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, 2019

☐ **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)

Delaware	**32-0426967**
(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	Trading Symbol	Name of Each Exchange on Which Registered
Common Stock, $0.0001 par value per share	**ACER**	**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 every Interactive Data File required to be submitted 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 such files). Yes ☑ No ☐

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

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☑	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 28, 2019, based upon the closing price as of such date was $21,551,439.

As of March 1, 2020, 10,095,176 shares of the registrant's common stock, par value $0.0001 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), ACER-001, and osanetant, 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 regulatory submissions or actions;
- 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; 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 ("SEC").

PART I

Item 1. Business.

Overview

We are a pharmaceutical company focused on the acquisition, development, and commercialization of therapies for serious rare and life-threatening diseases with significant unmet medical needs. Our pipeline includes three clinical-stage candidates: EDSIVO™ (celiprolol) for the treatment of vascular Ehlers-Danlos syndrome ("vEDS") in patients with a confirmed type III collagen (COL3A1) mutation; ACER-001 (a taste-masked, immediate release formulation of sodium phenylbutyrate) for the treatment of various inborn errors of metabolism, including urea cycle disorders ("UCD") and Maple Syrup Urine Disease ("MSUD"); and osanetant for the treatment of induced Vasomotor Symptoms ("iVMS") where Hormone Replacement Therapy ("HRT") is likely contraindicated. Our product candidates are believed to present a comparatively de-risked profile, having one or more of a favorable safety profile, clinical proof-of-concept data, mechanistic differentiation, and/or accelerated paths for development through specific programs and procedures established by the United States ("U.S.") Food and Drug Administration ("FDA").

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

Program / Indication	Novel MOA / Unique Characteristics	Development Status
EDSIVO™ (celiprolol)		
vascular Ehlers-Danlos syndrome (COL3A1+)	Induces vascular dilatation and smooth muscle relaxation	TBD*
ACER-001 (taste-masked, immediate-release form of sodium phenylbutyrate)		
Urea Cycle Disorders	Taste-masked formulation; evaluating bioequivalence to BUPHENYL® **	Pre-NDA
Maple Syrup Urine Disease	Inhibition of BCKD kinase to increase BCAA metabolism	Phase 2
Osanetant		
Induced Vasomotor Symptoms (iVMS)	Neurokinin 3 Receptor Antagonist	Phase 1/2

* Complete Response Letter received June 2019; Formal Dispute Resolution Request submitted to the FDA December 2019; response received March 2020 denying appeal of the Complete Response Letter but describing possible paths forward for Acer to explore that could provide the substantial evidence of effectiveness needed to support a potential resubmission of the EDSIVOTM NDA

** Pivotal bioavailability and bioequivalence ("BE") trial

- A pivotal trial evaluating the bioavailability and bioequivalence ("BE") of ACER-001 to BUPHENYL® (sodium phenylbutyrate) was successfully completed in the first quarter of 2020
- An investigational new drug application ("IND") filing for osanetant is anticipated in the second half of 2020

- Initiation of a Phase 1/2 pharmacokinetics/pharmacodynamics/safety trial of osanetant is expected by the end of 2020, subject to our ability to generate sufficient capital resources to fund this program
- New Drug Application ("NDA") submission for ACER-001 in UCD is anticipated in early 2021, subject to our ability to generate sufficient capital resources to fund this program, and assuming successful completion of additional nonclinical work and 12-month stability data

Our Strategy

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

- focus on serious rare and life-threatening diseases with significant unmet needs
- 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
- 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, if approved for marketing by the FDA, would be a New Chemical Entity ("NCE") in the U.S. Celiprolol is currently approved in the European Union for the treatment of hypertension and angina. Ehlers-Danlos syndrome ("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 about 25% of patients before the age of 20 but increase to roughly 90% of patients by the age of 40. The median survival age of vEDS patients in the U.S. 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 U.S. from an analysis of a commercially available patient claims database, with data of approximately 190 million unique patient lives. Based on that information, we estimate the prevalence of phenotypically-defined vEDS in the U.S. could be greater than 1 in 45,000.

Current Treatment Options for vEDS

Currently, there are no approved pharmacologic therapies in the U.S. 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 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™ (celiprolol) Treatment in vEDS

In 2004, researchers at Assistance Publique—Hôpitaux de Paris, Hôpital Européen Georges Pompidou ("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. Results from the Ong trial were 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 ("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):

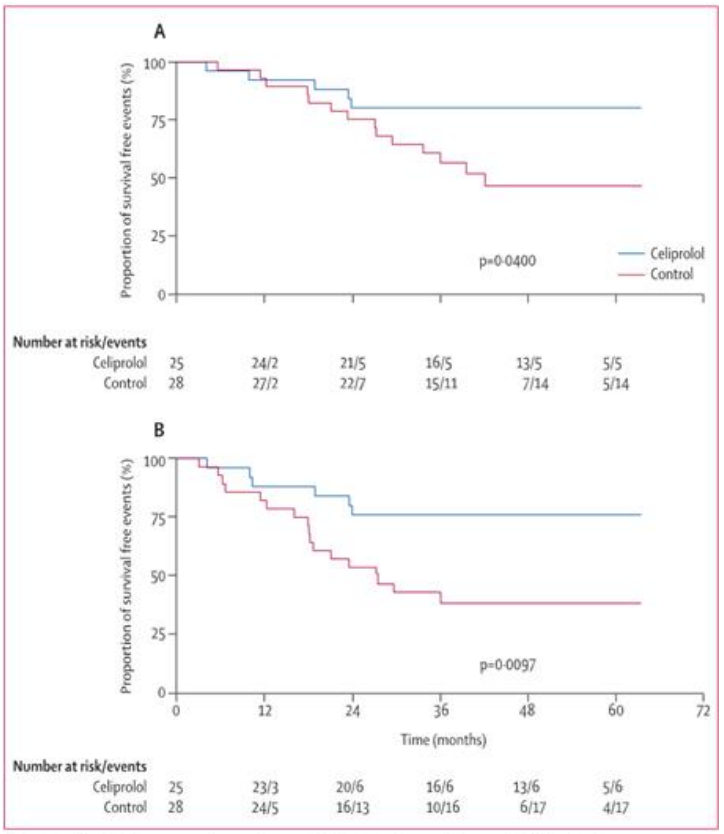


Figure 2: **Kaplan-Meier curves of event-free survival in 53 patients with vascular Ehlers-Danlos**
Primary endpoint (A). Primary and secondary endpoints (B).

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:

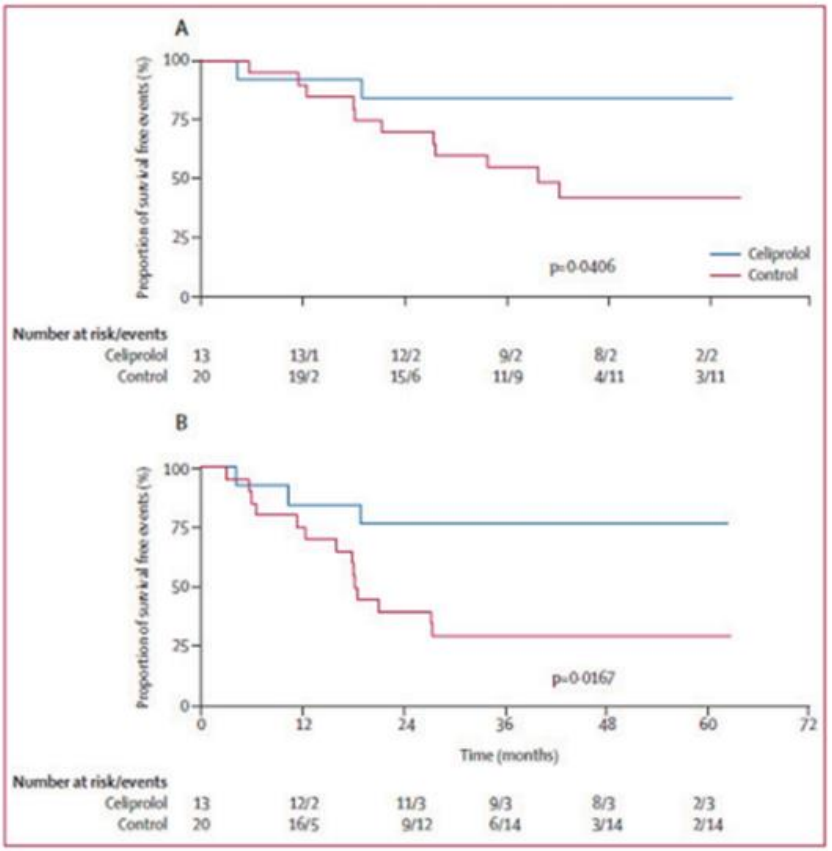


Figure 3: **Kaplan-Meier curves of event-free survival in 33 patients with positive COL3A1 mutation**

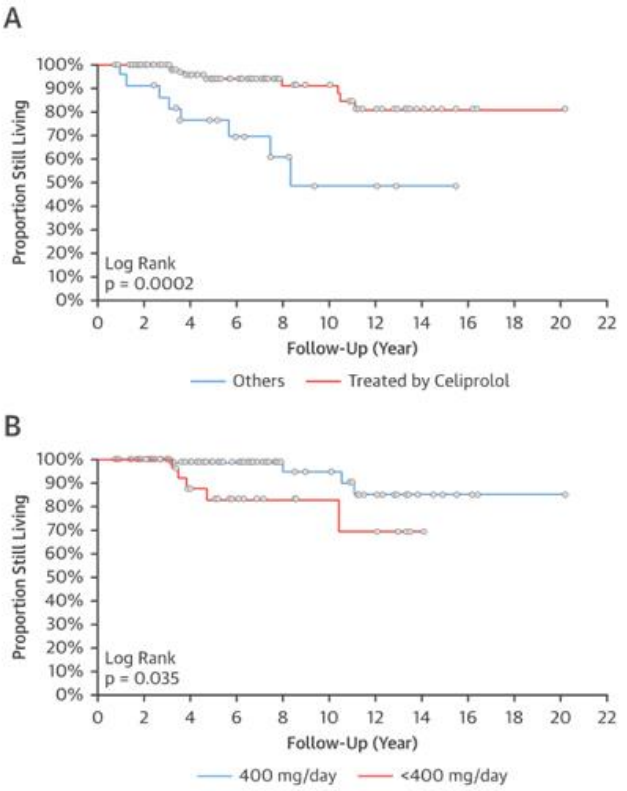
Primary endpoint (A). Primary and secondary endpoints (B).

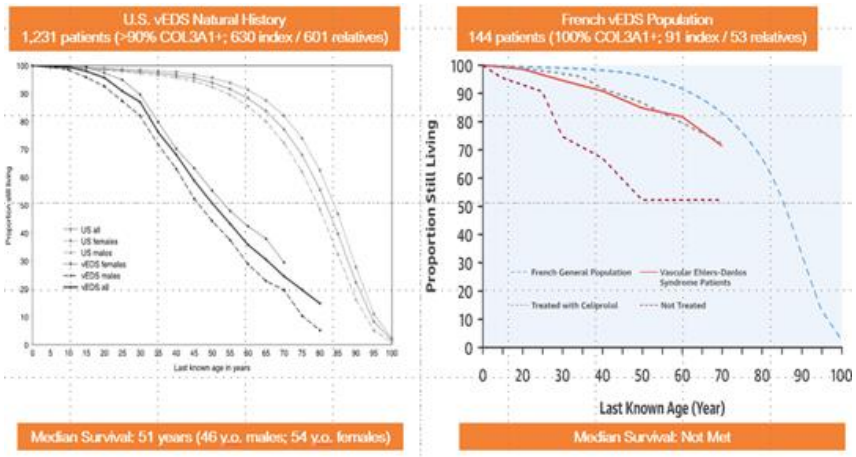
AP-HP granted us an exclusive right to access and use the data generated by the Ong trial. We have conducted a retrospective, source-verified analysis of that data, including the primary and secondary endpoints, which confirmed the published results of the Ong trial.

In addition to the Ong trial, in April 2019, long-term data from a cohort of COL3A1-positive vEDS patients was published in the Journal of the American College of Cardiology (JACC). The publication, entitled "Vascular Ehlers-Danlos Syndrome: Long-Term Observational Study," was authored by Michael Frank, MD, Xavier Jeunemaitre, MD, PhD, and Pierre Boutouyrie, MD, PhD, et al. This published study describes outcomes in 144 COL3A1-positive vEDS patients clinically monitored and treated at the French National Referral Center for Rare Vascular Diseases (Paris, France) between 2000 and 2017. Patients were followed for a median of 5.3 years, and up to 20 years. At the initial work up, 50% of patients were not treated regularly and only 33.3% were taking celiprolol; by the end of the study period, the majority (90.3%) were treated with celiprolol alone or in combination with other medications. Once the maximum tolerated dose of celiprolol was reached, 90 (62.5%) patients remained at this dose throughout their follow-up. Only five (3.5%) patients required dose reduction due to fatigue, and no serious drug-related adverse event was recorded.

Patients had a lower mortality rate than that expected from the natural history of the disease as described in previous U.S. reports[1]. Survival curve analysis showed that those not treated with celiprolol had a significantly worse outcome than celiprolol-treated patients: survival was 80.7% (95% CI 67.8%–93.6%) in those treated with celiprolol versus 48.5% (95% CI 19.7%–77.4%) in those not treated ($p<0.001$) after 11.1 years of follow-up. Survival was significantly higher in patients treated with a median dose of celiprolol of 400mg/day (n=83) vs. patients treated with a lower median dose of 217mg/d [100-300mg/day] (n=27), suggesting a dose effect and that 400mg/day should be considered the optimal dose. The authors also observed a relative decrease in hospitalization rates for acute arterial events during the time period in which the majority of patients were on celiprolol, suggesting a positive effect of celiprolol on the incidence and/or severity of new arterial events. The authors concluded that in this large, long-term cohort study, vEDS patients had a higher survival rate than expected relative to the known natural history of the disease and a lower annual occurrence of arterial complications, and that celiprolol use was potentially associated with these significant improvements in clinical outcomes.

FIGURE 3 Kaplan-Meier Survival Analysis of vEDS Patients in Groups I and II *COL3A1* Pathogenic Variants, According to Celiprolol Treatment





In May 2019, results were presented and published at the Society for Vascular Medicine (SVM) 2019 Annual Scientific Sessions from a pilot study designed to evaluate the effect of antihypertensive therapy on the rates of clinical events in patients with vEDS. The goal of this pilot study was to better understand the extent of use of antihypertensive medications in vEDS patients in the U.S., and their potential benefit in reducing the rate of vEDS-related clinical events. There are currently no approved medications to treat vEDS in the U.S.; however, antihypertensive medications are used by some physicians in vEDS patients with hopes of lowering the occurrence of clinical events.[2]

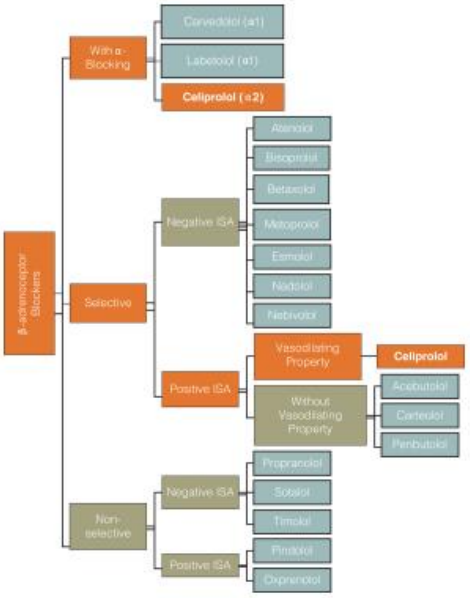
Researchers conducted a retrospective analysis of U.S. insurance claims (Truven MarketScan®) identifying vEDS patients over a four-year period from January 1, 2014 to December 31, 2017. The insurance claims-based information was then stratified based on insurance claims for antihypertensive medications and no antihypertensive medication. Researchers then calculated and compared the clinical event rate, including arterial rupture and aneurysm, and other hollow organ rupture, for each group. Of the 3,614 vEDS patients identified, 2,371 (65.6%) were determined not to be taking any antihypertensive medication and 1,243 (34.4%) were determined to be taking antihypertensive medications. There was no statistically significant difference between rate of clinical events in patients taking any of the antihypertensive medications compared to patients not taking an antihypertensive medication.

Table 4. Rate of Clinical Events in vEDS Patients on Antihypertensive Therapy

vEDS Patient Group	No. Patients (%)	Rate of Clinical Events	*P*-value (vs. No Antihypertensive)
No antihypertension therapy	2,371 (65.6%)	371 (15.6%)	-
Any antihypertension therapy	1,243 (34.4%)	205 (16.5%)	0.51
Beta blocker	895 (24.8%)	146 (16.3%)	0.64
ACE inhibitor	231 (6.4%)	38 (16.5%)	0.75
ARB	228 (6.3%)	55 (24.1%)	0.999
Calcium channel blocker	254 (7.0%)	33 (13.0%)	0.27

Celiprolol is a selective adrenergic modulator, acting as a cardioselective beta1 adrenoceptor antagonist with partial beta2 adrenoceptor agonist activity, beta3 agonist activity, and alpha2 antagonist activity and with intrinsic sympathomimetic and vasodilating properties.

The potential benefit of celiprolol in vEDS is thought to be mediated through a combination of agonist activity at beta2 and beta3 adrenergic receptors, and antagonist activity at alpha2 adrenergic receptors. While the exact mechanism is not fully understood, it has been proposed that it could exert its effects through vascular smooth muscle relaxation and dilatation, thereby decreasing the mechanical stress on collagen fibers in the arterial wall. Celiprolol has also been shown to increase the expression of endothelial nitric oxide synthase ("eNOS") messenger ribonucleic acid ("mRNA") and protein, and to activate phosphorylation of eNOS through the phosphatidylinositol 3-kinase ("PI3K")-Akt signaling pathway. eNOS is known to catalyze the synthesis of nitric oxide ("NO") in blood vessels, and NO plays a critical role in maintaining blood pressure ("BP") homeostasis and vascular integrity. Celiprolol's potential effect in vEDS is not thought to be substantially mediated via antagonist activity at the beta1 adrenergic receptor, as vEDS patients are typically normotensive, and as brachial systolic and diastolic blood pressures have been shown to not decrease in vEDS patients after treatment with celiprolol. Thus, the potential protective effect of celiprolol in vEDS patients is believed to be independent of any effect on BP lowering. We do not believe that there are any other drugs approved or in development in the U.S. or Europe that have a similar mechanism of action to celiprolol:



Registration Plan

Celiprolol has not been approved for any indication in the U.S. 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 ("Aventis")) in June 1987 but was withdrawn prior to FDA review and therefore never approved. We have obtained from Aventis the exclusive right in North and South America 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 ("MHRA"). We have also licensed from AP-HP exclusive worldwide rights to the data from the Ong trial.

In June 2019, we received a Complete Response Letter from the FDA regarding our NDA for EDSIVO™ for the treatment of vEDS. The Complete Response Letter stated that it will be necessary to conduct an adequate and well-controlled trial to determine whether celiprolol reduces the risk of clinical events in patients with vEDS. In December 2019, we submitted a Formal Dispute Resolution Request to the FDA's Office of New Drugs appealing the FDA's decision as outlined in the Complete Response Letter. In March 2020, we received a response to our Formal Dispute Resolution Request from the Office of New Drugs of the FDA stating that it had denied our appeal of the Complete Response Letter in relation to the NDA for EDSIVO™. In its Appeal Denied letter, the Office of New Drugs described possible paths forward for Acer to explore that could provide the substantial evidence of effectiveness needed to support a potential resubmission of the EDSIVO™ NDA for the treatment of patients with vEDS with a confirmed COL3A1 mutation. In its Appeal Denied letter, the Office of New Drugs referred to the FDA Guidance document issued in December 2019, where substantial evidence of effectiveness can be provided by two or more adequate and well-controlled studies demonstrating efficacy, or a single positive adequate and well-controlled study plus confirmatory evidence. While neither resubmission nor the prospect of approval of the EDSIVOTM NDA is assured, we are evaluating our possible next steps with the goal of resubmission of the EDSIVO™ NDA.

ACER-001

Background

Sodium phenylbutyrate ("NaPB") is currently approved in the U.S. and the European Union to treat patients with UCD. Our product candidate ACER-001 is a taste-masked, immediate release formulation of NaPB, designed to potentially treat UCD and 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, 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 U.S. is 1 in 35,000 live births. Approximately 2,000 patients suffer from UCD in the U.S.

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 consumed, 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®, at up to $1.2 million per patient per year (based on patient weight), is often prohibitive.

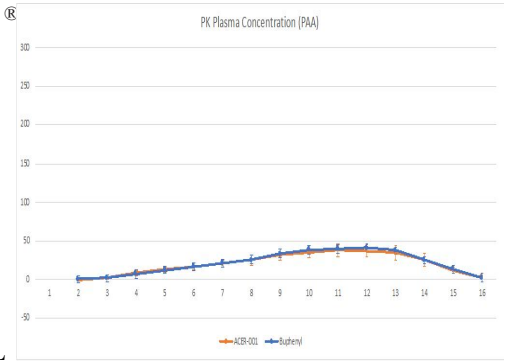
In cases where dietary management or medication is not effective, patients with UCD may require a liver transplant.

ACER-001 for Treatment of UCD

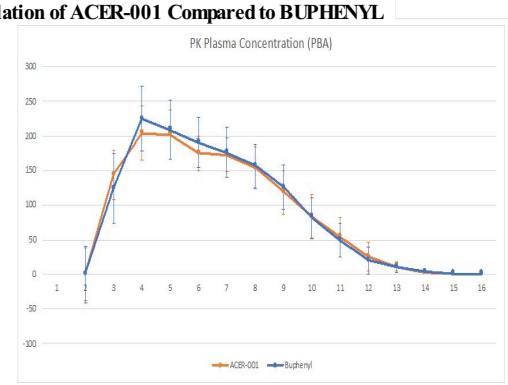
Rationale for ACER-001 Treatment in UCD

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.

In February 2020, we reported the successful completion and final data from Part B of our pivotal trial evaluating the bioavailability and bioequivalence of ACER-001 to BUPHENYL® (sodium phenylbutyrate). Consistent with observations from Part A of the trial, data from Part B showed ACER-001 to be bioequivalent to BUPHENYL® and were within the parameters recommended by the FDA's Guidance for Industry, "Statistical Approaches to Establishing Bioequivalence." The ACER-001 trial design consisted of two parts. Part A was a single-center, single-blind, randomized, single-dose crossover trial designed to evaluate the relative bioavailability of three different oral suspension formulations of ACER-001 compared to BUPHENYL® in 20 healthy adult subjects. Results from Part A of the trial, along with results from a concurrent taste assessment trial evaluating palatability of the three formulations of ACER-001 compared to BUPHENYL®, informed Acer's selection of the single, optimal formulation of ACER-001 that was evaluated in Part B.



Bioavailability of Optimal Formulation of ACER-001 Compared to BUPHENYL®



PBA = phenylbutyrate; PAA = phenylacetate

Lab Name	Parameter	Geometric Mean Difference	Lower 90% Confidence Limit	Upper 90% Confidence Limit
PBA	Cmax	98.01	93.85	102.36
	AUCt	98.87	96.33	101.47
	AUCinf	98.85	96.32	101.45
PAA	Cmax	97.51	92.82	102.44
	AUCt	95.94	90.35	101.87
	AUCinf	95.16	88.92	101.84

Part B of this trial was a single-center, single-blind, randomized, single-dose crossover study designed to show bioequivalence of ACER-001 compared to BUPHENYL® in 36 healthy adult subjects. As described above, results from Part B were announced in February 2020.

Also, we expect to finalize the design and size of a taste study comparing the optimal formulation of ACER-001 to BUPHENYL® and begin enrollment in the second half of 2020. The design and size of the study will be determined following further discussions with the FDA. Results of this study are not required for NDA submission.

Registration Plan

We intend to initially seek FDA approval to market ACER-001 in the U.S. using a regulatory pathway established under section 505(b)(2) of the Federal Food, Drug and Cosmetic Act ("FDCA") that allows applicants to rely at least in part on third party data for approval, which may expedite the preparation, submission, and approval of a marketing application. We also intend to seek approval in the European Union and potentially other territories outside the U.S., after the 505(b)(2) NDA for treatment of UCD is filed. Because the FDA has approved an NDA for BUPHENYL®, which is referred to as the reference listed drug ("RLD"), we intend to rely on the RLD's preclinical and clinical safety data, while supplementing the data with a bridging study that shows 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 early 2021, assuming successful completion of nonclinical work and 12-month stability data. NDA submission is subject to our ability to generate sufficient capital resources to fund this program.

Maple Syrup Urine Disease (MSUD)

Background

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 ("BCAA"), leucine, valine, and isoleucine, as well as the associated branded-chain ketoacids ("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 U.S. 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 U.S.

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 ("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 U.S. by submitting a 505(b)(2) NDA using 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 by the end of 2022, subject to our ability to generate sufficient capital resources to fund this program. We also intend to seek approval in the European Union and other territories outside the U.S. after the 505(b)(2) 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 IND and initiating a Phase 2 trial in MSUD by the end of 2020. The following four clinical trials are planned:

Study One

This multicenter, open-label, uncontrolled clinical trial is expected to 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.

Study Two

This multicenter, double-blind, placebo-controlled study is expected to 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 four 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 is expected to 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 is expected to 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.

Osanetant

Background

Osanetant is a clinical-stage, selective, non-peptide tachykinin NK3 receptor antagonist. NK3R is the main receptor for neurokinin B ("NKB"), a tachykinin peptide primarily found in the arcuate nucleus ("ARC") of the hypothalamus. In December 2018, we entered into an exclusive license agreement with Sanofi to acquire worldwide rights to osanetant.

iVMS Background

Hot flashes, flushing, and night sweats are known as Vasomotor Symptoms ("VMS"), and most often occur in women with menopause. VMS are causally related to decreasing estradiol concentrations, mainly in the serum and subsequently also in the temperature regulating center located in the hypothalamus. The lack of estrogen alters neurotransmitter activity, especially in the serotonergic and noradrenergic pathways. Because sex steroids act as potent neuromodulators, the substitution of ovarian sex steroids by HRT is the most effective treatment option for VMS. While VMS associated with menopause can be often be treated with HRT, there are patients who experience VMS who are not in menopause and for whom HRT is contraindicated.3

VMS that are induced by either chemical or surgical intervention are referred to as iVMS. For example, patients receiving tamoxifen treatment for breast cancer, men receiving leuprolide treatment for prostate cancer, and women who are BRCA-positive who elect to have bilateral salpingo-oophorectomy ("BSO")[4, 5], exhibit severe iVMS and have either a contraindication or relative contraindication to receive HRT to alleviate these symptoms because their cancer is sensitive to hormones which could cause disease recurrence.[2, 6]

iVMS Diagnosis and Incidence

In women with Hormone Receptor positive ("HR+") Breast Cancer ("CaB") receiving tamoxifen:

- 84% of women experienced hot flashes[7]
- 80% experienced night sweats
- 60% experienced severe symptoms
- Symptoms persisted throughout 5 years of treatment and were mainly attributed to tamoxifen
- After 4.5 years, 46% of women had discontinued tamoxifen[8]

In men with HR+ Prostate Cancer ("CaP") receiving leuprolide:

- 80% of men experience hot flashes[9]
- 15-27% of patients consider hot flashes the most distressing side effect
- 30-40% experienced moderate-to-severe symptoms
- 20% discontinued or disrupted treatment

In women who are BRCA+ and have BSO:

- 67% of women have symptoms of menopause such as hot flashes[10]
- Up to 35% complain of "extremely bothersome" symptoms up to two years after their surgery[11]

iVMS are well documented with the use of hormonal cancer therapies and certain surgical procedures. Symptoms such as hot flashes can appear immediately and be severe. Traditional HRTs are usually contraindicated. Non-adherence to cancer therapy can be associated with side effects that can increase the mortality risk or shorten the time to recurrence. Therefore, a non-hormonal treatment for patients with iVMS is needed to potentially help increase the quality of life and improve outcomes.[12]

Rationale for Osanetant Treatment for iVMS

NKB/NK3R is implicated in a variety of human functions and affects the hypothalamus-pituitary-gonadal axis, which plays a critical part in the development and regulation of a number of the body's systems, such as the reproductive and immune systems. Clinical proof of concept studies with other NK3R antagonists have demonstrated rapid and clinically-meaningful improvement in vasomotor symptoms and polycystic ovarian syndrome.

Osanetant was originally developed by Sanofi for the treatment of symptoms associated with schizophrenia. Development was discontinued in 2005. Osanetant was studied in 325 healthy subjects and 665 schizophrenic patients with clinical and laboratory safety data from 21 completed Phase 1 and Phase 2 studies. No major safety concerns were identified from these studies after single-dose and repeated-dose administration of up to 400mg once daily for up to 21 days, and 200mg once daily for up to six weeks. Osanetant is orally bioavailable and readily crosses the blood-brain barrier. We believe that several disorders involving the hypothalamus-pituitary-gonadal axis could benefit from treatment with an NK3R antagonist.

Registration Plan

We believe osanetant would qualify as an NCE in the U.S., and as such, would be eligible for five years of market exclusivity following potential FDA approval. Additional exclusivity would depend on the indications selected and the development pathway that is chosen. We anticipate filing an IND for osanetant with the FDA in the second half of 2020. We plan to explore treatment with osanetant in a Phase 1/2 trial in iVMS patients where HRT is likely contraindicated. This trial would evaluate pharmacokinetics, pharmacodynamics, and safety. Initiation of this trial by the end of 2020 is subject to our ability to raise sufficient capital.

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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 U.S. to target these centers and will evaluate whether to commercialize in other geographies ourselves or with an experienced partner.

We are in the process of formulating our commercialization strategy for osanetant.

Competition

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 needs for novel therapies to treat vEDS, UCD, MSUD, and iVMS, 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.

We are not aware of any other companies that are pursuing a treatment for vEDS, although we are aware of a study that is currently enrolling vEDS patients at AP-HP that 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. Our potential competitors and the related stage of development for their product candidates in our other target indications for ACER-001 and osanetant include the following:

- UCD: Horizon Pharma plc / SOBI, Inc. (Marketed); Promethera Biosciences S.A./N.V. (Phase 2); Aeglea BioTherapeutics Inc. (Phase 1/2); Ultragenyx (Phase 1/2); Synlogic, Inc. (Phase 1); Kaleido (Phase 2)
- MSUD: Synlogic, Inc. (preclinical)
- iVMS: Veru (Phase 2); Que Oncology (Phase 2)

Many of our competitors, either alone or with strategic partners, have or will have substantially greater financial, technical and human resources than we do. 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.

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

Licenses

Baylor College of Medicine ("BCM")

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 licensed products as defined in the agreement. The license agreement requires us to make certain upfront and annual payments to BCM, as well as reimburse certain legal costs, make payments upon achievement of defined milestones, and pay royalties in the low single-digit percent range on net sales of any developed product over the royalty term.

Aventis Pharma SA

In June 2016, we entered into an agreement with Aventis Pharma SA (now Sanofi) 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 the U.S. and Brazil 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. Subsequently we amended our agreement with Sanofi to provide the same rights to data access and use for potential marketing approval in all of North and South America.

Assistance Publique—Hôpitaux de Paris, Hôpital Européen Georges Pompidou (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 used this pivotal clinical data to support our NDA filing for EDSIVOTM for the treatment of vEDS. The agreement requires us to make certain upfront payments to AP-HP, as well as reimburse certain costs and make payments of royalties in the low single-digit range on net sales of celiprolol over the royalty term.

In September 2018, we entered into a license agreement with AP-HP to acquire the exclusive worldwide intellectual property rights to three European patent applications relating to certain uses of celiprolol including (i) the optimal dose of celiprolol in treating vEDS patients, (ii) the use of celiprolol during pregnancy, and (iii) the use of celiprolol to treat kyphoscoliotic Ehlers-Danlos syndrome (type VI). Pursuant to the agreement, we will reimburse AP-HP for certain costs and will pay annual maintenance fee payments. Subject to a minimum royalty amount, we will also pay royalty payments on annual net sales of celiprolol during the royalty term in the low single digit percent range, depending upon whether there is a valid claim of a licensed patent. Under the agreement, we will control and pay the costs of ongoing patent prosecution and maintenance for the licensed applications. We may terminate the agreement in our sole discretion upon written notice to AP-HP, and AP-HP may terminate the agreement in the event we fail to make the required payments after notice and opportunity to cure. Additionally, the agreement will terminate if we terminate clinical development, marketing approval is withdrawn by the health or regulatory authorities in all countries, we cease to do business or there is a procedure of winding-up by court decision against us. We subsequently filed three U.S. patent applications on this subject matter in October 2018.

Sanofi

In December 2018, we entered into an exclusive license agreement with Sanofi granting us worldwide rights to osanetant, a clinical-stage, selective, non-peptide tachykinin NK3 receptor antagonist. We plan to initially pursue development of osanetant as a potential treatment for iVMS. The agreement required us to make an upfront payment to Sanofi, with further payments due upon achievement of defined development and sales milestones, and to pay royalties on our net sales of osanetant 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. We have hired both internal resources and 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 to EDSIVO™ in the U.S. via multiple pathways. We believe that we will be eligible for NCE exclusivity for EDSIVO™, which provides upon approval of an NCE five years 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 U.S. 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 U.S., which requires the submission of one or more studies in pediatric subjects 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 U.S. 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 ("PAP") to offset costs associated with a high priced therapeutic to minimize the incentive for vEDS patients in the U.S. to seek to obtain celiprolol elsewhere.

In October 2018, we filed three U.S. patent applications relating to certain uses of celiprolol including (i) the use of celiprolol during pregnancy, (ii) the optimal dose of celiprolol in treating vEDS patients and (iii) the use of celiprolol to treat kyphoscoliotic Ehlers-Danlos syndrome (type VI).

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 U.S., selected countries in Europe (including Turkey), and Brazil. BCM has been issued one patent in each of the U.S. 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 March 2016 and plan to seek further patent protection in major markets, including the U.S. 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. Orphan Drug exclusivity provides, upon the approval by the FDA 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. We were granted Orphan Drug designation for ACER-001 for the treatment of MSUD by the FDA in August 2014.

Furthermore, we may qualify to receive an additional six months of pediatric exclusivity in the U.S., which runs consecutively to an existing exclusivity, if we conduct a successful pediatric study approved by the FDA for this purpose.

Osanetant

We intend to explore various pathways to protect our commercial rights to osanetant in multiple rare and life-threatening neuroendocrine disorders, including evaluating filing patent applications and acquiring existing intellectual property.

Government Regulation and Product Approval

Government authorities in the U.S. at the federal, state and local level and in other countries extensively regulate, among other things, the use of unapproved drugs, preclinical 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 U.S. 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. This section discusses, in general terms, the typical approval process. Our product candidates must be approved by the FDA before they may be legally marketed in the U.S. and by the appropriate foreign regulatory agency 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 U.S., 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 U.S., the FDA regulates drugs under the Federal Food, Drug and Cosmetic Act ("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 requires 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 standard process required by the FDA before a drug may be marketed in the U.S. generally involves the following:

- completion of preclinical laboratory tests, animal studies and formulation studies in compliance with the FDA's current good laboratory practice ("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 U.S. may begin;
- approval by an institutional review board ("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 ("cGCP") regulations, IND regulations, and human subject protection regulations;
- submission to the FDA of an NDA;
- 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 Practices ("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.

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, directly or by cross-reference, 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 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 ("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 doctors, statisticians, and others who are independent of the clinical trial sponsor. Similar to IRBs, the DSMBs review the progress of a clinical trial and participant safety, but they 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, or due to ethical considerations.

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 Current Good Manufacturing Practice ("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. Under standard approval processes, 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 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 six- or ten-month 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 the 60-day 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 ("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 six or 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 or class 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 inspects 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 and audit data at one or more clinical 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 commercial marketing of the drug subject to specific prescribing information for specific indication(s) and, if applicable, specific post-approval requirements. A Complete Response Letter indicates that the review cycle of the application is complete but 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 plans 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 data in the NDA does not satisfy the criteria for approval. Data from clinical trials are not always conclusive and the FDA may interpret this data differently than we interpret the data.

The FDA also may require implementation of a Risk Evaluation and Mitigation Strategy ("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 Phase 4 clinical trials, 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 data to the labeling 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 following commercial use, 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 Amendments and 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, but for which the information is publicly available. The Hatch-Waxman Amendments also established the abbreviated new drug application ("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 health care 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, 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 patent 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 promotional 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 are common 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 ("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 ("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, 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 what the impact of such changes, if any, may be.

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 U.S. 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, legislation that affects reimbursement for drugs with small patient populations could be adopted, limiting payments for pharmaceuticals such as our product candidates, which could adversely affect our potential future net revenue and results.

In addition, in the U.S., the Patient Protection and Affordable Care Act ("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. On May 4, 2017, the House of Representatives passed the American Health Care Act ("AHCA") which contains provisions that would change the level of federal funding of state Medicaid programs and affect funding for long term care recipients, including the elderly and disabled. The Senate then moved to craft its own "repeal and replace" legislation known as the Better Care Reconciliation Act ("BCRA") with more onerous funding changes affecting the elderly and disabled relative to the Affordable Care Act. The BCRA and two other amendments failed in the Senate and it is unclear if the Senate will debate potential amendments further. However, even if a different bill or amendment passed in the Senate, reconciliation with the House's AHCA bill would be required. Under any new legislation, we expect additional rules, regulations and interpretations to be issued that may materially affect our future financial condition and operations. Even if the Affordable Care Act is not amended or repealed, the current administration could propose changes impacting implementation of the Affordable Care Act. The ultimate composition and timing of any legislation enacted under the current administration that would impact the current implementation of the Affordable Care Act remains uncertain. Given the complexity of the Affordable Care Act and the substantial requirements for regulation thereunder, the impact of the Affordable Care Act on our financial conditions and operations cannot be predicted, whether in its current form or as amended or repealed.

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 ("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 ("CMS") an agency within the U.S. Department of Health and Human Services ("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 it, 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 U.S., 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 U.S. apply similarly in the context of the European Union and/or other jurisdictions, 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 (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 U.S. 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 U.S.). 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, 2019, we had 17 full-time employees and no part-time employees, in addition to a number of 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

Merger and Reverse Stock Split

On September 19, 2017, the Company (then a Texas corporation known as Opexa Therapeutics, Inc.) 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 Company, 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 Company (the "Merger"). Also on September 19, 2017, in connection with, and prior to the completion of, the Merger, the Company effected a 1-for-10.355527 reverse stock split of its then outstanding common stock (the "Reverse Split") and immediately following the Merger, the Company 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 have been adjusted to reflect the Reverse Split.

Following the completion of the Merger, the business conducted by the Company became primarily the business conducted by Private Acer.

Delaware Reincorporation and Subsidiary Merger

On May 15, 2018, we changed our state of incorporation from the State of Texas to the State of Delaware (the "Reincorporation") pursuant to a plan of conversion, dated May 15, 2018. Immediately following the Reincorporation, we eliminated our holding company structure by merging our wholly-owned subsidiary Private Acer with and into the Company (the "Subsidiary Merger"). The Company was the surviving corporation in connection with the Subsidiary Merger.

Company Information

See the sections immediately above titled "Merger and Reverse Stock Split" and "Delaware Reincorporation and Subsidiary Merger." For accounting and financial reporting purposes, Private Acer was considered to have acquired the Company in the Merger. Private Acer was incorporated in 2013 as part of a reorganization whereby Acer Therapeutics, LLC was converted into a Delaware corporation. 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 (the "Exchange Act") under which we file periodic reports, proxy and information statements and other information with the SEC. Copies of the reports, proxy statements and other information are available on the SEC's website, https://www.sec.gov.

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 stockholder 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

In light of the United States ("U.S.") Food and Drug Administration's ("FDA's") Complete Response Letter regarding our New Drug Application ("NDA") for EDSIVOTM, we halted precommercial activities while we work toward our goal of approval for EDSIVOTM. We may decide at any time not to continue development of EDSIVOTM.

Our recent business priority has been premised upon the approval and commercial success of EDSIVOTM. In June 2019, we received a Complete Response Letter from the FDA regarding our NDA for EDSIVOTM (celiprolol) for the treatment of vascular Ehlers-Danlos syndrome ("vEDS"). The Complete Response Letter stated that it will be necessary to conduct an adequate and well-controlled trial to determine whether celiprolol reduces the risk of clinical events in patients with vEDS. We had previously devoted a substantial majority of our research, development, clinical, and precommercial efforts and financial resources towards the development of EDSIVOTM. In order to reduce operating expenses and conserve cash resources, in June 2019, we implemented a corporate restructuring which included a reduction of approximately 60% of our full-time workforce of 48 employees and halted precommercial activities for EDSIVOTM. In December 2019, we submitted a Formal Dispute Resolution Request to the Office of New Drugs appealing the FDA's decision as outline in the Complete Response Letter. In

March 2020, we received a response to our Formal Dispute Resolution Request from the Office of New Drugs of the FDA stating that it had denied our appeal of the Complete Response Letter in relation to the NDA for EDSIVOTM. In its Appeal Denied letter, the Office of New Drugs described possible paths forward for Acer to explore that could provide the substantial evidence of effectiveness needed to support a potential resubmission of the EDSIVOTM NDA for the treatment of patients with vEDS with a confirmed COL3A1 mutation. In its Appeal Denied letter, the Office of New Drugs referred to the FDA Guidance document issued in December 2019, where substantial evidence of effectiveness can be provided by two or more adequate and well-controlled studies demonstrating efficacy, or a single positive adequate and well-controlled study plus confirmatory evidence. While neither resubmission nor the prospect of approval of the EDSIVOTM NDA is assured, we are evaluating our possible next steps with the goal of resubmission of the EDSIVOTM NDA. We may decide at any time not to continue development of EDSIVOTM, which could have a material adverse effect on our business operations and financial prospects.

Substantial doubt exists as to our ability to continue as a going concern.

As of December 31, 2019, we had an accumulated deficit of $76.3 million, cash and cash equivalents of $12.1 million, and current liabilities of $2.8 million. Based on available resources, we believe that our cash and cash equivalents currently on hand are sufficient to fund our operations through the end of 2020, excluding support for EDSIVOTM development and precommercial activities and the planned osanetant clinical trial. 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 have not established a source of revenues and 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 factors individually and collectively raise substantial doubt about our ability to continue as a going concern and therefore it may be more difficult for us to attract investors. Unless we are able to raise additional capital to finance our operations, our long-term business plan may not be accomplished, and we may be forced to cease, reduce, or delay operations. In their audit report in respect of our 2019 audited financial statements, our independent registered public accounting firm included in its report an explanatory paragraph, indicating that the substantial doubt as to our ability to continue as to our ability to continue as a going concern is of fundamental importance to understanding our financial statements.

We will require additional financing to obtain marketing approval of our product candidates and, if approved, to 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, 2019, we had an accumulated deficit of $76.3 million, cash and cash equivalents of $12.1 million and current liabilities of $2.8 million. As discussed above, 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 2020. 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.

We will need to raise additional capital in order to finance 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 stockholders, 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.

Funding from our ATM facility may 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 our at-the-market ("ATM") facility with JonesTrading Institutional Services LLC ("JonesTrading") and Roth Capital Partners, LLC ("Roth Capital") in order to use the program to sell shares of our common stock, as well as provide certain periodic deliverables required by the amended and restated sales agreement with JonesTrading and Roth Capital for the ATM facility. Due to the SEC's "baby shelf rules" which prohibit companies with a public float of less than $75 million from issuing securities under a shelf registration statement in excess of one-third of such company's public float in a 12-month period, we are only able to issue a limited number of shares using our shelf-registration statement at this time. The number of shares and price at which we may be able to sell shares under the ATM facility may be limited due to market conditions and other factors beyond our control.

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 and a history of losses. 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 serious rare and life-threatening diseases with significant unmet medical needs. We are not profitable and have incurred losses in each year since inception. 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 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 years ended December 31, 2019 and 2018 was $29.4 million and $21.3 million, respectively. As of December 31, 2019, we had an accumulated deficit of $76.3 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 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. We may also invest in acquiring or in-licensing additional product candidates to expand our pipeline.

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:

- seek regulatory and marketing approvals and reimbursement for our product candidates;
- 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 preclinical, clinical, or other trials or studies 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 enrollment delays, longer follow-up for planned studies, additional major studies or supportive studies necessary to support marketing approval.

Further, the net losses we incur will 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. Our ability to generate product revenue depends upon our ability to successfully identify, develop and 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 medical affairs, marketing and distribution infrastructure;
- obtain coverage and adequate product reimbursement from third-party payors, including government payors;
- achieve market acceptance for our approved 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 successfully 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 FDA or comparable foreign regulatory authorities, to perform studies or trials in addition to those that we currently anticipate.

Following receipt of a Complete Response Letter from the FDA regarding our NDA for EDSIVO™, in June 2019, we implemented a corporate restructuring which included a reduction of approximately 60% of our full-time workforce of 48 employees and halted precommercial activities for EDSIVOTM. In December 2019, we submitted a Formal Dispute Resolution Request to the FDA's Office of New Drugs appealing the FDA's decision as outlined in the Complete Response Letter. In March 2020, we received a response to our Formal Dispute Resolution Request from the Office of New Drugs of the FDA stating that it had denied our appeal of the Complete Response Letter in relation to the NDA for EDSIVOTM. In its Appeal Denied letter, the Office of New Drugs described possible paths forward for Acer to explore that could provide the substantial evidence of effectiveness needed to support a potential resubmission of the EDSIVOTM NDA for the treatment of patients with vEDS with a confirmed COL3A1 mutation. In its Appeal Denied letter, the Office of New Drugs referred to the FDA Guidance document issued in December 2019, where substantial evidence of effectiveness can be provided by two or more adequate and well-controlled studies demonstrating efficacy, or a single positive adequate and well-controlled study plus confirmatory evidence. While neither resubmission nor the prospect of approval of the EDSIVOTM NDA is assured, we are evaluating our possible next steps with the goal of resubmission of the EDSIVOTM NDA. We may decide at any time not to continue development of EDSIVOTM, which could have a material adverse effect on our business operations and

financial prospects. Even if we complete the development and regulatory processes described above, we anticipate incurring significant costs associated with launching and commercializing these products.

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 ("SOX"), as well as rules and regulations implemented by the Securities and Exchange Commission ("SEC") and The Nasdaq Capital Market ("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 determine that our internal controls are not effective. 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 effectively. Being a public company could also make it more difficult or more costly for us to obtain certain types of insurance, including directors' and officers' liability insurance. 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.

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 controls 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.

Although we are committed to continuing to improve our internal control processes, and although we will continue to diligently and vigorously review our internal controls 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 penalties or investigations by Nasdaq or the SEC.

We face risks related to health epidemics which could adversely affect our business.

Our business could be materially adversely affected by the effects of a widespread outbreak of contagious disease, including the recent outbreak of respiratory illness caused by a novel coronavirus first identified in Wuhan, Hubei Province, China. These effects could include disruptions or restrictions on our employees' ability to travel, as well as disruptions at or closures of our facilities or the facilities of our manufacturers and suppliers, which could adversely impact our development activities and other operations. In addition, a significant outbreak of contagious diseases in the human population could result in a widespread health crisis that could adversely affect the economies and financial markets of many countries, resulting in an economic downturn that could adversely affect our manufacturers and suppliers and otherwise adversely impact our development activities and other operations.

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 worldwide. 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. As a company, we have limited experience with acquiring other companies, or with acquiring products outside of the U.S. Any cash acquisition we pursue would divert the cash we have on our balance sheet from our present clinical development programs. Any stock acquisitions would dilute our stockholders' ownership.

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 U.S. 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 U.S. without first obtaining approval from the FDA to market each product candidate. Similarly, we cannot commercialize our product candidates outside of the U.S. without obtaining regulatory approval from comparable foreign regulatory authorities. Our product candidates could fail to receive marketing approval for many reasons, including the following:

- 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 the design or implementation of any clinical trials we conduct or rely upon for regulatory approval;
- 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 ("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 ("FFDCA") for one or more of our product candidates, and we may be required to conduct clinical trials instead of relying on third-party data, as is the position of the FDA with respect to our NDA for EDSIVOTM;
- 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 U.S. 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, to the satisfaction of the applicable regulatory authorities, 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 U.S., it is necessary to submit and obtain approval of an 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 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 ("cGMP") requirements. The FDA and the Competent Authorities of the Member States of the European Economic Area ("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 current good clinical practice ("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, even those that are accepted for filing and reviewed by the FDA, will 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 U.S., 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 U.S., 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 obtain approval under Section 505(b)(2) of the FFDCA or if we are required to generate additional data related to safety or efficacy in order to obtain approval under Section 505(b)(2), we may be unable to meet our anticipated development and commercialization timelines.

Traditional drug development typically relies upon Section 505(b)(1) of the FFDCA for seeking marketing authorization in the U.S., where the sponsor of the product candidate (i.e., the applicant for marketing authorization) is required to conduct all of the studies needed to demonstrate the safety and efficacy of such candidate. Although we may consider a Section 505(b)(1) pathway in the future, our current strategy for seeking marketing authorization in the U.S. for our product candidates (including EDSIVO™ and ACER-001) relies at least in part 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 needed to demonstrate the safety and efficacy of the product candidate at issue 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. In June 2019, we received a Complete Response Letter from the FDA regarding our NDA for EDSIVO™ for the treatment of vEDS. The Complete Response Letter stated that it will be necessary to conduct an adequate and well-controlled trial to determine whether celiprolol reduces the risk of clinical events in patients with vEDS. In light of the Complete Response Letter, we have currently halted precommercial activities for EDSIVO™ as part of a corporate restructuring initiative. In December 2019, we submitted a Formal Dispute Resolution Request to the FDA's Office of New Drugs appealing the FDA's decision as outlined in the Complete Response Letter. In March 2020, we received a response to our Formal Dispute Resolution Request from the Office of New Drugs of the FDA stating that it had denied our appeal of the Complete Response Letter in relation to the NDA for EDSIVO™. In its Appeal Denied letter, the Office of New Drugs described possible paths forward for Acer to explore that could provide the substantial evidence of effectiveness needed to support a potential resubmission of the EDSIVO™ NDA for the treatment of patients with vEDS with a confirmed COL3A1 mutation. In its Appeal Denied letter, the Office of New Drugs referred to the FDA Guidance document issued in December 2019, where substantial evidence of effectiveness can be provided by two or more adequate and well-controlled studies demonstrating efficacy, or a single positive adequate and well-controlled study plus confirmatory evidence. While neither resubmission nor the prospect of approval of the EDSIVO™ NDA is assured, we are evaluating our possible next steps with the goal of resubmission of the EDSIVO™ NDA. 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 might elect a Section 505(b)(1) pathway to generate sufficient clinical data so that we would no longer need to rely on third-party data. However, a Section 505(b)(1) pathway would likely 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 if 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.

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 certain of 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. In June 2019, we received a Complete Response Letter from the FDA regarding our NDA for EDSIVO™ for the treatment of vEDS. The Complete Response Letter stated that it will be necessary to conduct an adequate and well-controlled trial to determine whether celiprolol reduces the risk of clinical events in patients with vEDS. In light of the Complete Response Letter, we have currently halted precommercial activities for EDSIVO™ as part of a corporate restructuring initiative. In December 2019, we submitted a Formal Dispute Resolution Request to the FDA's Office of New Drugs appealing the FDA's decision as outlined in the Complete Response Letter. In March 2020, we received a response to our Formal Dispute Resolution Request from the Office of New Drugs of the FDA stating that it had denied our appeal of the Complete Response Letter in relation to the NDA for EDSIVO™. In its Appeal Denied letter, the Office of New Drugs described possible paths forward for Acer to explore that could provide the substantial evidence of effectiveness needed to support a potential resubmission of the EDSIVO™ NDA for the treatment of patients with vEDS with a confirmed COL3A1 mutation. In its Appeal Denied letter, the Office of New Drugs referred to the FDA Guidance document issued in December 2019, where substantial evidence of effectiveness can be provided by two or more adequate and well-controlled studies demonstrating efficacy, or a single positive adequate and well-controlled study plus confirmatory evidence. While neither resubmission nor the prospect of approval of the EDSIVO™ NDA is assured, we are evaluating our possible next steps with the goal of resubmission of the EDSIVO™ NDA. We currently do not have, and 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 any NDA we file), or another regulatory authority indicates that a product candidate should not be approved or there should be restrictions on approval, such as the requirement for a Risk Evaluation and Mitigation Strategy ("REMS"), to ensure the safe use of the drug. Delays in marketing approval or rejections of applications for marketing approval in the U.S. or other markets may result from many factors, including:

- the FDA's or comparable foreign regulatory authorities' disagreement with the design or implementation of any clinical trials we conduct or rely on for regulatory approval;
- 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 product candidates, which would significantly harm our business, results of operations and prospects.

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 current Good Laboratory Practice ("cGLP"), 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 animals may result in findings that may require further evaluation, which could affect the risk-benefit evaluation of clinical development, or which may 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 cGLP 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 cGCP. Clinical trials are subject to further oversight by these governmental agencies and Institutional Review Boards ("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 U.S. 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 ("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 ("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. As an organization, we have completed a single clinical trial and have limited experience in designing clinical trials, and thus 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 completed a single clinical trial and may be unable to continue to do so efficiently or at all for our current product candidates or any product candidate we develop.

We will need to conduct clinical trials of our product candidates, with the exception of EDSVIOTM, for which we do not presently intend to conduct additional clinical trials. The conduct of clinical trials and the submission of a successful NDA is a complicated process. As an organization, we have completed a single clinical trial, 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.

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 U.S. 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 such as 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 such as the FDA and national competition agencies in Europe strictly regulate the promotional claims that may be made about prescription products, such as EDSIVOTM, ACER-001, or osanetant, 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 U.S., 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 U.S. 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 U.S. 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 U.S. federal government and states and by the 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 ("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 ("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 without proper written authorization;
- the federal Open Payments program, created under Section 6002 of the Patient Protection and Affordable Care Act ("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 U.S. 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 U.S. 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 U.S. 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 U.S. and foreign governments will continue to consider changes to existing healthcare legislation including the Affordable Care Act. 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.

Our risk of delay in product approvals is increased if the U.S. government is fully or partially shut down due to lack of continuity in funding.

Our business operations are directly and indirectly affected by the operations of the U.S. government, including but not limited to the FDA. Any interruption in the continuity of funding of all or a part of government activities could have a significant negative effect on our business, including the timing of that review decision. For example, over the last several years, including beginning on December 22, 2018 and ending on January 25, 2019, the U.S. government has had shutdowns. We cannot predict the likelihood, duration, impact, or timing of any future shutdown. There can be no assurance that if such shutdown(s) were to occur in the future, adequate funds would be available to the FDA and other U.S. government agencies to allow them to continue their activities uninterrupted. Even when funding is restored following one or more shutdowns, we cannot predict the ongoing impact of such shutdowns on our business, or the degree to which funding would be restored to the FDA or other agencies having an impact on our business.

Other Risks Related to Our Business

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 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.

Our recent restructuring may have a negative impact on our ability to attract and retain qualified personnel. In order to reduce operating expenses and conserve cash resources following receipt of the Complete Response Letter we received from the FDA regarding our NDA for EDSIVO™ for the treatment of vEDS in June 2019, we implemented a corporate restructuring initiative including a reduction of approximately 60% of our full-time workforce of 48 employees and a halt of precommercial activities for EDSIVO™. As a result, as of December 31, 2019, we had a full-time workforce of 17 employees to conduct our planned business operations. If our projections prove to be inaccurate or if we are forced to implement any further workforce reductions, we may not have sufficient staffing to pursue our research and development goals.

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 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.

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 stockholders. 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 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 in amounts we consider to be reasonable for our stage of development. 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, 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.

Our internal computer systems, or those of our development collaborators, third-party clinical research organizations or other contractors or consultants, may fail or suffer cybersecurity or other 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 cybersecurity breaches and 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 cybersecurity or other 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, the further development and commercialization of our product candidates could be delayed, and our reputation could be harmed.

Risks Related to Commercialization of Our Product Candidates

Our product candidate EDSIVOTM has not been approved for any indication in the U.S., and, in June 2019, we received a Complete Response Letter from the FDA stating that it will be necessary to conduct an adequate and well-controlled trial to determine whether celiprolol reduces the risk of clinical events in patients with vEDS. This may result in greater research and development expenses, regulatory issues that could further 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 for the treatment of hypertension was submitted to the FDA in 1987, however, the NDA was withdrawn prior to review. Celiprolol has, however, 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. In June 2019, we received a Complete Response Letter from the FDA regarding our NDA for EDSIVOTM (celiprolol) for the treatment of vEDS. The Complete Response Letter stated that it will be necessary to conduct an adequate and well-controlled trial to determine whether celiprolol reduces the risk of clinical events in patients with vEDS. We had previously devoted a substantial majority of our research, development, clinical, and precommercial efforts and financial resources towards the development of EDSIVO™. In order to reduce operating expenses and conserve cash resources, in June 2019, we implemented a corporate restructuring which included a reduction of approximately 60% of our full-time workforce of 48 employees and halted precommercial activities for EDSIVOTM. In December 2019, we submitted a Formal Dispute Resolution Request to the FDA's Office of New Drugs appealing the FDA's decision outlined in the Complete Response Letter. In March 2020, we received a response to our Formal Dispute Resolution Request from the Office of New Drugs of the FDA stating that it had denied our appeal of the Complete Response Letter in relation to the NDA for EDSIVOTM. In its Appeal Denied letter, the Office of New Drugs described possible paths forward for Acer to explore that could provide the substantial evidence of effectiveness needed to support a potential resubmission of the EDSIVOTM NDA for the treatment of patients with vEDS with a confirmed COL3A1 mutation. In its Appeal Denied letter, the Office of New Drugs referred to the FDA Guidance document issued in December 2019, where substantial evidence of effectiveness can be provided by two or more adequate and well-controlled studies demonstrating efficacy, or a single positive adequate and well-controlled study plus confirmatory evidence. While neither resubmission nor the prospect of approval of the EDSIVOTM NDA is assured, we are evaluating our possible next steps with the goal of resubmission of the EDSIVOTM NDA. 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.

Even if we obtain the required regulatory approvals in the U.S. 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. In 2017, we commissioned a patient-finder study that phenotypically identified 4,169 vEDS patients in the U.S. from an analysis of a commercially available patient claims database, with data of approximately 190 million unique patient lives. Based on that information, we estimate the prevalence of phenotypically-defined vEDS in the U.S. could be greater than 1 in 45,000. Studies suggest that the incidence of UCD in the U.S. is 1 in 35,000 live births. Approximately 2,000 patients suffer from UCD in the U.S. 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 U.S.

It is estimated that vEDS, UCD, and MSUD collectively impact approximately 7,000 patients in the U.S. 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 EDSIVOTM 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, although precommercial activities had been conducted for EDSIVO™ prior to our receipt of the FDA's Complete Response Letter regarding our NDA for EDSIVO™, 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, medical affairs, 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 potential 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.

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. In addition, pricing of orphan and rare disease drug treatments is under increased pressure given the overall healthcare cost climate generally, and pricing of pharmaceutical products specifically.

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 U.S., 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 U.S. 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 ultra-orphan diseases for which there is a small patient population in the U.S. A drug designated an Orphan Drug may receive up to seven years of exclusive marketing in the U.S. 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 needs.

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 as well as to more cost-effective 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 designation in the U.S. for EDSIVO™ for vEDS and ACER-001 for MSUD. We expect to seek Orphan Drug exclusivity from the European Medicines Agency ("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 U.S. 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 EDSIVO™ 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 EDSIVO™ or ACER-001 also will not bar the FDA from approving another celiprolol drug product or a sodium phenylbutyrate ("NaPB") product, for another indication. In the U.S., reforms to the Orphan Drug Act, if enacted, could also materially affect our ability to maintain Orphan Drug exclusivity for EDSIVO™ for vEDS and ACER-001 for MSUD.

Price controls, importation of drug products from outside the U.S., or other rules may be imposed in domestic or foreign markets, which may adversely affect our future profitability.

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, legislation that affects reimbursement for drugs with small patient populations could be adopted, limiting payments for pharmaceuticals such as our product candidates, which could adversely affect our potential future net revenue and results. 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 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 product candidates 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 all or any expenses incurred in connection with their development. If our product candidates are rendered obsolete by advancements in pharmaceutical technologies, our business 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 U.S. 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 U.S., the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 ("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 U.S. 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 U.S. 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 U.S., 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 ("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. Although we have contracts in place, 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, with the exception of EDSIVOTM, for which we do not presently intend to conduct additional clinical trials. 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 clinical trials for any of our product candidates. We have and will continue 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 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 U.S. 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 any 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 EDSIVO™, ACER-001, and osanetant. 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 (ACER-001) for treatment of MSUD. In August 2016, we entered into an agreement with Assistance Publique—Hôpitaux de Paris, Hôpital Européen Georges Pompidou ("AP-HP"), pursuant to which we obtained an exclusive worldwide right to access and use data from the Ong trial, which we used to support an NDA filing for EDSIVO™ for the treatment of vEDS. In September 2018, we entered into an additional agreement with AP-HP pursuant to which we obtained the exclusive worldwide intellectual property rights to three European patent applications relating to certain uses of celiprolol including (i) the optimal dose of celiprolol in treating vEDS patients, (ii) the use of celiprolol during pregnancy and (iii) the use of celiprolol to treat kyphoscoliotic Ehlers-Danlos syndrome (type VI). In December 2018, we entered into an

exclusive license agreement with Sanofi granting us worldwide rights to osanetant, a clinical-stage, selective, non-peptide tachykinin NK3 receptor antagonist. 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 U.S. can be less extensive than those in the U.S. 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 U.S. 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 U.S., or from selling or importing products made using our inventions in and into the U.S. 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 U.S. 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 U.S. 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 U.S. 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 U.S. Patent and Trademark Office ("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 U.S. In addition, potential infringers of our intellectual property rights may have substantially more resources than we do to defend their position, which could adversely affect the outcome of any such dispute.

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 U.S., 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 U.S. remain confidential until issued as patents. Except for the preceding exceptions, patent applications in the U.S. 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 U.S. 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 (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 U.S. 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 U.S. 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 U.S. 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 common stock, and any trading market that exists in our common stock 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 common stock 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 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, including clinical study results and determinations by regulatory authorities with respect thereto, including but not limited to any continued development of EDSIVO™ that we may or may not decide to pursue in light of the FDA's March 2020 denial of our appeal of the June 2019 Complete Response Letter;

- the initiation, termination or reduction in the scope of any collaboration arrangements or any disputes or developments regarding such collaborations;
- our capital and 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 U.S. and in foreign countries; or
- dilutive effects of sales of shares of common stock by us or our stockholders, and sales of common stock acquired upon exercise or conversion by the holders of options.

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 are a defendant in securities litigation, which may be costly and time-consuming to defend.

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 like ours have experienced significant stock price volatility in recent years. Moreover, we were named in a putative securities class action complaint and a stockholders' derivative suit as a result of the decline in our stock price following the June 25, 2019 announcement that we had received a Complete Response Letter from the FDA regarding our NDA for EDSIVO™. See Item 3 – Legal Proceedings for additional information. 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 stockholders.

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 stockholder 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 stockholders from receiving a premium for their shares in connection with a change of control.

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

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 stockholders and cause the market price of our common stock to decline. An aggregate of 10,095,176 shares of common stock were outstanding as of December 31, 2019. As of such date, another 1,322,475 shares of common stock were issuable upon exercise of outstanding options or settlement of restricted stock units. A substantial majority of the outstanding shares of our common stock, as well as a substantial majority of the shares of common stock issuable upon exercise of outstanding options, 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 all of our product candidates, 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 our 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.

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.

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, 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 stockholders 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 increase our tax obligations and thus have a material adverse effect on our cash flow and results of operations.

Because of their ownership of our common stock, insiders may influence significant corporate decisions.

As of March 1, 2020, our executive officers and directors and their affiliates beneficially owned or controlled approximately 19% of the outstanding shares of our common stock. Accordingly, these executive officers, directors and their affiliates will have substantial influence over the outcome of corporate actions requiring stockholder 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. This concentration of ownership may also delay or prevent a change of control of our company, even if such a change of control would benefit our other stockholders.

Anti-takeover provisions in our organizational documents and Delaware law might discourage, delay, or prevent an acquisition attempt or change in control of our company that you might consider favorable.

Our certificate of incorporation 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 stockholder 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 stockholder nominations of directors and for stockholder proposals that can be acted on at stockholder meetings;
- limit who may call stockholder meetings;
- require that any action to be taken by our stockholders be effected at a duly called annual or special meeting and not by written consent;
- provide that vacancies on our Board of Directors may be filled only by a majority of directors then in office, even if less than a quorum;
- require a super-majority of votes to approve certain amendments to our charter as well as to amend our bylaws generally; and
- authorize us to indemnify officers and directors against losses that they may incur in investigations and legal proceedings resulting from their services to us, which may include services in connection with takeover defense measures.

Further, as a Delaware corporation, we are also subject to provisions of Section 203 of the Delaware General Corporation Law regulating corporate takeovers. Section 203 generally prohibits us from engaging in a business combination with interested stockholders subject to certain exceptions.

These anti-takeover provisions and other provisions under Delaware law, our charter and our bylaws could discourage, delay or prevent a transaction involving an acquisition attempt or a change in control of our company, including actions that our stockholders 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 stockholders to elect directors of your choosing and to cause us to take other corporate actions you desire.

Our certificate of incorporation designates the Court of Chancery of the State of Delaware as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could limit a stockholder's ability to bring a claim in a judicial forum that the stockholder believes is more convenient or favorable for disputes with us or our directors, officers or other employees.

Our certificate of incorporation provides that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware shall be the sole and exclusive forum for:

- any derivative action or proceeding brought on our behalf;
- any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers or other employees to us or our stockholders;
- any action asserting a claim arising pursuant to any provision of the Delaware General Corporation Law; or
- any action asserting a claim against us governed by the internal affairs doctrine.

Any person or entity purchasing or otherwise acquiring any interest in shares of our capital stock shall be deemed to have notice of and consented to the provisions of our certificate of incorporation described above. This choice of forum provision may limit a stockholder's ability to bring a claim in a judicial forum that the stockholder believes is more convenient or favorable for disputes with us or our directors, officers or other employees, which may discourage such lawsuits against us and our directors, officers and other employees. Alternatively, if a court were to find these provisions of our certificate of incorporation inapplicable to, or unenforceable in respect of, one or more of the specified types of actions or proceedings, we may incur additional costs associated with resolving such matters in other jurisdictions, which could adversely affect our business, financial condition or results of operations.

Item 1B. Unresolved Staff Comments.

None.

Item 2. Properties.

We lease 4,360 square feet of office space located in Newton, Massachusetts, which serve as our company headquarters and are used by our employees working in research and development, regulatory affairs, and general and administrative functions. We lease 3,677 square feet of office space located in Bend, Oregon, which serve as a satellite facility. The leases expire on May 31, 2022.

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.

On September 27, 2017, Piper Sandler & Co. ("Piper") filed a lawsuit against us, Piper Sandler & 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 we breached our obligations to Piper pursuant to an August 30, 2016 engagement letter between the parties and an April 28, 2017 addendum thereto by failing to pay Piper (i) a fee of $1.1 million in connection with the financing which closed on September 19, 2017 for aggregate consideration of approximately $15.7 million and (ii) $0.1 million in reimbursement for expenses incurred by Piper pursuant to the engagement letter. On November 10, 2017, we filed an answer and counterclaim in the lawsuit, denying Piper'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 filed a reply to the counterclaims denying the essential allegations, and discovery has commenced. On February 22, 2019, Piper filed a motion for summary judgment. In response, we filed our opposition to Piper's motion on March 22, 2019. Piper subsequently filed its reply to our opposition on April 5, 2019. Piper's motion is currently pending before the Court and oral argument on the summary judgment motion was held on September 4, 2019. No ruling on the motion has yet been issued by the Court. We have not recorded a liability as of December 31, 2019, because a potential loss is not probable or reasonably estimable given the preliminary nature of the proceedings.

In addition, on July 1, 2019, plaintiff Tyler Sell filed a putative class action lawsuit, Sell v. Acer Therapeutics Inc., against us, Chris Schelling and Harry Palmin, in the United States District Court for the Southern District of New York. The complaint alleges that prior to the receipt of the Complete Response Letter from the FDA, we made material false and misleading statements or omissions which allegedly constitute securities fraud. On August 12,

2019, a stockholder's derivative action, Gress v. Acer Therapeutics Inc., was filed in the United States District Court for the District of Delaware against us and certain of our officers and directors, asserting damages resulting from alleged breach of fiduciary duties, based on the same facts at issue in the Sell case. We believe both suits are without merit and intend to defend ourselves vigorously. With the selection of a lead plaintiff, the Sell case is now known as Skiadas v. Acer Therapeutics. On February 7, 2020, we filed a motion to dismiss the class action lawsuit. We have not recorded a liability as of December 31, 2019 because a potential loss is not probable or reasonably estimable given the preliminary nature of the proceedings.

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."

As of March 1, 2020, there were approximately 90 registered holders of our common stock. This number does not include stockholders 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 serious rare and life-threatening diseases with significant unmet medical needs. Our pipeline includes three clinical-stage candidates: EDSIVO™ (celiprolol) for the treatment of vascular Ehlers-Danlos syndrome ("vEDS") in patients with a confirmed type III collagen (COL3A1) mutation; ACER-001 (a taste-masked, immediate release formulation of sodium phenylbutyrate) for the treatment of various inborn errors of metabolism, including urea cycle disorders ("UCD") and Maple Syrup Urine Disease ("MSUD") and osanetant for the treatment of induced Vasomotor Symptoms ("iVMS") where Hormone Replacement Therapy ("HRT") is likely contraindicated. Our product candidates are believed to present a comparatively de-risked profile, having one or more of a favorable safety profile, clinical proof-of-concept data, mechanistic differentiation, and/or accelerated paths for development through specific programs and procedures established by the United States ("U.S.") Food and Drug Administration ("FDA").

Merger and Reverse Stock Split

On September 19, 2017, the Company (then a Texas corporation known as Opexa Therapeutics, Inc.) 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 Company, 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 Company (the "Merger"). This transaction was approved by the Company's stockholders at a special meeting of its stockholders on September 19, 2017. Also on September 19, 2017, in connection with, and prior to the completion of, the Merger, the Company effected a 1-for-10.355527 reverse stock split of its then outstanding common stock (the "Reverse Split") and immediately following the Merger, the Company 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 have been adjusted to reflect the Reverse Split.

Following the completion of the Merger, the business conducted by the Company became primarily the business conducted by Private Acer.

For accounting and financial reporting purposes, Private Acer was considered to have acquired the Company 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.

Delaware Reincorporation and Subsidiary Merger

On May 15, 2018, we changed our state of incorporation from the State of Texas to the State of Delaware (the "Reincorporation") pursuant to a plan of conversion, dated May 15, 2018. Immediately following the Reincorporation, we eliminated our holding company structure by merging our wholly-owned subsidiary Private Acer with and into the Company (the "Subsidiary Merger"). The Company was the surviving corporation in connection with the Subsidiary Merger.

Restructuring

In June 2019, we received a Complete Response Letter from the FDA regarding our New Drug Application ("NDA") for EDSIVO™ (celiprolol) for the treatment of vEDS. The Complete Response Letter stated that it will be necessary to conduct an adequate and well-controlled trial to determine whether celiprolol reduces the risk of clinical events in patients with vEDS. In order to reduce operating expense and conserve cash resources, in June

2019, we initiated a corporate restructuring, which included a reduction of approximately 60% of our full-time workforce of 48 employees and halted precommercial activities for EDSIVOTM. We recorded a one-time severance-related charge of approximately $1.5 million associated with the workforce reduction in the quarter ended June 30, 2019. We expect the restructuring to align the resources needed for us to conduct our planned business operations and maintain sufficient capital to conduct our business as planned through the end of 2020, excluding support for EDSIVOTM development and precommercial activities and the planned osanetant clinical trial.

Going Concern

The accompanying financial statements have been prepared in conformity with GAAP, which contemplate our continuation as a going concern. We have not established a source of revenues and, as such, have been dependent on funding operations through the sale of equity securities. Since inception, we have experienced significant losses and incurred negative cash flows from operations. We expect to incur further losses over the next several years as we develop our business. We have spent, and expect to continue to spend, a substantial amount of funds in connection with implementing our business strategy, including our planned product development efforts and potential precommercial activities.

As of December 31, 2019, we had cash and cash equivalents of $12.1 million. Our cash and cash equivalents available at December 31, 2019 are expected to fund operations through the end of 2020, excluding support for EDSIVO™ development and precommercial activities and the planned osanetant clinical trial.

We will need to raise additional capital to fund continued operations some time during 2020. We have no commitments for any additional financing and may not be successful in our efforts to raise additional funds or achieve profitable operations. Any amounts raised will be used for further development of our product candidates, precommercial activities, potential acquisitions of additional product candidates, and for other working capital purposes. If we are unable to obtain additional capital (which is not assured at this time), our long-term business plan may not be accomplished, and we may be forced to cease, reduce, or delay operations.

These factors individually and collectively raise substantial doubt about our ability to continue as a going concern. Our financial statements do not include any adjustments or classifications that may result from our possible inability to continue as a going concern. The report of our independent registered public accounting firm on our financial statements for the year ended December 31, 2019 also includes an explanatory paragraph regarding substantial doubt about our ability to continue as a going concern.

Revenue

We have no products approved for commercial sale and have not generated 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.

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 preclinical, 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 and other direct costs of acquiring intellectual property.

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.

Since our inception in December 2013, we have spent a total of approximately $42.3 million in research and development expenses through December 31, 2019. Of that amount, approximately $31.1 million is directly related to EDSIVO™ and approximately $9.8 million is directly related to ACER-001.

We expect our research and development expenses to be substantial 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 preclinical 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, pursue regulatory approvals, and operate our business as planned.

General and Administrative Expenses

General and administrative expenses consist primarily of employee-related expenses, including salaries, benefits, and stock-based compensation; precommercial costs that had been associated with preparing for the commercial launch of EDSIVO™ for the treatment of vEDS, if approved by the FDA; and professional fees for legal, business consulting, auditing, and tax services. We expect that general and administrative expenses will be substantial in the future.

Other income, net

Other income, net consists primarily of interest income. We earn interest income from interest-bearing accounts and money market funds, which we classify as cash and cash equivalents. Additionally, we record as part of other income, 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 and Estimates

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 accounting principles generally accepted in the U.S. ("GAAP"). The preparation of these consolidated 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.

Goodwill

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. We evaluate according to Accounting Standards Update No. 2017-04, *Intangibles – Goodwill and Other* (Topic 350) 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 may opt to perform a qualitative assessment or a quantitative impairment test to determine whether goodwill is impaired. If we were to determine based on a qualitative assessment that it was more likely than not that the fair value of the reporting unit was less than its carrying value, a quantitative impairment test would then be performed. The quantitative impairment test compares the fair value of the reporting unit with its carrying amount, including goodwill. If the estimated fair value of the reporting unit is less than its carrying amount, a goodwill impairment would be recognized for the difference.

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 from the GPCR Target Pools of Anchor that we obtained the rights to in the March 20, 2015, acquisition of Anchor. 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 under our 2018 Stock Incentive Plan, 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 on the date of grant using the Black-Scholes model. We recognize stock-based compensation expense for stock options and restricted stock units on a straight-line basis over the vesting term.

Research and Development Expenses

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 Preclinical Study Expenses

We make estimates of prepaid and/or 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 clinical trials and preclinical studies are based on estimates of costs incurred for services provided by contract research organizations ("CRO"), manufacturing organizations, and for other trial- and study-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 recording expenses associated with these activities, we obtain information from various sources and estimate the level of effort 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. No material change in estimates were recognized in the year ended December 31, 2019. At December 31, 2019, our accounts payable and accrued expenses included $0.6 million for costs associated with clinical trials.

Results of Operations

Comparison of the years ended December 31, 2019 and 2018

The following table summarizes our results of operations for the years ended December 31, 2019 and 2018:

	Years Ended December 31,			
	2019	2018	$ Change	% Change
Research and development	$ 13,851,018	$ 12,452,424	$ 1,398,594	11%
General and administrative	16,046,423	9,261,570	6,784,853	73%
Loss from operations	(29,897,441)	(21,713,994)	(8,183,447)	38%
Total other income, net	479,472	433,103	46,369	11%
Net loss	$ (29,417,969)	$ (21,280,891)	$ (8,137,078)	38%

Research and Development Expenses

Research and development expenses were approximately $13.9 million for the year ended December 31, 2019, as compared to approximately $12.5 million for the year ended December 31, 2018. This increase of approximately $1.4 million was primarily due to increases in spending related to contract manufacturing services, regulatory consulting, and medical affairs services during the first half of 2019 in preparation for the potential launch of EDSIVOTM, as well as to an increase in employee-related expenses. The increase in employee-related expenses was driven by increased headcount during the first half of 2019, as well as $0.5 million restructuring expense, and increased stock-based compensation expense. These increases were partially offset by a decrease in spending related to licenses. Research and development expense for the year ended December 31, 2019 was primarily comprised of approximately $7.5 million directly related to EDSIVOTM and approximately $5.5 million directly related to ACER-001.

General and Administrative Expenses

General and administrative expenses were approximately $16.0 million for the year ended December 31, 2019, as compared to approximately $9.3 million for the year ended December 31, 2018. This increase of approximately $6.7 million was primarily due to a $4.6 million increase in employee-related expenses, which included expense of $1.0 million related to the restructuring, increased headcount and travel during the first half of 2019, and increased stock-based compensation expense. The remaining increase in general and administrative expenses was primarily due to an increase in expenses related to precommercial activities.

Other Income, Net

Other income, net of approximately $0.5 million and $0.4 million during the years ended December 31, 2019 and 2018, respectively, was primarily attributable to interest income.

Liquidity and Capital Resources

We have never been profitable and have incurred operating losses in each year since inception. From inception through December 31, 2019, we have raised net cash proceeds of approximately $81.7 million, primarily from common stock offerings, private placements of convertible preferred stock, and debt financings. On August 3, 2018, we completed an underwritten public offering of 2,555,555 shares of common stock at a public offering price of $18.00 per share. We received aggregate net proceeds of approximately $42.7 million, after deducting underwriting discounts, commissions and offering-related expenses of approximately $3.3 million. As of December 31, 2019, we had approximately $12.1 million in cash and cash equivalents. Our net loss for the years ended December 31, 2019 and 2018, was $29.4 million and $21.3 million, respectively. As of December 31, 2019, we had an accumulated deficit of approximately $76.3 million. The following table summarizes our cash flows for the years ended December 31, 2019, and 2018:

	Years Ended December 31,	
	2019	2018
Operating activities	$ (29,506,949)	$ (16,587,803)
Investing activities	(178,967)	(95,627)
Financing activities	92,272	42,710,359
Net (decrease) increase in cash and cash equivalents	$ (29,593,644)	$ 26,026,929

Operating Activities

Net cash used in operating activities was approximately $29.5 million for the year ended December 31, 2019, as compared to approximately $16.6 million for the year ended December 31, 2018. The increase of approximately $12.9 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, as well as increased use of cash due to the timing of accrued expenses and accounts payable, partially offset by an increase in non-cash stock-based compensation.

Investing Activities

Net cash used in investing activities during the years ended December 31, 2019 and 2018 relates to the purchase of property and equipment.

Financing Activities

Net cash provided by financing activities during the year ended December 31, 2019 consisted of proceeds of $0.1 million from the exercise of stock options. Net cash provided by financing activities during the year ended December 31, 2018 consisted primarily of $42.6 million of net proceeds from the issuance of common stock.

Future Capital Requirements

We have not generated 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 to continue to incur significant expenses 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. In their audit report in respect of our 2019 audited financial statements, our independent registered public accounting firm included an explanatory paragraph expressing substantial doubt as to our ability to continue as a going concern, as discussed above.

In June 2019, in order to reduce operating expenses and conserve cash resources following receipt of the FDA's Complete Response Letter, we implemented a corporate restructuring which included a reduction of approximately 60% of our full-time workforce of 48 employees and halted precommercial activities for EDSIVO™. We recorded a one-time severance-related charge of approximately $1.5 million associated with the workforce reduction. In December 2019, we submitted a Formal Dispute Resolution Request to the FDA's Office of New Drugs appealing the FDA's decision as outlined in the Complete Response Letter. In March 2020, we received a response to our Formal Dispute Resolution Request from the Office of New Drugs of the FDA stating that it had denied our appeal of the Complete Response Letter in relation to the NDA for EDSIVO™. In its Appeal Denied letter, the Office of New Drugs described possible paths forward for Acer to explore that could provide the substantial evidence of effectiveness needed to support a potential resubmission of the EDSIVO™ NDA for the treatment of patients with vEDS with a confirmed COL3A1 mutation. In its Appeal Denied letter, the Office of New Drugs referred to the FDA Guidance document issued in December 2019, where substantial evidence of effectiveness can be provided by two or more adequate and well-controlled studies demonstrating efficacy, or a single positive adequate and well-controlled study plus confirmatory evidence. While neither resubmission nor the prospect of approval of the EDSIVO™ NDA is assured, we are evaluating our possible next steps with the goal of resubmission of the EDSIVO™ NDA. Depending on our progress, as well as our available resources and needs, we may decide at any time not to continue development of EDSIVO™, which could have a material adverse effect on our business operations and financial prospects. As of December 31, 2019, we had approximately $12.1 million in cash and cash equivalents. 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 2020, excluding support for EDSIVO™ development and precommercial activities and the planned osanetant clinical trial.

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

- any continued development of EDSIVO™ we may or may not decide to pursue in light of the FDA's June 2019 Complete Response Letter and the March 2020 denial of our appeal of the Complete Response Letter
- 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 preclinical studies and clinical trials for our product candidates
- the costs involved in patent filing, prosecution, and enforcement
- the number of programs we pursue

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 our product candidates, pursue regulatory approvals, potentially commercialize (if approved) our product candidates, and operate our business as planned.

We expect to incur significant expenses and operating losses for at least the next year as we initiate and continue the clinical development of, seek regulatory approval for, and potentially commercialize (if approved) our product candidates. In addition, operating as a publicly-traded company involves 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. We do not maintain any lines of credit or have any sources of debt or equity capital committed for funding, other than the amended and restated sales agreement entered into on March 18, 2020 with JonesTrading Institutional Services LLC and Roth Capital Partners, LLC. 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. Due to the SEC's "baby shelf rules" which prohibit companies with a public float of less than $75 million from issuing securities under a shelf registration statement in excess of one-third of such company's public float in a 12-month period, we are only able to issue a limited number of shares using our shelf registration statement at this time.

To the extent that we raise additional capital through the issuance of additional equity or convertible debt securities, the ownership interest of our stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of existing stockholders. 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 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 ("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 low single-digit percent royalties on net sales of any developed product over the royalty term.

In June 2016, we entered into an agreement with Aventis Pharma SA (now Sanofi) 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 the U.S. and Brazil 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. Subsequently we amended our agreement with Sanofi to provide the same rights to data access and use for potential marketing approval in all of North and South America.

In August 2016, we entered into an agreement with AP-HP granting us 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 utilized this pivotal clinical data to support an NDA filing for EDSIVOTM 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 low single-digit percent royalties on net sales of celiprolol over the royalty term.

In September 2018, we entered into an additional agreement with AP-HP to acquire the exclusive worldwide intellectual property rights to three European patent applications relating to certain uses of celiprolol including (i) the use of celiprolol during pregnancy, (ii) the optimal dose of celiprolol in treating vascular Ehlers-Danlos syndrome ("vEDS") patients and (iii) the use of celiprolol to treat kyphoscoliotic Ehlers-Danlos syndrome (type VI). Pursuant to the agreement, we will reimburse AP-HP for certain costs and will pay annual maintenance fee payments. Subject to a minimum royalty amount, we will also pay royalty payments on annual net sales of celiprolol during the royalty term in the low single digit percent range, depending upon whether there is a valid claim of a licensed patent. Under the agreement, we will control and pay the costs of ongoing patent prosecution and maintenance for the licensed applications. We subsequently filed three U.S. patent applications on this subject matter in October 2018.

In December 2018, we entered into an exclusive license agreement with Sanofi granting us worldwide rights to osanetant a clinical-stage, selective, non-peptide tachykinin NK3 receptor antagonist. The agreement requires us to make certain upfront payments to Sanofi, make payments upon achievement of defined development and sales milestones and pay royalties on net sales of osanetant over the royalty term. We plan to initially pursue development of osanetant as a potential treatment for iVMS where HRT is likely contraindicated.

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, 2019 and 2018 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

Stockholders and Board of Directors
Acer Therapeutics Inc.
Newton, Massachusetts

Opinion on the Consolidated Financial Statements

We have audited the accompanying consolidated balance sheet of Acer Therapeutics Inc. (the "Company") as of December 31, 2019, the related consolidated statements of operations, changes in stockholders' equity, and cash flows for the year ended December 31, 2019, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2019, and the results of its operations and its cash flows for the year ended December 31, 2019, in conformity with accounting principles generally accepted in the United States of America.

Going Concern Uncertainty

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

Basis for Opinion

These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's consolidated financial statements based on our audit. 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 audit 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 consolidated 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 audit 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 audit included performing procedures to assess the risks of material misstatement of the consolidated 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 consolidated financial statements. Our audit also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. We believe that our audit provides a reasonable basis for our opinion.

/s/ BDO USA, LLP

We have served as the Company's auditor since 2019.

Boston, Massachusetts
March 18, 2020

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, 2018, and the related consolidated statements of operations, stockholders' equity and cash flows for year 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, 2018, and the results of its operations and its cash flows for the year then ended, in conformity with accounting principles generally accepted in the United States of America.

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 audit. 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 audit 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 audit 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 audit 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 audit 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 audit provides a reasonable basis for our opinion.

/s/ Wolf & Company, P.C.

We served as the Company's auditor from 2014 to 2018.

Boston, Massachusetts
March 7, 2019

ACER THERAPEUTICS INC.
CONSOLIDATED BALANCE SHEETS
AS OF DECEMBER 31, 2019 AND 2018

	2019	2018
Assets		
Current assets:		
Cash and cash equivalents	$ 12,077,640	$ 41,671,284
Prepaid expenses and other current assets	807,356	1,075,021
Total current assets	12,884,996	42,746,305
Property and equipment, net	193,974	130,867
Other assets:		
Goodwill	7,647,267	7,647,267
In-process research and development	118,600	118,600
Other non-current assets	620,674	20,380
Total assets	$ 21,465,511	$ 50,663,419
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 561,090	$ 1,033,829
Accrued expenses	1,944,431	4,546,432
Other current liabilities	263,392	—
Total current liabilities	2,768,913	5,580,261
Other non-current liabilities	326,282	—
Total liabilities	3,095,195	5,580,261
Commitments and Contingencies (Note 6)		
Stockholders' equity:		
Preferred stock, $0.0001 par value; authorized 10,000,000 shares; none issued and outstanding	—	—
Common stock, $0.0001 par value; authorized 150,000,000 shares; 10,095,176 and 10,087,363 shares issued and outstanding at December 31, 2019 and 2018, respectively	1,010	1,009
Additional paid-in capital	94,619,818	91,914,692
Accumulated deficit	(76,250,512)	(46,832,543)
Total stockholders' equity	18,370,316	45,083,158
Total liabilities and stockholders' equity	$ 21,465,511	$ 50,663,419

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

ACER THERAPEUTICS INC.
CONSOLIDATED STATEMENTS OF OPERATIONS
FOR THE YEARS ENDED DECEMBER 31, 2019 AND 2018

	2019	2018
Operating expenses:		
Research and development	$ 13,851,018	$ 12,452,424
General and administrative	16,046,423	9,261,570
Total operating expenses	29,897,441	21,713,994
Loss from operations	(29,897,441)	(21,713,994)
Other income, net:		
Interest income	471,267	412,553
Foreign currency transaction gain	8,205	20,550
Total other income, net	479,472	433,103
Net loss	$ (29,417,969)	$ (21,280,891)
Net loss per share - basic and diluted	$ (2.91)	$ (2.49)
Weighted average common shares outstanding - basic and diluted	10,092,179	8,555,039

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

ACER THERAPEUTICS INC.
CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY
FOR THE YEARS ENDED DECEMBER 31, 2019 AND 2018

	Stockholders' Equity				
	Common stock		Additional Paid-in Capital	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount			
Balance as of December 31, 2017	7,497,433	$ 74,974	$ 47,812,215	$ (25,551,652)	$ 22,335,537
Reallocation for change in par value	—	(74,224)	74,224	—	—
Issuance of common stock, net of issuance costs	2,555,555	256	42,622,444	—	42,622,700
Issuance of common stock in connection with stock option exercises	34,375	3	87,656	—	87,659
Stock-based compensation	—	—	1,318,153	—	1,318,153
Net loss	—	—	—	(21,280,891)	(21,280,891)
Balance as of December 31, 2018	10,087,363	1,009	91,914,692	(46,832,543)	45,083,158
Issuance of common stock in connection with stock option exercises	7,813	1	92,271	—	92,272
Stock-based compensation	—	—	2,612,855	—	2,612,855
Net loss	—	—	—	(29,417,969)	(29,417,969)
Balance as of December 31, 2019	10,095,176	$ 1,010	$ 94,619,818	$ (76,250,512)	$ 18,370,316

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

ACER THERAPEUTICS INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS
FOR THE YEARS ENDED DECEMBER 31, 2019 AND 2018

	2019	2018
Cash flows from operating activities:		
Net loss	$ (29,417,969)	$ (21,280,891)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation	2,612,855	1,318,153
Depreciation	58,282	27,744
Loss on disposal of property and equipment	57,578	—
Changes in operating assets and liabilities		
Prepaid expenses and other current assets	267,665	(193,134)
Accounts payable	(472,739)	937,956
Accrued expenses	(2,602,001)	2,609,101
Other noncurrent assets	(10,620)	(6,732)
Net cash used in operating activities	(29,506,949)	(16,587,803)
Cash flows from investing activities:		
Purchase of property and equipment	(178,967)	(95,627)
Net cash used in investing activities	(178,967)	(95,627)
Cash flows from financing activities:		
Proceeds from issuance of common stock, net of issuance costs	—	42,622,700
Proceeds from exercise of stock options	92,272	87,659
Net cash provided by financing activities	92,272	42,710,359
Net (decrease) increase in cash and cash equivalents	(29,593,644)	26,026,929
Cash and cash equivalents, beginning of the year	41,671,284	15,644,355
Cash and cash equivalents, end of the year	$ 12,077,640	$ 41,671,284

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

1. NATURE OF OPERATIONS AND BASIS OF PRESENTATION

Business

Acer Therapeutics Inc., a Delaware corporation (the "Company"), is a pharmaceutical company focused on the acquisition, development, and commercialization of therapies for serious rare and life-threatening diseases with significant unmet medical needs. The Company's pipeline includes three clinical-stage candidates: EDSIVO™ (celiprolol) for the treatment of vascular Ehlers-Danlos syndrome ("vEDS") in patients with a confirmed type III collagen (COL3A1) mutation; ACER-001 (a taste-masked, immediate release formulation of sodium phenylbutyrate) for the treatment of various inborn errors of metabolism, including urea cycle disorders ("UCD") and Maple Syrup Urine Disease ("MSUD"); and osanetant for the treatment of induced Vasomotor Symptoms ("iVMS") where Hormone Replacement Therapy ("HRT") is likely contraindicated. The Company's product candidates are believed to present a comparatively de-risked profile, having one or more of a favorable safety profile, clinical proof-of-concept data, mechanistic differentiation, and/or accelerated paths for development through specific programs and procedures established by the United States ("U.S.") Food and Drug Administration ("FDA").

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.

In June 2019, the Company received a Complete Response Letter from the FDA regarding its new drug application ("NDA") for EDSIVO™ (celiprolol) for the treatment of vEDS. The Complete Response Letter stated that it will be necessary to conduct an adequate and well-controlled trial to determine whether celiprolol reduces the risk of clinical events in patients with vEDS. The Company had previously devoted a substantial majority of its research, development, clinical, and precommercial efforts and financial resources towards the development of EDSIVO™. In order to reduce operating expenses and conserve cash resources following receipt of the FDA's Complete Response Letter, in June 2019, the Company implemented a corporate restructuring which included a reduction of approximately 60% of its full-time workforce of 48 employees and halted precommercial activities for EDSIVO™. In December 2019, the Company submitted a Formal Dispute Resolution Request to the FDA's Office of New Drugs appealing the FDA's decision as outlined in the Complete Response Letter. In March 2020, the Company received a response to its Formal Dispute Resolution Request from the Office of New Drugs of the FDA stating that it had denied the Company's appeal of the Complete Response Letter in relation to the NDA for EDSIVOTM. In its Appeal Denied letter, the Office of New Drugs described possible paths forward for Acer to explore that could provide the substantial evidence of effectiveness needed to support a potential resubmission of the EDSIVOTM NDA for the treatment of patients with vEDS with a confirmed COL3A1 mutation. In its Appeal Denied letter, the Office of New Drugs referred to the FDA Guidance document issued in December 2019, where substantial evidence of effectiveness can be provided by two or more adequate and well-controlled studies demonstrating efficacy, or a single positive adequate and well-controlled study plus confirmatory evidence. While neither resubmission nor the prospect of approval of the EDSIVOTM NDA is assured, the Company is evaluating its possible next steps with the goal of resubmission of the EDSIVOTM NDA. Depending on its progress, as well as the Company's available resources and needs, the Company may decide at any time not to continue development of EDSIVO™, which could have a material adverse effect on its business operations and financial prospects.

Merger and Reverse Stock Split

On September 19, 2017, the Company (then a Texas corporation known as Opexa Therapeutics, Inc.) 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 Company, 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 Company (the "Merger"). This transaction was approved by the Company's stockholders at a

special meeting of its stockholders on September 19, 2017. Also on September 19, 2017, in connection with, and prior to the completion of, the Merger, the Company effected a 1-for-10.355527 reverse stock split of its then outstanding common stock (the "Reverse Split") and immediately following the Merger, the Company 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 Company became primarily the business conducted by Private Acer.

Delaware Reincorporation and Subsidiary Merger

On May 15, 2018, the Company changed its state of incorporation from the State of Texas to the State of Delaware pursuant to a plan of conversion, dated May 15, 2018. As a result of this reincorporation, the par value of the Company's preferred stock was changed to $0.0001 from no par value and the par value of the Company's common stock was reduced to $0.0001 from $0.01.

Immediately following the Reincorporation, the Company's holding company structure was eliminated by merging wholly-owned subsidiary Private Acer with and into the Company (the "Subsidiary Merger"). The Company was the surviving corporation in connection with the Subsidiary Merger.

Basis of Presentation

The consolidated financial statements include Acer Therapeutics Inc. and, prior to the date of the Subsidiary Merger, its wholly-owned subsidiary, Private Acer. All intercompany balances and transactions have been eliminated in consolidation. Any reference in these notes to applicable guidance is meant to refer to the authoritative accounting principles generally accepted in the U.S., as found in the Accounting Standards Codification ("ASC") and Accounting Standards Update ("ASU") of the Financial Accounting Standards Board ("FASB").

Liquidity

The Company had an accumulated deficit of $76.3 million and cash and cash equivalents of $12.1 million as of December 31, 2019. Net cash used in operating activities was $29.5 million and $16.6 million for the years ended December 31, 2019 and 2018, respectively. On November 9, 2018, the Company entered into a sales agreement with Roth Capital Partners, LLC, and on March 18, 2020, an amended and restated sales agreement (the "Agreement") was entered into with JonesTrading Institutional Services LLC and Roth Capital Partners, LLC. The Agreement provides a facility for the offer and sale of shares of common stock from time to time having an aggregate offering price of up to $50 million depending upon market demand, in transactions deemed to be an at-the-market offering (the "ATM Offering"). Any such sales would be effected pursuant to the Company's registration statement on Form S-3 (File No. 333-228319), declared effective by the SEC on November 21, 2018. As of December 31, 2019, the Company had not sold any shares of common stock under the Agreement. The Company has no obligation to sell any shares of common stock pursuant to the Agreement and may at any time suspend sales pursuant to the Agreement. Each party may terminate the Agreement at any time without liability. The Company's existing cash and cash equivalents are expected to enable it to continue to evaluate possible next steps with respect to EDSIVO™, complete the Acer-001 (UCD) pivotal bioequivalence trial, advance certain of its other development activities, and provide for other working capital purposes, but exclude support for EDSIVO™ development and precommercial activities and the planned osanetant clinical trial.

Management expects to continue to finance operations through the issuance of additional equity or debt securities and/or through strategic collaborations. Any transactions which occur may contain covenants that restrict the ability of management to operate the business and any securities issued may have rights, preferences, or privileges senior to the Company's common stock and may dilute the ownership of current stockholders of the Company.

Going Concern

The accompanying financial statements have been prepared in conformity with GAAP, which contemplate continuation of the Company as a going concern. The Company has not established a source of revenues and, as such, has been dependent on funding operations through the sale of equity securities. Since inception, the Company has experienced significant losses and incurred negative cash flows from operations. The Company expects to incur further losses over the next several years as it develops its business. The Company has spent, and expects to continue to spend, a substantial amount of funds in connection with implementing its business strategy, including its planned product development efforts and potential precommercial activities.

As of December 31, 2019, the Company had cash and cash equivalents of $12.1 million. The Company's cash and cash equivalents available at December 31, 2019 are expected to fund operations through the end of 2020, excluding support for EDSIVO™ development and precommercial activities and the planned osanetant clinical trial.

The Company will need to raise additional capital to fund continued operations some time during 2020. The Company has no commitments for any additional financing and may not be successful in its efforts to raise additional funds or achieve profitable operations. Any amounts raised will be used for further development of the Company's product candidates, precommercial activities, potential acquisitions of additional product candidates and for other working capital purposes. If the Company is unable to obtain additional capital (which is not assured at this time), its long-term business plan may not be accomplished, and the Company may be forced to cease, reduce, or delay operations.

These factors individually and collectively raise substantial doubt about the Company's ability to continue as a going concern. The accompanying financial statements do not include any adjustments or classifications that may result from the possible inability of the Company to continue as a going concern.

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 expenses during the reporting period. Estimates having relatively higher significance include stock-based compensation, contract manufacturing accruals, 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.

The Company follows the provisions of ASC Topic 820, Fair Value Measurement, which establishes a fair value hierarchy that prioritizes the inputs to valuation techniques used to measure fair value. The Company considers its investments in money market funds of $11.6 million and $41.7 million as of December 31, 2019 and 2018, respectively, included in cash and cash equivalents, to be Level 1, which are based on unadjusted quoted

prices in active markets for identical assets or liabilities accessible to the reporting entity at the measurement date. The estimated fair value of the Company's financial instruments, which include cash and cash equivalents, accounts payable, and accrued liabilities approximates their carrying value, based upon their short-term maturities or prevailing interest rates.

Research and Development Expenses

Costs incurred for research and development are expensed as incurred.

Stock-Based Compensation

The Company records stock-based payments at fair value. The measurement date for compensation expense related to awards is generally the date of the grant. The fair value of awards 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:

	2019	2018
Risk-free interest rate	1.68% - 2.57%	2.27% - 2.98%
Expected life (years)	6	6
Volatility	60%	60%
Dividend rate	0%	0%

Due to its limited operating history and a limited trading history of its common stock in relation to the life of its standard option grants, 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 stock-based awards 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. 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 was impaired as of December 31, 2019 or 2018.

Goodwill

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. The Company evaluates the recoverability of goodwill according to ASU No. 2017-04, *Intangibles – Goodwill and Other* (Topic 350), which it adopted in the fourth quarter of 2018, annually, or more frequently if events or changes in circumstances indicate

that the carrying value of goodwill might be impaired. The Company may opt to perform a qualitative assessment or a quantitative impairment test to determine whether goodwill is impaired. The Company's goodwill is allocated to a single reporting unit. If the Company were to determine based on a qualitative assessment that it was more likely than not that the fair value of the reporting unit was less than its carrying value, a quantitative impairment test would then be performed. The quantitative impairment test compares the fair value of the reporting unit with its carrying amount, including goodwill. If the estimated fair value of the reporting unit is less than its carrying amount, a goodwill impairment would be recognized for the difference. As a result of the Complete Response Letter from the FDA and the subsequent decline in the Company's stock price, goodwill was evaluated for impairment and it was determined that the fair value of the reporting unit exceeded its carrying value and no impairment existed. The Company performed a qualitative analysis of goodwill as of December 31, 2019 and 2018, in which management concluded that it was more likely than not that the fair value of the reporting unit is greater than its carrying amount.

Foreign Currency Transaction Gain/(Loss)

Gains and losses arising from transactions and revaluation of balances denominated in currencies other than U.S. dollars are recorded in foreign currency transaction gain/(loss) on the consolidated statements of operations.

Income Taxes

The Company is primarily subject to U.S. federal and Massachusetts state income taxes. The Company's tax returns for years 2015 through present are open to tax examinations by U.S. federal and state tax authorities; however, carryforward attributes that were generated prior to January 1, 2015 remain subject to adjustment upon examination if they either have been utilized or will be utilized in a future period. 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's 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, 2019 and 2018. The Company's policy is to recognize interest and penalties related to income tax, if any, in income tax expense. As of December 31, 2019 and 2018, the Company had 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 stock-based awards, were not included in the calculation of the diluted net 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 serious rare and life-threatening diseases with significant unmet medical needs.

Recently Adopted Accounting Pronouncements

In February 2016, the FASB issued ASU No. 2016-02, *Leases (Topic 842)* ("ASU 2016-02"), which establishes new accounting and disclosure requirements for leases. ASU 2016-02 requires lessees to classify most leases as either finance or operating leases and to initially recognize a lease liability and right-of-use asset. The Company adopted ASU 2016-02 in the first quarter of 2019 using the effective date approach to recognize and measure leases as of the adoption date. The Company has elected to utilize the available practical expedient to not separate lease components from non-lease components as well as the package of practical expedients that allows the Company not to reassess (1) whether any expired or existing contracts as of the adoption date are or contain a lease, (2) lease classification for any expired or existing leases as of the adoption date and (3) initial direct costs for any existing leases as of the adoption date. As a result of the adoption of this guidance, the Company recorded a non-cash transaction to recognize on January 1, 2019 lease liabilities totaling $0.4 million and right-of-use-assets totaling $0.4 million, which will be amortized over the remaining terms of the leases.

In June 2018, the FASB issued ASU No. 2018-07, *Compensation—Stock Compensation (Topic 718) Improvements to Nonemployee Share-Based Payment Accounting* ("ASU 2018-07"). This new guidance expands the scope of ASC Topic 718 to include share-based payments granted to nonemployees in exchange for goods or services used or consumed in an entity's own operations and supersedes the guidance in ASC Topic 505-50. Equity-classified nonemployee awards are measured on the grant date, rather than on the earlier of (1) the performance commitment date or (2) the date at which the nonemployee's performance is complete. Awards to nonemployees are measured by estimating the fair value of the equity instruments to be issued, rather than either the fair value of the goods or services received or the fair value of the equity instruments issued, whichever can be measured more reliably. Entities may use the expected term to measure nonemployee options or elect to use the contractual term as the expected term, on an award-by-award basis. The Company adopted ASU 2018-07 in the first quarter of 2019. There was no impact on the Company's financial statements as a result of the adoption of this guidance.

3. PROPERTY AND EQUIPMENT

Property and equipment consisted of the following at December 31, 2019 and 2018:

	December 31, 2019	December 31, 2018
Computer hardware and software	$ 60,749	$ 58,868
Leasehold improvements	60,535	7,648
Furniture and fixtures	145,487	96,013
Subtotal property and equipment, gross	266,771	162,529
Less accumulated depreciation	(72,797)	(31,662)
Property and equipment, net	$ 193,974	$ 130,867

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, leasehold improvements are depreciated over the shorter of the estimated useful life of the asset or the duration of the current lease arrangement, and furniture and fixtures are depreciated over an estimated useful life of 7 years.

4. ACCRUED EXPENSES

Accrued expenses consisted of the following at December 31, 2019 and 2018:

	December 31, 2019	December 31, 2018
Accrued contract manufacturing	$ 655,207	$ 1,079,314
Accrued contract research and regulatory consulting	418,000	128,678
Accrued precommercial costs	252,125	85,000
Accrued payroll and payroll taxes	153,238	1,302,728
Accrued legal	152,340	111,543
Accrued accounting, audit, and tax fees	147,850	102,000
Accrued consulting	109,073	49,940
Accrued miscellaneous expenses	56,598	119,861
Accrued license fees	—	1,567,368
Total accrued expenses	$ 1,944,431	$ 4,546,432

5. LEASES

On March 6, 2018, the Company entered into a lease agreement (the "Newton Lease") commencing on October 1, 2018 for certain premises, which consist of 2,760 square feet of office space located in Newton, Massachusetts (the "Newton Premises"), to serve as its corporate headquarters. On March 5, 2019, the Company entered into a lease agreement to amend the Newton Lease and to lease an additional 1,600 square feet of office space, commencing on June 1, 2019, located in Newton, Massachusetts (the "Additional Newton Premises") to serve as additional space for its corporate headquarters. The term of the leases for the Newton Premises and the Additional Newton Premises expires on May 31, 2022. In addition, the Company is required to share in certain taxes and operating expenses of the Newton Premises and the Additional Newton Premises.

The Company entered into a triple net lease (the "Bend Lease") effective April 1, 2018 for certain premises consisting of 2,288 square feet of office space located in Bend, Oregon (the "Bend Premises") to serve as a satellite facility. On April 23, 2019, the Company entered into a lease agreement to amend the Bend Lease and to lease an additional 1,389 square feet of office space, commencing on May 1, 2019, located in Bend, Oregon (the "Additional Bend Premises") to serve as additional space for its satellite facility. The term of the lease for the Bend Premises and the Additional Bend Premises expires on May 31, 2022 (the "Bend Term"). The Company has an option to extend the Bend Term for up to two additional periods of three years and a right of first refusal to lease an additional suite in the same building.

The leases for the Newton Premises, the Additional Newton Premises, the Bend Premises, and the Additional Bend Premises are classified as operating leases. In the first quarter of 2019, the Company adopted ASU 2016-02 and recorded a non-cash transaction to recognize a right-of-use-asset of $0.4 million in other noncurrent assets, as well as a lease liability of $0.2 million in other current liabilities and $0.2 million in other non-current liabilities. Since the adoption of ASU 2016-02, the Company has recognized additional right-of-use-assets totaling $0.3 million as well as additional lease liabilities totaling $0.1 million in other current liabilities and $0.2 million in other non-current liabilities in conjunction with the commencement of the leases for the Additional Newton Premises and the Additional Bend Premises. The Company's lease liability represents the net present value of future lease payments utilizing a discount rate of 8%, which corresponds to the Company's incremental borrowing rate. As of December 31, 2019, the weighted average remaining lease term was 2.4 years. For the years ended December 31, 2019 and 2018, the Company recorded expense of $0.2 million and $0.1 million, respectively, related to the leases. During the year ended December 31, 2019, the Company made cash payments of $0.2 million for amounts included in the measurement of lease liabilities. The Company is therefore reporting a right-of-use asset of $0.6 million in Other non-current assets and lease liabilities totaling $0.6 million in Other current liabilities and Other non-current liabilities on its consolidated balance sheet as of December 31, 2019.

The following table reconciles the undiscounted lease liabilities to the total lease liabilities recognized on the consolidated balance sheet as of December 31, 2019:

Undiscounted lease liabilities for years ending December 31,:		
2020	$	263,392
2021		273,158
2022		115,951
Total undiscounted lease liabilities		652,501
Less effects of discounting		(62,827)
Total lease liabilities as of December 31, 2019	$	589,674
Reported as of December 31, 2019:		
Other current liabilities	$	263,392
Other non-current liabilities		326,282
Total lease liabilities	$	589,674

Future minimum lease payments at December 31, 2018 were as follows:

Years Ending December 31:	Minimum Lease Payments	
2019	$	151,579
2020		155,813
2021		93,204
Total	$	400,596

6. COMMITMENTS AND CONTINGENCIES

License Agreements

In April 2014, Private Acer obtained exclusive rights to intellectual property relating to ACER-001 and preclinical and clinical data, through a 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, make payments upon achievement of defined milestones, and pay royalties in the low single-digit percent range on net sales of any developed product over the royalty term.

In August 2016, Private Acer signed an agreement with Assistance Publique—Hôpitaux de Paris, Hôpital Européen Georges Pompidou ("AP-HP") (via its Department of Clinical Research and Development) granting the Company the exclusive worldwide rights to access and use data from a randomized controlled clinical study of celiprolol. The Company used this pivotal clinical data to support an NDA regulatory filing for EDSIVOTM, 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 payment of royalties in the low single-digit percent range on net sales of celiprolol over the royalty term.

In September 2018, the Company entered into a License Agreement for Development and Exploitation with AP-HP to acquire the exclusive worldwide intellectual property rights to three European patent applications relating to certain uses of celiprolol including (i) the optimal dose of celiprolol in treating vEDS patients, (ii) the use of celiprolol during pregnancy and (iii) the use of celiprolol to treat kyphoscoliotic Ehlers-Danlos syndrome (type VI). Pursuant to the agreement, the Company will reimburse AP-HP for certain costs and will pay annual maintenance fee payments. Subject to a minimum royalty amount, the Company will also pay royalty payments on annual net sales of celiprolol during the royalty term in the low single digit percent range, depending upon whether there is a valid claim of a licensed patent. Under the agreement, the Company will control and pay the costs of ongoing patent prosecution and maintenance for the licensed applications. The Company may terminate the agreement in its sole discretion upon written notice to AP-HP, and AP-HP may terminate the agreement in the event the Company fails to make the required payments after notice and opportunity to cure. Additionally, the agreement will terminate if the Company terminates clinical development, marketing approval is withdrawn by the health or regulatory authorities

in all countries, the Company ceases to do business or there is a procedure of winding-up by court decision against the Company. The Company subsequently filed three U.S. patent applications on this subject matter in October 2018.

In December 2018, the Company entered into an exclusive license agreement with Sanofi granting the Company worldwide rights to osanetant, a clinical-stage, selective, non-peptide tachykinin NK3 receptor antagonist. The agreement required the Company to make a certain upfront payment to Sanofi, make payments upon achievement of defined development and sales milestones and pay royalties on net sales of osanetant over the royalty term. The Company plans to initially pursue development of osanetant as a potential treatment for iVMS where HRT is likely contraindicated.

Litigation

From time to time, the Company may become involved in litigation or proceedings relating to claims arising out of its operations.

On September 27, 2017, Piper Sandler & Co. ("Piper") filed a lawsuit against the Company, Piper Sandler & 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 the Company breached its obligations to Piper pursuant to an August 30, 2016 engagement letter between the parties and an April 28, 2017 addendum thereto by failing to pay Piper (i) a fee of $1.1 million in connection with the financing which closed on September 19, 2017 for aggregate consideration of approximately $15.7 million and (ii) $0.1 million in reimbursement for expenses incurred by Piper pursuant to the engagement letter. On November 10, 2017, the Company filed an answer and counterclaim in the lawsuit, denying Piper'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 filed a reply to the counterclaims denying the essential allegations, and discovery has commenced. On February 22, 2019, Piper filed a motion for summary judgment. In response, the Company filed its opposition to Piper's motion on March 22, 2019. Piper subsequently filed its reply to the Company's opposition on April 5, 2019. Piper's motion is currently pending before the Court and oral argument on the summary judgment motion was held on September 4, 2019. No ruling on the motion has yet been issued by the Court. The Company has not recorded a liability as of December 31, 2019, because a potential loss is not probable or reasonably estimable given the preliminary nature of the proceedings.

In addition, on July 1, 2019, plaintiff Tyler Sell filed a putative class action lawsuit, Sell v. Acer Therapeutics Inc., against the Company, Chris Schelling and Harry Palmin, in the U.S. District Court for the Southern District of New York. The complaint alleges that prior to the receipt of the Complete Response Letter from the FDA, the Company made material false and misleading statements or omissions which allegedly constitute securities fraud. On August 12, 2019, a stockholder's derivative action, Gress v. Acer Therapeutics Inc., was filed in the U.S. District Court for the District of Delaware against the Company and certain of its officers and directors, asserting damages resulting from alleged breach of fiduciary duties, based on the same facts at issue in the Sell case. The Company believes both suits are without merit and intends to defend itself vigorously. With the selection of a lead plaintiff, the Sell case is now known as Skiadas v. Acer Therapeutics. On February 7, 2020, the Company filed a motion to dismiss the class action lawsuit. The Company has not recorded a liability as of December 31, 2019 because a potential loss is not probable or reasonably estimable given the preliminary nature of the proceedings.

7. STOCKHOLDERS' EQUITY

Underwritten Public Offerings

On August 3, 2018, the Company completed an underwritten public offering of 2,555,555 shares of common stock, including 333,333 shares sold pursuant to the underwriters' full exercise of their option to purchase additional shares to cover over-allotments, at a public offering price of $18.00 per share. The Company received aggregate net proceeds of approximately $42.7 million, after deducting underwriting discounts, commissions and offering-related expenses of approximately $3.3 million.

At-the-Market Facility

On November 9, 2018, the Company entered into a sales agreement with Roth Capital Partners, LLC, and on March 18, 2020, the Company entered into an amended and restated sales agreement with JonesTrading Institutional Services LLC and Roth Capital Partners, LLC. The agreement provides a facility for the offer and sale of shares of common stock from time to time having an aggregate offering price of up to $50 million depending upon market demand, in transactions deemed to be an "at-the-market" offering (the "ATM Offering"). Any such sales would be effected pursuant to the Company's registration statement on Form S-3 (File No. 333-228319), declared effective by the SEC on November 21, 2018. As of December 31, 2019, the Company has not sold any shares of common stock under the agreement. The Company has no obligation to sell any shares of common stock pursuant to the agreement and may at any time suspend sales pursuant to the agreement. Each party may terminate the agreement at any time without liability.

2018 Stock Incentive Plan

The Company's 2018 Stock Incentive Plan (the "2018 Plan"), adopted on May 14, 2018, provides for the grant of up to 500,000 shares of common stock as stock options, restricted stock, stock appreciation rights, restricted stock units, performance-based awards and cash-based awards that may be settled in cash, stock or other property to employees, executive officers, directors, and consultants.

In addition to the 500,000 shares, the total number of shares reserved for issuance under the 2018 Plan also consists of the sum of the number of shares subject to outstanding awards under the Company's 2010 Stock Incentive Plan, as amended and restated (the "2010 Plan"), and the 2013 Stock Incentive Plan, as amended (the "2013 Plan"), as of the effective date of the 2018 Plan that are subsequently forfeited or terminated for any reason prior to being exercised or settled, plus the number of shares subject to vesting restrictions under the 2010 Plan and the 2013 Plan on the effective date of the 2018 Plan that are subsequently forfeited, plus the number of shares reserved but not issued or subject to outstanding grants under the 2010 Plan and the 2013 Plan as of the effective date of the 2018 Plan, up to a maximum of 635,170 shares in aggregate. In addition, the number of shares authorized for issuance under the 2018 Plan is automatically increased (the "evergreen provision") on the first day of each fiscal year beginning on January 1, 2019, and ending on (and including) January 1, 2028, in an amount equal to the lesser of (i) 4% of the outstanding shares of common stock on the last day of the immediately preceding fiscal year, or (ii) another amount (including zero) determined by the Company's Board of Directors. Any shares subject to awards granted under the 2018 Plan that are forfeited or terminated before being exercised or settled, or are not delivered to the participant because such award is settled in cash, will again become available for issuance under the 2018 Plan. Shares withheld to satisfy the grant, exercise price or tax withholding obligation related to an award will again become available for issuance under the 2018 Plan. On January 1, 2019, 403,495 additional shares were authorized according to the evergreen provision. At December 31, 2019, 162,004 shares of common stock remained available for the grant of future awards under the 2018 Plan.

The 2018 Plan is administered by the Company's Board of Directors, which may in turn delegate authority to administer the plan to a committee such as the Compensation Committee, referred to herein as the 2018 Plan administrator. Subject to the terms of the 2018 Plan, the 2018 Plan administrator will determine recipients, the number of shares or amount of cash subject to awards to be granted, whether an option is to be an incentive stock options or non-incentive stock options and the terms and conditions of the stock awards, including the period of their exercisability and vesting. Subject to the limitations set forth below, the 2018 Plan administrator will also determine the exercise price of options granted under the 2018 Plan. The 2018 Plan expressly provides that, without the approval of the stockholders, the 2018 Plan administrator does not have the authority to reduce the exercise price of any outstanding stock options or stock appreciation rights under the 2018 Plan (except in connection with certain corporate transactions, such as stock splits, certain dividends, recapitalizations, reorganizations, mergers, spin-offs and the like), or cancel any outstanding underwater stock options or stock appreciation rights in exchange for cash or new stock awards under the 2018 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 generally vest either 1) 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, or 2) for certain stock options granted on September 18, 2019, 50% vest on each of January 1, 2021 and January 1, 2022, assuming continued service, and with vesting

acceleration in full immediately prior to a change in control. Restricted stock units generally vest and are settled upon the first anniversary of the grant date.

2013 Stock Incentive Plan

Private Acer's 2013 Plan, which was assumed by the Company in connection with the Merger, provided 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 were generally granted with an exercise price equal to the fair value of the common stock at the date of grant and had contractual terms of 10 years. At December 31, 2019, all shares available under the 2013 Plan were subject to outstanding equity awards, and no new awards may be granted under the 2013 Plan.

2010 Stock Incentive Plan

The Company's 2010 Plan, as amended and restated, provided 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. Option awards were generally granted with an exercise price equal to the fair value of the common stock at the date of grant and had contractual terms of 10 years. At December 31, 2019, all shares available under the 2010 Plan were subject to outstanding equity awards, and no new awards may be granted under the 2010 Plan.

A summary of option activity under the 2018 Plan, 2013 Plan, and 2010 Plan for the year ended December 31, 2019 is as follows:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Remaining Contractual Term (Years)	Aggregate Intrinsic Value (in millions)
Options outstanding at December 31, 2018	781,725	$ 16.34	9.0	
Granted	1,063,150	$ 13.84		
Exercised	(7,813)	$ 11.81		$ 0.1
Cancelled/forfeited	(523,587)	$ 21.51		
Options outstanding at December 31, 2019	1,313,475	$ 12.28	8.7	$ 0.4
Options exercisable at December 31, 2019	339,184	$ 11.79	7.4	$ 0.1

A summary of restricted stock unit activity under the 2018 Plan for the year ended December 31, 2019 is as follows:

	Number of Shares	Weighted Average Grant Date Fair Value Per Share	Aggregate Intrinsic Value (in millions)
Non-vested outstanding at December 31, 2018	—	—	
Granted	15,000	$ 23.60	
Cancelled/forfeited	(6,000)		
Non-vested outstanding at December 31, 2019	9,000	$ 23.60	$ 0.1

At December 31, 2019, there was approximately $5.6 million of unrecognized compensation expense related to the stock-based compensation arrangements granted under all plans. The average remaining vesting period for options was 2.2 years. The weighted average grant-date fair value of options granted during the years ended December 31, 2019 and 2018 was $8.03 and $12.73, respectively. The amount of stock-based compensation expense recorded to general and administrative expenses and to research and development expenses was approximately $1.6 million and $1.0 million, respectively, for the year ended December 31, 2019. The amount of stock-based compensation expense recorded to general and administrative expenses and to research and development expenses was approximately $0.6 million and $0.7 million, respectively, for the year ended December 31, 2018.

8. INCOME TAXES

There was no provision for income taxes for the years ended December 31, 2019 and 2018, 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,	
	2019	2018
Deferred tax assets:		
Net operating loss carry forwards	$ 8,506,035	$ 3,604,179
Capitalized research and development costs	17,477,585	14,381,936
Accrued liabilities	133,933	232,899
Tax credit carryforwards	7,174,516	6,311,164
Stock-based compensation	935,508	394,918
Operating lease	141,777	—
Total deferred tax assets	34,369,354	24,925,096
Valuation allowance	(34,221,451)	(24,922,829)
Net deferred tax assets	147,903	2,267
Deferred tax liabilities:		
Operating lease right of use asset	(141,777)	—
Other	(6,126)	(2,267)
Total deferred tax liabilities	(147,903)	(2,267)
	$ —	$ —

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

	December 31,	
	2019	2018
Federal statutory rate	21.0%	21.0%
R&D and Orphan Drug credits	4.4%	10.9%
State income tax, net of federal tax benefit	6.6%	2.8%
Valuation allowance	(31.6%)	(35.2%)
Share-based compensation	(0.2%)	0.0%
Other, net	(0.2%)	0.5%
Effective tax rate	0.0%	0.0%

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, 2019, for income tax reporting purposes, the Company had U.S. federal and state net operating loss carryforwards of approximately $36.1 million and research and development credits and Orphan Drug credits of approximately $7.1 million that can be carried forward and offset against taxable income. For state purposes, the Company had state net operating loss carryforwards of approximately $1.1 million and research and development credits of approximately $0.1 million that can be carried forward and offset against taxable income. Federal net operating loss, research and development credits, and Orphan Drug credits generated prior to 2018 and Massachusetts net operating losses can be carried forward for 20 years and begin to expire in 2022. Federal net operating loss generated after 2017 can be carried forward indefinitely. 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.

9. NET LOSS PER SHARE

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

As of December 31, 2019 and 2018, the number of shares of common stock underlying potentially dilutive securities consist of:

	December 31,	
	2019	2018
Options to purchase common stock	1,313,475	781,725
Restricted stock units	9,000	—
Total	1,322,475	781,725

10. RESTRUCTURING

In June 2019, the Company received a Complete Response Letter from the FDA regarding its New Drug Application for EDSIVO™ (celiprolol) for the treatment of vEDS. The Complete Response Letter stated that it will be necessary to conduct an adequate and well-controlled trial to determine whether celiprolol reduces the risk of clinical events in patients with vEDS. In order to reduce operating expenses and conserve cash resources, in June 2019, the Company initiated a corporate restructuring, which included a reduction of approximately 60% of its full-time workforce of 48 employees and halted precommercial activities for EDSIVO™. In the second quarter of 2019, the Company recorded a one-time severance-related charge of approximately $1.5 million associated with the workforce reduction in the quarter ended June 30, 2019, of which approximately $1.0 million was included in general and administrative expenses and approximately $0.5 million was included in research and development expenses. As of December 31, 2019, the Company had no remaining liability related to the one-time severance-related charge.

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, 2019 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 (2013)* 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, 2019 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.

On March 18, 2020, we entered into an amendment and restatement of that certain Sales Agreement dated November 9, 2018 (the "Original Agreement" and, as amended and restated, the "Agreement") with Jones Trading Institutional Services LLC and Roth Capital Partners, LLC (together, the "Sales Agents"). Pursuant to the Agreement, we may offer and sell the shares in transactions deemed to be an "at-the-market" offering as defined in Rule 415 of the Securities Act of 1933, which shares can be sold by us from time to time, depending upon market demand, with the Sales Agents acting as agents for sales.

We will pay the Sales Agents a commission equal to 3.5% of the gross proceeds from the sale of shares of common stock by them as agents under the Agreement. The Agreement provides that we will provide customary indemnification rights to the Sales Agents. We have no obligation to sell any shares of common stock pursuant to

the Agreement and may at any time suspend sales pursuant to the Agreement. Any party may terminate the Agreement pursuant to the terms of the Agreement without liability of any party.

The shares of common stock will be sold pursuant to a shelf registration statement on Form S-3 which has been declared effective by the Securities and Exchange Commission ("SEC") on November 21, 2018, and a prospectus supplement to be filed by us with the SEC on March 18, 2020. This shall not constitute an offer to sell or the solicitation of an offer to buy nor shall there be any sale of these securities in any state in which such offer, solicitation or sale would be unlawful prior to the registration or qualification under the securities laws of any such state.

The foregoing description of the Agreement does not purport to be complete and is subject to and qualified in its entirety by reference to the Agreement, a copy of which is filed as Exhibit 10.27 hereto and is incorporated herein by reference.

PART III

Item 10. Directors, Executive Officers and Corporate Governance.

Executive Officers

The names of our executive officers and their ages as of March 1, 2020 are as follows:

Name	Age	Position
Chris Schelling	44	President, Chief Executive Officer and Director
William T. Andrews, M.D.	55	Chief Medical Officer
Donald R. Joseph	65	Chief Legal Officer and Secretary
John Klopp	45	Chief Technical Officer
Harry S. Palmin	50	Chief Operating Officer and Chief Financial Officer
Matthew T. Seibt	49	Chief Commercial 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 served as a director from that time until the Merger. From December 2013 to February 2016, he served as Private Acer's Chief Operating Officer, and from February 2016 until the Merger, he served as Private Acer's President and Chief Executive Officer. Prior to founding Private Acer, he served as Executive Director of Strategic Marketing at BioMarin Pharmaceutical Inc., a Nasdaq-listed biotechnology company, from May 2006 to October 2012. Mr. Schelling also founded Censa Pharmaceuticals Inc. in 2015 and currently serves as a director. He has also served as a director at Cascade Prodrug, Inc. since June 2017. 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 and history from Carroll College.

William T. Andrews, M.D., FACP has served as our Chief Medical Officer since October 2017. Prior to joining Acer, Dr. Andrews provided strategic consulting services to rare disease companies from April 2016 to September 2017. Prior to that, he served at 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 20 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.

Donald R. Joseph has served as our Chief Legal Officer and Secretary since April 2018. He previously served as an advisor and consultant to biopharmaceutical and global health organizations. He has over twenty years of biopharmaceutical industry experience, including senior management positions in global health non-profit organizations. Mr. Joseph served as Chief Legal Officer and Board Secretary of Humanigen (previously known as KaloBios Pharmaceuticals, Inc.), a publicly listed company, from June 2013 to November 2015. Prior to Humanigen, he was Chief Executive Officer of BIO Ventures for Global Health, or BVGH, from February to November 2012 and Chief Operating Officer from April 2010 to January 2012. He is a former Chairman and Secretary and former member of the BVGH Board of Directors. He previously served as General Counsel, Corporate Secretary, and in other senior management roles at publicly held biopharmaceutical companies, including Abgenix and Renovis. Mr. Joseph has served since August 2017 as lead independent director of Achieve Life Sciences, a publicly traded pharmaceutical company. Before entering the life sciences industry, Mr. Joseph practiced business law for a number of years in major firms, including as an international partner at Baker & McKenzie. He received his J.D. degree from the University of Texas School of Law, with honors.

John Klopp has served as our Chief Technical Officer since September 2019. He previously served as our Vice President, Manufacturing from January 2018 to September 2019. Prior to joining Acer, Mr. Klopp served as Senior Director, Manufacturing Management for Ultragenyx Pharmaceutical, Inc, a biopharmaceutical company, where he worked from March 2013 to January 2018. Mr. Klopp holds a B.S. degree in chemistry from Pennsylvania State University and an M.S. in chemistry from the University of California, Berkeley.

Harry S. Palmin has served as our Chief Financial Officer since the completion of the Merger in September 2017 and was appointed to the additional position of Chief Operating Officer in September 2018. From December 2013 to February 2016, Mr. Palmin served as the President, Chief Executive Officer and a director of Private Acer, and 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 an M.A. in international economics and finance from the Brandeis University International Business School.

Matthew T. Seibt has served as our Chief Commercial Officer since September 2019. He previously served as our acting Chief Commercial Officer from March 2019 to September 2019 and as our Vice President, Market Access and Reimbursement from April 2018 to March 2019. Prior to joining Acer, Mr. Seibt served as Director, Account Management, Market Access, and Reimbursement at Biogen Inc., a biotechnology company, from December 2014 to April 2018. Mr. Seibt holds a B.A. in Economics and Government from the University of Texas.

Directors

All of the current directors serve until the next annual stockholders' meeting or until their successors have been duly elected and qualified. The current members of the Board of Directors and their ages as of March 1, 2020 are as follows:

Name	Age	Position
Stephen J. Aselage	68	Chairman of the Board
Jason Amello	51	Director
John M. Dunn	68	Director
Michelle Griffin	54	Director
Chris Schelling	44	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. From October 2015 until the Merger, Mr. Aselage served as the Chairman of Private Acer's Board of Directors. Most recently, he was President and Chief Executive Officer of Retrophin, Inc., a Nasdaq-listed, biopharmaceutical company, from November 2014 until his retirement in January 2019, and remains 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 Pharmaceutical Inc., a Nasdaq-listed biotechnology company, 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 Nasdaq-listed biopharmaceutical company. From May 2012 to May 2013, he served as Executive Vice President, Chief Financial Officer and Treasurer of ZIOPHARM Oncology, Inc., a biopharmaceutical company. From April 2000 to June 2011, Mr. Amello held various positions at Genzyme Corporation, a biotechnology company, 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.

John M. Dunn has served as a director since the completion of the Merger in September 2017. From October 2015 until the Merger, Mr. Dunn served as a member of Private Acer's Board of Directors. From November 2014 to April 2019, he served as General Counsel of Vital Therapies, Inc., a Nasdaq-listed 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 has served as a director of Sharp Healthcare, a nonprofit regional health care delivery system, since 2019. 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 has served as a member of the Board of Directors and as Chair of the Audit Committee for publicly traded companies Adaptive Biotechnologies Corporation since March 2019 and for HTG Molecular Diagnostics, Inc. since August 2018. Ms. Griffin previously served as a member of the Board of Directors and as Chair of the Audit Committee for publicly traded companies 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. During various periods from 1997 to 2011, she served in the capacity of Chief Financial Officer for Trubion Pharmaceuticals, Inc., Dendreon Corporation and Corixa Corporation. Ms. Griffin earned a B.S. in marketing from George Mason University and an M.B.A. with a specialization in finance and international business from Seattle University.

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 of Directors, 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 of Directors 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 of Directors has determined that Ms. Griffin and Messrs. Amello and Dunn each, individually, qualifies as an "audit committee financial expert" as defined in Securities and Exchange Commission ("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.

Delinquent Reports Section 16(a)

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, 2019 except one Form 4 reporting the grant of an employee stock option for each of the following executive officers that was due on September 20, 2019 and was filed on September 23, 2019: Harry S. Palmin, William T. Andrews, Donald Joseph, and Matthew Seibt; and one Form 4 that was due on September 20, 2019 and filed on September 25, 2019 for John M. Klopp.

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 years ended December 31, 2019 and 2018.

Summary Compensation Table

Name and Principal Position	Year	Salary ($)	Bonus($)	Option Awards ($)(1)	Stock Awards ($)(1)	All Other Compensation ($)	Total ($)
Chris Schelling	2019	$ 436,000	$ —	$ 1,031,211 (2)	$ —	$ —	$ 1,467,211
President and Chief Executive Officer	2018	$ 400,000	$ 150,000 (4)	$ —	$ —	$ —	$ 550,000
William T. Andrews, M.D., FACP	2019	$ 412,000	$ —	$ 462,041 (2)	$ 212,400 (3)	$ —	$ 1,086,441
Chief Medical Officer	2018	$ 400,000	$ 105,000 (4)	$ —	$ —	$ —	$ 505,000
Harry S. Palmin	2019	$ 382,400	$ —	$ 462,041 (2)	$ —	$ —	$ 844,441
Chief Operating Officer and Chief Financial Officer	2018	$ 340,000	$ 89,250 (4)	$ —	$ —	$ —	$ 429,250

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

(2) Option awards were granted (i) on February 1, 2019 to Dr. Andrews and Messrs. Schelling and Palmin, and (ii) on September 18, 2019 to Dr. Andrews and Mr. Palmin, each with an exercise price equal to the closing market price of our common stock on the Nasdaq Capital Market on the date of grant. The options are time-based. The February 1 option grants 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 September 18 option grants vest 50% on January 1, 2021 and 50% on January 1, 2022, 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 2018 Stock Incentive Plan), but only to the extent that the optionee remains in service immediately prior to such Change in Control.

(3) We issued Dr. Andrews a restricted stock unit representing 9,000 shares of common stock on March 1, 2019, which vested in full on March 1, 2020 and settled on March 2, 2020.

(4) Represents bonuses earned during 2018 and paid during 2019.

Narrative Disclosure to Summary Compensation Table

Our Board of Directors reviews compensation annually for all of the executive officers. Compensation awarded to Named Executive Officers in 2019 and 2018 generally consisted of base salary and equity awards for options to purchase shares of our common stock or restricted stock units. In setting executive compensation, our Board of Directors retained the services of Radford (which is a part of Aon Hewitt, a business unit of Aon plc) as an independent compensation consultant and considered compensation for comparable positions in the market, the historical compensation levels of the 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 stockholders, and a long-term commitment to our company. We do not target a specific competitive position or a specific mix of compensation among elements of compensation. Prior to the September 2017 Merger, Private Acer retained the services of Radford 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 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 Capital Market and concluded that no conflict of interest exists that would prevent Radford from independently advising the Compensation Committee.

Outstanding Equity Awards at Fiscal Year-End

The following table presents information regarding outstanding equity awards at December 31, 2019 for each of the Named Executive Officers.

		Option Awards					Stock Awards		
Name	Grant Date	Number of Securities Underlying Unexercised Options (#) Exercisable	Number of Securities Underlying Unexercised Options (#) Unexercisable		Option Exercise Price ($)	Option Expiration Date	Number of shares or units of stock that have not vested (#)		Market value of shares or units of stock that have not vested ($)
Chris Schelling	2/1/19	—	72,500	(1)	$ 24.46	2/1/2029	—		—
	10/4/17	23,000	23,000	(1)	$ 15.34	10/4/2027	—		—
William T. Andrews, M.D., FACP	9/18/19	—	40,000	(2)	$ 3.42	9/18/2029	—		—
	2/1/19	—	27,000	(1)	$ 24.46	2/1/2029	—		—
	10/4/17	50,000	50,000	(1)	$ 15.34	10/4/2027	—		—
	3/1/19	—	—		—	—	9,000	(3)	$ 36,090 (4)
Harry S. Palmin	9/18/19	—	40,000	(2)	$ 3.42	9/18/2029	—		—
	2/1/19	—	27,000	(1)	$ 24.46	2/1/2029	—		—
	10/4/17	33,800	33,800	(1)	$ 15.34	10/4/2027	—		—

(1) These 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 and our 2018 Stock Incentive Plan, as applicable), but only to the extent that the optionee remains in service immediately prior to such Change in Control.

(2) These options are time-based and 50% vest on January 1, 2021 and 50% on January 1, 2022, 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 and our 2018 Stock Incentive Plan, as applicable), but only to the extent that the optionee remains in service immediately prior to such Change in Control.

(3) The restricted stock units vested in full on March 1, 2020.

(4) The market values of the unvested RSUs are calculated by multiplying the number of units shown in the table by $4.01, the closing price of our common stock on December 31, 2019.

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, 2019.

Name	Fees Earned or Paid in Cash ($)	Option Awards ($) (1)(2)	All Other Compensation ($)	Total ($)
Jason Amello	42,500	85,342	—	127,842
Stephen J. Aselage	73,750	85,342	—	159,092
Hubert Birner, Ph.D., MBA (3)	—	—	—	—
John M. Dunn	50,000	85,342	—	135,342
Michelle Griffin	55,000	85,342	—	140,342
Luc Marengere, Ph.D. (3)	—	—	—	—

(1) Amounts shown in this column represent the aggregate grant date fair value of stock option awards made during 2019, calculated in accordance with ASC Topic 718. See Note 2 to our consolidated 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, 2019 was: Mr. Amello, 15,000 shares; Mr. Aselage, 33,000 shares; Dr. Birner, 0 shares; Mr. Dunn, 28,000 shares; Ms. Griffin, 15,000 shares; and Dr. Marengere, 0 shares.

(3) Drs. Birner and Marengere did not stand for reelection at our 2019 annual meeting of stockholders. Drs. Birner and Marengere are each affiliated with TVM Capital Life Sciences and did not receive compensation for their service on our Board of Directors, which ended on May 17, 2019.

Standard Compensation Arrangements

Independent directors 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 of Directors. We reimburse our directors for travel and lodging expenses in connection with their attendance at Board of Directors 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 2018 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 2018 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, 2020, 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, 2020, there were 10,095,176 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 number of shares is deemed to include the number 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	2,672,309	(2)	26.5%
Nantahala Capital Management, LLC	990,655	(3)	9.8%
Bukwang Pharmaceutical Co. Ltd.	544,572	(4)	5.4%
Executive Officers and Directors:			
Chris Schelling	1,796,875	(5)	17.7%
William T. Andrews, M.D., FACP	78,250	(6)	*
Harry S. Palmin	174,000	(7)	1.7%
Jason Amello	14,250	(8)	*
Stephen J. Aselage	51,905	(9)	*
John M. Dunn	33,952	(10)	*
Michelle Griffin	14,250	(11)	*
All current directors and executive officers as a group (10 persons)	2,231,486	(12)	21.4%

* 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 Amendment No. 1 to Schedule 13D filed with the SEC on August 24, 2018. Consisting of shares of common stock beneficially owned by certain investment funds affiliated with TVM Capital Life Science as follows: (i) 1,697,709 shares of common stock held by TVM Life Science Ventures VII L.P. ("TVM VII LP"); (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 LP, TVM LSV VII (GP) Ltd. ("TVM VII GP") is the general partner of TVM VII LP. Luc Marengere, Reshentha Beeby, 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, and Leatt and Ms. Beeby each disclaim beneficial ownership of the reported securities, other than those shares which the reporting person

owns of record. The address of TVM VII LP 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.

(3) This information is based on a Schedule 13G/A filed with the SEC on February 12, 2020. Pursuant to the Schedule 13G/A, Nantahala Capital Management, LLC ("Nantahala") and each of Wilmot B. Harkey and Daniel Mack, as managing members of Nantahala, share voting and investment power with respect to the shares, which are held by funds and separately managed accounts under control of Nantahala. The address for Nantahala and Messrs. Harkey and Mack is 130 Main Street, 2nd Floor, New Canaan, Connecticut 06840.

(4) This information is based on confirmation provided by the stockholder 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.

(5) Consisting of (i) 1,750,000 shares of common stock; and (ii) 46,875 shares of common stock underlying stock options exercisable within 60 days of March 1, 2020.

(6) Consisting of (i) 69,250 shares of common stock underlying stock options exercisable within 60 days of March 1, 2020; and (ii) 9,000 restricted stock units which vested on March 1, 2020.

(7) Consisting of (i) 125,000 shares of common stock; and (ii) 49,000 shares of common stock underlying stock options exercisable within 60 days of March 1, 2020.

(8) Represents shares of common stock underlying stock options exercisable within 60 days of March 1, 2020.

(9) Consisting of (i) 18,905 shares of common stock; and (ii) 33,000 shares of common stock underlying stock options exercisable within 60 days of March 1, 2020.

(10) Consisting of (i) 5,952 shares of common stock; and (ii) 28,000 shares of common stock underlying stock options exercisable within 60 days of March 1, 2020.

(11) Represents shares of common stock underlying stock options exercisable within 60 days of March 1, 2020.

(12) Consisting of: (i) 1,899,857 shares of common stock; (ii) 322,629 shares of common stock underlying stock options exercisable within 60 days of March 1, 2020; and (iii) 9,000 restricted stock units which vested on March 1, 2020.

Securities Authorized for Issuance Under Equity Compensation Plans

The following table sets forth information, as of December 31, 2019, with respect to our compensation plans under which common stock is authorized for issuance. These plans consist of our 2018 Stock Incentive Plan, our 2013 Stock Incentive Plan, and our 2010 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 and Vesting of Restricted Stock Units (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	1,322,475	$ 12.28	162,004
Equity Compensation Plans Not Approved by Stockholders	—	—	—
Total	1,322,475	$ 12.28	162,004

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

Transactions with Related Persons

Since January 1, 2019, 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 immediate family members.

Director Independence

The Board of Directors determined that Ms. Griffin 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 of Directors. The Board of Directors 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 of Directors' 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	Audit Committee	Compensation Committee	Nominating and Corporate Governance Committee
Jason Amello	X		
Stephen J. Aselage		C	X
John M. Dunn	X		C
Michelle Griffin	C	X	

C = Chair

Item 14. Principal Accountant Fees and Services.

The following table presents (i)) the aggregate fees billed to us for the fiscal year ended December 31, 2019 by BDO USA, LLP ("BDO"), who was appointed as our independent registered public accounting firm on March 7, 2019, and (ii) the aggregate fees billed to us for the fiscal years ended December 31, 2019 and 2018 by Wolf and Company, P.C. ("Wolf"), who served as our independent registered public accounting firm during the period September 20, 2017 to March 7, 2019.

	Years Ended December 31,	
	2019	2018
	BDO	Wolf
Audit fees(1)	$ 283,128	$ 113,000
Audit-related fees (2)	—	58,000
Tax fees (3)	—	—
All other fees	—	—
Total fees	$ 283,128	$ 171,000

(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, services that are normally provided in connection with statutory and regulatory filings or engagements, comfort letters, reports on an issuer's internal controls, and review of documents to be filed with the SEC (e.g. periodic filings, registration statements, and company responses to SEC comment letters).

(2) Audit-related fees are related to other assurance and related services that are traditionally performed by an independent accountant such as employee benefit plan audits, due diligence related to mergers and acquisitions, accounting assistance and audits in connection with proposed or consummated acquisitions, attest services that are not required by statute or regulation, and consultations concerning proposed accounting and reporting standards.

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

Change in Independent Registered Public Accounting Firm

On March 7, 2019, we engaged BDO as our independent registered public accounting firm to audit our financial statements for the fiscal year ended December 31, 2019, and we dismissed Wolf. The decision to change accountants was approved by the Audit Committee of our Board of Directors.

During the year ended December 31, 2018, there were no: (1) disagreements (as defined in Item 304(a)(1)(iv) of Regulation S-K and the related instructions) with Wolf 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 Wolf would have caused Wolf 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, 2019 and 2018, neither we nor anyone on our behalf consulted with BDO 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 BDO 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 of Directors' 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 of Directors 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 of Directors 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	Certificate of Incorporation, as filed with the Delaware Secretary of State on May 15, 2018 (incorporated by reference to Exhibit 3.3 to the Company's Current Report on Form 8-K filed on May 15, 2018).
3.2	Bylaws, effective May 15, 2018 (incorporated by reference to Exhibit 3.4 to the Company's Current Report on Form 8-K filed on May 15, 2018).
3.3	Certificate of Ownership and Merger, as filed with the Delaware Secretary of State on May 15, 2018 (incorporated by reference to Exhibit 3.5 to the Company's Current Report on Form 8-K filed on May 15, 2018).
4.1*	Description of the Company's capital stock registered pursuant to Section 12 of the Securities Exchange Act of 1934.
4.2	Specimen common stock certificate (incorporated by reference to Exhibit 4.1 to the Company's Current Report on Form 8-K filed on May 15, 2018).
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).
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 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 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+	Acer Therapeutics Inc. 2018 Stock Incentive Plan (incorporated by reference to Appendix G to the Company's Definitive Proxy Statement on Schedule 14A filed on April 9, 2018).
10.7+	Form of Notice of Stock Option Grant and Stock Option Agreement for option awards to be made under the 2018 Stock Incentive Plan (incorporated by reference to Exhibit 10.3 to the Company's Current Report on Form 8-K filed on May 15, 2018).
10.8+	Form of Notice of Restricted Stock Unit Award and Restricted Stock Unit Agreement for awards under the 2018 Stock Incentive Plan (incorporated by reference to Exhibit 10.8 to the Company's Annual Report on Form 10-K for the year ended December 31, 2019).
10.9♦	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).

Exhibit No.	Description
10.10♦	License Agreement for Development and Exploitation, dated as of September 19, 2018, by and between Acer Therapeutics Inc. and Assistance Publique – Hôpitaux de Paris (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed on November 9, 2018).
10.11♦	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).
10.12	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).
10.13	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).
10.14	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).
10.15	Exclusive License Agreement, dated as of December 21, 2018, by and between Acer Therapeutics Inc. and Sanofi (incorporated by reference to Exhibit 99.1 to the Company to the Company's Current Report on Form 8-K filed on January 2, 2019.)
10.16+	Employment Agreement, dated February 22, 2018, by and between Acer Therapeutics Inc. and Chris Schelling (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K filed on February 27, 2018).
10.17+	Employment Agreement, dated February 22, 2018, by and between Acer Therapeutics Inc. and William Andrews (incorporated by reference to Exhibit 10.2 to the Company's Current Report on Form 8-K filed on February 27, 2018).
10.18+	Employment Agreement, dated February 22, 2018, by and between Acer Therapeutics Inc. and Harry Palmin (incorporated by reference to Exhibit 10.3 to the Company's Current Report on Form 8-K filed on February 27, 2018).
10.19+	Employment Agreement, dated April 20, 2018, by and between Acer Therapeutics Inc. and Donald Joseph (incorporated by reference to Exhibit 10.4 to the Company's Quarterly Report on Form 10-Q filed on May 14, 2018).
10.20+	Employment Agreement, dated September 18, 2019, by and between Acer Therapeutics Inc. and John Klopp (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed on November 13, 2019).
10.21+	Employment Agreement, dated September 18, 2019, by and between Acer Therapeutics Inc. and Matthew T. Seibt (incorporated by reference to Exhibit 10.2 to the Company's Quarterly Report on Form 10-Q filed on November 13, 2019).
10.22	Lease Agreement, dated March 6, 2018, by and between Acer Therapeutics Inc. and Commonwealth Development LLC, as trustee of the Gateway Realty Trust (incorporated by reference to Exhibit 10.5 to the Company's Quarterly Report on Form 10-Q filed on May 14, 2018).

Exhibit No.	Description
10.23	Lease Agreement, dated March 5, 2019, by and between Acer Therapeutics Inc. and Commonwealth Development LLC, as trustee of the Gateway Realty Trust (incorporated by reference to Exhibit 10.1 to the Company's Quarterly Report on Form 10-Q filed on August 13, 2019).
10.24	Triple Net Lease, dated April 1, 2018, by and between Acer Therapeutics Inc. and Eastern Western Corp. (incorporated by reference to Exhibit 10.6 to the Company's Quarterly Report on Form 10-Q filed on May 14, 2018).
10.25+	Form of Indemnification Agreement between the Company and its directors and officers (incorporated by reference to Appendix F to the Company's Definitive Proxy Statement on Schedule 14A filed on April 9, 2018).
10.26	Sales Agreement, dated November 9, 2018 by and between Acer Therapeutics Inc. and Roth Capital Partners, LLC (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K filed on November 9, 2018).
10.27*	Amended and Restated Sales Agreement, dated March 18, 2020 by and among Acer Therapeutics Inc., Roth Capital Partners, LLC, and JonesTrading Institutional Services LLC.
23.1*	Consent of Independent Registered Public Accounting Firm BDO USA, LLP.
23.2*	Consent of Independent Registered Public Accounting Firm Wolf & Company, P.C.
24.1*	Power of Attorney (see the signature page hereof).
31.1*	Certification of Principal Executive Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2*	Certification of Principal Financial Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1**	Certification of Principal Executive Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2**	Certification of Principal 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, 2019, 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; (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.

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

** In accordance with Item 601(b)(32)(ii) of Regulation S-K and SEC Release No. 34-47986, the certifications furnished in Exhibits 32.1 and 32.2 hereto are deemed to accompany this Form 10-K and will not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934. Such exhibits will not be deemed to be incorporated by reference into any filing under the Securities Act of 1933 or the Securities Exchange Act of 1934.

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 18, 2020

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

POWER OF ATTORNEY

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Chris Schelling and Harry Palmin, and each of them, his or her true and lawful attorneys-in-fact, each with full power of substitution, for him or her in any and all capacities, to sign any amendments to this report on Form 10-K and to file the same, with exhibits thereto and other documents in connection therewith, with the Securities and Exchange Commission, hereby ratifying and confirming all that each of said attorneys-in-fact or their substitute or substitutes may do or cause to be done by virtue hereof.

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 18, 2020
/s/ Harry Palmin Harry Palmin	Chief Operating Officer and Chief Financial Officer *(Principal Financial and Accounting Officer)*	March 18, 2020
/s/ Jason Amello Jason Amello	Director	March 18, 2020
/s/ Stephen J. Aselage Stephen J. Aselage	Chairman of the Board	March 18, 2020
/s/ John M. Dunn John M. Dunn	Director	March 18, 2020
/s/ Michelle Griffin Michelle Griffin	Director	March 18, 2020

ACER THERAPEUTICS INC.
DESCRIPTION OF SECURITIES REGISTERED PURSUANT TO
SECTION 12 OF THE SECURITIES EXCHANGE ACT OF 1934

Acer Therapeutics Inc., a Delaware corporation ("we", "us" or "our"), has one class of securities registered pursuant to Section 12 of the Securities Exchange Act of 1934: our common stock, $0.0001 par value per share. The general terms and provisions of our common stock are summarized below. This summary does not purport to be complete and is qualified in its entirety by reference to our certificate of incorporation and our bylaws, each of which has been filed as an exhibit to our most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission ("SEC"), as may be amended by a document filed with one of our periodic reports filed with the SEC subsequent to the date of that Annual Report.

Common Stock

We are authorized to issue 150,000,000 shares of common stock. Each holder of common stock is entitled to one vote for each share of common stock held on all matters submitted to a vote of stockholders. We have not provided for cumulative voting for the election of directors in our certificate of incorporation. This means that the holders of a majority of the shares voted can elect all of the directors then standing for election. Subject to preferences that may apply to shares of preferred stock outstanding at the time, the holders of outstanding shares of our common stock are entitled to receive dividends out of assets legally available at the times and in the amounts that our board of directors may determine from time to time. Upon our liquidation, dissolution or winding-up, the holders of common stock are entitled to share ratably in all assets remaining after payment of all liabilities and the liquidation preferences of any outstanding preferred stock. Holders of common stock have no preemptive or conversion rights or other subscription rights. There are no redemption or sinking fund provisions applicable to the common stock. All outstanding shares of common stock are fully paid and nonassessable.

Anti-Takeover Effects of Delaware Law and Our Certificate of Incorporation and Bylaws

Certain provisions of Delaware law, our certificate of incorporation and our bylaws could have the effect of delaying, deferring or discouraging another party from acquiring control of us.

Certificate of Incorporation and Bylaws. Our certificate of incorporation and bylaws include provisions that:

- authorize the board of directors to issue, without stockholder approval, up to 10,000,000 shares of preferred stock with such designations, powers, preferences and other rights and qualifications, limitations or restrictions as our board of directors may authorize, which preferred stock could decrease the amount of earnings and assets available for distribution to holders of our common stock or adversely affect the rights and powers, including voting rights, of the holders of our common stock;
- establish advance notice requirements for stockholder nominations of directors and for stockholder proposals that can be acted on at stockholder meetings;
- limit who may call stockholder meetings;
- require that any action to be taken by our stockholders be effected at a duly called annual or special meeting and not by written consent;
- provide that vacancies on our board of directors may be filled only by a majority of directors then in office, even if less than a quorum;
- require a super-majority of votes to amend certain provisions of our charter as well as to amend our bylaws generally;
- authorize us to indemnify officers and directors against losses that they may incur in investigations and legal proceedings resulting from their services to us, which may include services in connection with takeover defense measures; and

- establish the Court of Chancery of the State of Delaware as the sole and exclusive forum for certain derivative actions or proceeding brought on our behalf, any action asserting a claim of breach of fiduciary duty, any action asserting a claim against us arising pursuant to the General Corporation Law of the State of Delaware (the "DGCL"), or any action asserting a claim governed by the internal affairs doctrine.

Delaware anti-takeover statute. We are subject to the provisions of Section 203 of the DGCL regulating corporate takeovers. In general, Section 203 prohibits a publicly-held Delaware corporation from engaging, under certain circumstances, in a business combination with an interested stockholder for a period of three years following the date the person became an interested stockholder unless:

- prior to the date of the transaction, the board of directors of the corporation approved either the business combination or the transaction which resulted in the stockholder becoming an interested stockholder; or
- upon completion of the transaction that resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction commenced, excluding for purposes of determining the voting stock outstanding, but not the outstanding voting stock owned by the interested stockholder, (1) shares owned by persons who are directors and also officers and (2) shares owned by employee stock plans in which employee participants do not have the right to determine confidentially whether shares held subject to the plan will be tendered in a tender or exchange offer; or at or subsequent to the date of the transaction, the business combination is approved by the board of directors of the corporation and authorized at an annual or special meeting of stockholders, and not by written consent, by the affirmative vote of at least 66-2/3% of the outstanding voting stock which is not owned by the interested stockholder.

Generally, a "business combination" includes a merger, asset or stock sale, or other transaction resulting in a financial benefit to the "interested stockholder" and an "interested stockholder" is a person who, together with affiliates and associates, owns or, within three years prior to the determination of interested stockholder status, did own 15% or more of a corporation's outstanding voting stock.

Transfer Agent and Registrar

The transfer agent and registrar for our common stock is Continental Stock Transfer and Trust Company.

Listing

Our common stock is listed on The Nasdaq Stock Market under the symbol "ACER."

ACER THERAPEUTICS INC.

$50,000,000

Common Stock
($0.0001 par value per share)

Amended and Restated Sales Agreement

March 18, 2020

Roth Capital Partners, LLC
888 San Clemente Drive, Suite 400
Newport Beach, CA 92660

JonesTrading Institutional Services LLC
757 Third Avenue, 23rd Floor
New York, NY 10017

Ladies and Gentlemen:

Acer Therapeutics Inc., a Delaware corporation (the "Company") and Roth Capital Partners, LLC ("Roth") are parties to that certain Sales Agreement dated November 9, 2018 (the "Original Sales Agreement"). The Company and Roth, together with JonesTrading Institutional Services LLC ("JonesTrading" and together with Roth, the "Agents") desire to amend and restate the Original Sales Agreement with this agreement (the "Agreement"), and hereby agree as follows:

1. Issuance and Sale of Shares. The Company agrees that, from time to time during the term of this Agreement, on the terms and subject to the conditions set forth herein, it may issue and sell through or to the Agents, shares (the "Placement Shares") of common stock of the Company, $0.0001 par value per share (the "Common Stock"), having an aggregate offering price of up to $50,000,000, *provided, however*, that in no event shall the Company issue or sell through or to the Agents such number of Placement Shares that (a) exceeds the number of shares or dollar amount of shares of Common Stock that may be sold pursuant to the Registration Statement (as defined below), or (b) exceeds the number of authorized but unissued shares of Common Stock of the Company (the "Maximum Amount"). Notwithstanding anything to the contrary contained herein, the parties hereto agree that compliance with the limitations set forth in this Section 1 on the amount of Placement Shares issued and sold under this Agreement shall be the sole responsibility of the Company and that Agents shall have no obligation in connection with such compliance. The issuance and sale of Placement Shares through or to the Agents will be effected pursuant to the Registration Statement (as defined below) filed by the Company and declared effective by the Securities and Exchange Commission (the "Commission"), although nothing in this Agreement shall be construed as requiring the Company to use the Registration Statement to issue any Placement Shares.

The Company has filed or will file, in accordance with the provisions of the Securities Act of 1933, as amended, and the rules and regulations thereunder (the "Securities Act"), with the Commission a registration statement on Form S-3, including a base prospectus relating to certain securities, including the Common Stock, to be issued from time to time by the Company, and which incorporates by reference documents that the Company has filed or will file in accordance with the provisions of the Securities Exchange Act of 1934, as amended, and the rules and regulations thereunder (the "Exchange Act"). The Company has prepared a prospectus supplement specifically relating to the Placement Shares (the "Prospectus Supplement") to the base prospectus included as part of such registration statement. The Company will furnish to the Agents, for use by the Agents, copies of the base prospectus included as part of such registration statement, as supplemented by the Prospectus Supplement, if any, relating to the Placement Shares. Except where the context otherwise requires, such registration statement, and any post-effective amendment thereto, including all documents filed as part thereof or incorporated by reference therein, and including any information contained in a Prospectus (as defined below) subsequently filed with the Commission pursuant to Rule 424(b) under the Securities Act or deemed to be a part of such registration statement pursuant to Rule 430B of the Securities Act, or any subsequent registration statement on Form S-3 filed pursuant to Rule 415(a)(6) under the Securities Act by the Company to cover any Placement Shares, is herein called the "Registration Statement." The base prospectus, including all documents incorporated therein by reference, included in the Registration Statement, as it may be supplemented by the Prospectus Supplement, in the form in which such prospectus and/or Prospectus Supplement have most recently been filed by the Company with the Commission pursuant to Rule 424(b) under the Securities Act, together with any then issued Issuer Free Writing Prospectus (defined below), is herein called the "Prospectus." Any reference herein to the Registration Statement, the Prospectus or any amendment or supplement thereto, shall be deemed to refer to and include the documents incorporated or deemed to be incorporated by reference therein, and any reference herein to the terms "amend," "amendment" or "supplement" with respect to the Registration Statement or the Prospectus shall be deemed to refer to and include the filing after the execution hereof of any document with the Commission deemed to be incorporated by reference therein (the "Incorporated Documents"). For purposes of this Agreement, all references to the Registration Statement, the Prospectus or to any amendment or supplement thereto shall be deemed to include the most recent copy filed with the Commission pursuant to its Electronic Data Gathering Analysis and Retrieval System, or if applicable, the Interactive Data Electronic Application system when used by the Commission (collectively "EDGAR").

2. Placements. Each time that the Company wishes to issue and sell Placement Shares hereunder (each, a "Placement"), it will notify an Agent (the "Designated Agent") by email notice (or other method mutually agreed to in writing by the Parties) of the number or dollar value of Placement Shares, the time period during which sales are requested to be made, any limitation on the number of Placement Shares that may be sold in any one day and any minimum price below which sales may not be made (a "Placement Notice"), the form of which is attached hereto as Schedule 1. The Placement Notice shall originate from any of the individuals from the Company set forth on Schedule 3 (with a copy to each of the other individuals from the Company listed on such schedule), and shall be addressed to each of the individuals from the Designated Agent set forth on Schedule 3, as such Schedule 3 may be amended from time to time. The Placement Notice shall be effective unless and until (i) the Designated Agent declines to accept the terms contained therein for any reason, in its sole

discretion, (ii) the entire amount of the Placement Shares thereunder have been sold, (iii) the Company suspends or terminates the Placement Notice or (iv) the Agreement has been terminated under the provisions of Section 12. The amount of any discount, commission or other compensation to be paid by the Company to the Designated Agent in connection with the sale of the Placement Shares shall be calculated in accordance with the terms set forth in Schedule 2. It is expressly acknowledged and agreed that neither the Company nor the Designated Agent will have any obligation whatsoever with respect to a Placement or any Placement Shares unless and until the Company delivers a Placement Notice to the Designated Agent and the Designated Agent does not decline such Placement Notice pursuant to the terms set forth above, and then only upon the terms specified therein and herein. In the event of a conflict between the terms of this Agreement and the terms of a Placement Notice, the terms of the Placement Notice will control.

3. Sale of Placement Shares by the Agents Subject to the terms and conditions of this Agreement, the Designated Agent, for the period specified in the Placement Notice, will use its commercially reasonable efforts consistent with its normal trading and sales practices and applicable state and federal laws, rules and regulations and the rules of the Nasdaq Capital Market (the "Exchange"), to sell the Placement Shares up to the amount specified, and otherwise in accordance with the terms of such Placement Notice. The Designated Agent will provide written confirmation to the Company no later than the opening of the Trading Day (as defined below) immediately following the Trading Day on which it has made sales of Placement Shares hereunder setting forth the number of Placement Shares sold on such day, the compensation payable by the Company to the Designated Agent pursuant to Section 2 with respect to such sales, and the Net Proceeds (as defined below) payable to the Company, with an itemization of the deductions made by the Designated Agent (as set forth in Section 5(b)) from the gross proceeds that it receives from such sales. Subject to the terms of the Placement Notice, the Designated Agent may sell Placement Shares by any method permitted by law deemed to be an "at the market offering" as defined in Rule 415 of the Securities Act.

4. Suspension of Sales.

(a) The Company or the Designated Agent may, upon notice to the other party in writing (including by email correspondence to each of the individuals of the other party set forth on Schedule 3, if receipt of such correspondence is actually acknowledged by any of the individuals to whom the notice is sent, other than via auto-reply) or by telephone (confirmed immediately by verifiable facsimile transmission or email correspondence to each of the individuals of the other party set forth on Schedule 3), suspend any sale of Placement Shares; provided, however, that such suspension shall not affect or impair any party's obligations with respect to any Placement Shares sold hereunder prior to the receipt of such notice. Each party agrees that no such notice under this Section 4 shall be effective against any other party unless it is made to one of the individuals named on Schedule 3 hereto, as such Schedule may be amended from time to time.

(b) Notwithstanding any other provision of this Agreement, during any period in which the Company is in possession of material non-public information, the Company and the Agents agree that (i) no sale of Placement Shares will take place, (ii) the Company shall not

request the sale of any Placement Shares, and (iii) the Agents shall not be obligated to sell or offer to sell any Placement Shares.

5. Sale and Delivery to the Designated Agent; Settlement.

(a) Sale of Placement Shares. On the basis of the representations and warranties herein contained and subject to the terms and conditions herein set forth, upon the Designated Agent's acceptance of the terms of a Placement Notice, and unless the sale of the Placement Shares described therein has been declined, suspended, or otherwise terminated in accordance with the terms of this Agreement, the Designated Agent, for the period specified in the Placement Notice, will use its commercially reasonable efforts consistent with its normal trading and sales practices and applicable law and regulations and the rules of the Exchange to sell such Placement Shares up to the amount specified in such Placement Notice, and otherwise in accordance with the terms of such Placement Notice. The Company acknowledges and agrees that (i) there can be no assurance that the Designated Agent will be successful in selling Placement Shares, (ii) the Designated Agent will incur no liability or obligation to the Company or any other person or entity if it does not sell Placement Shares for any reason other than a failure by the Designated Agent to use its commercially reasonable efforts consistent with its normal trading and sales practices and applicable law and regulations and the rules of the Exchange to sell such Placement Shares as required under this Agreement and (iii) the Designated Agent shall be under no obligation to purchase Placement Shares on a principal basis pursuant to this Agreement, except as otherwise agreed by the Designated Agent and the Company.

(b) Settlement of Placement Shares. Unless otherwise specified in the applicable Placement Notice, settlement for sales of Placement Shares will occur on the second (2nd) Trading Day (or such earlier day as is industry practice for regular-way trading) following the date on which such sales are made (each, a "Settlement Date"). The amount of proceeds to be delivered to the Company on a Settlement Date against receipt of the Placement Shares sold (the "Net Proceeds") will be equal to the aggregate sales price received by the Designated Agent for the Placement Shares, after deduction for (i) the Designated Agent's commission, discount or other compensation for such sales payable by the Company pursuant to Section 2 hereof, and (ii) any transaction fees imposed by any governmental or self-regulatory organization in respect of such sales.

(c) Delivery of Placement Shares. On each Settlement Date, in return for payment of the Net Proceeds by the Designated Agent, the Company will, or will cause its transfer agent to, electronically transfer the Placement Shares being sold by crediting the Designated Agent's or its designee's account (provided the Designated Agent shall have given the Company written notice of such designee prior to the Settlement Date) at The Depository Trust Company through its Deposit and Withdrawal at Custodian System or by such other means of delivery as may be mutually agreed upon by the Company and the Designated Agent which in all cases shall be freely tradable, transferable, registered shares in good deliverable form. On each Settlement Date, the Designated Agent will deliver the related Net Proceeds in same day funds to an account designated by the Company on, or prior to, the Settlement Date. The Company agrees that if the Company, or its transfer agent (if applicable), defaults in its obligation to deliver Placement Shares on a Settlement Date the Company agrees that in

addition to and in no way limiting the rights and obligations set forth in Section 10(a) hereto, it will (i) hold the Designated Agent harmless against any loss, claim, damage, or reasonable, documented expense (including reasonable and documented legal fees and expenses), as incurred, arising out of or in connection with such default by the Company or its transfer agent (if applicable) and (ii) pay to the Designated Agent any commission, discount, or other compensation to which it would otherwise have been entitled absent such default.

(d) Limitations on Offering Size. Under no circumstances shall the Company cause or request the offer or sale of any Placement Shares if, after giving effect to the sale of such Placement Shares, the aggregate number or aggregate gross sales proceeds of Placement Shares sold pursuant to this Agreement would exceed the lesser of (A) together with all sales of Placement Shares under this Agreement, the Maximum Amount, (B) the amount available for offer and sale under the Registration Statement and (C) the amount authorized from time to time to be issued and sold under this Agreement by the Company's board of directors, a duly authorized committee thereof or a duly authorized executive committee, and notified to the Designated Agent in writing. Under no circumstances shall the Company cause or request the offer or sale of any Placement Shares pursuant to this Agreement at a price lower than the minimum price authorized from time to time by the Company's board of directors, duly authorized committee thereof or a duly authorized executive committee, and notified to the Designated Agent in writing. Further, under no circumstances shall the Company cause or permit the aggregate offering amount of Placement Shares sold pursuant to this Agreement to exceed the Maximum Amount.

(e) Sales Through Agents. The Company agrees that any offer to sell, any solicitation of an offer to buy, or any sales of Placement Shares shall only be effected by or through an Agent, and only a single Agent, on any single given date, and in no event shall the Company request that more than one Agent sell Securities on the same day; provided however that, for the avoidance of doubt, (i) the foregoing limitation shall not apply to (A) exercise of any option, warrant, right or any conversion privilege set forth in the instruction governing such securities, (B) sales solely to employees, directors or security holders of the Company or its subsidiaries, or to a trustee or other person acquiring such securities for the accounts of such person and (ii) such limitation shall not apply (A) on any day during which no sales are made pursuant to this Agreement or (B) during a period in which the Company has notified the Agents that it will not sell Common Stock under this Agreement and (1) no Placement Notice is pending or (2) after a Placement Notice has been withdrawn.

6. Representations and Warranties of the Company. Except as disclosed in the Registration Statement or Prospectus (including the Incorporated Documents), the Company represents and warrants to, and agrees with each of the Agents that as of the date of this Agreement and as of each Applicable Time (as defined below), unless such representation, warranty or agreement specifies a different date or time:

(a) Registration Statement and Prospectus. The Company and, assuming no act or omission on the part of an Agent that would make such statement untrue, the transactions contemplated by this Agreement meet the requirements for and comply with the conditions for the use of Form S-3 under the Securities Act. The Registration Statement has been or will be filed with the Commission and has been or will be declared effective under the Securities Act

prior to the delivery of any Placement Notice by the Company. The Prospectus Supplement will name Roth and JonesTrading as the agents in the section entitle "Plan of Distribution." The Company has not received, and has no notice of, any order of the Commission preventing or suspending the use of the Registratio Statement, or threatening or instituting proceedings for that purpose. The Registration Statement and the offer and sale of Placement Shares as contemplate hereby meet the requirements of Rule 415 under the Securities Act and comply in all material respects with said Rule. Any statutes, regulations, contracts or othe documents that are required to be described in the Registration Statement or the Prospectus or to be filed as exhibits to the Registration Statement have been s described or filed. Copies of the Registration Statement, the Prospectus, and any such amendments or supplements and all documents incorporated by reference therein that were filed with the Commission on or prior to the date of this Agreement have been delivered, or are available through EDGAR, to Agents and thei counsel. The Company has not distributed and, prior to the later to occur of each Settlement Date and completion of the distribution of the Placement Shares, wi not distribute any offering material in connection with the offering or sale of the Placement Shares other than the Registration Statement and the Prospectus an any Issuer Free Writing Prospectus to which the Agents have consented, such consent not to be unreasonably withheld, conditioned or delayed. The Compan has not, in the 12 months preceding the date hereof, received notice from the Exchange to the effect that the Company is not in compliance with the listing o maintenance requirements. The Company has no reason to believe that it will not in the foreseeable future continue to be in compliance with all such listing and maintenance requirements.

(b) No Misstatement or Omission The Registration Statement, when it became or becomes effective, and the Prospectus, and any amendment or supplement thereto, on the date of such Prospectus or amendment or supplement, conformed and will conform in all materia respects with the requirements of the Securities Act. At each Settlement Date, the Registration Statement and the Prospectus, as of such date, will conform in a material respects with the requirements of the Securities Act. The Registration Statement, when it became or becomes effective, did not, and will not, contain a untrue statement of a material fact or omit to state a material fact required to be stated therein or necessary to make the statements therein not misleading. The Prospectus and any amendment or supplement thereto, on the date thereof and at each Applicable Time (defined below), did not and will not include an untrue statement of a material fact or omit to state a material fact necessary to make the statements therein, in light of the circumstances under which they were made, not misleading. The Incorporated Documents did not, and any further documents filed and incorporated by reference therein will not, when filed with the Commission contain an untrue statement of a material fact or omit to state a material fact required to be stated in such document or necessary to make the statements in such document, in light of the circumstances under which they were made, not misleading. The foregoing shall not apply to statements in, or omissions from, any such document made in reliance upon, and in conformity with, information furnished to the Company by an Agent specifically for use in the preparation thereof.

(c) Conformity with Securities Act and Exchange Act The Registration Statement, the Prospectus, any Issue Free Writing Prospectus or any amendment or supplement thereto, and the Incorporated Documents, when such documents were or are filed with the Commission under the Securities Act or the Exchange Act or became or become effective under

the Securities Act, as the case may be, conformed and will conform in all material respects with the requirements of the Securities Act and the Exchange Act, as applicable.

(d) Financial Information. The financial statements of the Company included or incorporated by reference in the Registration Statement, the Prospectus and the Issuer Free Writing Prospectuses, if any, together with the related notes and schedules, present fairly, in all material respects, the financial position of the Company as of the dates indicated and the results of operations, cash flows and changes in stockholders' equity of the Company for the periods specified and have been prepared in compliance with the requirements of the Securities Act and Exchange Act and in conformity with generally accepted accounting principles in the United States ("GAAP") applied on a consistent basis (except for (i) such adjustments to accounting standards and practices as are noted therein, (ii) in the case of unaudited interim financial statements, to the extent such financial statements may not include footnotes required by GAAP or may be condensed or summary statements and (iii) such adjustments which will not be material, either individually or in the aggregate) during the periods involved; the other financial and statistical data with respect to the Company contained or incorporated by reference in the Registration Statement, the Prospectus and the Issuer Free Writing Prospectuses, if any, are accurately and fairly presented in all material respects and prepared on a basis consistent with the financial statements and books and records of the Company; there are no financial statements (historical or pro forma) that are required to be included or incorporated by reference in the Registration Statement, or the Prospectus that are not included or incorporated by reference as required; the Company does not have any material liabilities or obligations, direct or contingent (including any off-balance sheet obligations), not described in the Registration Statement (excluding the exhibits thereto), and the Prospectus; and all disclosures contained or incorporated by reference in the Registration Statement, the Prospectus and the Issuer Free Writing Prospectuses, if any, regarding "non-GAAP financial measures" (as such term is defined by the rules and regulations of the Commission) comply in all material respects with Regulation G of the Exchange Act and Item 10 of Regulation S-K under the Securities Act, to the extent applicable.

(e) Conformity with EDGAR Filing. The Prospectus delivered to the Agents for use in connection with the sale of the Placement Shares pursuant to this Agreement after the date hereof will be identical to the versions of the Prospectus created to be transmitted to the Commission for filing via EDGAR, except to the extent permitted by Regulation S-T.

(f) Organization. The Company is duly organized, validly existing as a corporation and in good standing under the laws of its jurisdiction of organization. The Company is, and will be, duly licensed or qualified as a foreign corporation for transaction of business and in good standing under the laws of each other jurisdiction in which its ownership or lease of property or the conduct of its business requires such license or qualification, and has all corporate power and authority necessary to own or hold its properties and to conduct its business as described in the Registration Statement and the Prospectus, except where the failure to be so qualified or in good standing or have such power or authority would not, individually or in the aggregate, have a material adverse effect on or affecting the assets, business, operations, earnings, properties, condition (financial or otherwise), prospects, stockholders' equity or results of operations of the Company or prevent or materially interfere with consummation of the transactions contemplated hereby (a "Material Adverse Effect").

(g) Subsidiaries. As of the date hereof, the Company has no subsidiaries. The Company does not own or control, directly or indirectly, any corporation, association or other entity other than the subsidiaries listed in Exhibit 21.1 to the Company's most recent Annual Report on Form 10-K, if any. The Company owns directly or indirectly, all of the equity interests of its subsidiaries, if any, free and clear of any lien, charge, security interest, encumbrance, right of first refusal or other restriction, and all the equity interests of its subsidiaries, if any, are validly issued and are fully paid, non-assessable and free of preemptive and similar rights.

(h) No Violation or Default. The Company is not (i) in violation of its charter or by-laws or similar organizational documents; (ii) in default, and no event has occurred that, with notice or lapse of time or both, would constitute such a default, in the due performance or observance of any term, covenant or condition contained in any indenture, mortgage, deed of trust, loan agreement or other agreement or instrument to which the Company is a party or by which the Company is bound or to which any of the property or assets of the Company is subject; or (iii) in violation of any law or statute or any judgment, order, rule or regulation of any court or arbitrator or governmental or regulatory authority, except, in the case of each of clauses (ii) and (iii) above, for any such violation or default that would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect. To the Company's knowledge, no other party under any material contract or other agreement to which it is a party is in default in any respect thereunder where such default would reasonably be expected to have a Material Adverse Effect.

(i) No Material Adverse Effect. Subsequent to the respective dates as of which information is given in the Registration Statement, the Prospectus and the Issuer Free Writing Prospectuses, if any (including any document deemed incorporated by reference therein) there has not been (i) any Material Adverse Effect, (ii) any transaction which is material to the Company, (iii) any obligation or liability, direct or contingent (including any off-balance sheet obligations), incurred by the Company which is material to the Company, (iv) any material change in the capital stock or outstanding long-term indebtedness (other than (A) the grant of additional awards under equity incentive plans, (B) changes in the number of outstanding Common Stock due to the issuance of shares upon exercise or conversion of securities exercisable for or convertible into Common Stock outstanding on the date hereof, (C) any repurchase of capital stock of the Company, (D) as a result of the sale of Placement Shares, or (E) other than as publicly reported or announced), or (v) any dividend or distribution of any kind declared, paid or made on the capital stock of the Company other than in each case above in the ordinary course of business or as otherwise disclosed in the Registration Statement or Prospectus (including any document deemed incorporated by reference therein).

(j) Capitalization. The issued and outstanding shares of capital stock of the Company have been validly issued, are fully paid and non-assessable and, other than as disclosed in the Registration Statement or the Prospectus, are not subject to any preemptive rights, rights of first refusal or similar rights. The Company has an authorized, issued and outstanding capitalization as set forth in the Registration Statement and the Prospectus as of the dates referred to therein (other than the grant of additional options and restricted stock units under the Company's existing stock option plans, or changes in the number of outstanding shares of Common Stock of the Company due to the issuance of shares upon the exercise or conversion of securities exercisable for, or convertible into, Common Stock outstanding on the date hereof) and

such authorized capital stock conforms to the description thereof set forth in the Registration Statement and the Prospectus. The description of the securities of the Company in the Registration Statement and the Prospectus is complete and accurate in all material respects. As of the date referred to therein, the Company does not have outstanding any options to purchase, or any rights or warrants to subscribe for, or any securities or obligations convertible into, or exchangeable for, or any contracts or commitments to issue or sell, any shares of capital stock or other securities.

(k) Authorization; Enforceability. The Company has full legal right, power and authority to enter into this Agreement and perform the transactions contemplated hereby. This Agreement has been duly authorized, executed and delivered by the Company and is a legal, valid and binding agreement of the Company enforceable against the Company in accordance with its terms, except (i) to the extent that enforceability may be limited by bankruptcy, insolvency, reorganization, moratorium or similar laws affecting creditors' rights generally and by general equitable principles and (ii) the indemnification and contribution provisions of Section 10 hereof may be limited by federal or state securities laws and public policy considered in respect thereof.

(l) Authorization of Placement Shares. The Placement Shares, when issued and delivered pursuant to the terms approved by the board of directors of the Company or a duly authorized committee thereof, against payment therefor as provided herein, will be duly and validly authorized and issued and fully paid and non-assessable, free and clear of any pledge, lien, encumbrance, security interest or other claim, including any statutory or contractual preemptive rights, resale rights, rights of first refusal or other similar rights, and will be registered pursuant to Section 12 of the Exchange Act. The Placement Shares, when issued, will conform in all material respects to the description thereof set forth in or incorporated into the Prospectus.

(m) No Consents Required. No consent, approval, authorization, order, registration or qualification of or with any court or arbitrator or governmental or regulatory authority having jurisdiction over the Company is required for the execution, delivery and performance by the Company this Agreement, the issuance and sale by the Company of the Placement Shares, except for such consents, approvals, authorizations, orders and registrations or qualifications as may be required under applicable state securities laws or by the by-laws and rules of the Financial Industry Regulatory Authority ("FINRA") or the Exchange, including any notices that may be required by the Exchange, in connection with the sale of the Placement Shares by the Agents.

(n) No Preferential Rights. (i) No person, as such term is defined in Rule 1-02 of Regulation S-X promulgated under the Securities Act (each, a "Person"), has the right, contractual or otherwise, to cause the Company to issue or sell to such Person any Common Stock or shares of any other capital stock or other securities of the Company (other than upon the exercise of outstanding options, warrants or other rights to purchase Common Stock, or upon the exercise of equity awards that may be granted from time to time under the Company's employee or director stock option or benefits plans, in each case as disclosed in the Registration Statement or Prospectus), (ii) no Person has any preemptive rights, resale rights, rights of first refusal, or any other rights (whether pursuant to a "poison pill" provision or otherwise) to purchase any

Common Stock or shares of any other capital stock or other securities of the Company, (iii) no Person has the right to act as an underwriter or as a financia advisor to the Company in connection with the offer and sale of Common Stock, and (iv) no Person has the right, contractual or otherwise, to require the Company to register under the Securities Act any Common Stock or shares of any other capital stock or other securities of the Company, or to include any such shares or other securities in the Registration Statement or the offering contemplated thereby, whether as a result of the filing or effectiveness of the Registration Statement or the sale of the Placement Shares as contemplated thereby or otherwise.

(o) Independent Public Accountant. The Company's independent accountants, whose report on the financial statements of the Company is filed with the Commission as part of the Company's most recent Annual Report on Form 10-K filed with the Commission and incorporated into the Registration Statement and the Prospectus, are and, during the periods covered by their report, were an independent registered public accounting firm with respect to the Company within the meaning of the Securities Act and the Public Company Accounting Oversight Board (United States). To the Company's knowledge, the Company's independent accountants are not in violation of the auditor independence requirements of the Sarbanes-Oxley Act of 2002 (the "Sarbanes-Oxley Act") with respect to the Company.

(p) Enforceability of Agreements. All agreements between the Company and third parties expressly referenced in the Prospectus are legal, valid and binding obligations of the Company enforceable against the Company in accordance with their respective terms, except to the extent that (i) enforceability may be limited by bankruptcy, insolvency, reorganization, moratorium or similar laws affecting creditors' rights generally and by general equitable principles and (ii) the indemnification provisions of certain agreements may be limited by federal or state securities laws or public policy considerations in respect thereof, and except for any unenforceability that, individually or in the aggregate, would not reasonably be expected to have a Material Adverse Effect.

(q) No Litigation. There are no legal, governmental or regulatory actions, suits or proceedings pending, nor, to the Company's knowledge, any legal, governmental or regulatory investigations, to which the Company is a party or to which any property of the Company is the subject that, individually or in the aggregate, if determined adversely to the Company would reasonably be expected to have a Material Adverse Effect or materially and adversely affect the ability of the Company to perform its obligations under this Agreement; to the Company's knowledge, no such actions, suits or proceedings are threatened or contemplated by any governmental or regulatory authority or threatened by others that, individually or in the aggregate, if determined adversely to the Company, would reasonably be expected to have a Material Adverse Effect; and (i) there are no current or pending legal, governmental or regulatory investigations, actions, suits or proceedings that are required under the Securities Act to be described in the Prospectus that are not so described; and (ii) there are no contracts or other documents that are required under the Securities Act to be filed as exhibits to the Registration Statement that are not so filed.

(r) Licenses and Permits. The Company possesses or has obtained, all licenses, certificates, consents, orders, approvals, permits and other authorizations issued by, and have made all declarations and filings with, the appropriate federal, state, local or foreign

governmental or regulatory authorities that are necessary for the ownership or lease of their respective properties or the conduct of their respective businesses as described in the Registration Statement and the Prospectus (the "Permits"), except where the failure to possess, obtain or make the same would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect. The Company has not received written notice of any proceeding relating to revocation or modification of any such Permit or has any reason to believe that such Permit will not be renewed in the ordinary course, except where the failure to obtain any such renewal would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect.

(s) No Material Defaults. The Company has not defaulted on any installment on indebtedness for borrowed money or on any rental on one or more long-term leases, which defaults, individually or in the aggregate, would reasonably be expected to have a Material Adverse Effect. The Company has not filed a report pursuant to Section 13(a) or 15(d) of the Exchange Act since the filing of its last Annual Report on Form 10-K, indicating that it (i) has failed to pay any dividend or sinking fund installment on preferred stock or (ii) has defaulted on any installment on indebtedness for borrowed money or on any rental on one or more long-term leases, which defaults, individually or in the aggregate, would reasonably be expected to have a Material Adverse Effect.

(t) S-3 Eligibility. (i) At the time of filing the Registration Statement and (ii) at the time of the most recent amendment thereto for the purposes of complying with Section 10(a)(3) of the Securities Act (whether such amendment was by post-effective amendment, incorporated report filed pursuant to Section 13 or 15(d) of the Exchange Act or form of prospectus), the Company met the then applicable requirements for use of Form S-3 under the Securities Act, including compliance with General Instruction I.B.6 of Form S-3. The aggregate market value of the outstanding voting and non-voting common equity (as defined in Securities Act Rule 405) of the Company held by persons other than affiliates of the Company (pursuant to Securities Act Rule 144, those that directly, or indirectly through one or more intermediaries, control, or are controlled by, or are under common control with, the Company) (the "Non-Affiliate Shares"), was equal to $32,364,839 (calculated by multiplying (x) the highest price at which the common equity of the Company closed on the Exchange on February 20, 2020 times (y) the number of Non-Affiliate Shares). The Company is not a shell company (as defined in Rule 405 under the Securities Act) and has not been a shell company for at least 12 calendar months previously and if it has been a shell company at any time previously, has filed current Form 10 information (as defined in Instruction I.B.6 of Form S-3) with the Commission at least 12 calendar months previously reflecting its status as a entity that is not a shell company.

(u) Certain Market Activities. Neither the Company nor, to the Company's knowledge, any of its directors, officers or controlling persons has taken, directly or indirectly, any action designed, or that has constituted or would reasonably be expected to cause or result in, under the Exchange Act or otherwise, the stabilization or manipulation of the price of any security of the Company to facilitate the sale or resale of the Placement Shares.

(v) Broker/Dealer Relationships. Neither the Company nor any related entities (i) is required to register as a "broker" or "dealer" in accordance with the provisions of the

Exchange Act or (ii) directly or indirectly through one or more intermediaries, controls or is a "person associated with a member" or "associated person of a member" (within the meaning set forth in the FINRA Manual).

(w) No Reliance. The Company has not relied upon the Agents or legal counsel for the Agents for any legal, tax or accounting advice in connection with the offering and sale of the Placement Shares.

(x) Taxes. The Company has filed all federal, state, local and foreign tax returns which have been required to be filed and paid all taxes shown thereon through the date hereof, to the extent that such taxes have become due and are not being contested in good faith, except where failure to do so would not reasonably be expected to have a Material Adverse Effect. Except as otherwise disclosed in or contemplated by the Registration Statement or the Prospectus, no tax deficiency has been determined adversely to the Company which has had, or would reasonably be expected to have, individually or in the aggregate, a Material Adverse Effect. The Company has no knowledge of any federal, state or other governmental tax deficiency, penalty or assessment which has been or might be asserted or threatened against it which would reasonably be expected to have a Material Adverse Effect.

(y) Title to Real and Personal Property. The Company has good and valid title in fee simple to all items of real property and good and valid title to all personal property described in the Registration Statement or Prospectus as being owned by it that are material to the business of the Company, in each case free and clear of all liens, encumbrances and claims, except those that (i) do not materially interfere with the use made and proposed to be made of such property by the Company or (ii) would not reasonably expected, individually or in the aggregate, to have a Material Adverse Effect. Any real property described in the Registration Statement or Prospectus as being leased by the Company is held by it under valid, existing and enforceable leases, except those that (A) do not materially interfere with the use made or proposed to be made of such property by the Company or (B) would not be reasonably expected, individually or in the aggregate, to have a Material Adverse Effect.

(z) Intellectual Property. The Company owns or possesses adequate enforceable rights to use all patents, patent applications, trademarks (both registered and unregistered), service marks, trade names, trademark registrations, service mark registrations, copyrights, licenses and know-how (including trade secrets and other unpatented and/or unpatentable proprietary or confidential information, systems or procedures) (collectively, the "Intellectual Property"), necessary for the conduct of its business as conducted as of the date hereof, except to the extent that the failure to own or possess adequate rights to use such Intellectual Property would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect; the Company has not received any written notice of any claim of infringement or conflict which asserted Intellectual Property rights of others, which infringement or conflict, if the subject of an unfavorable decision, would reasonably be expected to result in a Material Adverse Effect; there are no pending, or to the Company's knowledge, threatened judicial proceedings or interference proceedings against the Company challenging the Company's rights in or to or the validity of the scope of any of the Company's patents, patent applications or proprietary information, except for such right or claim that would not, individually or in the aggregate, reasonably be expected to result in a Material Adverse Effect.

(aa) Environmental Laws. The Company (i) is in compliance with any and all applicable federal, state, local and foreign laws, rules, regulations, decisions and orders relating to the protection of human health and safety, the environment or hazardous or toxic substances or wastes, pollutants or contaminants (collectively, "Environmental Laws"); (ii) has received and is in compliance with all permits, licenses or other approvals required of it under applicable Environmental Laws to conduct its businesses as described in the Registration Statement and the Prospectus; and (iii) has no received notice of any actual or potential liability for the investigation or remediation of any disposal or release of hazardous or toxic substances or wastes, pollutants or contaminants, except, in the case of any of clauses (i), (ii) or (iii) above, for any such failure to comply or failure to receive required permits, licenses, other approvals or liability as would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect.

(bb) Disclosure Controls. The Company maintains systems of internal controls designed to provide reasonable assurance that (i) transactions are executed in accordance with management's general or specific authorizations; (ii) transactions are recorded as necessary to permit preparation of financial statements in conformity with GAAP and to maintain asset accountability; (iii) access to assets is permitted only in accordance with management's general or specific authorization; and (iv) the recorded accountability for assets is compared with the existing assets at reasonable intervals and appropriate action is taken with respect to any differences. The Company is not aware of any material weaknesses in its internal control over financial reporting (other than as set forth in the Prospectus). Since the date of the latest audited financial statements of the Company included in the Prospectus, there has been no change in the Company's internal control over financial reporting that has materially affected, or is reasonably likely to materially affect, the Company's internal control over financial reporting (other than as set forth in the Prospectus). The Company has established disclosure controls and procedures (as defined in Exchange Act Rules 13a-15 and 15d-15) for the Company and designed such disclosure controls and procedures to ensure that material information relating to the Company is made known to the certifying officers. The Company's certifying officers have evaluated the effectiveness of the Company's controls and procedures as of a date within 90 days prior to the filing date of the Form 10-K for the fiscal year most recently ended (such date, the 'Evaluation Date"). The Company presented in its Form 10-K for the fiscal year most recently ended the conclusions of the certifying officers about the effectiveness of the disclosure controls and procedures based on their evaluations as of the Evaluation Date. Since the Evaluation Date there have been no significant changes in the Company's internal controls (as such term is defined in Item 307(b) of Regulation S-K under the Securities Act) or, to the Company's knowledge, in other factors that could significantly adversely affect the Company's internal controls. To the knowledge of the Company, the Company's "internal controls over financial reporting" and "disclosure controls and procedures" are effective.

(cc) Sarbanes-Oxley. The Company is not aware of any failure on the part of the Company or any of the Company's directors or officers, in their capacities as such, to comply with any applicable provisions of the Sarbanes-Oxley Act and the applicable rules and regulations promulgated thereunder in all material respects. Each of the principal executive officer and the principal financial officer of the Company (or each former principal executive officer of the Company and each former principal financial officer of the Company as applicable) has made all certifications required by Sections 302 and 906 of the Sarbanes-Oxley

Act with respect to all reports, schedules, forms, statements and other documents required to be filed by it or furnished by it to the Commission during the past 12 months. For purposes of the preceding sentence, "principal executive officer" and "principal financial officer" shall have the meanings given to such terms in the Sarbanes-Oxley Act.

(dd) Finder's Fees. The Company has not incurred any liability for any finder's fees, brokerage commissions or similar payments in connection with the transactions herein contemplated, except as may otherwise exist with respect to the Agents pursuant to this Agreement.

(ee) Labor Disputes. No labor disturbance by or dispute with employees of the Company exists or, to the knowledge of the Company, is threatened which would be reasonably be expected to have a Material Adverse Effect

(ff) Investment Company Act. The Company is not or after giving effect to the offering and sale of the Placement Shares, will not be an "investment company" or an entity "controlled" by an "investment company," as such terms are defined in the Investment Company Act of 1940, as amended (the "Investment Company Act").

(gg) Operations. The operations of the Company are and have been conducted at all times in compliance with applicable financial record keeping and reporting requirements of the Currency and Foreign Transactions Reporting Act of 1970, as amended, the money laundering statutes of all jurisdictions to which the Company is subject, the rules and regulations thereunder and any related or similar rules, regulations or guidelines, issued, administered or enforced by any governmental agency having authority over the Company (collectively, the "Money Laundering Laws"), except as would not reasonably be expected to result in a Material Adverse Effect; and no action, suit or proceeding by or before any court or governmental agency, authority or body having authority over the Company or any arbitrator involving the Company with respect to the Money Laundering Laws is pending or, to the knowledge of the Company, threatened.

(hh) Off-Balance Sheet Arrangements. There are no transactions, arrangements and other relationships between and/or among the Company, and/or, to the knowledge of the Company, any of its affiliates and any unconsolidated entity, including, but not limited to, any structural finance, special purpose or limited purpose entity (each, an "Off Balance Sheet Transaction") that would reasonably be expected to affect materially the Company's liquidity or the availability of or requirements for its capital resources, including those Off Balance Sheet Transactions described in the Commission's Statement about Management's Discussion and Analysis of Financial Conditions and Results of Operations (Release Nos. 33-8056; 34-45321; FR-61 required to be described in the Prospectus which have not been described as required.

(ii) Underwriter Agreements. Other than with respect to this Agreement, the Company is not a party to any agreement with an agent or underwriter for any other "at the market" or continuous equity transaction.

(jj) ERISA. To the knowledge of the Company, each material employee benefit plan, within the meaning of Section 3(3) of the Employee Retirement Income Security

Act of 1974, as amended ("ERISA"), that is maintained, administered or contributed to by the Company or any of its affiliates for employees or former employees of the Company has been maintained in material compliance with its terms and the requirements of any applicable statutes, orders, rules and regulations, including but not limited to ERISA and the Internal Revenue Code of 1986, as amended (the "Code"); no prohibited transaction, within the meaning of Section 406 of ERISA or Section 4975 of the Code, has occurred which would result in a material liability to the Company with respect to any such pla excluding transactions effected pursuant to a statutory or administrative exemption; and for each such plan that is subject to the funding rules of Section 412 of the Code or Section 302 of ERISA, no "accumulated funding deficiency" as defined in Section 412 of the Code has been incurred, whether or not waived, and th fair market value of the assets of each such plan (excluding for these purposes accrued but unpaid contributions) exceeds the present value of all benefits accrued under such plan determined using reasonable actuarial assumptions, other than, in each case, as would not reasonably be expected to have a Material Adverse Effect.

(kk) Forward Looking Statements. No forward-looking statement (within the meaning of Section 27A of the Securities Act and Section 21E of the Exchange Act) (a "Forward Looking Statement") contained in the Registration Statement and the Prospectus has been made or reaffirmed without a reasonable basis or has been disclosed other than in good faith. The Forward Looking Statements incorporated by reference in the Registration Statement and the Prospectus from the Company's Annual Report on Form 10-K for the fiscal year most recently ended (i) except for any Forward Looking Statement included in any financial statements and notes thereto, are within the coverage of the safe harbor for forward looking statements set forth in Section 27A of the Securities Act, Rule 175(b) under the Securities Act or Rule 3b-6 under the Exchange Act, as applicable, (ii) were made by the Company with a reasonable basis and in good faith and reflect the Company's good faith commercially reasonable best estimate of the matters described therein as of the respective dates on which such statements were made, and (iii) have been prepared in accordance with Item 10 of Regulation S-K under the Securities Act.

(ll) Agent Purchases. The Company acknowledges and agrees that the Agents have informed the Company that each Agent may, to the extent permitted under the Securities Act and the Exchange Act, purchase and sell Common Stock for its own account while this Agreement is in effect, provided, that (i) no such purchase or sales shall take place while a Placement Notice is in effect (except to the extent each Agent may engage in sales of Placement Shares purchased or deemed purchased from the Company as a "riskless principal" or in a similar capacity) and (ii) the Company shall not be deemed to have authorized or consented to any such purchases or sales by such Agent.

(mm) Margin Rules. Neither the issuance, sale and delivery of the Placement Shares nor the application of the proceeds thereof by the Company as described in the Registration Statement and the Prospectus will violate Regulation T, U or X of the Board of Governors of the Federal Reserve System.

(nn) Insurance. The Company carries, or is covered by, insurance in such amounts and covering such risks as the Company reasonably believes is adequate for the conduct of its business and as is customary for companies of similar size engaged in similar businesses in similar industries.

(oo) No Improper Practices. (i) Neither the Company, nor to the Company's knowledge, any of its executive officers has, in the past five years, made any unlawful contributions to any candidate for any political office (or failed fully to disclose any contribution in violation of law) or made any contribution or other payment to any official of, or candidate for, any federal, state, municipal, or foreign office or other person charged with similar public or quasi-public duty in violation of any law or of the character required to be disclosed in the Prospectus; (ii) no relationship, direct or indirect, exists between or among the Company or, to the Company's knowledge, any affiliate of the Company, on the one hand, and the directors, officers and stockholders of the Company, that is required by the Securities Act to be described in the Registration Statement and the Prospectus that is not so described; (iii) no relationship direct or indirect, exists between or among the Company, or any affiliate of the Company, on the one hand, and the directors, officers, stockholders or directors of the Company that is required by the rules of FINRA to be described in the Registration Statement and the Prospectus that is not so described; (iv) there are n material outstanding loans or advances or material guarantees of indebtedness by the Company to or for the benefit of any of its officers or directors or any of the members of the families of any of them; (v) the Company has not offered, or caused any placement agent to offer, Common Stock to any person with the intent to influence unlawfully (A) a customer or supplier of the Company to alter the customer's or supplier's level or type of business with the Company or (B) a trade journalist or publication to write or publish favorable information about the Company or any of its products or services, and, (vi) neither the Company nor, to the Company's knowledge, any employee or agent of the Company has made any payment of funds of the Company or received or retained any funds in violation of any law, rule or regulation (including, without limitation, the Foreign Corrupt Practices Act of 1977), which payment, receipt or retention of funds is of a character required to be disclosed in the Registration Statement or the Prospectus.

(pp) Compliance with Applicable Laws. The Company (A) to its knowledge, is and at all times has been in material compliance with all statutes, rules and regulations applicable to the ownership, testing, development, manufacture, packaging, processing, use, distribution, marketing, labeling, promotion, sale, offer for sale, storage, import, export or disposal of any product under development, manufactured or distributed by the Company ("Applicable Laws"), (B) has not received any Form 483 from the U.S. Food and Drug Administration (FDA"), notice of adverse finding, warning letter, or other written correspondence or notice from the FDA or any other federal, state, local or foreign governmental or regulatory authority alleging or asserting material noncompliance with any Applicable Laws or any licenses, certificates, approvals, clearances, authorizations, permits and supplements or amendments thereto required by any such Applicable Laws ("Authorizations"), which would, individually or in the aggregate, reasonably be expected to result in a Material Adverse Effect; (C) possesses all material Authorizations and such Authorizations are valid and in full force and effect and the Company is not in material violation of any term of any such Authorizations; (D) has not received written notice of any claim, action, suit, proceeding, hearing, enforcement, investigation, arbitration or other action from the FDA or any other federal, state, local or foreign governmental or regulatory authority or third party alleging that any Company product, operation or activity is in material violation of any Applicable Laws or Authorizations and has no knowledge that the FDA or any other federal, state, local or foreign governmental or regulatory authority or third party is considering any such claim, litigation, arbitration, action, suit, investigation or proceeding against the Company; (E) has not received notice that the FDA or any other federal, state, local

or foreign governmental or regulatory authority has taken, is taking or intends to take action to limit, suspend, modify or revoke any material Authorizations and has no knowledge that the FDA or any other federal, state, local or foreign governmental or regulatory authority is considering such action; and (F) to its knowledge, has filed, obtained, maintained or submitted all reports, documents, forms, notices, applications, records, claims, submissions and supplements or amendments as required by any Applicable Laws or Authorizations except where the failure to file such reports, documents, forms, notices, applications, records, claims, submissions and supplements or amendments would not result in a Material Adverse Effect, and that all such reports, documents, forms, notices, applications, records, claims, submissions and supplements or amendments were materially complete and correct on the date filed (or were corrected or supplemented by a subsequent submission).

(qq) Clinical Studies. All clinical trials conducted by the Company or on behalf of the Company were, and, if still pending are, to the Company's knowledge, being conducted in all material respects in compliance with all Applicable Laws and in accordance with experimental protocols, procedures and controls generally used by qualified experts in the clinical trials of new drugs and biologics as applied to comparable products to those being developed by the Company, except where such noncompliance would not reasonably be expected to have a Material Adverse Effect; the descriptions of the results of such clinical trials contained in the Registration Statement and the Prospectus are accurate in all material respects, and the Company has no knowledge of any other clinical trials, the results of which reasonably call into question the clinical trial results described or referred to in the Registration Statement and the Prospectus when viewed in the context in which such results are described; and the Company has not received any written notices or correspondence from the FDA or any other domestic or foreign governmental agency requiring the termination or suspension of any clinical trials conducted by or on behalf of the Company that are described in the Registration Statement and the Prospectus or the results of which are referred to in the Registration Statement and the Prospectus.

(rr) Status Under the Securities Act. The Company was not and is not an ineligible issuer as defined in Rule 405 under the Securities Act at the times specified in Rules 164 and 433 under the Securities Act in connection with the offering of the Placement Shares.

(ss) No Misstatement or Omission in an Issuer Free Writing Prospectus. Each Issuer Free Writing Prospectus, as of its issue date and as of each Applicable Time (as defined in Section 24 below), did not, does not and will not include any information that conflicted, conflicts or will conflict with the information contained in the Registration Statement or the Prospectus, including any incorporated document deemed to be a part thereof that has not been superseded or modified. The foregoing sentence does not apply to statements in or omissions from any Issuer Free Writing Prospectus based upon and in conformity with written information furnished to the Company by an Agent specifically for use therein.

(tt) No Conflicts. Neither the execution of this Agreement, nor the issuance, offering or sale of the Placement Shares, nor the consummation of any of the transactions contemplated herein and therein, nor the compliance by the Company with the terms and provisions hereof and thereof will conflict with, or will result in a breach of, any of the terms and provisions of, or has constituted or will constitute a default under, or has resulted in or will result

in the creation or imposition of any lien, charge or encumbrance upon any property or assets of the Company pursuant to the terms of any contract or other agreement to which the Company may be bound or to which any of the property or assets of the Company is subject, except (i) such conflicts, breaches or defaults as may have been waived and (ii) such conflicts, breaches and defaults that would not reasonably be expected to have a Material Adverse Effect; nor will such action result (x) in any violation of the provisions of the organizational or governing documents of the Company, or (y) in any material violation of the provisions of any statute or any order, rule or regulation applicable to the Company or of any court or of any federal, state or other regulatory authority or other government body having jurisdiction over the Company.

(uu) OFAC. Neither the Company nor any director or officer, or, to the knowledge of the Company, after due inquiry, any agent, employee, affiliate or representative of the Company, is a government, individual or entity (in this paragraph (uu), "Person") that is, or is owned or controlled by a Person that is, currently subject to any U.S. sanctions administered by the Office of Foreign Assets Control of the U.S. Department of the Treasury ("OFAC"), the United Nations Security Council ("UNSC"), the European Union ("EU"), Her Majesty's Treasury ("HMT"), or other relevant sanctions authority (collectively, "Sanctions"), nor located, organized or resident in a country or territory that is the subject of Sanctions; provided however, that for the purposes of this paragraph (uu), no person shall be an affiliate of the Company solely by reason of owning less than a majority of any class of voting securities of the Company. The Company will not directly or indirectly, use the proceeds of the offering of the Placement Shares hereunder, or lend, contribute or otherwise make available such proceeds to any subsidiary, joint venture partner or other person or entity, for the purpose of financing the activities of any person currently subject to any U.S. sanctions administered by OFAC.The Company represents and covenants that, except as detailed in the Prospectus, for the past five years, the Company has not knowingly engaged in, is not now knowingly engaged in, and will not knowingly engage in, any dealings or transactions with any Person, or in any country or territory, that at the time of the dealing or transaction is or was the subject of Sanctions.

(vv) Stock Transfer Taxes. On each Settlement Date, all stock transfer or other taxes (other than income taxes) which are required to be paid in connection with the sale and transfer of the Placement Shares to be sold hereunder will be, or will have been, fully paid or provided for by the Company and all laws imposing such taxes will be or will have been fully complied with by the Company in all material respects.

Any certificate signed by an officer of the Company and delivered to the Agents or to counsel for the Agents pursuant to or in connection with this Agreement shall be deemed to be a representation and warranty by the Company, as applicable, to the Agents as to the matters set forth therein.

7. Covenants of the Company. The Company covenants and agrees with the Agents that:

(a) Registration Statement Amendments. After the date of this Agreement and during any period in which a Prospectus relating to any Placement Shares is required to be delivered by the Agents under the Securities Act (including in circumstances where such requirement may be satisfied pursuant to Rule 172 under the Securities Act) (the "Prospectus

Delivery Period") (i) the Company will notify the Agents promptly of the time when any subsequent amendment to the Registration Statement, other than documents incorporated by reference, has been filed with the Commission and/or has become effective or any subsequent supplement to the Prospectus has been filed and of any request by the Commission for any amendment or supplement to the Registration Statement or Prospectus or for additional information, (ii) the Company will prepare and file with the Commission, promptly upon the Agents' request, any amendments or supplements to the Registration Statement or Prospectus that, in such Agents' reasonable opinion, may be necessary or advisable in connection with the distribution of the Placement Shares by the Agents (provided, however, that the failure of the Agents to make such request shall not relieve the Company of any obligation or liability hereunder, or affect the Agents' right to rely on the representations and warranties made by the Company in this Agreement and provided, further, that the only remedy the Agents shall have with respect to the failure to make such filing shall be to cease making sales under this Agreement until such amendment or supplement is filed); (iii) the Company will not file any amendment or supplement to the Registration Statement or Prospectus, other than documents incorporated by reference, relating to the Placement Shares or a security convertible into the Placement Shares unless a copy thereof has been submitted to Agents within a reasonable period of time before the filing and the Agents have not reasonably and in good faith objected thereto (provided, however, that (A) the failure of the Agents to make such objection shall not relieve the Company of any obligation or liability hereunder, or affect the Agents' right to rely on the representations and warranties made by the Company in this Agreement and (B) the Company has no obligation to provide the Agents any advance copy of such filing or to provide the Agents an opportunity to object to such filing if the filing does not name the Agents or does not relate to the transaction herein provided; and provided, further, that the only remedy Agents shall have with respect to the failure to by the Company to obtain such consent shall be to cease making sales under this Agreement) and the Company will furnish to the Agents at the time of filing thereof a copy of any document that upon filing is deemed to be incorporated by reference into the Registration Statement or Prospectus, except for those documents available via EDGAR; and (iv) the Company will cause each amendment or supplement to the Prospectus to be filed with the Commission as required pursuant to the applicable paragraph of Rule 424(b) of the Securities Act or, in the case of any document to be incorporated therein by reference, to be filed with the Commission as required pursuant to the Exchange Act, within the time period prescribed (the determination to file or not file any amendment or supplement with the Commission under this Section 7(a), based on the Company's reasonable opinion or reasonable objections, shall be made exclusively by the Company).

(b) Notice of Commission Stop Orders. The Company will advise the Agents, promptly after it receives notice or obtains knowledge thereof, of the issuance or threatened issuance by the Commission of any stop order suspending the effectiveness of the Registration Statement, of the suspension of the qualification of the Placement Shares for offering or sale in any jurisdiction, or of the initiation or threatening of any proceeding for any such purpose; and it will promptly use its commercially reasonable efforts to prevent the issuance of any stop order or to obtain its withdrawal if such a stop order should be issued. The Company will advise the Agents promptly after it receives any request by the Commission for any amendments to the Registration Statement or any amendment or supplements to the Prospectus or any Issuer Free Writing Prospectus or for additional information related to the offering of the Placement Shares

or for additional information related to the Registration Statement, the Prospectus or any Issuer Free Writing Prospectus.

(c) Delivery of Prospectus; Subsequent Changes During the Prospectus Delivery Period, the Company will use its commercially reasonable efforts to comply with all requirements imposed upon it by the Securities Act, as from time to time in force, and to file on or before their respective due dates all reports and any definitive proxy or information statements required to be filed by the Company with the Commission pursuant to Sections 13(a), 13(c), 14, 15(d) or any other provision of or under the Exchange Act. If the Company has omitted any information from the Registration Statement pursuant to Rule 430A under the Securities Act, it will use its commercially reasonable efforts to comply with the provisions of and make all requisite filings with the Commission pursuant to said Rule 430A and to notify the Agents promptly of all such filings. If during the Prospectus Delivery Period any event occurs as a result of which the Prospectus as then amended or supplemented would include an untrue statement of a material fact or omit to state a material fact necessary to make the statements therein, in the light of the circumstances then existing, not misleading, or if during the Prospectus Delivery Period it is necessary to amend or supplement the Registration Statement or Prospectus to comply with the Securities Act, the Company will promptly notify Agents to suspend the offering of Placement Shares during such period and the Company will promptly amend or supplement the Registration Statement or Prospectus (at the expense of the Company) so as to correct such statement or omission or effect such compliance; provided, however, that the Company may delay the filing of any amendment or supplement, if in the judgment of the Company, it is in the best interests of the Company.

(d) Listing of Placement Shares During the Prospectus Delivery Period, the Company will use its commercially reasonable efforts to cause the Placement Shares to be listed on the Exchange and to qualify the Placement Shares for sale under the securities laws of such jurisdictions as the Agents reasonably designate and to continue such qualifications in effect so long as required for the distribution of the Placement Shares; provided, however, that the Company shall not be required in connection therewith to qualify as a foreign corporation or dealer in securities or file a general consent to service of process in any jurisdiction.

(e) Delivery of Registration Statement and Prospectus The Company will furnish to the Agents and their counsel (at the reasonable expense of the Company) copies of the Registration Statement, the Prospectus (including all documents incorporated by reference therein) and all amendments and supplements to the Registration Statement or Prospectus that are filed with the Commission during the Prospectus Delivery Period (including all documents filed with the Commission during such period that are deemed to be incorporated by reference therein), in each case as soon as reasonably practicable and in such quantities as the Agents may from time to time reasonably request and, at the Agents' request, will also furnish copies of the Prospectus to each exchange or market on which sales of the Placement Shares may be made; provided, however, that the Company shall not be required to furnish any document (other than the Prospectus) to the Agents to the extent such document is available on EDGAR.

(f) Earnings Statement. The Company will make generally available to its security holders as soon as practicable, but in any event not later than 15 months after the end of

the Company's current fiscal quarter, an earnings statement covering a 12-month period that satisfies the provisions of Section 11(a) and Rule 158 of the Securities Act.

(g) Use of Proceeds. The Company will use the Net Proceeds as described in the Prospectus in the section entitled "Use of Proceeds."

(h) Notice of Other Sales. Without prior written notice to the Agents, the Company will not, directly or indirectly, offer to sell, sell, contract to sell, grant any option to sell or otherwise dispose of any Common Stock (other than the Placement Shares offered pursuant to this Agreement) or securities convertible into or exchangeable for Common Stock, warrants or any rights to purchase or acquire, Common Stock during the period beginning on the date on which any Placement Notice is delivered to an Agent hereunder and ending on the second (2nd) Trading Day immediately following the final Settlement Date with respect to Placement Shares sold pursuant to such Placement Notice (or, if the Placement Notice has been terminated or suspended prior to the sale of all Placement Shares covered by a Placement Notice, the date of such suspension or termination); and will not directly or indirectly in any other "at the market" or continuous equity transaction offer to sell, sell, contract to sell, grant any option to sell or otherwise dispose of any Common Stock (other than the Placement Shares offered pursuant to this Agreement) or securities convertible into or exchangeable for Common Stock, warrants or any rights to purchase or acquire, Common Stock prior to the termination of this Agreement; provided, however, that such restrictions will not be required in connection with the Company's issuance or sale of (i) Common Stock, restricted stock units, options to purchase Common Stock or Common Stock issuable upon the exercise of options, pursuant to any employee or director stock option or benefits plan, stock ownership plan or dividend reinvestment plan (but not Common Stock subject to a waiver to exceed plan limits in its dividend reinvestment plan) of the Company whether now in effect or hereafter implemented, (ii) Common Stock issuable upon conversion of securities or the exercise of warrants, options or other rights in effect or outstanding, and disclosed in filings by the Company available on EDGAR or otherwise in writing to the Agents, and (iii) Common Stock, or securities convertible into or exercisable for Common Stock, offered and sold in negotiated transaction to vendors, customers, strategic partners or potential strategic partners, acquisition candidates or other investors conducted in a manner so as not to be integrated with the offering of Common Stock hereby.

(i) Change of Circumstances. The Company will, at any time during the pendency of a Placement Notice advise the Agents promptly after it shall have received notice or obtained knowledge thereof, of any information or fact that would alter or affect in any material respect any opinion, certificate, letter or other document required to be provided to the Agents pursuant to this Agreement.

(j) Due Diligence Cooperation. During the term of this Agreement, the Company will cooperate with any reasonable due diligence review conducted by the Agents or their respective representatives in connection with the transactions contemplated hereby, including, without limitation, providing information and making available documents and senior corporate officers, during regular business hours and at the Company's principal offices, as the Agents may reasonably request.

(k) Required Filings Relating to Placement of Placement Shares The Company agrees that on such dates as the Securities Act shall require, the Company will (i) file a prospectus supplement with the Commission under the applicable paragraph of Rule 424(b) under the Securities Act (each and every filing under Rule 424(b), a "Filing Date"), which prospectus supplement will set forth, within the relevant period, the amount of Placement Shares sold through the Agents, the Net Proceeds to the Company and the compensation payable by the Company to the Agents with respect to such Placement Shares, and (ii) deliver such number of copies of each such prospectus supplement to each exchange or market on which such sales were effected as may be required by the rules or regulations of such exchange or market.

(l) Representation Dates; Certificate Prior to the submission of the first Placement Notice and within five (5) trading days of each time the Company:

(i) files the Prospectus relating to the Placement Shares or amends or supplements (other than a prospectus supplement relating solely to an offering of securities other than the Placement Shares), the Registration Statement or the Prospectus relating to the Placement Shares by means of a post-effective amendment, sticker, or supplement but not by means of incorporation of documents by reference into the Registration Statement or the Prospectus relating to the Placement Shares;

(ii) files an annual report on Form 10-K under the Exchange Act (including any Form 10-K/A containing amended financial information or a material amendment to the previously filed Form 10-K);

(iii) files a quarterly report on Form 10-Q under the Exchange Act; or

(iv) files a current report on Form 8-K containing amended financial information (other than information "furnished" pursuant to Items 2.02 or 7.01 of Form 8-K or to provide disclosure pursuant to Item 8.01 of Form 8-K relating to the reclassification of certain properties as discontinued operations in accordance with Statement of Financial Accounting Standards No. 144) under the Exchange Act; (Each date of filing of one or more of the documents referred to in clauses (i) through (iv) shall be a "Representation Date"),

the Company shall furnish the Agents (but in the case of clause (iv) above only if the Agents reasonably determine that the information contained in such Form 8-K is material) with a certificate, in the form attached hereto as Exhibit 7(l) (the "Representation Date Certificate"); provided however, if no Placement Notice is pending at such Representation Date, then before the Company delivers a Placement Notice or the Agents sells any Placement Shares, the Company shall provide the Agents with a Representation Date Certificate. The requirement to provide a Representation Date Certificate shall be waived for any Representation Date occurring at a time at which no Placement Notice is pending, which waiver shall continue until the earlier to occur of the date the Company delivers a Placement Notice hereunder (which for such calendar quarter shall be considered a Representation Date) and the next occurring Representation Date; provided however, that such waiver shall not apply for any Representation Date on which the Company files its annual report on Form 10-K. Notwithstanding the foregoing, if the Company subsequently decides to sell Placement Shares following a Representation Date when the Company relied on such waiver and did not provide the Agents

with a Representation Date Certificate, then before the Company delivers the Placement Notice or the Agents sell any Placement Shares, the Company sha provide the Agents with a Representation Date Certificate, dated the date of the Placement Notice.

(m) Legal Opinion. Prior to the submission of the first Placement Notice, the Company shall cause to be furnished to the Agents a written opinion and negative assurance letter of Pillsbury Winthrop Shaw Pittman LLP ("Company Counsel"), or other counsel reasonably satisfactory to the Agents, in form and substance satisfactory to the Agents and their counsel, and a written opinion of Clark+Elbing LLP ("Company IP Counsel"), or other counsel reasonably satisfactory to the Agents, in form and substance satisfactory to the Agents and their counsel. Thereafter, within five (5) Trading Days of each Representation Date with respect to which the Company is obligated to deliver a Representation Date Certificate for which no waiver i applicable, the Company shall cause to be furnished to the Agents a negative assurance letter of Company Counsel in form and substance satisfactory to the Agents and their counsel; provided however, if no Placement Notice is pending at such Representation Date, then before the Company delivers a Placemen Notice or the Agents sell any Placement Shares, the Company shall provide the Agents with such negative assurance letter; provided, further, that in lieu of such negative assurance letter for subsequent periodic filings under the Exchange Act, counsel may furnish the Agents with a letter (a "Reliance Letter") to the effect that the Agents may rely on a prior negative assurance letter delivered under this Section 7(m) to the same extent as if it were dated the date of such letter (except that statements in such prior negative assurance letter shall be deemed to relate to the Registration Statement and the Prospectus as amended or supplemented as of the date of the Reliance Letter).

(n) Comfort Letter. (1) Prior to the submission of the first Placement Notice and (2) within five (5) Trading Days of each Representation Date, with respect to which the Company is obligated to deliver a Representation Date Certificate for which no waiver is applicable, th Company shall cause its independent accountants to furnish the Agents letters (the "Comfort Letters"), dated the date the Comfort Letter is delivered, which shall meet the requirements set forth in this Section 7(n); provided however, if no Placement Notice is pending at such Representation Date, then before the Company delivers a Placement Notice or the Agents sell any Placement Shares, the Company shall provide the Agents with the Comfort Letter; provided, further, that requested by the Agents, the Company shall cause a Comfort Letter to be furnished to the Agents within ten (10) Trading Days of the date of occurrence of any material transaction or event, including the restatement of the Company's financial statements. The Comfort Letter from the Company's independent accountants shall be in a form and substance reasonably satisfactory to the Agents, (i) confirming that they are an independent public accounting firm within the meaning of the Securities Act and the Public Company Accounting Oversight Board (the "PCAOB"), (ii) stating, as of such date, the conclusions and findings of such firm with respect to the financial information and other matters ordinarily covered by accountants' "comfort letters" to underwriters in connection with registered public offerings (the first such letter, the "Initial Comfort Letter") and (iii) updating the Initial Comfort Letter with any information that would have been included in the Initial Comfort Letter had it been given on such date and modified as necessary to relate to the Registration Statement and the Prospectus, as amended and supplemented to the date of such letter.

(o) Market Activities. The Company will not, directly or indirectly, (i) take any action designed to cause or result in, or that constitutes or would reasonably be expected to constitute, the stabilization or manipulation of the price of any security of the Company to facilitate the sale or resale of Common Stock or (ii) sell, bid for, or purchase Common Stock in violation of Regulation M, or pay anyone any compensation for soliciting purchases of the Placement Shares other than the Agents.

(p) Investment Company Act. The Company will conduct its affairs in such a manner so as to reasonably ensure that it will not become, at any time prior to the termination of this Agreement, an "investment company," as such term is defined in the Investment Company Act.

(q) No Offer to Sell. Other than an Issuer Free Writing Prospectus approved in advance by the Company and the Agents in their capacity as agents hereunder, neither the Agents nor the Company (including its agents and representatives, other than the Agents in their capacity as such) will make, use, prepare, authorize, approve or refer to any written communication (as defined in Rule 405 under the Securities Act), required to be filed with the Commission, that constitutes an offer to sell or solicitation of an offer to buy Placement Shares hereunder.

(r) Sarbanes-Oxley Act. The Company will maintain and keep accurate books and records reflecting its assets and maintain internal accounting controls in a manner designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with GAAP and including those policies and procedures that (i) pertain to the maintenance of records that in reasonable detail accurately and fairly reflect the transactions and dispositions of the assets of the Company, (ii) provide reasonable assurance that transactions are recorded as necessary to permit the preparation of the Company's consolidated financial statements in accordance with GAAP, (iii) that receipts and expenditures of the Company are being made only in accordance with management's and the Company's directors' authorization, and (iv) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of the Company's assets that could have a material effect on its financial statements. The Company will use commercially reasonable efforts to comply with all other effective applicable provisions of the Sarbanes-Oxley Act and the applicable regulations thereunder that are designed to ensure that information required to be disclosed by the Company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the Commission's rules and forms.

8. Payment of Expenses. The Company will pay all expenses incident to the performance of its obligations under this Agreement, including (i) the preparation, filing, including any fees required by the Commission, and printing of the Registration Statement (including financial statements and exhibits) as originally filed and of each amendment and supplement thereto, in such number as the Agents shall deem reasonably necessary, (ii) the printing and delivery to the Agents of this Agreement and such other documents as may be required in connection with the offering, purchase, sale, issuance or delivery of the Placement Shares, (iii) the preparation, issuance and delivery of the certificates, if any, for the Placement Shares to the Agents, including any stock or other transfer taxes and any capital duties, stamp

duties or other duties or taxes payable upon the sale, issuance or delivery of the Placement Shares to the Agents, (iv) the fees and disbursements of the counsel, accountants and other advisors to the Company, (v) the reasonable out-of-pocket expenses of Agents, including fees and disbursements of counsel to the Agents, up to an aggregate of $60,000 (which amount shall include all fees and disbursements of such counsel described in clause (ix) below), (vi) the printing and delivery to the Agents of copies of any Permitted Issuer Free Writing Prospectus (defined below) and the Prospectus and any amendments or supplements thereto in such number as the Agents shall deem necessary, (vii) the preparation, printing and delivery to the Agents of copies of the blue sky survey and any Canadian "wrapper" and any supplements thereto, in such number as the Agents shall deem necessary, (viii) the fees and expenses of the transfer agent and registrar for the Common Stock, (ix) the fees and expenses incident to any review by FINRA of the terms of the sale of the Placement Shares, including fees and expenses of counsel to the Agents, and (x) the fees and expenses incurred in connection with the listing of the Placement Shares on the Exchange.

9. Conditions to Agents' Obligations. The obligations of the Agents hereunder with respect to a Placement will be subject to the continuing accuracy and completeness of the representations and warranties made by the Company herein, to the due performance by the Company of its obligations hereunder, to the completion by the Agents of a due diligence review satisfactory to it in its reasonable judgment, and to the continuing satisfaction (or waiver by the Agents in their sole discretion) of the following additional conditions:

(a) Registration Statement Effective. The Registration Statement shall have become effective and shall be available for the sale of all Placement Shares contemplated to be issued by any Placement Notice.

(b) No Material Notices. None of the following events shall have occurred and be continuing: (i) receipt by the Company of any request for additional information from the Commission or any other federal or state governmental authority during the period of effectiveness of the Registration Statement, the response to which would require any post-effective amendments or supplements to the Registration Statement or the Prospectus; (ii) the issuance by the Commission or any other federal or state governmental authority of any stop order suspending the effectiveness of the Registration Statement or the initiation of any proceedings for that purpose; (iii) receipt by the Company of any notification with respect to the suspension of the qualification or exemption from qualification of any of the Placement Shares for sale in any jurisdiction or the initiation or threatening of any proceeding for such purpose; or (iv) the occurrence of any event that makes any material statement made in the Registration Statement or the Prospectus or any material document incorporated or deemed to be incorporated therein by reference untrue in any material respect or that requires the making of any changes in the Registration Statement, the Prospectus or documents so that, in the case of the Registration Statement, it will not contain any materially untrue statement of a material fact or omit to state any material fact required to be stated therein or necessary to make the statements therein not misleading and, that in the case of the Prospectus, it will not contain any materially untrue statement of a material fact or omit to state any material fact required to be stated therein or necessary to make the statements therein, in the light of the circumstances under which they were made, not misleading.

(c) No Misstatement or Material Omission. Agents shall not have advised the Company that the Registration Statement or Prospectus, or any amendment or supplement thereto, contains an untrue statement of fact that in the Agents' reasonable opinion is material, or omits to state a fact that in the Agents' reasonable opinion is material and is required to be stated therein or is necessary to make the statements therein not misleading.

(d) Material Changes. Except as contemplated in the Prospectus, or disclosed in the Company's reports filed with the Commission, there shall not have been any material adverse change, on a consolidated basis, in the authorized capital stock of the Company or any Material Adverse Effect, or any development that could reasonably be expected to cause a Material Adverse Effect, or a downgrading in or withdrawal of the rating assigned to any of the Company's securities (other than asset backed securities) by any rating organization or a public announcement by any rating organization that it has under surveillance or review its rating of any of the Company's securities (other than asset backed securities), the effect of which, in the case of any such action by a rating organization described above, in the reasonable judgment of the Agents (without relieving the Company of any obligation or liability it may otherwise have), is so material as to make it impracticable or inadvisable to proceed with the offering of the Placement Shares on the terms and in the manner contemplated in the Prospectus.

(e) Legal Opinion. The Agents shall have received the opinion of Company Counsel required to be delivered pursuant Section 7(m) on or before the date on which such delivery of such opinion is required pursuant to Section 7(m).

(f) IP Opinion. The Agents shall have received the opinion of Company IP Counsel required to be delivered pursuant Section 7(m) on or before the date on which such delivery of such opinion is required pursuant to Section 7(m).

(g) Comfort Letter. The Agents shall have received the Comfort Letter required to be delivered pursuant Section 7(n) on or before the date on which such delivery of such Comfort Letter is required pursuant to Section 7(n).

(h) Representation Certificate. The Agents shall have received the certificate required to be delivered pursuant to Section 7(l) on or before the date on which delivery of such certificate is required pursuant to Section 7(l).

(i) Secretary's Certificate. On the date of this Agreement, the Agents shall have received a certificate, signed on behalf of the Company by its corporate Secretary, in form and substance satisfactory to the Agents and their counsel.

(j) No Suspension. Trading in the Common Stock shall not have been suspended on the Exchange, and the Common Stock shall not have been delisted from the Exchange.

(k) Other Materials. On each date on which the Company is required to deliver a certificate pursuant to Section 7(l), the Company shall have furnished to the Agents such appropriate further information, certificates and documents as the Agents may reasonably request. All such opinions, certificates, letters and other documents will be in compliance with the provisions hereof.

(l) Securities Act Filings Made. All filings with the Commission required by Rule 424 under the Securities Act to have been filed prior to the issuance of any Placement Notice hereunder shall have been made within the applicable time period prescribed for such filing by Rule 424.

(m) Approval for Listing. The Placement Shares shall either have been approved for listing on the Exchange, subject only to notice of issuance, or the Company shall have filed an application for listing of the Placement Shares on the Exchange at, or prior to, the issuance of any Placement Notice.

(n) No Termination Event. There shall not have occurred any event that would permit the Agents to terminate this Agreement pursuant to Section 12(a).

10. Indemnification and Contribution.

(a) Company Indemnification. The Company agrees to indemnify and hold harmless the Agents, their partners, members, directors, officers, employees and agents and each person, if any, who controls the Agents within the meaning of Section 15 of the Securities Act or Section 20 of the Exchange Act as follows:

(i) against any and all loss, liability, claim, damage and expense whatsoever, as incurred, joint or several, arising out of or based upon any untrue statement or alleged untrue statement of a material fact contained in the Registration Statement (or any amendment thereto), or the omission or alleged omission therefrom of a material fact required to be stated therein or necessary to make the statements therein not misleading, or arising out of any untrue statement or alleged untrue statement of a material fact included in any related Issuer Free Writing Prospectus or the Prospectus (or any amendment or supplement thereto), or the omission or alleged omission therefrom of a material fact necessary in order to make the statements therein, in the light of the circumstances under which they were made, not misleading;

(ii) against any and all loss, liability, claim, damage and expense whatsoever, as incurred, joint or several, to the extent of the aggregate amount paid in settlement of any litigation, or any investigation or proceeding by any governmental agency or body, commenced or threatened, or of any claim whatsoever based upon any such untrue statement or omission, or any such alleged untrue statement or omission; provided that (subject to Section 10(d) below) any such settlement is effected with the written consent of the Agents, which consent shall not unreasonably be delayed or withheld; and

(iii) against any and all expense whatsoever, as incurred (including the reasonable fees and disbursements of counsel), reasonably incurred in investigating, preparing or defending against any litigation, or any investigation or proceeding by any governmental agency or body, commenced or threatened, or any claim whatsoever based upon any such untrue statement or omission, or any such alleged untrue statement or omission, to the extent that any such expense is not paid under (i) or (ii) above, provided, however, that this indemnity agreement shall not apply to any loss, liability, claim, damage or expense to the extent arising out of any untrue statement or omission or alleged untrue statement or omission made solely in reliance upon and in conformity with written information furnished to the Company by any

Agent expressly for use in the Registration Statement (or any amendment thereto), or in any related Issuer Free Writing Prospectus or the Prospectus (or an amendment or supplement thereto).

(b) Agent Indemnification. Each Agent agrees to indemnify and hold harmless the Company and its directors and each officer of the Company who signed the Registration Statement, and each person, if any, who (i) controls the Company within the meaning of Section 15 o the Securities Act or Section 20 of the Exchange Act or (ii) is controlled by or is under common control with the Company against any and all loss, liability, claim damage and expense described in the indemnity contained in Section 10(a), as incurred, but only with respect to untrue statements or omissions, or alleged untrue statements or omissions, made in the Registration Statement (or any amendments thereto) or the Prospectus (or any amendment or supplement thereto) in reliance upon and in conformity with information relating to an Agent and furnished to the Company in writing by such Agent or its agents and counsel expressly for use therein.

(c) Procedure. Any party that proposes to assert the right to be indemnified under this Section 10 will, promptly after receipt of notice of commencement of any action against such party in respect of which a claim is to be made against an indemnifying party or parties under this Section 10, notify each such indemnifying party of the commencement of such action, enclosing a copy of all papers served, but the omission so to notify such indemnifying party will not relieve the indemnifying party from (i) any liability that it might have to any indemnified party otherwise than under this Section 10 and (ii) any liability that it may have to any indemnified party under the foregoing provision of this Section 10 unless, and only to the extent that, such omission results in the forfeiture of substantive rights or defenses by the indemnifying party. If any such action is brought against any indemnified party and it notifies the indemnifying party of its commencement, the indemnifying party will be entitled to participate in and, to the extent that it elects by delivering written notice to the indemnified party promptly after receiving notice of the commencement of the action from the indemnified party, jointly with any other indemnifying party similarly notified, to assume the defense of the action, with counsel reasonably satisfactory to the indemnified party, and after notice from the indemnifying party to the indemnified party of its election to assume the defense, the indemnifying party will not be liable to the indemnified party for any legal or other expenses except as provided below and except for the reasonable costs of investigation subsequently incurred by the indemnified party in connection with the defense. The indemnified party will have the right to employ its own counsel in any such action, but the fees, expenses and other charges of such counsel will be at the expense of such indemnified party unless (1) the employment of counsel by the indemnified party has been authorized in writing by the indemnifying party, (2) the indemnified party has reasonably concluded (based on written advice of counsel) that there may be legal defenses available to it or other indemnified parties that are different from or in addition to those available to the indemnifying party, (3) a conflict or potential conflict exists (based on written advice of counsel to the indemnified party) between the indemnified party and the indemnifying party (in which case the indemnifying party will not have the right to direct the defense of such action on behalf of the indemnified party) or (4) the indemnifying party has not in fact employed counsel to assume the defense of such action within a reasonable time after receiving notice of the commencement of the action, in each of which cases the reasonable fees, disbursements and other charges of counsel will be at the expense of the indemnifying party or parties. It is

understood that the indemnifying party or parties shall not, in connection with any proceeding or related proceedings in the same jurisdiction, be liable for the reasonable fees, disbursements and other charges of more than one separate firm admitted to practice in such jurisdiction at any one time for all such indemnified party or parties. All such fees, disbursements and other charges will be reimbursed by the indemnifying party promptly after the indemnifying party receives a written invoice relating to fees, disbursements and other charges in reasonable detail. An indemnifying party will not, in any event, be liable for any settlement of any action or claim effected without its written consent. No indemnifying party shall, without the prior written consent of each indemnified party, settle or compromise or consent to the entry of any judgment in any pending or threatened claim, action or proceeding relating to the matters contemplated by this Section 10 (whether or not any indemnified party is a party thereto), unless such settlement, compromise or consent (1) includes an unconditional release of each indemnified party from all liability arising out of such litigation, investigation, proceeding or claim and (2) does not include a statement as to or an admission of fault, culpability or a failure to act by or on behalf of any indemnified party.

(d) Contribution. In order to provide for just and equitable contribution in circumstances in which the indemnification provided for in the foregoing paragraphs of this Section 10 is applicable in accordance with its terms but for any reason is held to be unavailable from the Company or an Agent, the Company and such Agent will contribute to the total losses, claims, liabilities, expenses and damages (including any investigative, legal and other expenses reasonably incurred in connection with, and any amount paid in settlement of, any action, suit or proceeding or any claim asserted, but after deducting any contribution received by the Company from persons other than the Agents, such as persons who control the Company within the meaning of the Securities Act, officers of the Company who signed the Registration Statement and directors of the Company, who also may be liable for contribution) to which the Company and the Agents may be subject in such proportion as shall be appropriate to reflect the relative benefits received by the Company on the one hand and the Agents on the other hand. The relative benefits received by the Company on the one hand and the Agents on the other hand shall be deemed to be in the same proportion as the total net proceeds from the sale of the Placement Shares (before deducting expenses) received by the Company bear to the total compensation received by the Agents (before deducting expenses) from the sale of Placement Shares on behalf of the Company. If but only if, the allocation provided by the foregoing sentence is not permitted by applicable law, the allocation of contribution shall be made in such proportion as is appropriate to reflect not only the relative benefits referred to in the foregoing sentence but also the relative fault of the Company, on the one hand, and such Agent, on the other hand, with respect to the statements or omission that resulted in such loss, claim, liability, expense or damage, or action in respect thereof, as well as any other relevant equitable considerations with respect to such offering. Such relative fault shall be determined by reference to, among other things, whether the untrue or alleged untrue statement of a material fact or omission or alleged omission to state a material fact relates to information supplied by the Company or such Agent, the intent of the parties and their relative knowledge, access to information and opportunity to correct or prevent such statement or omission. The Company and each Agent agree that it would not be just and equitable if contributions pursuant to this Section 10(d) were to be determined by pro rata allocation or by any other method of allocation that does not take into account the equitable considerations referred to herein. The amount paid or payable by an indemnified party as a result of the loss, claim, liability, expense, or damage, or

action in respect thereof, referred to above in this Section 10(d) shall be deemed to include, for the purpose of this Section 10(d), any legal or other expenses reasonably incurred by such indemnified party in connection with investigating or defending any such action or claim to the extent consistent with Section 10(c) hereof. Notwithstanding the foregoing provisions of this Section 10(d), an Agent shall not be required to contribute any amount in excess of the commissions received by it under this Agreement and no person found guilty of fraudulent misrepresentation (within the meaning of Section 11(f) of the Securities Act) will be entitled to contribution from any person who was not guilty of such fraudulent misrepresentation. For purposes of this Section 10(d), any person who controls a party to this Agreement within the meaning of the Securities Act, and any officers, directors, partners, employees or agents of an Agent, will have the same rights to contribution as that party, and each officer of the Company who signed the Registration Statement will have the same rights to contribution as the Company, subject in each case to the provisions hereof. Any party entitled to contribution, promptly after receipt of notice of commencement of any action against such party in respect of which a claim for contribution may be made under this Section 10(d), will notify any such party or parties from whom contribution may be sought, but the omission to so notify will not relieve that party or parties from whom contribution may be sought from any other obligation it or they may have under this Section 10(d) except to the extent that the failure to so notify such other party materially prejudiced the substantive rights or defenses of the party from whom contribution is sought. Except for a settlement entered into pursuant to the last sentence of Section 10(c) hereof, no party will be liable for contribution with respect to any action or claim settled without its written consent if such consent is required pursuant to Section 10(c) hereof.

11. Additional Representations and Covenants.

(a) Representations and Covenants of the Agents. Each Agent represents and warrants that it is duly registered as a broker-dealer under FINRA, the Exchange Act and the applicable statutes and regulations of each state in which the Placement Shares will be offered and sold except such states in which such Agent is exempt from registration or such registration is not otherwise required. Each Agent shall continue, for the term of this Agreement, to be duly registered as a broker-dealer under FINRA, the Exchange Act and the applicable statutes and regulations of each state in which the Placement Shares will be offered and sold, except such states in which such Agent is exempt from registration or such registration is not otherwise required, during the term of this Agreement. Each Agent shall comply with all applicable law and regulations in connection with the transactions contemplated by this Agreement, including the issuance and sale through such Agent of the Placement Shares.

(b) Representations and Agreements to Survive Delivery. The indemnity and contribution agreements contained in Section 10 of this Agreement and all representations and warranties of the Company and the Agents herein or in certificates delivered pursuant hereto shall survive, as of their respective dates, regardless of (i) any investigation made by or on behalf of the Agents, any controlling persons, or the Company (or any of their respective officers, directors or controlling persons), (ii) delivery and acceptance of the Placement Shares and payment therefor or (iii) any termination of this Agreement.

12. Termination.

(a) An Agent may terminate this Agreement with respect to itself, by notice to the Company, as hereinafter specified at any time (1) if there has been, since the time of execution of this Agreement or since the date as of which information is given in the Prospectus, any Material Adverse Effect, or any development that is reasonably likely to have a Material Adverse Effect or, in the sole judgment of such Agent, is material and adverse and makes it impractical or inadvisable to market the Placement Shares or to enforce contracts for the sale of the Placement Shares, (2) if there has occurred any material adverse change in the financial markets in the United States or the international financial markets, any outbreak of hostilities or escalation thereof or other calamity or crisis or any change or development involving a prospective change in national or international political, financial or economic conditions, in each case the effect of which is such as to make it, in the judgment of such Agent, impracticable or inadvisable to market the Placement Shares or to enforce contracts for the sale of the Placement Shares, (3) if trading in the Common Stock has been suspended or limited by the Commission or the Exchange, or if trading generally on the Exchange has been suspended or limited, or minimum prices for trading have been fixed on the Exchange, (4) if any suspension of trading of any securities of the Company on any exchange or in the over-the-counter market shall have occurred and be continuing, (5) if a major disruption of securities settlements or clearance services in the United States shall have occurred and be continuing, or (6) if a banking moratorium has been declared by either U.S. Federal or New York authorities. Any such termination shall be without liability of any party to any other party except that the provisions of Section 8 (Expenses), Section 10 (Indemnification), Section 11 (Survival of Representations), Section 17 (Governing Law; Consent to Jurisdiction) and Section 18 (Waiver of Jury Trial) hereof shall remain in full force and effect notwithstanding such termination. If an Agent elects to terminate this Agreement as provided in this Section 12(a) with respect to itself, such Agent shall provide the required notice as specified in Section 13 (Notices).

(b) The Company shall have the right, by giving written notice as hereinafter specified, to (i) terminate this Agreement or (ii) reduce the amount of Common Stock permitted to be issued and sold under this Agreement and offered by the Prospectus Supplement in its sole discretion at any time after the date of this Agreement. Any such termination shall be without liability of any party to any other party except that the provisions of Section 8, Section 10, Section 11, Section 17 and Section 18 hereof shall remain in full force and effect notwithstanding such termination.

(c) Each Agent shall have the right, by giving written notice as hereinafter specified, to terminate this Agreement with respect to itself in its sole discretion at any time after the date of this Agreement. Any such termination shall be without liability of any party to any other party except that the provisions of Section 8, Section 10, Section 11, Section 17 and Section 18 hereof shall remain in full force and effect notwithstanding such termination.

(d) Unless earlier terminated pursuant to this Section 12, this Agreement shall automatically terminate upon the issuance and sale of all of the Placement Shares through the Agents on the terms and subject to the conditions set forth herein; provided that the provisions of Section 8, Section 10, Section 11, Section 17 and Section 18 hereof shall remain in full force and effect notwithstanding such termination.

(e) This Agreement shall remain in full force and effect unless terminated pursuant to Sections 12(a), (b), (c), or (d) above or otherwise by mutual agreement of the parties; provided, however, that any such termination by mutual agreement shall in all cases be deemed to provide that Section 8, Section 10, Section 11, Section 17 and Section 18 shall remain in full force and effect; provided further that, for the avoidance of doubt to the extent this Agreement is terminated by one Agent pursuant to Sections 12(a) or (c) above, this Agreement shall terminate only with respect to such Agent and shall remain in full force and effect with respect to the Company and the other Agent, unless and until terminated pursuant to Sections 12(a), (b), (c), or (d) above.

(f) Any termination of this Agreement shall be effective on the date specified in such notice of termination; provided, however, that such termination shall not be effective until the close of business on the date of receipt of such notice by an Agent or the Company, as the case may be. Upon termination of this Agreement, the Company shall not have any liability to such Agent for any discount, commission or other compensation with respect to any Placement Shares not otherwise sold by such Agent under this Agreement; provided, however, if such termination shall occur prior to the Settlement Date for any sale of Placement Shares, such Placement Shares shall settle in accordance with the provisions of this Agreement.

13. Notices. All notices or other communications required or permitted to be given by any party to any other party pursuant to the terms of this Agreement shall be in writing, unless otherwise specified, and if sent to the Agents, shall be delivered to:

Roth Capital Partners, LLC
888 San Clemente
Newport Beach, CA 92660
Fax No.: (949) 720-7227
Attention: Equity Capital Markets

JonesTrading Institutional Services LLC
900 Island Park Drive, Suite 160
Daniel Island, SC 29492
Attn: Burke Cook
E-mail:

with a copy (which shall not constitute notice) to:

Duane Morris LLP
1540 Broadway
New York, NY 10036
Attn: James T. Seery
E-mail:

and if to the Company, shall be delivered to:

Acer Therapeutics Inc.

One Gateway Center, Suite 351
300 Washington Street
Newton, MA 02458
Attn: Chris Schelling
President and Chief Executive Officer

with a copy (which shall not constitute notice) to:

Pillsbury Winthrop Shaw Pittman LLP
12255 El Camino Real, Suite 300
San Diego, CA 92130
Attn: Mike Hird
Gabriella A. Lombardi

Each party to this Agreement may change such address for notices by sending to the parties to this Agreement written notice of a new address for such purpose. Each such notice or other communication shall be deemed given (i) when delivered personally, by email, or by verifiable facsimile transmission (with an original to follow) on or before 4:30 p.m., New York City time, on a Business Day or, if such day is not a Business Day, on the next succeeding Business Day, (ii) on the next Business Day after timely delivery to a nationally-recognized overnight courier and (iii) on the Business Day actually received if deposited in the U.S. mail (certified or registered mail, return receipt requested, postage prepaid).

An electronic communication ("Electronic Notice") shall be deemed written notice for purposes of this Section 13 if sent to the electronic mail address specified by the receiving party under separate cover. Electronic Notice shall be deemed received at the time the party sending Electronic Notice receives verification of receipt by the receiving party. Any party receiving Electronic Notice may request and shall be entitled to receive the notice on paper, in a nonelectronic form ("Nonelectronic Notice") which shall be sent to the requesting party within ten (10) days of receipt of the written request for Nonelectronic Notice.

14. Successors and Assigns. This Agreement shall inure to the benefit of and be binding upon the Company and each Agent and their respective successors and the affiliates, controlling persons, officers and directors referred to in Section 10 hereof. References to any of the parties contained in this Agreement shall be deemed to include the successors and permitted assigns of such party. Nothing in this Agreement, express or implied, is intended to confer upon any party other than the parties hereto or their respective successors and permitted assigns any rights, remedies, obligations or liabilities under or by reason of this Agreement, except as expressly provided in this Agreement. Neither party may assign its rights or obligations under this Agreement without the prior written consent of the other party.

15. Adjustments for Stock Splits. The parties acknowledge and agree that all share-related numbers contained in this Agreement shall be adjusted to take into account any stock split, stock dividend or similar event effected with respect to the Placement Shares.

16. Entire Agreement; Amendment; Severability. The Sales Agreement dated November 9, 2019 between the Company and Roth is hereby amended in its entirety and restated

herein. This Agreement (including all schedules and exhibits attached hereto and Placement Notices issued pursuant hereto) constitutes the entire agreement and supersedes all other prior and contemporaneous agreements and undertakings, both written and oral, among the parties hereto with regard to the subject matter hereof, including, without limitation, engagement letter dated September 28, 2018 between the Company and Roth. Neither this Agreement nor any term hereo may be amended except pursuant to a written instrument executed by the Company and the Agents. In the event that any one or more of the provisions contained herein, or the application thereof in any circumstance, is held invalid, illegal or unenforceable as written by a court of competent jurisdiction, then such provision shall be given full force and effect to the fullest possible extent that it is valid, legal and enforceable, and the remainder of the terms and provisions herein shall be construed as if such invalid, illegal or unenforceable term or provision was not contained herein, but only to the extent that giving effect to such provision and the remainder of the terms and provisions hereof shall be in accordance with the intent of the parties as reflected in this Agreement.

17. **GOVERNING LAW AND TIME; WAIVER OF JURY TRIAL. THIS AGREEMENT SHALL BE GOVERNED AND CONSTRUED IN ACCORDANCE WITH THE LAWS OF THE STATE OF NEW YORK WITHOUT REGARD TO THE PRI OF CONFLICTS OF LAWS. SPECIFIED TIMES OF DAY REFER TO NEW YORK CITY TIME. EACH PARTY HEREBY IRREVO WAIVES, TO THE FULLEST EXTENT PERMITTED BY APPLICABLE LAW, ANY AND ALL RIGHT TO TRIAL BY JURY IN ANY LEGAL PROCEEDING ARISING OUT OF OR RELATING TO THIS AGREEMENT OR THE TRANSACTIONS CONTEMPLATED HEREBY.**

18. **CONSENT TO JURISDICTION. EACH PARTY HEREBY IRREVOCABLY SUBMITS TO THE N EXCLUSIVE JURISDICTION OF THE STATE AND FEDERAL COURTS SITTING IN THE CITY OF NEW YORK, BORO MANHATTAN, FOR THE ADJUDICATION OF ANY DISPUTE HEREUNDER OR IN CONNECTION WITH ANY TRANS CONTEMPLATED HEREBY, AND HEREBY IRREVOCABLY WAIVES, AND AGREES NOT TO ASSERT IN ANY SUIT, ACTI PROCEEDING, ANY CLAIM THAT IT IS NOT PERSONALLY SUBJECT TO THE JURISDICTION OF ANY SUCH COURT, THA SUIT, ACTION OR PROCEEDING IS BROUGHT IN AN INCONVENIENT FORUM OR THAT THE VENUE OF SUCH SUIT, AC PROCEEDING IS IMPROPER. EACH PARTY HEREBY IRREVOCABLY WAIVES PERSONAL SERVICE OF PROCESS AND CO TO PROCESS BEING SERVED IN ANY SUCH SUIT, ACTION OR PROCEEDING BY MAILING A COPY THEREOF (CERTI REGISTERED MAIL, RETURN RECEIPT REQUESTED) TO SUCH PARTY AT THE ADDRESS IN EFFECT FOR NOTICE UNDER THIS AGREEMENT AND AGREES THAT SUCH SERVICE SHALL CONSTITUTE GOOD AND SUFFICIENT SER PROCESS AND NOTICE THEREOF. NOTHING CONTAINED HEREIN SHALL BE DEEMED TO LIMIT IN ANY WAY ANY RI SERVE PROCESS IN ANY MANNER PERMITTED BY LAW.**

19. Use of Information. The Agents may not use any information gained in connection with this Agreement and the transactions contemplated by this Agreement, including

due diligence, to advise any party with respect to transactions not expressly approved by the Company.

20. Counterparts. This Agreement may be executed in two or more counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument. Delivery of an executed Agreement by one party to the other may be made by facsimile transmission.

21. Effect of Headings. The section and Exhibit headings herein are for convenience only and shall not affect the construction hereof.

22. Permitted Free Writing Prospectuses The Company represents, warrants and agrees that, unless it obtains the prior consent of each Agent, which consent shall not be unreasonably withheld, conditioned or delayed, and each Agent represents, warrants and agrees that, unless it obtains the prior consent of the Company, which consent shall not be unreasonably withheld, conditioned or delayed, it has not made and will not make any offer relating to the Placement Shares that would constitute an Issuer Free Writing Prospectus, or that would otherwise constitute a "free writing prospectus," as defined in Rule 405, required to be filed with the Commission. Any such free writing prospectus consented to by the Agents or by the Company, as the case may be, is hereinafter referred to as a "Permitted Free Writing Prospectus." The Company represents and warrants that it has treated and agrees that it will treat each Permitted Free Writing Prospectus as an "issuer free writing prospectus," as defined in Rule 433, and has complied and will comply with the requirements of Rule 433 applicable to any Permitted Free Writing Prospectus, including timely filing with the Commission where required, legending and record keeping.

23. Absence of Fiduciary Relationship. The Company acknowledges and agrees that:

(a) Each Agent is acting solely as agent in connection with the public offering of the Placement Shares and in connection with each transaction contemplated by this Agreement and the process leading to such transactions, and no fiduciary or advisory relationship between the Company or any of its respective affiliates, stockholders (or other equity holders), creditors or employees or any other party, on the one hand, and the Agents, on the other hand, has been or will be created in respect of any of the transactions contemplated by this Agreement, irrespective of whether or not any Agent has advised or is advising the Company on other matters, and the Agents have no obligation to the Company with respect to the transactions contemplated by this Agreement except the obligations expressly set forth in this Agreement;

(b) it is capable of evaluating and understanding, and understands and accepts, the terms, risks and conditions of the transactions contemplated by this Agreement;

(c) the Agents have not provided any legal, accounting, regulatory or tax advice with respect to the transactions contemplated by this Agreement and it has consulted its own legal, accounting, regulatory and tax advisors to the extent it has deemed appropriate;

(d) it is aware that the Agents and their respective affiliates are engaged in a broad range of transactions which may involve interests that differ from those of the Company

and the Agents have no obligation to disclose such interests and transactions to the Company by virtue of any fiduciary, advisory or agency relationship or otherwise; and

(e) it waives, to the fullest extent permitted by law, any claims it may have against the Agents for breach of fiduciary duty or alleged breach of fiduciary duty in connection with the sale of Placement Shares under this Agreement and agrees that the Agents shall not have any liability (whether direct or indirect, in contract, tort or otherwise) to it in respect of such a fiduciary duty claim or to any person asserting a fiduciary duty claim on its behalf or in right of it or the Company, employees or creditors of Company, other than in respect of the Agents' obligations under this Agreement and to keep information provided by the Company to the Agents and the Agents' counsel confidential to the extent not otherwise publicly-available.

24. Miscellaneous; Definitions.

As used in this Agreement, the following terms have the respective meanings set forth below:

"Applicable Time" means (i) each Representation Date and (ii) the time of each sale of any Placement Shares pursuant to this Agreement.

"Business Day" shall mean any day on which the Exchange and commercial banks in the City of New York are open for business.

"Issuer Free Writing Prospectus" means any "issuer free writing prospectus," as defined in Rule 433, relating to the Placement Shares that (1) is required to be filed with the Commission by the Company, (2) is a "road show" that is a "written communication" within the meaning of Rule 433(d)(8)(i) whether or not required to be filed with the Commission, or (3) is exempt from filing pursuant to Rule 433(d)(5)(i) because it contains a description of the Placement Shares or of the offering that does not reflect the final terms, in each case in the form filed or required to be filed with the Commission or, if not required to be filed, in the form retained in the Company's records pursuant to Rule 433(g) under the Securities Act.

"Rule 172," "Rule 405," "Rule 415," "Rule 424," "Rule 424(b)," "Rule 430B," and "Rule 433" refer to such rules under the Securities Act.

"Trading Day" means any day on which shares of Common Stock are purchased and sold on the Exchange.

All references in this Agreement to financial statements and schedules and other information that is "contained," "included" or "stated" in the Registration Statement or the Prospectus (and all other references of like import) shall be deemed to mean and include all such financial statements and schedules and other information that is incorporated by reference in the Registration Statement or the Prospectus, as the case may be.

All references in this Agreement to the Registration Statement, the Prospectus or any amendment or supplement to any of the foregoing shall be deemed to include the copy filed with the Commission pursuant to EDGAR; all references in this Agreement to any Issuer Free Writing

Prospectus (other than any Issuer Free Writing Prospectuses that, pursuant to Rule 433, are not required to be filed with the Commission) shall be deemed t include the copy thereof filed with the Commission pursuant to EDGAR; and all references in this Agreement to "supplements" to the Prospectus shall include without limitation, any supplements, "wrappers" or similar materials prepared in connection with any offering, sale or private placement of any Placement Shares by the Agents outside of the United States.

If the foregoing correctly sets forth the understanding among the Company and the Agents, please so indicate in the space provided below for that purpose, whereupon this letter shall constitute a binding agreement among the Company and the Agents.

Very truly yours,

ACER THERAPEUTICS INC.

By: /s/ Chris Schelling
Name: Chris Schelling
Title: President and Chief Executive Officer

ACCEPTED as of the date first-above written:

ROTH CAPITAL PARTNERS, LLC

By: /s/ Eric B. Cheng
Name: Eric B. Cheng
Title: Managing Director, Co-Head of Healthcare Investment Banking

JONESTRADING INSTITUTIONAL SERVICES LLC

By: /s/ Burke Cook
Name: Burke Cook
Title: General Counsel

SCHEDULE 1

FORM OF PLACEMENT NOTICE

From: ACER THERAPEUTICS INC.

To: [ROTH CAPITAL PARTNERS, LLC][JONESTRADING INSTITUTIONAL SERVICES LLC]

Attention: _____

Subject: Placement Notice

Date:

Ladies and Gentlemen:

Pursuant to the terms and subject to the conditions contained in the Amended and Restated Sales Agreement among Acer Therapeutics Inc. (the "Company") and Roth Capital Partners, LLC and JonesTrading Institutional Services LLC (together, the "Agents"), dated March 18, 2020, the Company hereby requests that [*identify Designated Agent*] sell up to _____ of the Company's Common Stock, $0.0001 par value per share, at a minimum market price of $_____ per share, during the time period beginning [month, day, time] and ending [month, day, time].

SCHEDULE 2

Compensation

The Company shall pay to the Designated Agent in cash, upon each sale of Placement Shares pursuant to this Agreement, an amount equal to 3.5% of the gross proceeds from each sale of Placement Shares.

SCHEDULE 3

Notice Parties

The Company

Harry S. Palmin
Don Joseph
Jason S. Kneeland

Roth

Eric Cheng
Lou Ellis
Nazan Akdeniz
Phil DiNapoli

with a copy to

JonesTrading
Moe Cohen
Bryan Turley
John D'Agostini
Ryan Gerety
Burke Cook

with a copy to

EXHIBIT 7(l)

Form of Representation Date Certificate

_____, 20__

This Representation Date Certificate (this "Certificate") is executed and delivered in connection with Section 7(l) of the Amended and Restated Sale Agreement (the "Agreement"), dated March 18, 2020, and entered into among Acer Therapeutics Inc. (the "Company") and Roth Capital Partners, LLC an JonesTrading Institutional Services LLC. All capitalized terms used but not defined herein shall have the meanings given to such terms in the Agreement

The undersigned, a duly appointed and authorized officer of the Company, hereby certifies as follows in such capacity on behalf of the Company:

1. As of the date of this Certificate, (i) the Registration Statement does not contain any untrue statement of a material fact or omit to state a material fac required to be stated therein or necessary in order to make the statements therein not misleading and (ii) neither the Registration Statement nor the Prospectus contain any untrue statement of a material fact or omit to state a material fact required to be stated therein or necessary in order to make the statements therein, in light of the circumstances under which they were made, not misleading and (iii) no event has occurred as a result of which it is necessary to amend or supplement the Prospectus in order to make the statements therein not untrue or misleading.

2. Each of the representations and warranties of the Company contained in the Agreement were, when originally made, and are, as of the date of this Certificate, true and correct in all material respects.

3. Except as waived by the Agents in writing, each of the covenants required to be performed by the Company in the Agreement on or prior to the date of the Agreement, this Representation Date, and each such other date as set forth in the Agreement, has been duly, timely and fully performed in all material respects and each condition required to be complied with by the Company on or prior to the date of the Agreement, this Representation Date, and each such other date as set forth in the Agreement has been duly, timely and fully complied with in all material respects.

4. Subsequent to the date of the most recent financial statements in the Prospectus, except as described in the Prospectus, including the Incorporated Documents, there has been no Material Adverse Effect.

5. No stop order suspending the effectiveness of the Registration Statement or of any part thereof has been issued, and no proceedings for that purpose have been instituted or are pending or threatened by any securities or other governmental authority (including, without limitation, the Commission).

The undersigned has executed this Representation Date Certificate as of the date first written above.

ACER THERAPEUTICS INC.

By:_____
Name:
Title:

Consent of Independent Registered Public Accounting Firm

Acer Therapeutics Inc.
Newton, Massachusetts

We hereby consent to the incorporation by reference in the Registration Statements on Form S-3 (No. 333-228319) and Form S-8 (Nos. 333-221566, 333-224942 and 333-230133) of Acer Therapeutics Inc. of our report dated March 18, 2020, relating to the consolidated financial statements, which appears in this Form 10-K. Our report contains an explanatory paragraph regarding the Company's ability to continue as a going concern.

/s/ BDO USA, LLP

Boston, Massachusetts
March 18, 2020

EXHIBIT 23.2

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We consent to the incorporation by reference in Registration Statements on Form S-3 (File No. 333-228319) and Form S-8 (File Nos. 333-230133, 333-221566, and 333-224942) of our report dated March 7, 2019 relating to the consolidated financial statements of Acer Therapeutics Inc., appearing in the Annual Report on Form 10-K of Acer Therapeutics Inc. for the year ended December 31, 2019.

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 18, 2020

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 most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

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

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

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

Date: March 18, 2020

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 most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

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

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

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

Date: March 18, 2020

By: /s/ Harry Palmin
Harry Palmin
Chief Operating Officer and 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, 2019 (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 18, 2020

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, 2019 (the "Report"), as filed with the Securities and Exchange Commission on the date hereof, I, Harry Palmin, Chief Operating Officer and 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 18, 2020

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