital Market. The options are time-based and vest over a four-year period, with 25% of the shares vesting on the one-year anniversary of the grant date and the remaining shares vesting quarterly over the remaining three-year period, assuming continued service. The options have a standard post-service exercise period of 90 days. The options will accelerate and become fully vested immediately prior to a Change in Control (as defined in our 2010 Stock Incentive Plan), but only to the extent that the optionee remains in service immediately prior to such Change in Control.

(4) Dr. Andrews was appointed as an executive officer on October 2, 2017. Dr. Andrews' current base annual salary is $400,000, and he received a signing bonus in connection with commencement of employment.

(5) Mr. Palmin was appointed as an executive officer on September 19, 2017, the effective date of the merger. Mr. Palmin's current base annual salary is $340,000.

(6) Mr. Warma's employment was terminated on September 19, 2017 in connection with the merger.

(7) Represents severance compensation and benefits paid and payable to Mr. Warma pursuant to his employment agreement as follows: (i) 18 months of base salary in the amount of $624,937; (ii) a payment in lieu of any potential bonus in the amount of $187,481; (iii) accrued vacation pay in the amount of $30,723; and (iv) up to $46,166 for reimbursement of Mr. Warma's COBRA expenses actually incurred during the 18-month period following the effective date of his employment termination.

Narrative Disclosure to Summary Compensation Table

Acer's Board of Directors reviews compensation annually for all of its executive officers. Compensation awarded to Named Executive Officers in 2017 generally consisted of base salary and equity awards for options to purchase shares of Acer's common stock. In setting executive compensation, Acer's Board of Directors considered compensation for comparable positions in the market, the historical compensation levels of its executives, individual performance as compared to its expectations and objectives, the desire to motivate employees to achieve short- and long-term results that are in the best interests of our shareholders, and a long-term commitment to Acer. Acer does not target a specific competitive position or a specific mix of compensation among elements of compensation. Prior to the September 2017 merger with Opexa, Private Acer retained the services of Radford (which is a part of Aon Hewitt, a business unit of Aon plc) as an independent compensation consultant to (i) evaluate Private Acer's executive compensation program and recommend a course of action for consideration in preparation for becoming a public company and (ii) assess Private Acer's non-employee director compensation practices against a selection of peer group companies and make a recommendation relating thereto. Subsequent to closing of the merger with Opexa in September 2017, our Compensation Committee reviewed the analysis and reports prepared by Radford and provided to Private Acer and implemented certain compensation adjustments for our executives and non-employee directors. In reviewing the reports prepared by Radford for Private Acer, our Compensation Committee considered the independence of Radford pursuant to SEC rules and the corporate governance rules of the Nasdaq Stock Market and concluded that no conflict of interest exists that would prevent Radford from independently advising the Compensation Committee.

Agreement with Former Opexa Executive Officer. Prior to the merger, Mr. Warma was employed by Opexa as President, Chief Executive Officer and Acting Chief Financial Officer pursuant to the terms of an employment agreement dated June 16, 2008. Upon effectiveness of the merger on September 19, 2017, which was deemed to be a change of control under his employment agreement, Mr. Warma's employment was terminated. Mr. Warma's base salary at the time of his termination was $416,625 per annum, and he was eligible to receive an annual discretionary cash bonus of up to 50% of his base salary based on the achievement of selected objectives. Pursuant to the terms of his employment agreement, he received lump sum severance compensation equal to 18 months of base salary and a payment equal to 45% of his base salary in lieu of any potential bonus. He is also entitled to receive reimbursement of COBRA expenses for an 18-month period, subject to a cap equal to Opexa's standard contribution to employee health benefits. In addition, the vesting of Mr. Warma's stock options accelerated in full, although all such options were under water and were cancelled in connection with the merger. Mr. Warma's agreement also provides that for a 12-month period following his termination of employment, he will not engage or participate in any competitive business or solicit or recruit any of the Company's employees. Mr. Warma delivered a general release and waiver of claims in favor of the Company in consideration for his severance compensation.

2017 Outstanding Equity Awards at Fiscal Year-End

The following table presents information regarding outstanding equity awards at December 31, 2017 for each of the Named Executive Officers.

	Option Awards				
Name	Number of Securities Underlying Unexercised Options (#) Exercisable	Number of Securities Underlying Unexercised Options (#) Unexercisable		Option Exercise Price	Option Expiration Date
Chris Schelling	0	46,000	(1)	$ 15.34	10/4/2027
William T. Andrews, M.D., FACP	0	100,000	(1)	$ 15.34	10/4/2027
Harry S. Palmin	0	67,600	(1)	$ 15.34	10/4/2027
Neil K. Warma	0	0		$ —	—

(1) The options are time-based and vest over a four-year period, with 25% of the shares vesting on the one-year anniversary of the grant date and the remaining shares vesting quarterly over the remaining three-year period, assuming continued service. The options have a standard post-service exercise period of 90 days. The options will accelerate and become fully vested immediately prior to a Change in Control (as defined in our 2010 Stock Incentive Plan), but only to the extent that the optionee remains in service immediately prior to such Change in Control.

2017 Director Compensation

The following table presents summary information regarding compensation of the non-employee members of our Board of Directors who served during any part of the fiscal year ended December 31, 2017.

Name	Fees Earned or Paid in Cash	Option Awards(1)(2)	All Other Compensation	Total
Acer Therapeutics Inc.				
Jason Amello	$ 10,625	$ 77,764	$ —	$ 88,389
Stephen J. Aselage	$ 18,438	$ 52,735	$ —	$ 71,173
Hubert Birner, Ph.D., MBA (3)	$ —	$ —	$ —	$ —
John M. Dunn	$ 12,500	$ 52,735	$ —	$ 65,235
Michelle Griffin	$ 13,750	$ 77,764	$ —	$ 91,514
Luc Marengere, Ph.D. (3)	$ —	$ —	$ —	$ —
Formerly Opexa Therapeutics, Inc.				
Timothy C. Barabe (4)	$ 41,250	$ —	$ —	$ 41,250
Hans-Peter Hartung, M.D. (4)	$ 41,250	$ —	$ —	$ 41,250
Gail J. Maderis (4)	$ 41,250	$ —	$ —	$ 41,250
Michael S. Richman (4)	$ 41,250	$ —	$ —	$ 41,250

(1) Amounts shown in this column represent the aggregate grant date fair value of stock option awards made during 2017, calculated in accordance with ASC Topic 718. See Note 2 to our financial statements appearing elsewhere in this report for a discussion of the relevant assumptions used in calculating these amounts.

(2) The aggregate number of shares underlying outstanding option awards as of December 31, 2017 was: Mr. Aselage, 27,000 shares; Mr. Amello, 9,000 shares; Dr. Birner, 0 shares; Mr. Dunn, 22,000 shares; Ms. Griffin, 9,000 shares; Dr. Marengere, 0 shares; and 0 shares for each of the former Opexa directors Messrs. Barabe and Richman, Dr. Hartung and Ms. Maderis.

(3) Drs. Birner and Marengere are each affiliated with TVM Capital Life Sciences and do not receive compensation for their service on our Board of Directors.

(4) Ms. Maderis, Dr. Hartung, and Messrs. Barabe and Richman served as directors of Opexa through September 19, 2017, the effective date of the merger.

Standard Compensation Arrangements

Outside directors who are not affiliated with TVM Capital Life Sciences receive compensation for their service on our Board of Directors that consists of cash compensation and equity awards as described below. A director who is also our employee does not receive any additional compensation for services as a member of our Board. We reimburse our directors for travel and lodging expenses in connection with their attendance at Board and committee meetings. Our standard annual compensation arrangements consist of the following:

Board Member Cash Compensation:

- Annual Board member retainer - $35,000
- Additional non-executive Board Chair retainer - $25,000

Additional Committee Chair Cash Compensation:

- Audit - $15,000
- Compensation - $10,000
- Nominating/Governance - $7,500

Additional Committee Member Cash Compensation:

- Audit - $7,500
- Compensation - $5,000
- Nominating/Governance - $3,750

Board Member Equity Compensation:

- Initial stock option award to newly-appointed directors – 9,000 shares, vesting quarterly over a three-year period from the date of grant, with vesting to accelerate immediately prior to a Change in Control (as defined in our 2010 Stock Incentive Plan).
- Annual stock option award – 6,000 shares, vesting on the one-year anniversary from the date of grant, with vesting to accelerate immediately prior to a Change in Control (as defined in our 2010 Stock Incentive Plan).

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters.

The following table sets forth, as of March 1, 2018, the number and percentage of outstanding shares of our common stock beneficially owned by: (a) each person who is known by us to be the beneficial owner of more than 5% of our outstanding shares of common stock; (b) each of our directors; (c) the Named Executive Officers; and (d) all current directors and executive officers as a group. As of March 1, 2018, there were 7,497,433 shares of common stock issued and outstanding.

Beneficial ownership has been determined in accordance with Rule 13d-3 under the Exchange Act. Under this rule, certain shares may be deemed to be beneficially owned by more than one person (if, for example, persons share the power to vote or the power to dispose of the shares). In addition, shares are deemed to be beneficially owned by a person if the person has the right to acquire shares (for example, upon exercise of an option or warrant) within 60 days of the date as of which the information is provided. In computing the percentage ownership of any person,

the amount of shares is deemed to include the amount of shares beneficially owned by such person by reason of such acquisition rights. As a result, the percentage of outstanding shares of any person as shown in the following table does not necessarily reflect the person's actual voting power at any particular date.

To our knowledge, except as indicated in the footnotes to this table and pursuant to applicable community property laws, the persons named in the table have sole voting and investment power with respect to all shares of common stock shown as beneficially owned by them.

Beneficial Ownership Table

Name and Address of Beneficial Owner(1)	Number of Shares Owned		Percentage of Class
5% Stockholders (excluding Executive Officers and Directors):			
Funds affiliated with TVM Capital Life Science	2,397,309	(2)	32.0%
Bukwang Pharmaceutical Co. Ltd.	544,572	(3)	7.3%
Avego Healthcare Capital, LLC and affiliates	527,983	(4)	7.0%
Executive Officers and Directors:			
Chris Schelling	1,750,000		23.3
William T. Andrews, M.D., FACP	0		—
Harry S. Palmin	125,000		1.7
Jason Amello	750	(5)	*
Stephen J. Aselage	34,905	(6)	*
Hubert Birner, Ph.D., MBA	0	(7)	—
John M. Dunn	21,952	(8)	*
Michelle Griffin	750	(9)	*
Luc Marengere, Ph.D.	0	(10)	—
Neil K. Warma	2,197	(11)	*
All current directors and executive officers as a group (9 persons)	1,933,357	(12)	25.7%

* Less than 1%

(1) Unless otherwise indicated in the footnotes, the mailing address of the beneficial owner is c/o Acer Therapeutics Inc., One Gateway Center, Suite 351 (300 Washington Street), Newton, Massachusetts 02458.

(2) This information is based on a Schedule 13D filed with the SEC on February 16, 2018. Consisting of shares of common stock beneficially owned by certain investment funds affiliated with TVM Capital Life Science as follows: (i) 1,422,709 shares of common stock held by TVM Life Science Ventures VII L.P. ("TVM VII"); (ii) 725,844 shares of common stock held by TVM Life Science Ventures VI GmbH & Co. KG ("TVM VI German"); and (iii) 248,756 shares of common stock held by TVM Life Science Ventures VI L.P. ("TVM VI Cayman"). With respect to the shares held by TVM VII, TVM LSV VII (GP) Ltd. ("TVM VII GP") is the general partner of TVM VII. Luc Marengere, Mark Wanless, Gary Leatt, Hubert Birner, Stefan Fischer and Helmut Schühsler are members of the investment committee of TVM VII GP, which has voting and investment power with respect to these shares, and may be deemed to beneficially own such shares. TVM VII GP and Messrs. Birner, Fischer, Schühsler, Marengere, Wanless and Leatt each disclaim beneficial ownership of the reported securities, other than those shares which the reporting person owns of record. The address of TVM VII is 204, Rue Notre-Dame Ouest, Bureau 350, Montreal A8 H2Y 1TE, Canada. With respect to the shares held by TVM VI German, Messrs. Birner, Fischer and Schühsler are members of the investment committee of TVM Life Science Ventures Management VI L.P. ("TVM VI Management"), which is the managing limited partner of TVM VI German with voting and dispositive power over the shares held by TVM VI German, and may be deemed to beneficially own such shares. TVM VI Management and Messrs. Birner, Fischer and Schühsler each disclaim beneficial ownership of the shares held by TVM VI German, other than those shares which the reporting person owns of record. The address of TVM VI German is Ottostrasse 4, 80333 Munich, Germany. With respect to the shares held by TVM VI Cayman, Messrs. Birner, Schühsler and Fisher are members of the investment committee of TVM VI Management, which is the managing limited partner of TVM VI Cayman with voting and dispositive power over the shares held by TVM VI Cayman, and may be deemed to beneficially own such shares. TVM VI Management and Messrs. Birner, Schühsler and Fischer each disclaim beneficial ownership of the shares held by TVM VI Cayman, other than those shares which the reporting persons owns of

record. The address of TVM VI Cayman is Ottostrasse 4, 80333 Munich, Germany. Drs. Birner and Marengere are members of our Board of Directors.

(3) This information is based on confirmation provided by the shareholder of its stock ownership position. Hee-Won Yoo is the President and Chief Executive Officer of Bukwang Pharmaceutical Co. Ltd. Bukwang's address is 7, Sangdo-ro, Dongjak-gu, Seoul 06955, Korea.

(4) This information is based on a Schedule 13D filed with the SEC on September 28, 2017. Pursuant to the Schedule 13D, voting and investment power with respect to these shares, which are held of record directly by Avego Healthcare Capital LLC ("Avego"), may be deemed to be shared by Avego's controlling member, Mayura Trust B ("Mayura Trust"), the trustee of Mayura Trust, Mayura One LLC ("Mayura One"), and individuals Bala Venkataraman, Yelena Epova and Christopher R. Manning. Each reporting person disclaims beneficial ownership of all securities except to the extent of such person's pecuniary interest therein, other than those securities reported as being held directly by such person. Avego's address is 1055B Powers Place, Alpharetta, Georgia 30009.

(5) Represents shares of common stock underlying stock options exercisable within 60 days of March 1, 2018.

(6) Consisting of (i) 13,905 shares of common stock; and (ii) 21,000 shares of common stock underlying stock options exercisable within 60 days of March 1, 2018.

(7) Does not include shares of common stock beneficially owned by TVM VII LP, TVM VI or TVM VI LP, for each of which Dr. Birner serves on the investment committee. See footnote 2 above.

(8) Consisting of (i) 5,952 shares of common stock; and (ii) 16,000 shares of common stock underlying stock options exercisable within 60 days of March 1, 2018.

(9) Represents shares of common stock underlying stock options exercisable within 60 days of March 1, 2018.

(10) Does not include shares of common stock beneficially owned by TVM VII LP, for which Dr. Marengere serves on the investment committee. See footnote 2 above.

(11) Consisting of: (i) 1,748 shares of common stock; and (ii) 449 shares of common stock underlying Series M warrants. Mr. Warma's employment terminated on September 19, 2017 in connection with the merger.

(12) Consisting of: (i) 1,894,857 shares of common stock; and (ii) 38,500 shares of common stock underlying stock options exercisable within 60 days of March 1, 2018. Includes only current directors and executive officers serving in such capacity on the date of the table. Does not include shares of common stock which may be deemed to be beneficially owned by Drs. Birner or Marengere which are included in footnote 2 above.

Securities Authorized for Issuance Under Equity Compensation Plans

The following table sets forth information, as of December 31, 2017, with respect to our compensation plans under which common stock is authorized for issuance. These plans consist of our 2010 Stock Incentive Plan and 2013 Stock Incentive Plan. We believe that the exercise price for all of the options granted under these plans reflect at least 100% of fair market value on the dates of grant for the options at issue.

Equity Compensation Plan Information

Plan Category	Number of Securities to be Issued Upon Exercise of Outstanding Options (A)	Weighted Average Exercise Price of Outstanding Options (B)	Number of Securities Remaining Available for Future Issuance Under Equity Compensation Plans (Excluding Securities Reflected in Column (A))(C)
Equity Compensation Plans Approved by Stockholders	463,600	$ 11.23	159,572
Equity Compensation Plans Not Approved by Stockholders	—	—	—
Total	463,600	$ 11.23	159,572

Item 13. Certain Relationships and Related Transactions, and Director Independence.

Transactions with Related Persons

Since January 1, 2017, we have engaged in no reportable transactions with our directors, executive officers, beneficial holders of more than 5% of our voting securities, and affiliates or their immediately family members.

Director Independence

The Board determined that Ms. Griffin, Drs. Birner and Marengere, and Messrs. Amello, Aselage and Dunn are each an independent director within the meaning of Nasdaq listing standards, which directors constitute a majority of the Board. The Board has determined that each member of the Board's Audit, Compensation and Nominating and Corporate Governance Committees is independent (or similarly designated) based on the Board's application of the standards of Nasdaq, the rules and regulations promulgated by the SEC or the Internal Revenue Service, as appropriate for such committee membership. The current members of these committees are as follows:

Director	Independent	Audit Committee	Compensation Committee	Nominating and Corporate Governance Committee
Jason Amello	X	X		
Stephen J. Aselage	X		X	X
Hubert Birner, Ph.D., MBA	X			
John M. Dunn	X	X		X
Michelle Griffin	X	X	X	
Luc Marengere, Ph.D.	X		X	X
Chris Schelling				

Item 14. Principal Accountant Fees and Services.

The following table presents (i) the aggregate fees billed to us for the fiscal years ended December 31, 2017 and 2016 by MaloneBailey, LLP, who served as our independent registered public accounting firm during the period of January 1, 2016 to September 20, 2017, and (ii) the aggregate fees billed to us for the fiscal years ended December 31, 2017 and 2016 by Wolf & Company, P.C., who was appointed as our independent registered public accounting firm on September 20, 2017.

	Years Ended December 31,			
	2017		2016	
	MaloneBailey	Wolf & Co.	MaloneBailey	Wolf & Co.
Audit fees(1)	$ 62,000	$ 79,000	$ 80,046	$ —
Audit-related fees (2)	16,000	42,000	5,500	—
Tax fees (3)	—	—	—	—
All other fees	—	—	—	—
Total fees	$ 78,000	$ 121,000	$ 85,546	$ —

(1) Audit fees consist of fees billed for services relating to the audit of our annual financial statement and review of our quarterly financial statements, and services that are normally provided in connection with statutory and regulatory filings or engagements.

(2) Audit-related fees consist of fees billed for providing consents for SEC registration statements such as Forms S-1, S-3, S-4 and S-8, other periodic reports and other documents filed with the SEC, or other documents issued in connection with securities offerings.

(3) Tax fees are for services relating to tax compliance, tax advice and tax planning.

Change in Independent Registered Public Accounting Firm

On September 20, 2017, we engaged Wolf & Company, P.C., as our independent registered public accounting firm to audit our financial statements for the fiscal year ended December 31, 2017, and we dismissed MaloneBailey, LLP. Prior to the completion of the merger, Wolf & Company, P.C. served as the auditor and independent registered public accounting firm to Private Acer. The decision to change accountants was approved by the Audit Committee of our Board of Directors.

The report of MaloneBailey, LLP on our consolidated financial statements for the year ended December 31, 2016 did not contain an adverse opinion or disclaimer of opinion, nor was it qualified or modified as to uncertainty, audit scope, or accounting principles, except that our financial statements for the fiscal year ended December 31, 2016 expressed, in an explanatory paragraph, substantial doubt about our ability to continue as a going concern due to recurring losses, negative operating cash flows and an accumulated deficit.

During the years ended December 31, 2017 and 2016, there were no: (1) disagreements (as defined in Item 304(a)(1)(iv) of Regulation S-K and the related instructions) with MaloneBailey, LLP on any matter of accounting principles or practices, financial statement disclosure, or auditing scope or procedures, which disagreement if not resolved to the satisfaction of MaloneBailey, LLP would have caused MaloneBailey, LLP to make reference thereto in its reports, or (2) reportable events (as described in Item 304(a)(1)(v) of Regulation S-K).

During the years ended December 31, 2017 and 2016, neither we nor anyone on our behalf consulted with Wolf & Company, P.C. regarding either (i) the application of accounting principles to a specific transaction, completed or proposed, or the type of audit opinion that might be rendered on our financial statements, and neither a written report nor oral advice was provided to us that Wolf & Company, P.C. concluded was an important factor considered by us in reaching a decision as to any accounting, auditing or financial reporting issue or (ii) any matter that was either the subject of a disagreement (as defined in Item 304(a)(1)(iv) of Regulation S-K and the related instructions) or a reportable event (as described in Item 304(a)(1)(v) of Regulation S-K).

Policy on Audit Committee Pre-Approval and Permissible Non-Audit Services of Independent Auditors

The Board's policy is to pre-approve all audit and permissible non-audit services provided by the independent auditors. These services may include audit services, audit-related services, tax services and other services. Pre-approval is generally provided for up to one year and any pre-approval is detailed as to the particular service or category of services and is generally subject to a specific budget. The independent auditors and management are required to periodically report to the Board regarding the extent of services provided by the independent auditors in accordance with this pre-approval, and the fees for the services performed to date. The Board may also pre-approve particular services on a case-by-case basis. The Audit Committee pre-approved 100% of any audit-related services, tax services or other services provided by our independent auditors during the last two fiscal years.

PART IV

Item 15. Exhibits and Financial Statement Schedules.

(a) Documents filed as part of this report:

1. Financial Statements are filed as part of this Annual Report on Form 10-K. The following consolidated financial statements are included in Item 8:

2. Financial Statement Schedules

The required information is included in the financial statements or notes thereto.

3. List of Exhibits:

Exhibit No.	Description
2.1#	Agreement and Plan of Merger and Reorganization, dated as of June 30, 2017, by and among Acer Therapeutics Inc. (formerly Opexa Therapeutics, Inc.), Opexa Merger Sub, Inc. and Acer Therapeutics Inc. (incorporated by reference to Exhibit 2.1 to the Company's Current Report on Form 8-K filed on July 3, 2017).
3.1	Restated Certificate of Formation of Acer Therapeutics Inc. (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K filed on July 26, 2012, File No. 001-33004).
3.2	Certificate of Designation of Preferences, Rights and Limitations of Series A Convertible Preferred Stock of Acer Therapeutics Inc. (incorporated by reference to Exhibit 4.2 to the Company's Current Report on Form 8-K filed on July 26, 2012, File No. 001-33004).
3.3	Certificate of Amendment of the Restated Certificate of Formation of Acer Therapeutics Inc. (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K filed on December 14, 2012, File No. 001-33004).
3.4	Certificate of Amendment to the Restated Certificate of Formation of Acer Therapeutics Inc. (incorporated by reference to Exhibit 3.1 to the Company's Quarterly Report on Form 10-Q filed on November 10, 2015).
3.5	Certificate of Amendment to the Restated Certificate of Formation of Acer Therapeutics Inc. (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K filed on September 28, 2015).
3.6	Certificate of Amendment to the Restated Certificate of Formation of Acer Therapeutics Inc. (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K filed on September 20, 2017).
3.7	Certificate of Amendment to the Restated Certificate of Formation of Acer Therapeutics Inc. (incorporated by reference to Exhibit 3.2 to the Company's Current Report on Form 8-K filed on September 20, 2017).
3.8	Amended and Restated By-laws, as amended (incorporated by reference to Exhibit 3.3 to the Company's Annual Report on Form 10-K filed on March 8, 2011, File No. 001-33004).
4.1	Form of Common Stock Certificate (incorporated by reference to Exhibit 4.1 to the Company's Quarterly Report on Form 10-Q filed on November 13, 2017).
4.2	Form of Series J Warrant issued on January 23, 2013 (incorporated by reference to Exhibit 4.1 to the Company's Current Report on Form 8-K filed on January 23, 2013, File No. 001-33004).
4.3	Form of Series K Warrant issued on January 30, 2013 (incorporated by reference to Exhibit 4.1 to the Company's Current Report on Form 8-K filed on January 30, 2013, File No. 001-33004).
4.4	Form of Series M Warrant issued on April 9, 2015 (incorporated by reference to Exhibit 4.11 to the Company's Registration Statement on Form S-1, as amended (File No. 333-201731), originally filed on January 28, 2015).
4.5	Warrant Agreement, dated February 25, 2015, by and between Acer Therapeutics Inc. and Continental Stock Transfer & Trust Company (incorporated by reference to Exhibit 4.2 to the Company's Quarterly Report on Form 10-Q filed on May 12, 2015).
4.6	Amended and Restated Series N Warrants issued on March 14, 2016 (incorporated by reference to Exhibit 4.13 to the Company's Annual Report on Form 10-K filed on March 15, 2016).
10.1+^	Acer Therapeutics Inc. Amended and Restated 2010 Stock Incentive Plan (incorporated by reference to Appendix A to the Company's Definitive Proxy Statement on Schedule 14A filed on April 11, 2016).

Exhibit No.	Description
10.2+^	Amendment No. 1 to the Acer Therapeutics Inc. Amended and Restated 2010 Stock Incentive Plan (incorporated by reference to Exhibit 10.35 to the Company's Registration Statement on Form S-4, as amended, (File No. 333-219358) filed on July 19, 2017).
10.3+^	Form of award agreement for awards to be made under the Acer Therapeutics Inc. Amended and Restated 2010 Stock Incentive Plan (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed August 14, 2014).
10.4+^	Form of restricted stock agreement for awards to be made under the Acer Therapeutics Inc. Amended and Restated 2010 Stock Incentive Plan (incorporated by reference to Exhibit 10.3 to the Company's Quarterly Report on Form 10-Q filed on May 12, 2015).
10.5+	Acer Therapeutics Inc. 2013 Stock Incentive Plan, as amended (incorporated by reference to Exhibit 10.3 to the Company's Current Report on Form 8-K filed on September 20, 2017).
10.6+	Employment Agreement dated June 16, 2008 by and between Acer Therapeutics Inc. and Neil K. Warma (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K filed on June 18, 2008, File No. 001-33004).
10.7	License Agreement dated September 5, 2001 by and between Acer Therapeutics Inc. (as successor) and Baylor College of Medicine (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed August 11, 2016).
10.8	License Agreement, dated January 13, 2006, by and between Acer Therapeutics Inc. and Shanghai Institute for Biological Services (incorporated by reference to Exhibit 10.23 to the Company's Registration Statement on Form SB-2 (Amendment No. 1) filed February 9, 2006, File No. 333-126687).
10.9	Stock Purchase Agreement, dated September 1, 2015, by and between Acer Therapeutics Inc. and the purchasers party thereto (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K filed on September 1, 2015).
10.10	Amendment to Stock Purchase Agreement, dated March 14, 2016, by and between Acer Therapeutics Inc. and the purchasers party thereto (incorporated by reference to Exhibit 10.21 to the Company's Annual Report on Form 10-K filed on March 15, 2016).
10.11	Sales Agreement, dated March 25, 2016, by and between Acer Therapeutics Inc. and IFS Securities, Inc. (doing business as Brinson Patrick, a division of IFS Securities, Inc.) (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K filed on March 25, 2016).
10.12♦	Agreement of Access and Use of Clinical Trial Data, dated as of August 3, 2016, by and between Acer Therapeutics Inc. and L'Assistance Publique—Hôpitaux de Paris (incorporated by reference to Exhibit 10.16 to the Company's Registration Statement on Form S-4, as amended (File No. 333-219358), filed on July 19, 2017).
10.13♦	Exclusive License Agreement, dated as of April 4, 2014, by and between Acer Therapeutics Inc. and Baylor College of Medicine (incorporated by reference to Exhibit 10.17 to the Company's Registration Statement on Form S-4, as amended (File No. 333-219358), filed on July 19, 2017).
10.14	First Amendment to License Agreement dated April 28, 2014 by and between Baylor College of Medicine and Acer Therapeutics Inc. (incorporated by reference to Exhibit 10.18 to the Company's Registration Statement on Form S-4, as amended (File No. 333-219358), filed on July 19, 2017).
10.15	Second Amendment to License Agreement, dated March 17, 2015, by and between Acer Therapeutics Inc. and Baylor College of Medicine (incorporated by reference to Exhibit 10.19 to the Company's Registration Statement on Form S-4, as amended (File No. 333-219358), filed on July 19, 2017).

Exhibit No.	Description
10.16	Third Amendment to License Agreement, dated September 8, 2016, by and between Acer Therapeutics Inc. and Baylor College of Medicine (incorporated by reference to Exhibit 10.20 to the Company's Registration Statement on Form S-4, as amended (File No. 333-219358), filed on July 19, 2017).
10.17	Business Advisory Agreement, dated as of September 1, 2015, by and between Acer Therapeutics Inc. and Extera Partners LLC (as assignee of EPLS LLC) (incorporated by reference to Exhibit 10.25 to the Company's Registration Statement on Form S-4, as amended (File No. 333-219358), filed on July 19, 2017).
10.18	Amendment to Business Advisory Agreement, dated as of August 24, 2016, by and between Acer Therapeutics Inc. and Extera Partners LLC (incorporated by reference to Exhibit 10.26 to the Company's Registration Statement on Form S-4, as amended (File No. 333-219358), filed on July 19, 2017).
10.19*	Sublease Agreement, dated as of October 16, 2017, by and between Acer Therapeutics Inc. and Bradley A. MacDonald.
21.1*	List of Subsidiaries.
23.1*	Consent of Independent Registered Public Accounting Firm Wolf & Company, P.C.
31.1*	Certification of Chief Executive Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2*	Certification of Chief Financial Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1*	Certification of Chief Executive Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2*	Certification of Chief Financial Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101*	Financial statements from the Annual Report on Form 10-K of the Company as of and for the period ended December 31, 2017, formatted in Extensible Business Reporting Language (XBRL): (i) Consolidated Balance Sheets; (ii) Consolidated Statements of Operations; (iii) Consolidated Statements of Changes in Redeemable Preferred Stock and Stockholders' Equity (Deficit); (iv) Consolidated Statements of Cash Flows; and (v) Notes to Consolidated Financial Statements.

* Filed herewith

\+ Management contract or compensatory plan or arrangement.

\# The schedules and exhibits to this exhibit have been omitted pursuant to Item 601(b)(2) of Regulation S-K. A copy of any omitted schedule and/or exhibit will be furnished to the SEC upon request.

♦ Confidential treatment was granted with respect to certain portions of this exhibit. Omitted portions have been filed separately with the Securities and Exchange Commission.

^ Note that the name of this plan has been amended to reflect the current name of the Company.

Item 16. Form 10-K Summary

Not Applicable.

SIGNATURES

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

ACER THERAPEUTICS INC.

Date: March 7, 2018

By: /s/ Harry Palmin
Harry Palmin
Chief Financial Officer

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

Signature	Title	Date
/s/ Chris Schelling Chris Schelling	President and Chief Executive Officer and Director *(Principal Executive Officer)*	March 7, 2018
/s/ Harry Palmin Harry Palmin	Chief Financial Officer *(Principal Financial and Accounting Officer)*	March 7, 2018
/s/ Jason Amello Jason Amello	Director	March 7, 2018
/s/ Stephen J. Aselage Stephen J. Aselage	Chairman of the Board	March 7, 2018
/s/ Hubert Birner Hubert Birner, Ph.D., MBA	Director	March 7, 2018
/s/ John M. Dunn John M. Dunn	Director	March 7, 2018
/s/ Michelle Griffin Michelle Griffin	Director	March 7, 2018
/s/ Luc Marengere Luc Marengere, Ph.D.	Director	March 7, 2018

SUBLEASE AGREEMENT made as of the 16th day of October, 2017, by and between

Bradley A. MacDonald ("Sublessor") and Acer Therapeutics Inc. ("Sublessee").

WHEREAS, Sublessor has leased from Commonwealth Development LLC as Trustee of The Gateway Realty Trust ("Major Lessor") Premises 351 (the Premises") of One Gateway Center in Newton, Massachusetts, under an indenture of lease dated July 14, 1993 (as has been amended most recently by Amendment Number Four) with a term ending on September 30, 2018, (hereinafter collectively referred to as the "the Major Lease"), copies of said lease and of the Amendment Number Four being attached hereto as Exhibit "A" and made a part hereof; and

WHEREAS, Sublessee is desirous of subleasing said demised Premises 351 under the Major Lease from and after December 1, 2017 through the balance of said term at the rate of $26.00 per square foot, and otherwise on the same monetary and non-monetary terms and conditions as the Major Lease; and

WHEREAS, Sublessor is willing to sublet said area on that basis;

NOW, THEREFORE, for good and valuable consideration, the receipt and sufficiency whereof is hereby acknowledged, the parties agree as follows:

1. Sublessor hereby sublets to Sublessee the entirety of Premises 351 of One Gateway Center beginning on December 1, 2017 (the "Start Date") and continuing thereafter until September 30, 2018 (the "End Date").

2. Sublessee shall pay to Sublessor as and for the total rent and/or other fees due hereunder (collectively referred to as "Rent," the amounts as follows:

A. $5,980 per month, payable in advance, the first installment to be paid on the execution of this Sublease and regular monthly installments in the amount of $5,980 per month to be paid on the 1st day of each month thereafter in respect of each month of the subleasing prior to the End Date.
B. Sublessee further agrees to pay in advance to Sublessor on the 1st day of each month such Operating Expense as is required pursuant to the Major Lease, which for the Major Lessor's FY 2017 is $844 per month. If such amount is adjusted at the end of the Major Lessor's fiscal year, any credit given by the Major Lessor shall be pro-rated according to the portion of the year in which this agreement is effective. Sublessee likewise agrees to pay to Sublessor, on the 1st day of each month, the monthly Operating Expense assessment for the Major Lessor's FY 2018. Any credits given by Major Lessor at the conclusion of the Major Lease for its FY 2018 Operating Expenses shall be paid over to the Sublessee.
C. Sublessee further agrees to pay Sublessor for all Real Estate Tax Assessment charges imposed pursuant to the Major Lease during the term of this sublease. Such charges are

payable quarterly and shall be pro-rated for the quarter in which this agreement becomes effective. The assessment to the Sublessor for the City of Newton Second Quarter of FY 2018 (October, November, and December) was $564. Accordingly, Sublessee shall pay $188 to Sublessor on the Start Date of this agreement.

D. It is understood that upon the execution of this Sublease, Sublessee shall have deposited with Sublessor the sum of $13,648 as a Security Deposit as security for the faithful performance and observance by Sublessee of the terms, conditions, provisions and covenants of this Sublease, it being further understood however, that said deposit is not to be considered prepaid rent. In the event Sublessee defaults in respect to any of the terms, conditions, provisions and covenants of this Sublease, including, but not limited to the payment of Rent, Sublessor may use, apply or retain the whole or any part of the Security Deposit to the extent required for the payment of any Rent or any other sum as to which Sublessee is in default or for any sum which Sublessor may expend or may be required to expend by reason of Subessee's default with respect to this Sublease, including but not limited to any amount for which the Sublessee is liable under the article contained herein entitled "DEFAULT" provided, however, that such Security Deposit shall in no way be construed as liquidated damages for any default or breach of any term, condition, provision and covenant of this Sublease, nor shall Sublessor be required, because of said deposit, to waive its right under the article contained herein entitled "DEFAULT" to terminate this Sublease in the event of default. If, on the End Date the Sublessee shall not have been in default under the Sublease at any time and Sublessee shall have fully and faithfully complied with all of the terms, conditions, provisions and covenants of this Sublease, including, without limitation, payment of Rent when due, the Security Deposit shall be returned without interest.

3. With respect to the Premises, except for the provisions of paragraph 5 below and with respect only to the period commencing on the Start Date and ending on the End Date, all terms, covenants and conditions of the Major Lease are made a part hereof, Sublessor herein being considered as if Lessor and Sublessee herein being considered as if Lessee, and subject to paragraph 5 below, this Sublease shall operate as though it were an assignment pro tanto.

4. Sublessee hereby accepts the Premises "as is" and in their present condition, except that Sublessor shall remove all furniture and furnishings (not including such items as are built in) prior to the Start Date. Sublessor is not providing telephone or cable services for the premises. Sublessee assumes herewith all maintenance and repair obligations imposed by the Major Lease regarding the Premises, and Sublessee shall be responsible for the same, including any removal of the furniture and/or décor or any alterations existing in the Premises, to the extent required under the Major Lease at the End Date. Sublessee agrees to reimburse Sublessor for any and all costs that may be billed or invoiced to Sublessor for work or services performed in the Premises from and after the Start Date.

5. Notwithstanding the foregoing, it is agreed that the Premises shall be used by Sublessee for the purposes of professional office work functions and related activities, and for no other purposes, all as defined in the Major Lease.

6. Major Lessor has agreed to provide certain services and to perform other obligations under the Major Lease and Sublessee is entitled to receive all of the same hereunder with respect to the Premises during the term of this Sublease. Upon reasonable notice from Sublessee of the failure of Major Lessor to perform any such obligation or provide any such service, Sublessor will promptly undertake to enforce its rights under the Major Lease; provided, however, that the method and manner of seeking enforcement thereof shall be solely within the commercially reasonable judgment and determination of Sublessor. Notwithstanding anything herein to the contrary, unless Sublessor shall fail to comply with the foregoing obligation, Sublessor shall not be liable to Sublessee for money damages on account of the failure of Major Lessor to perform any such obligations or provide any such service, nor shall any such failure constitute a constructive eviction of Sublessee.

7. Sublessee shall not do or permit anything to be done which would cause the Major Lease to be terminated by Major Lessor or forfeited. To the extent permitted by law, Sublessee hereby agrees to indemnify and hold Sublessor harmless from and against all direct out-of-pocket damages of any kind which Sublessor may suffer by reason of any breach or default hereunder by Sublessee, including termination or forfeiture of the Major Lease, and from and against all other liabilities, claims and damages arising during the term in the Premises or out of or in connection with the use and occupancy of the Premises by Sublessee during the term in the Premises, except to the extent that (a) the same shall be due in whole or in part to the negligence or willful misconduct of Sublessor, or any party under the control of Sublessor, or (b) Sublessor is indemnified by its insurance carriers or by Major Lessor for such liabilities, claims or damages.

8. Sublessee shall not sublet the Premises, in whole or in part, nor assign the Sublease nor permit any interest of Sublessee in this Sublease to become vested in any third party.

9. Sublessor shall pay Cushman and Wakefield its broker fee of $2,760 and an agreed fee to Boston Realty Advisors.

10. Any notice or demand from Sublessor to Sublessee or from Sublessee to Sublessor shall be deemed duly served if mailed by certified mail, return receipt requested, or via nationally recognized overnight courier, addressed,

A. if to Sublessee, at One Gateway Center, Suite 351, Newton, Massachusetts 02458 or
B. if to Sublessor, at 248 Summit Avenue, Brookline, Massachusetts 02446, or such place as Sublessor may designate in writing in the future,

and the customary certified mail receipt or evidence of delivery via overnight courier shall be conclusive evidence of such service.

11. Sublessee shall be responsible for appropriate signage, at Sublessee's expense, for the Sublessee's business purposes, which signage shall be done pursuant to the Major Lessor's policies and procedures. Sublessor agrees to assist as needed in communications with Major Lessor

regarding signage. Any bills or invoices related to signage work that may be addressed to Sublessor will be reimbursed by Sublessee.

12. Sublessee shall be responsible for all risks to its personal property and for carrying customary liability and property insurance.

13. The provisions of the Lease notwithstanding, and in addition thereto, Sublessee agrees to the extent permitted by law, that Sublessee shall make no claim against Sublessor for any injury or damage to Sublessee or to any other person(s) or for any damage to, or loss (by theft or otherwise) of any property of Sublessee or of any other person unless the same shall be due in whole or in part to the negligence or willful misconduct of Sublessor, or any of his agents, contractors, servants, employees, licensees, invitees or any other party under the control of Sublessor (a "Sublessor Control Party"), or a breach by Sublessor of the provisions of this sublease. Sublessee further agrees, to the extent permitted by law, and except to the extent the same shall be due in whole or in part to the negligence or willful misconduct of Sublessor or any Sublessor Control Party, or a breach by Sublessor of the provisions of this sublease, to indemnify and save harmless Sublessor against and from any and all claims by or on behalf of any person(s), firm(s) or corporation(s) arising from the conduct or management of or from any work or thing whatsoever done (other than by Sublessor or its agents or employees) in and on the Premises during the term of this Sublease, and to indemnify and save harmless Sublessor against and from any and all claims arising from any condition of the Premises due to or arising from any act done by Sublessee in breach of the provisions of this sublease or negligence of Sublessee or any of its agents, contractors, servants, employees, licensees or invitees (while in the Premises), and from and against all costs, expenses and liabilities incurred in or in connection with any such claim or claims or action or proceeding brought thereon; and, in case any action or proceeding be brought against Sublessor by reason of any such claim, Sublessee upon notice from Sublessor agrees, to the extent permitted by law, to resist or defend such action or proceeding and to employ counsel therefor reasonably satisfactory to Sublessor.

14. Sublessor agrees to provide to Sublessee all six parking garage passes/building access cards which are part of the Master Lease for the term of the Sublease.

15. All keys to the Premises will be turned over to the Sublessee with the exception of one key which shall be retained by Sublessor, who agrees to provide reasonable notice by telephone or email of any intent to enter the Premises.

16. Default: Sublessor and Sublessee agree to incorporate herein by reference the terms and conditions related to Default, Article 15.0 including all of its subparts, as set forth in the Major Lease, substituting throughout "Sublessor" for "Lessor," and "Sublessee" for "Lessee."

17. Major Lessor must signify its acceptance of this sublease, as required by Article 10 of the Major Lease, prior to this Sublease becoming effective.

WITNESS the execution in duplicate under seal the day and year first above written.

Sublessor:

/s/ Bradley A. MacDonald
Bradley A. MacDonald

Sublessee:

Acer Therapeutics Inc.

By: /s/ Chris Schelling
Chris Schelling
CEO & Founder
10/23/2017

Approved:
Commonwealth Development LLC as Trustee of Gateway Realty Trust

By: _____
Authorized Agent

CONSENT TO SUBLEASE

THIS CONSENT TO SUBLEASE (this "Consent") is dated as of October 25, 2017, is made with reference to that certain sublease (the "Sublease") dated as of October 16, 2017 by and between Bradley A. MacDonald, individually ("Tenant") with an address at 248 Summit Avenue, Brookline, MA 02446, and Acer Therapeutics Inc ("Subtenant") with an address at One Gateway Center, Suite 351, Newton, MA 02458, and is entered into by and among Commonwealth Development LLC, trustee of Gateway Realty Trust ("Landlord"), Tenant and Subtenant, all with reference to the following facts:

A. Landlord and Tenant are the parties to that certain Lease dated as of July 14, 1993, as most recently amended by Amendment Number Four, as of January 21, 2012 (as amended, "Master Lease");

B. Tenant and Subtenant wish to enter into the Sublease;

C. The Master Lease provides, inter alia, that Tenant may not enter into any sublease without Landlord's prior written approval; and

D. Tenant and Subtenant have presented the fully executed Sublease (a true copy of which is attached hereto) to Landlord for Landlord's approval.

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

1. Landlord hereby consents to the subleasing of the premises which Subtenant is leasing from Tenant (the "Subleased Premises") pursuant to the Sublease (i) upon the terms and conditions set forth herein and (ii) subject to the terms of the Master Lease, including, without limitation, Landlord's right to the net excess rent as set forth in Article 10.0 of the Master Lease, "ASSIGNMENT, MORTGAGING, SUBLETTING, ETC.". Tenant and Subtenant hereby represent to Landlord that Tenant is not receiving any such net excess rent under the Sublease. Except for the foregoing consent by Landlord to the subleasing of the Subleased Premises pursuant to the Sublease, nothing in this Agreement shall operate as a consent by Landlord to or approval by Landlord of any of the particular provisions of the Sublease.

2. Tenant and Subtenant hereby acknowledge that in the event of a conflict between the terms of this Consent and the Master Lease or Sublease, this Consent shall control.

3. (a) In the event of Master Lease Termination (as hereinafter defined) prior to the termination of the Sublease, and subject to the provisions of Section 3(b) hereof, or in the event of Landlord's right to terminate Tenant's (and as a result Subtenant's) right of possession to the Subleased Premises and the interest of Tenant (and as a result Subtenant) therein, Landlord may, at its sole discretion, require Subtenant to assume and agree to perform Tenant's obligations under the Master Lease with respect to the Subleased Premises. Such assumption by Subtenant of each and every one of the obligations of Tenant under the Master Lease with respect to the Subleased Premises shall entitle the Subtenant to occupy the Subleased Premises leased pursuant to the Master Lease, but shall not relieve Tenant from any liability to Landlord under the Master Lease. In the event of such assumption, Subtenant agrees to execute and deliver at any time and from time to time, upon request of Landlord, any instruments which may be necessary or appropriate to evidence such assumption and Subtenant hereby irrevocably appoints Landlord as its attorney-in-fact, coupled with an interest, to execute on behalf of Subtenant any documents or instruments necessary to evidence such assumption.

   In the event of such assumption, Landlord shall not (i) be liable to Subtenant for any act, omission or breach of the Sublease by Tenant, (ii) be subject to any offsets or defenses which Subtenant might have against Tenant, (iii) be bound by any rent or additional rent which Subtenant might have paid in advance to Tenant,
   (iv) be bound to honor any rights of Subtenant in any security deposit made with Tenant by Subtenant except to the extent Tenant has specifically assigned and turned over such security deposits to Landlord, or (v) be bound by any provision of the Sublease.

   Tenant hereby agrees that in the event of Master Lease Termination, and subject to the provisions of Section 3(b) hereof, at Landlord's request, Tenant shall immediately pay or transfer to Landlord any security deposits, rent or other sums then held by Tenant in connection with the subleasing of the Subleased Premises. Such security deposit may be applied by Landlord pursuant to the terms of the Master Lease in the event of any

holding over or other default by the Subtenant after a Master Lease Termination. Subtenant hereby agrees that under no circumstances whatsoever shall Landlord be held in any way responsible or accountable for any security deposit or any sums paid by Subtenant to Tenant unless and until and to the extent that Landlord has actually received such sums from Tenant and has acknowledged their source, and Subtenant shall have no claim to any security or other deposit made by Tenant under the Master Lease.

(b) "Master Lease Termination" means any event, which by voluntary or involuntary act or by operation of law, causes or permits the Master Lease to be terminated, expire, or be canceled, including, but not limited to, (1) a default by Tenant under the Master Lease or any of the terms and provisions hereof; (2) foreclosure proceedings brought by the holder of any mortgage or trust deed to which the Master Lease is subject; (3) the termination of Tenant's leasehold estate by dispossession proceeding or otherwise; and (4) termination of the Master Lease in accordance with its terms.

4. Neither the Master Lease, the Sublease nor this Consent shall be deemed, nor are such documents intended, to grant to Subtenant any rights whatsoever against Landlord. Subtenant hereby acknowledges and agrees that its sole remedy for any alleged or actual breach of its rights in connection with the Sublease shall be solely against Tenant. Subtenant acknowledges and agrees that it is not a third party beneficiary under the Master Lease and is not entitled to assert any of Tenant's rights thereunder against Landlord, whether in its own right or on behalf of Tenant.

5. Neither this Consent nor the Sublease shall release Tenant from any existing or future duty, obligation or liability to Landlord pursuant to the Master Lease, nor shall this Consent or the Sublease change, modify or amend the Master Lease in any manner, except insofar as it constitutes Landlord's consent to Subtenant’s subleasing of the Subleased Premises as set forth in this Consent. Notwithstanding the generality of the foregoing: (a) neither the Sublease nor the Consent shall absolve Tenant from any requirement set forth in the Master Lease that Tenant obtain Landlord's prior written approval as required in the Master Lease, including without limitation, for any alterations, for any additional subleases, assignments or other dispositions of its interest in the Master Lease or the Premises (as defined in the Master Lease), or for any further sublease, assignment or other disposition of the Sublease, (b) Landlord shall be entitled to accept performance by either Tenant or Subtenant, and (c) any alterations, decorations, installations, removals, additions or improvements (including without limitation, the drilling of holes in the Subleased Premises or elsewhere in the building of which the Subleased Premises is a part (the "Building")) in or to the Subleased Premises or the Building shall be subject to the requirements of the Master Lease, including without limitation, the necessity of obtaining Landlord’s prior written consent thereto.

6. Subtenant and Tenant shall provide Landlord with a simultaneous copy of any default notice issued under the Sublease. In addition to Landlord's rights under Section 3 hereof, in the event Tenant is in default under any of the terms and provisions of the Master Lease, Landlord may elect to receive directly from Subtenant all sums due or payable to Tenant by Subtenant pursuant to the Sublease, and upon receipt of Landlord's notice, Subtenant shall thereafter pay to Landlord any and all sums becoming due or payable under the Sublease and Tenant shall receive from Landlord a corresponding credit for such sums actually received by Landlord against any and all payments then owing from Tenant. Neither the service of such written notice nor the receipt of such direct payments shall cause Landlord to assume any of Tenant's duties, obligations and/or liabilities under the Sublease, nor shall such event impose upon Landlord the duty or obligation to honor the Sublease, nor subsequently to accept any purported attornment by Subtenant. Tenant grants Landlord a security interest in all such payments due to Tenant from Subtenant, which security interest Landlord may perfect by filing a UCC-1 Financing Statement. Landlord shall credit payments actually received pursuant to this conditional assignment to Tenant's obligations under the Master Lease.

7. Subtenant hereby acknowledges that it has read and has knowledge of all of the terms, provisions, rules and regulations of the Master Lease and agrees not to do or omit to do anything which would cause Tenant to be in breach of the Master Lease. Any such act or omission also shall constitute a breach of the Master Lease and this Consent shall entitle Landlord to recover any damage, loss, cost, or expense which it thereby suffers, from Tenant and/or Subtenant. Without limiting the foregoing, in the event of the failure of Subtenant to vacate the Subleased Premises upon the Master Lease Termination, and assuming Landlord has not elected to require Subtenant to assume and agree to perform Tenant's obligations under the Master Lease with respect to the Subleased Premises pursuant to Section 3(a) above, then Landlord shall be entitled to all of the rights and remedies against Subtenant that would be available to Landlord against Tenant holding over after a Master

Lease Termination, including without limitation the holdover rent provisions at the rate set forth in the Master Lease.

8. In the event of any litigation between the parties hereto with respect to the subject matter hereof, the unsuccessful party agrees to pay the successful party all reasonable costs, expenses and attorneys' fees incurred therein by the successful party, which amounts may be included as a part of a judgment rendered therein.

9. The parties acknowledge that the Sublease and this Consent constitute the entire agreement between Tenant and Subtenant with respect to the subject matter thereof insofar as Landlord may be concerned, and that no amendment, termination, modification or change therein will be binding upon Landlord unless Landlord shall have given its prior written consent thereto.

10. This Consent shall be binding upon and shall inure to the benefit of the parties' respective successors in interest and assigns, subject at all times, nevertheless, to all agreements and restrictions contained in the Master Lease, the Sublease, and herein, with respect to subleasing, assignment or other transfer and the foregoing shall not be deemed to limit or negate Landlord's rights to prohibit or condition its consent to a future dispossession of Tenant's or Subtenant's interests. The agreements contained herein constitute the entire understanding between parties with respect to the subject matter hereof, and supersede all prior agreements, written or oral, inconsistent herewith. Tenant and Subtenant warrant and agree that neither Landlord nor any of its agents or other representatives have made any representations concerning the Premises, the Subleased Premises, their condition, the Sublease or the Master Lease. This Consent may be executed in multiple counterparts, which when taken together shall constitute a complete document.

11. Notice required or desired to be given hereunder shall be effective by reputable delivery service, proof of delivery required, addressed to parties at the addresses set forth in this Consent (and if no addresses are so listed, then to the Landlord at the address set forth in the Master Lease for the payment of rent, or to Tenant or Subtenant at the address of the Premises or of the Subleased Premises, respectively). Any party may change its address for notice by giving notice in the manner hereinabove provided.

12. Tenant and Subtenant hereby agree, jointly and severally, to indemnify, hold harmless and defend Landlord from and against any loss, cost, expense, damage or liability, including, without limitation, reasonable attorneys' fees, incurred as a result of (a) any claim by any person or entity that it is entitled to a commission, finder's fee or like payment relating to or arising out of the Sublease, or (b) any claim by any person or entity in any way relating to or arising out of the Sublease, Subtenant’s use or occupancy of the Premises or any portion thereof, or any related agreements or dealings. Subtenant shall provide to Landlord, as if Subtenant were the Tenant, all of the insurance certificates required of Tenant under Article 9 of the Master Lease.

13. Upon execution hereof, Tenant shall reimburse Landlord, as additional rent, for Landlord’s reasonable legal, professional, administrative, managerial and all other expenses (which expenses may include, without limitation, hourly fees for administrative and management personnel and an allocation for overhead and profit) related to the Sublease and this Consent.

[SIGNATURE PAGE FOLLOWS]

EXECUTED under seal as of the date first written above.

TENANT:	**SUBTENANT:**
BRADLEY A. MACDONALD	**ACERTHERAPEUTICS INC.**
By:/s/ Bradley A. MacDonald Name: Bradley A. MacDonald	By: /s/ Chris Schelling Name: Chris Schelling

TLANDLORD:
GATEWAY REALTY TRUST

BY: COMMONWEALTH DEVELOPMENT LLC, as trustee and not individually

By /a/ James Magliozzi

Name: James A. Magliozzi
Title: Manager, duly authorized

Exhibit 21.1

SUBSIDIARIES OF THE REGISTRANT

Listed below are subsidiaries of Acer Therapeutics Inc. as of December 31, 2017.

Subsidiary Name	State/Jurisdiction of Incorporation
Acer Therapeutics Inc.	Delaware

EXHIBIT 23.1

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We consent to the incorporation by reference in Registration Statements on Form S-3 (File No. 333-208314) and Form S-8 (File No. 333-221566) of our report dated March 7, 2018 relating to the consolidated financial statements of Acer Therapeutics Inc. (which report expresses an unqualified opinion and includes an explanatory paragraph relating to the Company's ability to continue as a going concern), appearing in the Annual Report on Form 10-K of Acer Therapeutics Inc. for the year ended December 31, 2017.

We also consent to the references to us under the heading "Experts" in such Registration Statements.

/s/ Wolf & Company, P.C.
Wolf & Company, P.C.
Boston, Massachusetts
March 7, 2018

EXHIBIT 31.1

CERTIFICATION PURSUANT TO
SECTION 302 OF THE SARBANES-OXLEY ACT

I, Chris Schelling, certify that:

1. I have reviewed this Annual Report on Form 10-K of Acer Therapeutics Inc.;

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

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

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

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

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

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

   d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's fourth fiscal quarter that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

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

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

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

Date: March 7, 2018

By: /s/ Chris Schelling
Chris Schelling
President and Chief Executive Officer
(Principal Executive Officer)

EXHIBIT 31.2

CERTIFICATION PURSUANT TO
SECTION 302 OF THE SARBANES-OXLEY ACT

I, Harry Palmin, certify that:

1. I have reviewed this Annual Report on Form 10-K of Acer Therapeutics Inc.;

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

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

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

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

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

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

   d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's fourth fiscal quarter that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

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

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

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

Date: March 7, 2018

By: /s/ Harry Palmin
Harry Palmin
Chief Financial Officer
(Principal Financial and Accounting Officer)

EXHIBIT 32.1

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

In connection with the Annual Report of Acer Therapeutics Inc. (the "Company") on Form 10-K for the period ending December 31, 2017 (the "Report"), as filed with the Securities and Exchange Commission on the date hereof, I, Chris Schelling, President and Chief Executive Officer of the Company, certify, pursuant to 18 U.S.C. §1350, as adopted pursuant to §906 of the Sarbanes-Oxley Act of 2002, to the best of my knowledge, that:

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

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

Date: March 7, 2018

By: /s/ Chris Schelling

Chris Schelling
President and Chief Executive Officer
(Principal Executive Officer)

EXHIBIT 32.2

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

In connection with the Annual Report of Acer Therapeutics Inc. (the "Company") on Form 10-K for the period ending December 31, 2017 (the "Report"), as filed with the Securities and Exchange Commission on the date hereof, I, Harry Palmin, Chief Financial Officer of the Company, certify, pursuant to 18 U.S.C. §1350, as adopted pursuant to §906 of the Sarbanes-Oxley Act of 2002, to the best of my knowledge, that:

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

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

Date: March 7, 2018

By: /s/ Harry Palmin
Harry Palmin
Chief Financial Officer
(Principal Financial and Accounting Officer)