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, 2020

☐ **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, Suite 351, 300 Washington Street, 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 has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☐

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

The aggregate market value of the voting and non-voting common equity held by non-affiliates of the registrant as of June 30, 2020, based upon the closing price as of such date was $20,699,045.

As of February 15, 2021, 14,310,244 shares of the registrant's common stock, par value $0.0001 per share, were outstanding.

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 ACER-001 (sodium phenylbutyrate), EDSIVO™ (celiprolol), ACER-801 (osanetant), and ACER-2820 (emetine), constitute forward-looking statements. Such forward-looking statements are subject to a number of risks and uncertainties that could affect our ability to successfully implement our business strategy and cause actual results to differ materially from those anticipated. You should carefully consider all of the information in this report and, in particular, the following principal risks and all of the other specific factors further discussed in Item 1A of this report, "Risk Factors," before deciding whether to invest in our company:

Summary Risk Factors

- Substantial doubt exists as to our ability to continue as a going concern
- We will require additional financing to complete development and seek 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
- We may not be able to successfully negotiate and enter into a definitive agreement with Relief Therapeutics Holding AG ("Relief") for the potential collaboration and license of ACER-001 on the terms outlined in the option agreement, on other mutually acceptable terms, or at all. If we do not enter into a definitive agreement with Relief, we may not be able to repay the $4.0 million 12-month loan we received from Relief, which is secured by all of our assets
- Funding from our purchase agreement with Lincoln Park Capital Fund, LLC ("Lincoln Park") may be limited or be insufficient to fund our operations or implement our strategy
- Funding from our "at-the-market" ("ATM") facility with JonesTrading Institutional Services LLC ("Jones Trading") and Roth Capital Partners, LLC ("Roth Capital") may be limited or be insufficient to fund our operations or to implement our strategy
- 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 currently have no source of product sales revenue and may never be profitable
- 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. Neither resubmission nor approval of our NDA for EDSIVOTM is assured. We may decide at any time not to continue development of EDSIVOTM

- We face risks related to health epidemics including but not limited to the COVID-19 pandemic which could adversely affect our business
- 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
- 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 seek approval under Section 505(b)(2), we may be unable to meet our anticipated development and commercialization timelines, and could decide not to pursue further development, depending on the expected time, cost, and risks associated with generating any such additional data
- 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 studies to assess the safety and efficacy of our product candidates. We could decide not to pursue further development of one or all of our product candidates, depending on, among other things, the expected time, cost, and risks associated with generating any such additional data
- Clinical drug development involves a lengthy and expensive process with an uncertain outcome. Clinical development of product candidates for rare diseases carry additional risks, such as recruiting patients in a very small patient population
- 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
- As an organization, we have limited experience in designing and completing clinical trials, and may be unable to do so efficiently or at all for our current product candidates or any 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
- We may not be able to win government, academic institution or non-profit contracts or grants, which could affect the timing or continued development of one or more of our product candidates, and emetine in particular
- 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, among other factors, market awareness and acceptance of our product candidates
- 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
- We face substantial competition, which may result in others discovering, developing or commercializing products for our targeted indications before, or more successfully, than we do
- We rely on third-party suppliers and other third parties for manufacture of our product candidates and our dependence on these third parties may impair or delay the advancement of our research and development programs and the development of our product candidates

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

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

- We are a party to license or similar agreements under which we license intellectual property, data, and/or receive commercialization rights relating to ACER-001, EDSIVOTM, osanetant, and emetine. 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

- Our share price is very volatile, may not reflect the underlying value of our net assets or business prospects, and you may not be able to resell your shares at a profit or at all

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

- 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

- 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

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"), including but not limited to the Risk Factors associated with our business.

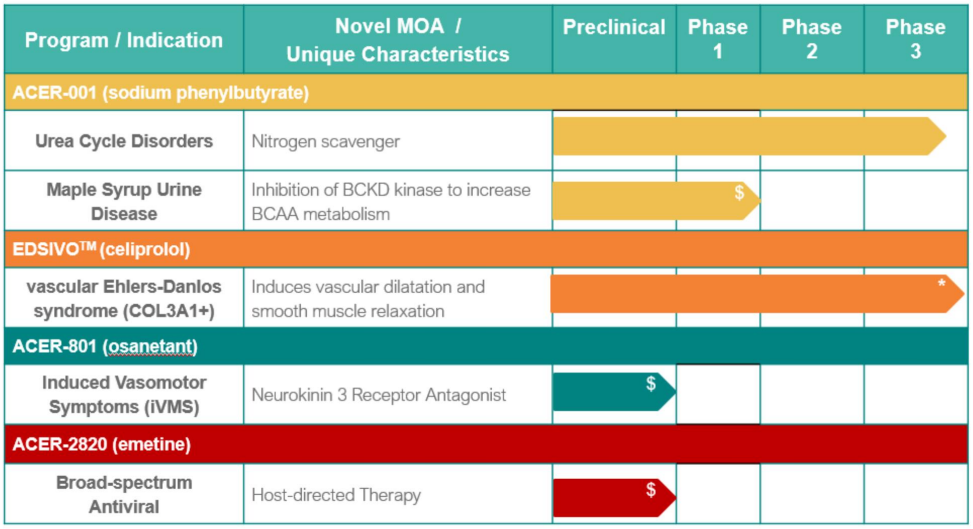
PART I

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 four programs: ACER-001 (sodium phenylbutyrate) for the treatment of various inborn errors of metabolism, including urea cycle disorders ("UCDs") and Maple Syrup Urine Disease ("MSUD"); EDSIVO™ (celiprolol) for the treatment of vascular Ehlers-Danlos syndrome ("vEDS") in patients with a confirmed type III collagen (COL3A1) mutation; ACER-801 (osanetant) for the treatment of induced Vasomotor Symptoms ("iVMS"); and ACER-2820 (emetine), a host-directed therapy against a variety of infectious diseases, including COVID-19. Our product candidates are believed to present comparatively de-risked programs as evidenced by having one or more of the following: favorable safety profile, clinical proof-of-concept data, mechanistic differentiation, and/or accelerated pathways 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:



$ Additional capital resources required to fund these programs going forward

* In response to a Complete Response Letter from the FDA for our New Drug Application ("NDA") for EDSIVO™ for the treatment of patients with vEDS with a confirmed COL3A1 mutation, we submitted a Formal Dispute Resolution Request which was denied in March 2020 by the FDA's Office of New Drugs. However, the Office of New Drug's denial 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

- ACER-001 (sodium phenylbutyrate)
  - Signed an Option Agreement with Relief Therapeutics Holding AG ("Relief") on January 25, 2021, providing Relief with exclusivity until June 30, 2021 for the right to pursue a potential collaboration and license agreement for worldwide development and commercialization for ACER-001. In return, Acer received an upfront nonrefundable payment of $1.0 million and a $4.0 million secured loan from Relief
  - Announced in February 2021 topline results from Acer's bioequivalence ("BE") trial in which ACER-001 showed similar relative bioavailability compared to BUPHENYL® (sodium phenylbutyrate) under fed conditions
  - Targeting a pre-NDA meeting with FDA in the second quarter of 2021, assuming successful and timely completion of the ongoing development activities
  - Submission of an ACER-001 NDA for treatment of patients with UCDs is anticipated in mid-2021, provided that no additional data is requested by the FDA during Acer's pre-NDA meeting and ongoing development activities are successfully completed (including evaluation of long-term product stability data)
- EDSIVO™ (celiprolol)
  - Submitted Type B meeting request to FDA in February 2021 to discuss Acer's proposed plan to collect additional data in support of celiprolol's potential benefit in treating COL3A1-positive vEDS patients
  - If FDA discussions are successful, and resulting data is positive, could potentially satisfy the substantial evidence of effectiveness needed to support a possible resubmission of the EDSIVO™ NDA (although neither EDSIVO™ NDA resubmission nor approval is assured)
- ACER-801 (osanetant)
  - Investigational New Drug Application ("IND") submission for osanetant is anticipated in the third quarter of 2021
  - Initiation of a Phase 2 clinical trial of osanetant in BRCA-positive patients who have undergone a prophylactic bilateral salpingo-oophorectomy ("PBSO") is expected in the fourth quarter of 2021, dependent upon successful IND filing and subject to additional capital
- ACER-2820 (emetine)
  - Further advancement of the emetine program in COVID-19 and other infectious diseases is dependent on our ability to raise non-dilutive capital
  - We believe that most of the emetine IND-enabling work is in-progress or complete, and we intend to minimize future emetine spending as we continue to work with federal agencies and private research organizations toward the goal of securing non-dilutive funding

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

ACER-001 (sodium phenylbutyrate)

Background

Sodium phenylbutyrate ("NaPB") is currently approved in the U.S. and the European Union ("EU") to treat patients with UCDs. Our product candidate ACER-001 is a proprietary powder formulation of NaPB. The formulation is designed to be both taste-masked and immediate release. ACER-001 is being developed using a microencapsulation process for the treatment of various inborn errors of metabolism, including UCDs and MSUD.

Urea Cycle Disorders

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. UCDs 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 UCDs is based on clinical observations, confirmed by biochemical and molecular genetic testing. A plasma ammonia concentration of 150 μmol/L or higher associated with a normal anion gap and a normal plasma glucose concentration is an indication for the presence of UCDs. Plasma quantitative amino acid analysis and measurement of urinary orotic acid can distinguish between the various types of UCDs. A definitive diagnosis of UCDs 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 UCDs in the U.S. is about 1 in 35,000 live births.[1] Approximately 2,000 patients suffer from UCDs in the U.S.

Current Treatment Options for UCDs

The current treatment of UCDs 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 UCDs, 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 UCDs 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 regimen for UCDs patients. Current nitrogen scavenger treatments for UCDs are based on phenylbutyrate or benzoate, which conjugate with glutamine or glycine, respectively, allowing for urinary excretion of nitrogen as phenylacetylglutamine or hippurate, respectively.

According to a 2016 study by Shchelochkov et al., published in *Molecular Genetics and Metabolism Reports*[2], while nitrogen scavenging medications are effective in helping to manage UCDs, 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 ("GPB"), which is marketed as RAVICTI®. While a study provided by Horizon Therapeutics, Inc. in the RAVICTI® package insert involving 46 adults with UCDs 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 average annual cost of RAVICTI® is $900,000 (based on patient weight), which is often prohibitively expensive.2

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

Rationale for ACER-001 Treatment in UCDs

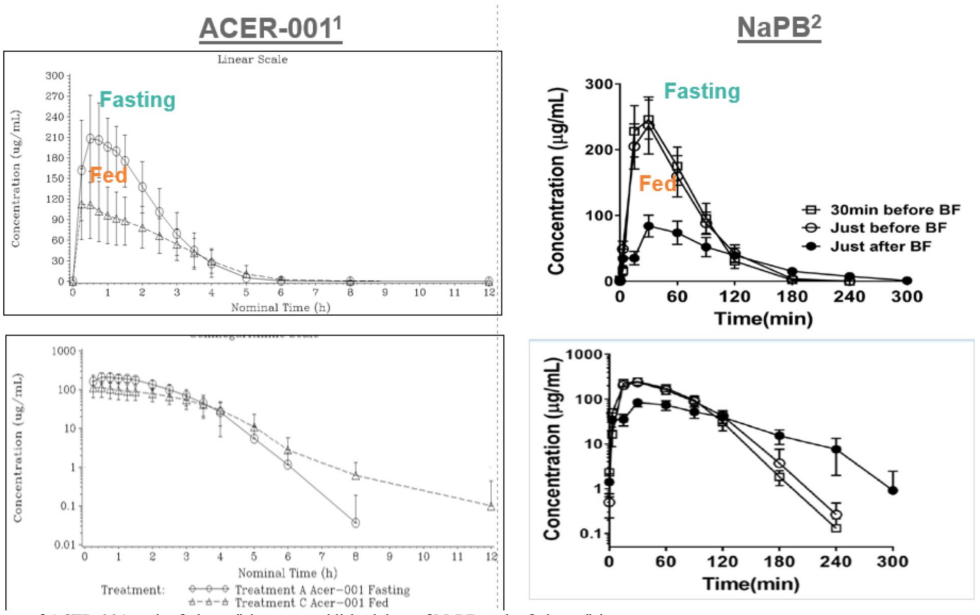
ACER-001 is a powder formulation of NaPB. The formulation is designed to be both taste-masked and immediate release. BUPHENYL®, a non-taste-masked formulation of NaPB, has been approved by the FDA for UCDs with demonstrated efficacy and safety in UCDs patients of all ages. We believe that if it is approved, ACER-001's taste-masked properties will make it an alternative to existing NaPB-based treatments, as the unpleasant taste associated with NaPB is cited as a major impediment to patient compliance with those treatments.

Bridging Studies

1. Under Fasted Conditions

In February 2020, we reported the completion and final data from our clinical trial evaluating the bioavailability and bioequivalence of ACER-001 to BUPHENYL® (sodium phenylbutyrate) both under fasted conditions. The 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 under fasted conditions. Data showed ACER-001 to have similar pharmacokinetic ("PK") profiles for both phenylbutyrate ("PBA") and phenylacetate ("PAA") compared to BUPHENYL® under fasted conditions.

This trial also included an arm of ACER-001 administered under fed conditions. When the fed and fasted arms of the study were compared, it was shown that administration of ACER-001 in a fasted state achieved more than two times the maximum concentration ("C_{max}") of PBA compared to administration of the same dose of ACER-001 in a fed state. These results are consistent with previously published data by Nakano, et al3 that evaluated PK of NaPB in patients with progressive familial intrahepatic cholestasis, also demonstrating that administration of NaPB in a fasted state significantly increased PBA peak plasma concentration compared to administration of NaPB in a fed state.



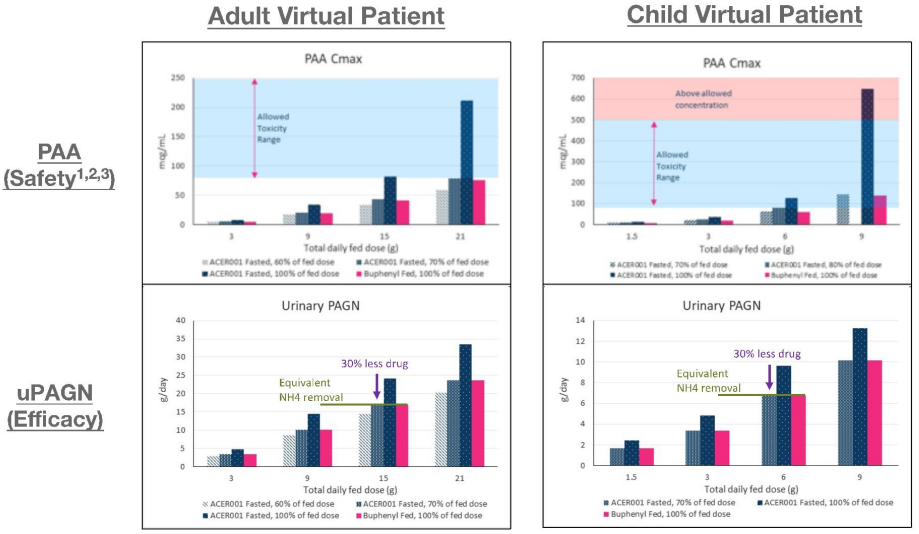
*Based on data comparison of ACER-001 under fed conditions vs. published data of NaPB under fed conditions

[1] ACER-001 BE/BA Study (Part B) in healthy volunteers

[2] Sci Rep 9, 17075 (2019). https://doi.org/10.1038/s41598-019-53628-x

Currently approved therapies for UCDs, including BUPHENYL®4 and RAVICTI®5, are required to be administered with food. BUPHENYL® is required to be administered in a fed state due to its aversive odor and taste, with side effects including nausea, vomiting and headaches, which can lead to discontinuation of treatment.6 Additionally, prescribing information states that the BUPHENYL® food effect is unknown. RAVICTI® PK and pharmacodynamic ("PD") properties were determined to be indistinguishable in fed or fasted states.7 ACER-001 is uniquely formulated with its multi-particulate, taste-masked coating to allow for administration in a fasted state, while still allowing for rapid systemic release.

Based on the results from the food effect study within the first ACER-001 BE trial, we commissioned Rosa & Co. LLC to create a PhysioPD® PK model to evaluate the potential food effect on exposure, tolerability and efficacy of ACER-001 in UCDs patients. Results from this *in silico* model suggested that administration of ACER-001 in a fasted state required approximately 30% less PBA to achieve comparable therapeutic benefit in a fed state. In addition, the model predicted that administration of ACER-001 in a fasted state compared to administration of BUPHENYL® or RAVICTI® (same amounts of PBA) in their required fed states would be expected to result in higher peak blood PBA, PAA and PAGN concentrations, predicting a 43% increase in urinary PAGN levels (a negative correlation between blood ammonia area under the curve and 24-hour urinary PAGN amount has been demonstrated).[8]



- ACER-001 in a fasted state required ~30% less PBA to achieve comparable therapeutic benefit in a fed state
- Model predicted 43% increase in urinary PAGN levels (negative correlation with blood ammonia AUC)

1 Mol Genet Metab. 2013 Dec; 110(4): 446-453.
2 Pediatr Res. 1986 Nov; 20(11):1117-21.
3 Cancer. 1995 Jun 15; 75(12):2932-8.

1. Under Fed Conditions

In February 2021, we announced topline results from our bioequivalence trial in which ACER-001 showed similar relative bioavailability to BUPHENYL® (sodium phenylbutyrate) under fed conditions. The single-center, single-blind, randomized, single-dose crossover trial evaluated BE of ACER-001 compared to BUPHENYL® when administered under fed conditions in 36 healthy adults. The topline data from this trial showed ACER-001 to have similar PK profiles for both PBA and PAA compared to BUPHENYL® under fed conditions.

Registration Plan

We intend to seek FDA approval in the U.S. to market ACER-001 for administration initially under fed conditions for the treatment of UCDs 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 EMA approval in the European Union and potentially other territories outside the U.S., after the 505(b)(2) NDA for treatment of UCDs 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 and efficacy data, while supplementing the data with a bridging study that shows similar relative bioavailability of ACER-001 to BUPHENYL®.

In August 2020, the FDA provided further clarity on our proposed regulatory paths forward for administration of ACER-001 under fed or fasted (pre-meal) conditions. In its feedback, the FDA stated that because BUPHENYL® is labeled for administration with food and a food effect has been observed with ACER-001, we should conduct an additional BE trial under fed conditions. As presented above, we therefore conducted a BE trial comparing the PK of BUPHENYL® and ACER-001, both under fed conditions. The results of this BE study will be included as a part of our planned NDA submission under the Section 505(b)(2) regulatory pathway for ACER-001 in the treatment of UCDs, where ACER-001 is administered with food. We are targeting a pre-NDA meeting with FDA in the second quarter of 2021, assuming successful and timely completion of the ongoing development activities. We intend to submit an ACER-001 NDA for treatment of patients with UCDs in mid-2021, provided no additional data is requested by the FDA during our pre-NDA meeting and ongoing development activities are successfully completed (including evaluation of long-term product stability data).

In parallel or after initial potential FDA approval for administration under fed conditions, and subject to additional capital, we also plan to evaluate potential development of ACER-001 for administration under fasted (pre-meal) conditions, which will likely require additional nonclinical and clinical studies in order to provide the necessary evidence of safety and efficacy of ACER-001 to be considered for FDA approval for administration under fasted (pre-meal) conditions.

ACER-001 is an investigational drug in the U.S. and is not currently FDA approved for UCDs.

Maple Syrup Urine Disease

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 branched-chain ketoacids ("BCKA") in a patient's blood. Left untreated, this can result in neurological damage, mental disability, coma or death. 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.

Diagnosis and Incidence

MSUD is typically diagnosed at birth via newborn screening. 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 1,000 patients are located in the U.S.9

Current Treatment Options in MSUD

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.

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.[10]

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 showed 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 clinically meaningful response.

Investigators at BCM further explored the mechanistic rationale for NaPB lowering BCAA/BCKA levels. NaPB was found to be an allosteric inhibitor of the branched-chain keto acid dehydrogenase complex kinase ("BCKD-kinase"), and enzyme that regulates the activity of the branched-chain keto acid dehydrogenase complex ("BCKDC") enzyme that is responsible for the normal metabolism of BCKAs.[11] By inhibiting the BCKD-kinase, the BCKDC is constitutively activated, thus the increased activity results in a reduction in the plasma levels of BCAA and BCKA in all people, including those with MSUD, suggesting that NaPB may be an effective treatment for people with MSUD, who experience elevated BCAA levels.[9,11]

In November 2020, study results evaluating the effect of NaPB in the management of acute MSUD attacks in pediatric patients (n=10) were published in the Journal of Pediatric Endocrinology and Metabolism showing a significant reduction in leucine levels in MSUD patients experiencing an acute attack.[12] The results suggested that NaPB can be safely administered in combination with emergency protocol and may provide additional clinical benefit beyond emergency protocol alone. However, verifying this outcome would require additional validation in a controlled trial. If ACER-001 is approved for the treatment of chronic MSUD, we believe patients will not be required to interrupt their therapy in the event of an acute crisis.

Registration Plan

We anticipate initiation of clinical studies evaluating ACER-001 in MSUD to occur in late 2021, subject to additional capital. If these clinical studies are successful, we plan to seek FDA approval to market ACER-001 for the treatment of MSUD in the U.S. by submitting a 505(b)(2) NDA incorporating data from BUPHENYL's® NDA (the reference listed drug) while supplementing our intended NDA for ACER-001 with additional PK, PD, efficacy and safety data specifically in the MSUD population, 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 is an investigational drug in the U.S. and is not currently FDA approved for MSUD.

Option Agreement with Relief Therapeutics Holding AG

On January 25, 2021, we and Relief entered into an option agreement pursuant to which we granted Relief an exclusive option to pursue a potential collaboration and license agreement with us for the development, regulatory approval and commercialization for ACER-001 for the treatment of UCDs and MSUD. The option agreement provides a period of time up to June 30, 2021 for the parties to perform additional due diligence and to work toward negotiation and execution of a definitive agreement with respect to the potential collaboration for ACER-001. In

consideration for the grant of the exclusivity option, (i) we received from Relief an upfront nonrefundable payment of $1.0 million, (ii) Relief provided to us a 12-month secured loan in the principal amount of $4.0 million, as evidenced by a promissory note we issued to Relief, and (iii) we granted to Relief a security interest in all of our assets to secure performance of the promissory note, as evidenced by a security agreement. The note is repayable in one lump sum within 12 months from issuance and bears interest at a rate equal to 6% per annum. If a definitive agreement with respect to the potential collaboration is not executed by the parties on or before June 30, 2021, the exclusivity option will terminate and the note is repayable by us upon maturity. The note contains certain customary events of default (including, but not limited to, default in payment of principal or interest thereunder or a material breach of the security agreement). Under the terms of the proposed collaboration and license agreement, the key terms of which are set forth in the option agreement, if a definitive agreement is executed pursuant to these terms and closed by June 30, 2021, we will receive $14.0 million in cash (which can be offset at Relief's option by the outstanding balance of the $4.0 million loan from Relief to us). In addition, Relief will agree to pay up to $20.0 million in U.S. development and commercial launch costs for the UCDs and MSUD indications. Further, we will retain development and commercialization rights in the U.S., Canada, Brazil, Turkey and Japan. The companies will split net profits from Acer's territories 60%:40% in favor of Relief. Relief will also license the rights for the rest of the world, where we will receive from Relief a 15% net sales royalty on all revenues received in Relief's territories. We could also receive a total of $6.0 million in milestones based on the first European marketing approvals for UCDs and MSUD. There can be no assurance, however, that a definitive agreement will be successfully negotiated and executed between the parties on these terms, on other mutually acceptable terms, or at all. Except for the $1.0 million upfront payment to us and the $4.0 million 12-month secured loan from Relief to us, the remaining proposed terms of the collaboration are not binding and are subject to change as a result of further diligence by Relief and negotiation of a definitive collaboration and license agreement between the parties. If we are unsuccessful in negotiating and executing a definitive agreement with Relief, we will be free to negotiate with third parties for partnering ACER-001 or to continue to develop and commercialize independently.

EDSIVO™ (celiprolol)

Background

EDSIVO™ is a selective adrenergic modulator ("SAM") 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 vEDS 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.[13]

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.[13] 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 anywhere in the world for vEDS. However, celiprolol, off label, has become the standard of care therapy for vEDS in Europe.[14] 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.

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 BBEST trial. Results from the BBEST trial were published on October 30, 2010 in The Lancet. The BBEST trial was funded by the French Ministry of Health, and the principal investigator for the study was Professor Pierre Boutouyrie.[15]

Fifty-three participants were enrolled in the BBEST 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.[15] 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=0.0097):

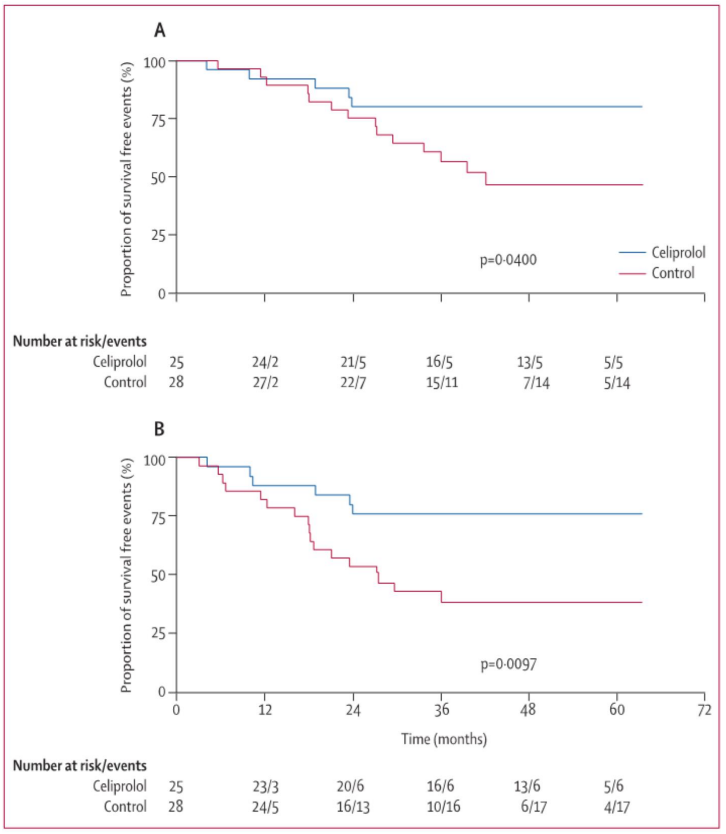


***Figure 2*: Kaplan-Meier curves of event-free survival in 53 patients with vascular Ehlers-Danlos**
Primary endpoint (A). Primary and secondary endpoints (B).

As described in the figure 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=0.0406). 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=0.0167), 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:

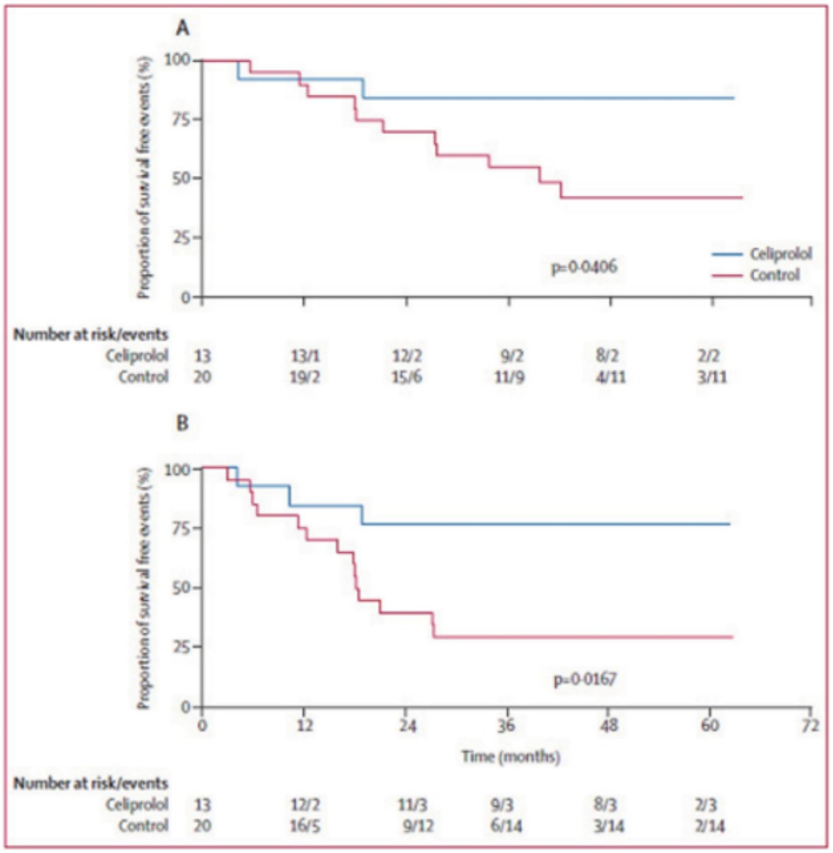


Figure 3: **Kaplan-Meier curves of event-free survival in 33 patients with positive COL3A1 mutation**

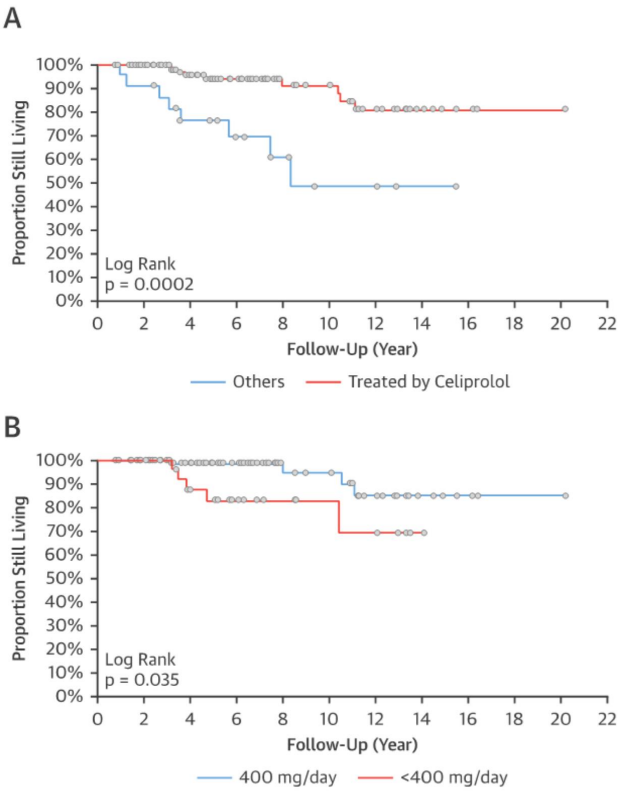
Primary endpoint (A). Primary and secondary endpoints (B).

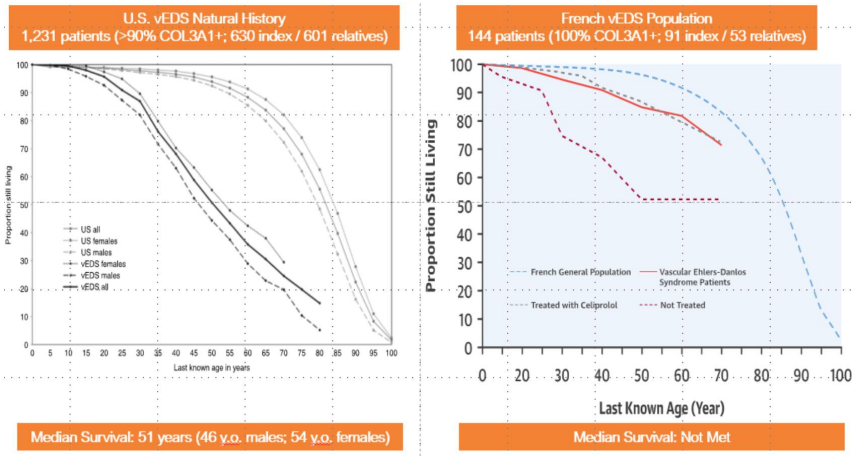
AP-HP granted us an exclusive right to access and use the data generated by the BBEST trial. We have conducted a retrospective, source-verified analysis of that data, including the primary and secondary endpoints, which confirmed the published results of the BBEST trial.

In addition to the BBEST 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.16 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[17]. 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.[17] 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.[18]

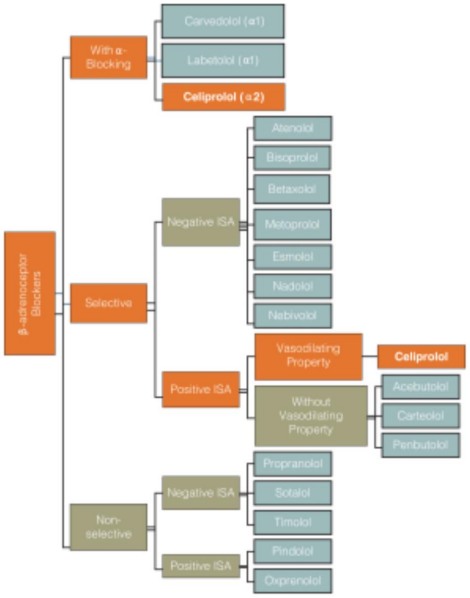
Researchers conducted a retrospective analysis of U.S. insurance claims (Truven MarketScan®) identifying vEDS patients over a four-year period from January 1, 2014 to December 31, 2017. The insurance claims-based information was then stratified based on insurance claims for antihypertensive medications 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.

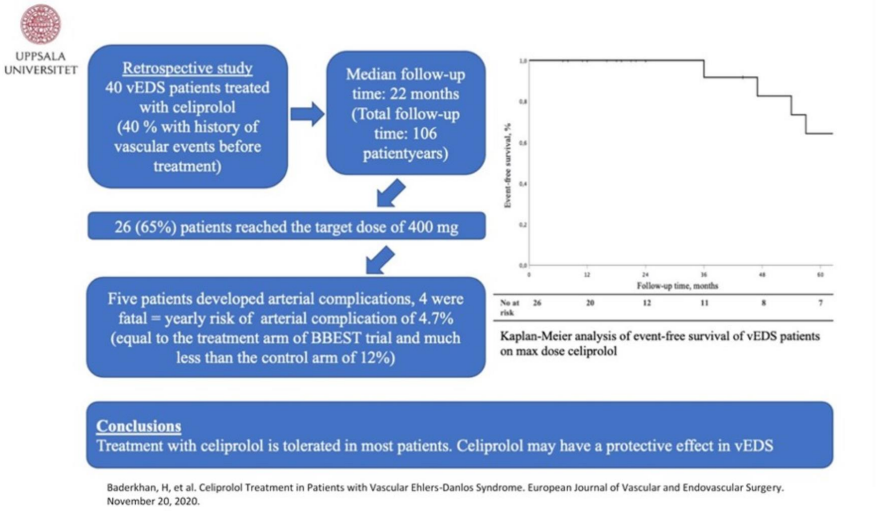
The potential benefit of celiprolol in vEDS is thought to be mediated through a combination of agonist activity at beta2 and beta3 adrenergic receptors, and antagonist activity at alpha2 adrenergic receptors. While the exact mechanism is not fully understood, it has been proposed that it could exert 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:



In November 2020, long-term data from COL3A1-positive vEDS patients was published in the *European Journal of Vascular and Endovascular Surgery ("EJVES")*, "Celiprolol treatment in patients with vascular Ehlers-Danlos Syndrome."19

This published study describes outcomes in 40 patients with COL3A1-positive vEDS that were clinically monitored and treated with celiprolol in a single center retrospective study at Uppsala University Hospital, a national referral center for vEDS patients in Sweden, between the years 2011 and 2019. Patients were followed for a median of 22 months (range 1-98 months) with a total follow up of 106 patient years. Assessments were conducted by a multidisciplinary team, including vascular surgeons, angiologists and clinical geneticists. Celiprolol was administered twice daily and titrated up by 100 mg steps to a maximum of 400 mg per day. Some patients were treated concomitantly or separately with other medications. Sixty-five percent of the patients reached the target dose of 400 mg and the medication was generally well tolerated.

The annual risk of a major vascular event was 4.7% in this study, noted as being similar to that observed in the celiprolol treatment-arm of the BBEST trial (5%) and lower than in the BBEST trial control arm (12%). Five patients suffered major vascular events, four of which were fatal. No significant predictor of vascular events was identified by the authors.



Baderkhan, H, et al. Celiprolol Treatment in Patients with Vascular Ehlers-Danlos Syndrome. European Journal of Vascular and Endovascular Surgery. November 20, 2020.

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 the exclusive right in North and South America from Aventis 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 BBEST trial. EDSIVO™ received FDA Orphan Drug Designation for the treatment of vEDS in 2015.

We submitted our NDA for EDSIVO™ in October 2018 and it was accepted for filing and substantive review by the FDA in December 2018, with priority review status. In June 2019, we received a Complete Response Letter ("CRL") from the FDA regarding our NDA for EDSIVO™ for the treatment of vEDS using the data obtained from the BBEST trial15 as the basis of approval. The CRL 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 CRL. 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 CRL. 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. The Office of New Drugs referred to the FDA Guidance document issued in December 201920, 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.

We believe we have identified a plan to collect additional data that supports the results from the COL3A1-positive analysis from the BBEST trial and could help meet the standard set forth in the FDA Guidance document issued in December 2019. In February 2021, we submitted a meeting request to the FDA to discuss Acer's proposed plan to provide sufficient confirmatory evidence. If successful, we believe that data provided under our proposal could potentially satisfy the additional confirmatory evidence needed to support a resubmission of our NDA, assuming the additional data analysis is positive. There can be no assurance that FDA will accept our plan or, if accepted, that the resulting data would be adequate to support resubmission, filing or approval of our NDA. We may also conclude at any point that the cost, risk and uncertainty of obtaining that additional data does not justify continuing with the development of EDSIVO™.

EDSIVO™ is an investigational drug in the U.S. and is not currently FDA approved for any indication.

ACER-801 (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.

Hot flashes, flushing, and night sweats are known as Vasomotor symptoms ("VMS"), and most often occur in women who are entering or in 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.

Vasomotor symptoms 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")[21,22] may exhibit severe iVMS.

In women with Hormone Receptor positive ("HR+") Breast Cancer ("CaB") receiving tamoxifen: [23]

- 84% of women experienced hot flashes
- 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

In men with HR+ Prostate Cancer ("CaP") receiving leuprolide: [24]

- 80% of men experience hot flashes

- 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 flashes25
- Up to 35% complain of "extremely bothersome" symptoms up to two years after their surgery26

iVMS are well documented with the use of cancer therapies and certain surgical procedures. Symptoms such as hot flashes can appear immediately and be severe. Non-adherence to therapy can be associated with side effects that can increase the mortality risk or shorten the time to recurrence.

iVMS Diagnosis and Incidence

Population Attribute	Women who are BRCA+ and have prophylactic bilateral oophorectomy (PBSO)	Men with HR+ Prostate Cancer (CaP) receiving Leuprolide	Women with HR+ Breast Cancer (CaB) receiving Tamoxifen
Estimated # of Patients[1] *U.S.* *WW (US/EU5/JAP)*	 47,387 110,940	 74,970 177,429	 120,932 286,207
Therapy Indication	Surgery recommended between ages of 35-40 or after childbearing completion[4]	ADT to decrease testosterone levels in high-risk localized and advanced prostate cancer[3] Typical therapy use is 1-6 months in combination with other treatments[3]	Hormone drug used to treat and prevent hormone receptor-positive breast cancers[2] Typical therapy use is 5-10 years[2]
Clinical Benefits	• 85%-95% reduction in incidence of ovarian cancer[12] • 53-68% reduction in breast cancer[12]	Decreased serum testosterone to ≤50 ng/dL from week 4 through week 48 in an estimated 94% of patients[6]	50% reduction in both invasive and non-invasive breast cancers[5]
iVMS Side Effects	67% experience[9]	80% experience[8] 30-40% moderate/severe	84% experience[7] 60% severe
HRT Use	Controversial; BRCA2 tend to be estrogen receptor positive	Contraindicated	Contraindicated
Compliance & Adherence	• Nearly 60% of BRCA+ women will elect a prophylactic oophorectomy [13]. • Inducement of menopause is one of the reasons to delay or not have surgery	Concern over hot flashes make patients less likely to begin ADT and can lead to early discontinuation[11]	• Many chose to never go on therapy due to side effects • Almost half discontinued by 4.5 years[10]

1 Trinity Partners 2020; 2 Dowsett, M, et al., Lancet.386(10001):1341-52, 2015; 3 Lee RJ, et al., Cancer and Chemotherapy and Biotherapy: Principles and Practice 5th ed. 2011; 4 ACOG Recommendations FAQs0s BRCA1 and BRCA2, August 2019; 5 [illegible] J, et al. Cancer Prev Res 2011;4: 1360-1365; 6 Lupron Depot PI March, 2019; 7 Moon, Z. et al., JOURNAL OF PSYCHOSOMATIC OBSTETRICS & GYNECOLOGY 2017 VOL. 38, NO. 3, 226–235; 8 Chalapalli, A. et al., Clinical and Translational Radiation Oncology 10 (2018) 29–35 9 [illegible] Johnson et al. American Society for Reproductive Medicine, 2014 Vol 102 No. 3 Supplement, e249; 10 Nichols, [illegible] et al. JNCI J Natl Cancer Inst, 2015, 1–8 ; 11 Gonzalez BD et al., Urol 194: 690-695 2015; 12 N Engl J Med. 2002 May 23;346(21):1609-15; 13 Open Med. 2007 Aug 14;1(2):e92-8

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 16 Phase 1 studies in 387 healthy subjects and 822

patients with the following disorders: depression, panic disorder, schizophrenia, asthma, or Parkinson's disease. Clinical and laboratory safety data has been collected from 23 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.27 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

Osanetant, if approved for marketing by the FDA, 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 submitting an IND for osanetant with the FDA in the third quarter of 2021. We plan to explore treatment with osanetant in a Phase 2 clinical trial in BRCA-positive patients who have undergone a prophylactic bilateral salpingo-oophorectomy ("PBSO") that would evaluate the efficacy and safety of the drug at various doses. Initiation of this trial, planned for the fourth quarter of 2021, is subject to successful IND submission and FDA clearance, and our ability to raise sufficient capital.

Osanetant is an investigational drug in the U.S. and is not currently FDA approved for any indication.

ACER-2820 (emetine)

Background

In May 2020, we announced that we had entered into a research collaboration agreement with the National Center for Advancing Translational Sciences, or NCATS, one of the National Institutes of Health, or NIH, to develop emetine hydrochloride as a potential treatment of SARS-CoV-2 infection, a virus that causes COVID-19. Emetine is an active pharmaceutical ingredient of syrup of ipecac, given orally to induce emesis, and has also been formulated as an injectable to treat thousands of individuals with amebiasis. Several independent emetine in vitro studies have demonstrated nanomolar potency against both DNA and RNA-replicating viruses, including Zika virus, Ebola virus28, Rabies Lyssavirus, human cytomegalovirus, human immunodeficiency virus 1, influenza A virus, Rift Valley fever virus, echovirus 1, human metapneumovirus, and herpes simplex virus type 229. Clinically, emetine has been used to treat approximately 700 patients (including pediatrics) with viral hepatitis30 and varicella-zoster virus31. Additionally, emetine is a potent inhibitor of multiple genetically-distinct coronaviruses and demonstrated in vitro the strongest anti-coronavirus activity in one study that screened and identified approved compounds with broad-spectrum efficacy against the replication of four coronaviruses32 and specifically against SARS-CoV-2. 33

Registration Plan

Further advancement of the emetine program in COVID-19 and other infectious diseases is dependent on our ability to raise non-dilutive capital. Initiation of a proposed Phase 2/3 COVID-19 clinical trial is also subject to successful IND submission and FDA clearance. Emetine is an investigational drug in the U.S. and is not currently FDA approved for any indication.

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Commercialization Strategy

Assuming the FDA approves ACER-001 and/or EDSIVOTM, we expect that the majority of UCDs, MSUD and/or vEDS patients would be treated at tertiary care centers, and therefore could be addressed with a targeted sales force. UCD and MSUD patients are primarily managed by metabolic geneticists and dietitians, while vEDS patients would primarily be treated by vascular medicine or cardiology specialists. 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 and emetine.

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 UCDs, MSUD, vEDS, iVMS, and COVID-19, 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 in clinical development with a treatment for vEDS, although we are aware of studies that are currently enrolling vEDS patients at AP-HP, including one that adds 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:

- UCDs: Horizon Pharma plc / Immedica Group (Marketed); Aeglea BioTherapeutics Inc. (Phase 3); Ultragenyx (Phase 1/2); 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 compared with us. Accordingly, our competitors may be more successful 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 UCDs 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

In April 2014, we obtained exclusive rights to intellectual property relating to ACER-001 and preclinical and clinical data, through a license agreement with BCM related to MSUD. 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.

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 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 a multicenter, prospective, randomized, open trial related to the use of celiprolol for the treatment of vEDS. We utilized this clinical data to support an NDA filing for EDSIVO™ for the treatment of vEDS. The agreement requires us to make certain upfront payments to AP-HP, reimburse certain of AP-HP's costs, make payments upon achievement of defined milestones, and pay 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 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). We are no longer pursuing the type VI patent application. 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. Of those, two applications remain in prosecution and the third, relating to type VI EDS, has been returned to AP-HP. We may choose to limit our pursuit of patent applications to specific territories, in which case AP-HP would have the right to revise our territorial license rights accordingly.

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. The agreement required us 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. We plan to initially pursue development of osanetant as a potential treatment for iVMS.

Research Collaboration

On May 11, 2020, we announced that we entered into a research collaboration agreement with the National Center for Advancing Translational Sciences ("NCATS"), one of the National Institutes of Health (NIH), to develop emetine hydrochloride as a potential treatment of SARS-COV-2 infection. Under the terms of the agreement, We and NCATS are collaborating to accelerate the clinical development of emetine.

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

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 patents cover methods and compositions for treating humans (and animals) with various formulations and prodrugs of NaPB for inborn errors of BCAA metabolism, including MSUD, and the latest expires in 2032. 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 three patents in the U.S. and one in 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 are seeking 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.

EDSIVO™

We intend to protect our commercial rights to EDSIVOTM in the U.S. via multiple pathways. We believe that we will be eligible for NCE exclusivity for EDSIVOTM, 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 EDSIVOTM 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 seek 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 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). We are no longer pursuing the type VI patent application.

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.

Emetine

We intend to explore various pathways to protect our commercial rights to emetine in multiple infectious diseases, including but not limited to COVID-19, 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 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
- meet Pediatric Research Equity Act ("PREA") requirements, if applicable
- 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
- 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 subjects or 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 undergo an informed consent process and provide their informed consent for 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 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 stored 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 product candidates. 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. 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
- 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.

Human Capital/Employees

Our key human capital management objectives are to attract, retain and develop the highest quality talent. To support these objectives, our human resources programs are designed to develop talent to prepare them for critical roles and leadership positions for the future; reward and support employees through competitive pay and benefits; enhance our culture through efforts aimed at making the workplace more engaging and inclusive; acquire talent and facilitate internal talent mobility to create a high-performing and diverse workforce. As of February 15, 2021, we had 20 full-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.

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, or that may be accessed by links 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 Inc., One Gateway Center, Suite 351, 300 Washington Street, 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

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

As of December 31, 2020, we had an accumulated deficit of $99.1 million, cash and cash equivalents of $5.8 million, and current liabilities of $6.1 million. Based on available resources, we believe that our cash and cash equivalents currently on hand, combined with the $8.2 million in proceeds raised subsequent to December 31, 2020 from the sale of common stock and entering into the Relief transaction, are sufficient to fund our currently anticipated operating and capital requirements into the third quarter of 2021. (For a description of funds raised subsequent to December 31, 2020, see Note 11 to our financial statements and the Liquidity and Capital Resources section of Management's Discussion and Analysis of Financial Condition and Results of Operations included in this report.) Thus, 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 revenue and we expect to continue to incur losses for the foreseeable future as we continue our development of, and seek marketing approvals for, our product candidates. These 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.

We will require additional financing to complete development and seek 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, 2020, we had an accumulated deficit of $99.1 million, cash and cash equivalents of $5.8 million and current liabilities of $6.1 million. As discussed above, we believe that our cash and cash equivalents currently on hand, combined with the funds raised subsequent to December 31, 2020, are sufficient to fund our anticipated operating and capital requirements into the third quarter of 2021. Thus, 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 substantial additional funds sooner than planned, through public or private equity or debt financings or other sources, such as non-dilutive funding or 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:

- whether or not we are able to successfully negotiate and enter into a definitive agreement with Relief for the potential collaboration and license of ACER-001, which will impact our requirement to repay in cash the $4.0 million 12-month secured loan from Relief
- the scope, progress, results, and costs of researching and developing our current product candidates, and future product candidates, if any, including conducting preclinical and clinical trials
- the cost of seeking regulatory and marketing approvals and reimbursement for our product candidates and future product candidates, if any
- the cost of commercialization activities if our current product candidates and future product candidates, if any, 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, if any, 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 product candidates 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
- the timing, receipt and amount of sales of, or royalties on, future approved product candidates, 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
- delay, limit, reduce or terminate our establishment of sales and marketing capabilities or other activities that may be necessary to commercialize future approved product candidates, if any

We may not be able to successfully negotiate and enter into a definitive agreement with Relief for the potential collaboration and license of ACER-001 on the terms outlined in the option agreement, on other mutually acceptable terms, or at all. If we do not enter into a definitive agreement with Relief, we may not be able to repay the $4.0 million 12-month loan we received from Relief, which is secured by all of our assets.

On January 25, 2021, we and Relief entered into an option agreement pursuant to which we granted Relief an exclusive option to pursue a potential collaboration and license agreement with us for the development, regulatory approval and commercialization of ACER-001 for the treatment of UCDs and MSUD. The option agreement provides a period of time up to June 30, 2021 for the parties to perform additional due diligence and to work toward negotiation and execution of a definitive agreement with respect to the potential collaboration for ACER-001. In consideration for the grant of the exclusivity option, (i) we received from Relief an upfront nonrefundable payment of $1.0 million, (ii) Relief provided to us a 12-month secured loan in the principal amount of $4.0 million, as evidenced by a promissory note we issued to Relief, and (iii) we granted to Relief a security interest in all of our assets to secure performance of the promissory note, as evidenced by a security agreement. The note is repayable in one lump sum within 12 months from issuance and bears interest at a rate equal to 6% per annum. At Relief's option, the outstanding balance of the $4.0 million loan can be used to offset the $14.0 million payment that may otherwise be payable to us from Relief if a definitive agreement is executed. If a definitive agreement with respect to the potential collaboration is not executed by the parties on or before June 30, 2021, the exclusivity option will terminate and the note is repayable by us upon maturity. The note contains certain customary events of default (including, but not limited to, default in payment of principal or interest thereunder or a material breach of the security agreement).

There can be no assurance, however, that a definitive agreement will be successfully negotiated and executed between the parties on the terms outlined in the option agreement, on other mutually acceptable terms, or at all. Except for the $1.0 million upfront payment to us and the $4.0 million 12-month secured loan from Relief to us, the remaining proposed terms of the potential collaboration and license arrangement described in the option agreement are not binding and are subject to change as a result of further diligence by Relief and negotiation of a definitive collaboration and license agreement between the parties. If we are unable to successfully negotiate and enter into a definitive agreement, we may not be able to repay the $4.0 million 12-month loan we received from Relief, which is secured by all of our assets. If we do not have sufficient cash flow to repay the loan or if we fail to comply with the terms of the promissory note or security agreement, we might be subject to default. In that situation, Relief will have a first claim on all of our assets which have been pledged as collateral under the security agreement. If Relief were to attempt to foreclose on the collateral, there may be very little, if any, assets remaining after repayment in full of such secured indebtedness. Even if we are able to repay the full amount in cash, any such repayment could leave us with little or no working capital for our business. Any default under the loan arrangement with Relief and resulting foreclosure would have a material adverse effect on our financial condition and our ability to continue our operations.

If we are unable to successfully negotiate and enter into a definitive agreement with Relief, we may be unable to enter into a collaboration for ACER-001 with any other potential partner on acceptable terms, if at all. We face competition in our search for partners from other organizations worldwide, many of whom are larger and are able to offer more attractive deals in terms of financial commitments, contribution of human resources, or development, manufacturing, regulatory or commercial expertise and support. If we unable to enter into a definitive agreement with Relief, and we are not successful in attracting another partner and entering into collaboration on acceptable terms, we may not be able to complete development of or commercialize any product candidate, including ACER-001. In such event, our ability to generate revenues and achieve or sustain profitability would be significantly hindered and we may not be able to continue operations as proposed, requiring us to modify our business plan, curtail various aspects of our operations or cease operations.

Funding from our purchase agreement with Lincoln Park may be limited or be insufficient to fund our operations or implement our strategy.

Under our purchase agreement with Lincoln Park, we may direct Lincoln Park to purchase up to $15.0 million of shares of our common stock, subject to certain limitations and conditions, over a 36-month period commencing on June 8, 2020. There can be no assurance that we will be able to receive all of the remaining

committed funds from Lincoln Park because the purchase agreement contains limitations, restrictions, requirements, events of default and other provisions that could limit our ability to cause Lincoln Park to buy common stock from us, including that Lincoln Park own no more than 9.99% of our common stock. In addition, under the applicable rules of the Nasdaq Capital Market, if we seek to issue shares in excess of 19.99% of the total common stock outstanding as of the date we entered into the purchase agreement, we may be required to seek stockholder approval in order to be in compliance with the Nasdaq Capital Market rules. Our inability to access a portion or the full amount remaining available under the purchase agreement, in the absence of any other financing sources, could have a material adverse effect on our business. As of December 31, 2020, we had sold 900,000 shares of common stock under the purchase agreement for net proceeds of $2.2 million. Subsequent to December 31, 2020 and through the date of this report, we have sold an additional 200,000 shares under the purchase agreement for net proceeds of $0.5 million.

The extent to which we rely on Lincoln Park as a source of funding will depend on a number of factors, including the amount of working capital needed, the prevailing market price of our common stock and the extent to which we are able to secure working capital from other sources. We will also need to file one or more additional registration statements to register shares for resale under the terms of the purchase agreement and keep current an offering prospectus. If obtaining sufficient funding from Lincoln Park were to prove unavailable or prohibitively dilutive, we would need to secure another source of funding in order to satisfy our working capital needs. Even if we were to receive all remaining proceeds under the purchase agreement with Lincoln Park, we would still need additional capital to fully implement our business, operating and development plans. Should the financing we require to sustain our working capital needs be unavailable or prohibitively expensive when we require it, the consequences could be a material adverse effect on our business, operating results, financial condition and prospects.

Funding from our ATM facility with JonesTrading Institutional Services LLC ("JonesTrading") and Roth Capital Partners, LLC ("Roth Capital") may be limited or 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 ATM facility with JonesTrading and 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 which aggregate to no more than one-third of our public float using our shelf-registration statement at this time. From May 19, 2020 through December 31, 2020, we sold an aggregate of 1,838,957 shares of common stock under our ATM facility for net proceeds of $6.9 million. Subsequent to December 31, 2020 and through the date of this report, we have sold an aggregate of 877,107 additional shares of common stock under our ATM facility for net proceeds of $2.7 million. These sales of common stock are counted toward the maximum of one-third of our public float that can be sold in a 12-month period and reduce the remaining shares available to sell under our ATM facility during that 12-month period. 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, 2020 and 2019 was $22.9 million and $29.4 million, respectively. As of December 31, 2020, we had an accumulated deficit of $99.1 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. 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 public or private equity or debt financings, strategic collaborations, or non-dilutive funding. 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
- 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 product candidates, if any
- establish, maintain and protect our intellectual property rights
- 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.

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

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. Neither resubmission nor approval of our NDA for EDSIVOTM is assured. We may decide at any time not to continue development 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 certain 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 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 (i) described possible paths forward for us 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 and (ii) 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. We believe we have identified a plan to collect additional data that supports the results from the COL3A1-positive analysis from the BBEST trial and could help meet the standard set forth in the FDA Guidance document issued in December 2019. In February 2021, we submitted a meeting request to the FDA to discuss Acer's proposed plan to provide sufficient confirmatory evidence. If successful, data provided under our proposal could potentially satisfy the additional confirmatory evidence needed to support a resubmission of our NDA, assuming the additional data analysis is positive. There can be no assurance that FDA will accept our plan or, if accepted, that the resulting data would be adequate to support resubmission, filing or approval of our NDA. We may also conclude at any point that the cost, risk and uncertainty of obtaining that additional data does not justify continuing with the development of EDSIVO™.

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. We are not yet subject to the provisions of section 404(b) of SOX, which would require our independent registered public accounting firm's attestation on our assessment of internal controls over financial reporting. 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 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 including but not limited to the COVID-19 pandemic 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 pandemic of COVID-19, a respiratory illness caused by a novel coronavirus. While our employees work remotely a large part of the time, 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. Health professionals may reduce staffing and reduce or postpone meetings with clients, colleagues, and others in response to the spread of an infectious disease. Such events may result in a period of business disruption, and in reduced operations, any of which could materially affect our business, financial condition, and results of 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 or volatility that could adversely affect our manufacturers and suppliers and otherwise adversely impact our development activities and other operations.

The extent to which the COVID-19 pandemic will continue to affect our business, results of operations, and financial condition is difficult to predict. The outbreak could potentially affect the business of the FDA, European Medicines Agency ("EMA") or other health authorities, which could result in delays in meetings related to our product candidates and our planned clinical trials and ultimately in the review and approval of our product candidates. The spread of COVID-19 may also slow potential enrollment of clinical trials and reduce the number of eligible patients for our clinical trials, thereby making recruitment more difficult and competitive. Prolonged disruptions to businesses, manufacturing and supply chain, including shelter-in-place or similar orders imposed by federal, state or local government authorities, and economic downturns can lead to materially adverse effects on our business operations, including layoffs and/or suspension of our business operations. The COVID-19 outbreak and mitigation measures also have had and may continue to have an adverse impact on global economic conditions which could have an adverse effect on our business and financial condition, including impairing our ability to raise capital when and in the amount needed. The extent to which COVID-19 impacts our business will depend on future developments, which are highly uncertain and cannot be predicted, including new information which may emerge concerning the severity of COVID-19 and the actions to contain COVID-19 or treat its impact, among others. In addition, any COVID-19 infection of any of our employees could have a significant impact on our ability to conduct business.

We face substantial competitive and other risks in our emetine program, aimed against a variety of infectious diseases including COVID-19, and we may be unable to raise non-dilutive capital to continue the program.

We have recently announced a new development program for emetine, a host-directed therapy against a variety of infectious diseases, including COVID-19. There are many companies addressing COVID-19, both in therapeutic treatment and vaccines, many of which have significantly greater resources and capital than we do. Recent positive events in the development of one or more vaccines may reduce the demand for therapeutic products addressing COVID-19. The competition for funding research and development in this disease is intense and most of our competitors have greater resources available to them. Regulatory requirements in this area are in flux and will likely remain uncertain. Further advancement of the emetine program in COVID-19 and other infectious diseases is dependent on our ability to raise non-dilutive capital. There can be no assurance that we will be able to obtain adequate financing to carry out our development plan or that, even if funding is obtained, our development of emetine will be successful, timely, and accepted by appropriate regulatory authorities.

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

We expect to evaluate potential strategic acquisitions of complementary businesses, products or technologies 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 any or all of our product candidates, and we may be required to conduct clinical trials or provide other forms of substantial evidence of effectiveness instead of, or in addition to, relying on third-party data, as is the position of the FDA with respect to our NDA for EDSIVO™
- 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
- 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 seek approval under Section 505(b)(2), we may be unable to meet our anticipated development and commercialization timelines, and could decide not to pursue further development, depending on the expected time, cost, and risks associated with generating any such additional data.

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 ACER-001 and EDSIVO™) 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. We may be required 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 seek or obtain approval. In December 2019, we submitted a Formal Dispute Resolution Request to the 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 (i) described possible paths forward for us 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 and (ii) 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. We believe we have identified a plan to collect additional data that supports the results from the COL3A1-positive analysis from the BBEST trial and could help meet the standard set forth in the FDA Guidance document issued in December 2019. In February 2021, we submitted a meeting request to the FDA to discuss Acer's proposed plan to provide sufficient confirmatory evidence. If successful, data provided under our proposal could potentially satisfy the additional confirmatory evidence needed to support a resubmission of our NDA, assuming the additional data analysis is positive. There can be no assurance that FDA will accept our plan or, if accepted, that the resulting data would be adequate to support resubmission, filing or approval of our NDA. We may also conclude at any point that the cost, risk and uncertainty of obtaining that additional data does not justify continuing with the development of EDSIVO™.

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 studies to assess the safety and efficacy of our product candidates. We could decide not to pursue further development of one or all of our product candidates, depending on, among other things, the expected time, cost, and risks associated with generating any such additional data.

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 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 (i) described possible paths forward for us 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 and (ii) 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. We believe we have identified a plan to collect additional data that supports the results from the COL3A1-positive analysis from the BBEST trial and could help meet the standard set forth in the FDA Guidance document issued in December 2019. In February 2021, we submitted a meeting request to the FDA to discuss Acer's proposed plan to provide sufficient confirmatory evidence. If successful, data provided under our proposal could potentially satisfy the additional confirmatory evidence needed to support a resubmission of our NDA, assuming the additional data analysis is positive. There can be no assurance that FDA will accept our plan or, if accepted, that the resulting data would be adequate to support resubmission, filing or approval of our NDA. We may also conclude at any point that the cost, risk and uncertainty of obtaining that additional data does not justify continuing with the development of EDSIVO™.

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
- 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 development of product candidates for rare diseases carry additional risks, such as recruiting patients in a very small patient population.

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. Clinical trials are usually conducted at multiple sites, potentially 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
- 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. In addition, the ongoing COVID-19 pandemic may materially adversely affect our ability to recruit qualified subjects for our clinical trials, not only for emetine but for all of our product candidates. It is impossible to predict that impact on our clinical trials and our business.

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. 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 limited experience in designing and completing clinical trials and may be unable 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. The conduct of clinical trials and the submission of a successful NDA is a complicated process. As an organization, we have limited experience in designing and completing clinical trials, 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 product candidates. 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
- 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
- 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 ACER-001, EDSIVO™, osanetant, or emetine, 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 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
- 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. It is also possible that the executive branch may take certain steps by executive action which could modify or solidify aspects of the Affordable Care Act. Certain stakeholders are also pursuing litigation challenging certain provisions which, if successful, would have the effect of modifying some or all of the provisions of the Affordable Care Act. We cannot predict the reform initiatives that may be adopted or litigation outcomes 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
- the level of taxes that we are required to pay

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

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

Other Risks Related to Our Business

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 2019 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 of February 15, 2021, we had a workforce of 20 full-time 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, which could affect the timing or continued development of one or more of our product candidates, and emetine in particular.

From time to time, we may apply for contracts or grants from government agencies, non-profit entities and academic institutions. For example, we are pursuing several financing options, including federally-funded research contracts and grants and other potentially non-dilutive funding sources, to fund our planned emetine development program for the potential treatment of patients with COVID-19. 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 unfavorable 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. In addition, there are known cyberattacks against pharmaceutical companies engaged in development of therapeutic or vaccine products addressing COVID-19. Our emetine program is one such program that could attract the attention of cyberattackers.

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. We are exploring with the FDA other possible approaches that could provide the necessary confirmatory evidence of efficacy needed in order to seek approval. There can be no assurance that our plan will be accepted by the FDA or that we will be able to provide adequate data to meet that standard. This may also result in greater research and development expenses or regulatory issues that could further delay or prevent approval.

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 (i) 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 and (ii) 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. We believe we have identified a plan to collect additional data that supports the results from the COL3A1-positive analysis from the BBEST trial and could help meet the standard set forth in the FDA Guidance document issued in December 2019. In February 2021, we submitted a meeting request to the FDA to discuss Acer's proposed plan to provide sufficient confirmatory evidence. If successful, data provided under our proposal could potentially

satisfy the additional confirmatory evidence needed to support a resubmission of our NDA, assuming the additional data analysis is positive. There can be no assurance that FDA will accept our plan or, if accepted, that the resulting data would be adequate to support resubmission, filing or approval of our NDA. We may also conclude at any point that the cost, risk and uncertainty of obtaining that additional data does not justify continuing with the development of EDSIVO™. The novelty of this product candidate may continue to lengthen the regulatory review process, ultimately require the conduct of one or more additional studies or clinical trials as a prerequisite to approval (although we do not presently intend to conduct any such studies or trials), increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization, or lead to significant post-approval limitations or restrictions. There is also an increased risk that previously unknown or unanticipated adverse effects could be discovered during any clinical trials and beyond. Any such events could have a materially adverse impact on 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, among other factors, 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
- 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 to treat rare diseases are smaller than we believe they are, then our revenues may be adversely affected and our business may suffer.

The diseases that some of 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 some of 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 ACER-001 or EDSIVO™ 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 these 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 product candidates, 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.

For example, on January 25, 2021, we and Relief entered into an option agreement pursuant to which we granted Relief an exclusive option to pursue a potential collaboration and license agreement with us for the development, regulatory approval and commercialization of ACER-001 for the treatment of UCDs and MSUD. The option agreement provides a period of time up to June 30, 2021 for the parties to perform additional due diligence and to work toward negotiation and execution of a definitive agreement with respect to the potential collaboration for ACER-001. In consideration for the grant of the exclusivity option, (i) we received from Relief an upfront nonrefundable payment of $1.0 million, (ii) Relief provided to us a 12-month secured loan in the principal amount of $4.0 million, as evidenced by a promissory note we issued to Relief, and (iii) we granted to Relief a security interest in all of our assets to secure performance of the promissory note, as evidenced by a security agreement. The note is repayable in one lump sum within 12 months from issuance and bears interest at a rate equal to 6% per annum. At Relief's option, the outstanding balance of the $4.0 million loan can be used to offset the $14.0 million payment that may otherwise be payable to us from Relief if a definitive agreement is executed. If a definitive agreement with respect to the potential collaboration is not executed by the parties on or before June 30, 2021, the exclusivity option will terminate and the note is repayable by us upon maturity. The note contains certain customary events of default (including, but not limited to, default in payment of principal or interest thereunder or a material breach of the security agreement). There can be no assurance, however, that a definitive agreement will be successfully negotiated and executed between the parties on the terms outlined in the option agreement, on other mutually acceptable terms, or at all. Except for the $1.0 million upfront payment to us and the $4.0 million 12-month secured loan from Relief to us, the remaining proposed terms of the potential collaboration and license arrangement described in the option agreement are not binding and are subject to change as a result of further diligence by Relief and negotiation of a definitive collaboration and license agreement between the parties. If we are unable to successfully negotiate and enter into a definitive agreement with Relief, we may be unable to enter into a collaboration with any other potential partner on acceptable terms, if at all.

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
- business combinations or significant changes in a collaborator's business strategy may adversely affect a collaborator's willingness to complete its 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
- 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 (including indirect techniques of pricing pressure, such as allowing reimportation from markets outside the U.S.) 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 for our targeted indications 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, generally 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 to treat rare diseases is highly desirable 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
- 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 in the rare disease category will depend in part on our ability to obtain Orphan Drug designation for our product candidates to treat rare diseases 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
- 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 ACER-001 for MSUD and EDSIVO™ for vEDS. We expect to seek Orphan Drug exclusivity from the EMA for ACER-001 for MSUD; however, there can be no assurance that we will be successful. If we are unable to maintain our current Orphan Drug exclusivity or are unable to secure orphan status in Europe for ACER-001 for MSUD, it may have a material negative effect on our business.

Generally, if a product with an Orphan Drug designation subsequently receives the first marketing approval for the indication for which it has such designation, that product is entitled to a period of marketing exclusivity, which precludes the applicable regulatory authority from approving another marketing application for the same drug for the same indication for that time period. The applicable period is seven years in the 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 ACER-001 and EDSIVO™ 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 ACER-001 or EDSIVO™ 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 ACER-001 for MSUD and EDSIVO™ for vEDS.

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. There is also the potential for a reference pricing system using drug prices from other countries, sometimes referred to as "most favored nation" treatment. 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 any of 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 and executive actions. There have been, and likely will continue to be, legislative and executive 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 manufacture of our product candidates and our dependence on these third parties may impair or delay 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, due to the ongoing impact of the COVID-19 pandemic or other reasons, 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 for a product candidate. 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. 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.

In addition, the above risks are compounded by uncertainties related to the ongoing COVID-19 pandemic, which could affect our CROs' businesses internally (for example, maintaining staffing levels and ongoing financial viability), as well as their ability to perform their obligations to us under our agreements (such as recruitment of subjects for clinical trials in an increasingly uncertain and competitive business environment).

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 typically have control over the filing and prosecuting of patent applications and maintaining patents which cover making, using or selling ACER-001, EDSIVO™, osanetant, or emetine, 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
- 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 or similar agreements under which we license intellectual property, data, and/or receive commercialization rights relating to ACER-001, EDSIVO™, osanetant, and emetine. 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
- 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

Our share price is very volatile, may not reflect the underlying value of our net assets or business prospects, 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 and our ongoing dialogue with the FDA
- the initiation, termination or reduction in the scope of any collaboration arrangements or any disputes or developments regarding such collaborations
- market conditions
- the impact of short selling or the impact of a potential "short squeeze" resulting from a sudden increase in demand for our stock
- 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
- 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
- our ability to sell shares of common stock to Lincoln Park pursuant to the terms of the purchase agreement and our ability to register and maintain the registration of the shares issued and issuable thereunder

Broad market and industry factors, as well as economic and political factors, also may materially adversely affect the market price of our common stock. As noted, the short, medium, and long term impacts of the COVID-19 pandemic on the U.S. and global economies generally, and on our business specifically, are difficult to predict.

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 several stockholders' derivative suits 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 13,233,137 shares of common stock were outstanding as of December 31, 2020. As of such date, another 1,240,354 shares of common stock were issuable upon exercise of outstanding options. 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.

On April 30, 2020, we entered into the purchase agreement with Lincoln Park, pursuant to which Lincoln Park has committed to purchase up to $15.0 million of our common stock. Upon the execution of the purchase agreement, we issued 148,148 commitment shares to Lincoln Park as consideration for its commitment to purchase shares of our common stock under the purchase agreement. The remaining shares of our common stock that may be issued under the purchase agreement may be sold by us to Lincoln Park at our discretion from time to time over a 36-month period commencing on June 8, 2020. The purchase price for the shares that we may sell to Lincoln Park under the purchase agreement will fluctuate based on the price of our common stock. Depending on market liquidity at the time, sales of such shares may cause the trading price of our common stock to fall. As of December 31, 2020, we had sold 900,000 shares of common stock under the purchase agreement for net proceeds of $2.2 million. Subsequent to December 31, 2020 and through the date of this report, we have sold an additional 200,000 shares under the purchase agreement for net proceeds of $0.5 million.

We generally have the right to control the timing and amount of any sales of our shares to Lincoln Park under the purchase agreement. Sales of our common stock to Lincoln Park under the purchase agreement will depend upon market conditions and other factors to be determined by us. We may ultimately decide to sell to Lincoln Park all or only some of the shares of our common stock that may be available for us to sell pursuant to the purchase agreement. If and when we do sell additional shares to Lincoln Park, after Lincoln Park has acquired the shares, Lincoln Park may resell all, some or none of those shares at any time or from time to time in its discretion. Therefore, sales to Lincoln Park by us could result in substantial dilution to the interests of other holders of our common stock. Additionally, the sale of a substantial number of shares of our common stock to Lincoln Park, or the anticipation of such sales, could make it more difficult for us to sell equity or equity-related securities in the future at a time and at a price that we might otherwise wish to effect sales.

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 a prior year 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 our former wholly-owned subsidiary 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 2017 merger of Opexa Therapeutics, Inc. and private Acer Therapeutics Inc. 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 the net operating loss carryforwards and certain other tax attributes of our former wholly-owned subsidiary, which was subsequently merged with and into the company, 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 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 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 February 15, 2021, our executive officers and directors and their affiliates beneficially owned or controlled approximately 15% 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
- 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
- any action asserting a claim against us governed by the internal affairs doctrine

Section 27 of the Exchange Act creates exclusive federal jurisdiction over all suits brought to enforce any duty or liability created by the Exchange Act or the rules and regulations thereunder. As a result, the exclusive forum provision will not apply to suits brought to enforce any duty or liability created by the Exchange Act or any other claim for which the federal courts have exclusive jurisdiction. Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all suits brought to enforce any duty or liability created by the Securities Act or the rules and regulations thereunder. As a result, the exclusive forum provision will not apply to suits brought to enforce any duty or liability created by the Securities Act or any other claim for which the federal and state courts have concurrent jurisdiction.

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 become involved in litigation or proceedings relating to claims arising out of our operations.

Piper vs. Acer Therapeutics Inc.

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 $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 fact discovery has largely concluded. On February 22, 2019, Piper filed a motion for summary judgment. On March 26, 2020 the Court denied Piper's motion in part, as to Piper's claim and our counterclaim for damages, and granted in part, as to certain counterclaims by us. Discovery is ongoing in the case. Pursuant to the Court's directive, the parties have submitted a joint request for a pre-trial conference, which has not yet been scheduled. We have not recorded a liability as of December 31, 2020, because a potential loss is not probable or reasonably estimable given the status of the proceedings.

The Securities Class Action

On July 1, 2019, plaintiff Tyler Sell filed a putative class action lawsuit, *Sell v. Acer Therapeutics Inc. et al*, No. 1:19-cv-06137GHW, against us, Chris Schelling and Harry Palmin, in the U.S. District Court for the Southern District of New York. The complaint alleges that we violated federal securities laws by allegedly making material false and misleading statements regarding the likelihood of FDA approval for the EDSIVOTM NDA. With the selection of a lead plaintiff, the case is now captioned *Skiadas v. Acer Therapeutics Inc. et al*. The Lead Plaintiff filed a Second Amended Complaint on February 28, 2020 and we moved to dismiss the Second Amended Complaint on May 1, 2020. On June 16, 2020, the Court granted in part and denied in part our motion to dismiss. We filed its answer to the Second Amended Complaint on August 7, 2020, and the Court held an initial conference on August 17, 2020. After obtaining leave from the Court to do so, the Lead Plaintiff filed his Third Amended Complaint on February 4, 2021. We have not recorded a liability as of December 31, 2020 because a potential loss is not probable or reasonably estimable given the preliminary nature of the proceedings.

On August 12, 2019, a stockholder's derivative action, *Gress v. Aselage et al*., No. 1:19-cv-01505-MN, was filed in the U.S. District Court for the District of Delaware against certain of our present and former officers and directors, asserting damages resulting from the alleged breach of their fiduciary duties, based on the same facts at issue in the *Skiadas* case. On March 17, 2020, a second stockholder's derivative action, *Giroux v. Amello et al.*, No. 1:20-cv-10537-GAO, was filed in the U.S. District Court for the District of Massachusetts against certain of our present and former officers and directors, asserting claims based on the same facts at issue in the *Skiadas* and *Gress* cases. The parties in the *Gress a*nd *Giroux* cases have entered stipulations to stay the cases and the parties will meet and confer to propose case schedules to the Court in each of the respective cases. On June 23, 2020, a third stockholder's derivative action, *King v. Schelling*, *et al*., No. 1:20-cv-04779-GHW, was filed in the U.S. District Court for the Southern District of New York against certain of our present and former officers and directors that arises from the same facts underlying the *Skiadas*, *Gress*, and *Giroux* cases. The parties have agreed to extend the deadline to respond to the Derivative Complaint to December 10, 2020. On July 6, 2020, a fourth stockholder derivative action, *Diaz v. Amello et al*., No. 1:20-cv-00909-MN, was filed in the U.S. District Court for the District of Delaware. By Stipulation and Order dated August 7, 2020, the *Gress* and *Diaz* cases were consolidated under the caption *In re Acer Therapeutics Inc. Derivative Litigation*, Lead Case No. 1:19-cv-01505-MN. The parties recently reached an agreement to settle all of the derivative cases, and on January 21, 2021, plaintiff Giroux filed a motion to approve that settlement in the District Court of Massachusetts, the Court which will administer the settlement. If fully and finally approved by the Court as proposed, the settlement would provide for, among other things, (i) implementation or continuation by us of an agreed set of corporate governance measures, (ii) payment by our

insurance carriers of a total of $500,000 to plaintiffs' counsels, and (iii) a full and final release of all claims by the plaintiffs and a dismissal with prejudice of all of the pending derivative cases. We have not recorded a liability as of December 31, 2020 because a potential loss is not probable or reasonably estimable given the nature of the proceedings.

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 February 15, 2021, 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 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 four programs: ACER-001 (sodium phenylbutyrate) for the treatment of various inborn errors of metabolism, including urea cycle disorders ("UCDs") and Maple Syrup Urine Disease ("MSUD"); EDSIVO™ (celiprolol) for the treatment of vascular Ehlers-Danlos syndrome ("vEDS") in patients with a confirmed type III collagen (COL3A1) mutation; ACER-801 (osanetant) for the treatment of induced Vasomotor Symptoms ("iVMS"); and ACER-2820 (emetine), a host-directed therapy against a variety of infectious diseases, including COVID-19. Our product candidates are believed to present comparatively de-risked programs as evidenced by having one or more of the following: favorable safety profile, clinical proof-of-concept data, mechanistic differentiation, and/or accelerated pathways for development through specific programs and procedures established by the United States ("U.S.") Food and Drug Administration ("FDA").

Going Concern

The accompanying financial statements have been prepared in conformity with accounting principles generally accepted in the U.S. ("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 have an accumulated deficit of $99.1 million as of December 31, 2020 and expect to incur further losses over the foreseeable future 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, 2020, we had cash and cash equivalents of $5.8 million and current liabilities of $6.1 million. Our cash and cash equivalents available at December 31, 2020, combined with the funds raised subsequent to December 31, 2020, are expected to fund operations into the third quarter of 2021.

We will need to raise additional capital to fund continued operations in the second half of 2021. We may not be successful in our efforts to raise additional funds or achieve profitable operations. We continue to explore potential opportunities and alternatives to obtain the additional resources that will be necessary to support our ongoing operations through and beyond the next 12 months, including and raising additional capital through either private or public equity or debt financing, or non-dilutive funding, as well as using our ATM facility and/or our $15.0 million equity line facility entered into on April 30, 2020 with Lincoln Park Capital Fund, LLC ("Lincoln Park"), which is subject to certain limitations and conditions. From May 19, 2020 through the date of this report, we have raised gross proceeds of $10.0 million from the ATM facility and gross proceeds of $2.9 million from the agreement with Lincoln Park.

In addition, as described below, on January 25, 2021, we entered into an option agreement with Relief Therapeutics Holding AG ("Relief"), under the terms of which we received a $1.0 million upfront non-refundable payment and Relief provided to us a 12-month $4.0 million secured loan, repayable by us in cash on January 25, 2022 unless a definitive agreement is negotiated and executed prior to June 30, 2021 with respect to a potential collaboration and license agreement with us for the development, regulatory approval and commercialization of ACER-001 for UCDs and MSUD. At Relief's option, the outstanding balance of the $4.0 million loan can be used to offset the $14.0 million payment that may otherwise be payable to us from Relief if a definitive agreement is executed. We have no commitments for any additional financing, except for the agreement with Lincoln Park. 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 funding to support our current or proposed activities and operations, we may not be able to continue our operations as proposed, which may require us to suspend or terminate any ongoing development activities, modify our business plan, curtail various aspects of our operations, cease operations, or seek relief under applicable bankruptcy laws. In such event, our stockholders may lose a substantial portion or even all of their investment.

These factors individually and collectively raise substantial doubt about our ability to continue as a going concern for at least twelve months from the date these financial statements are available, or March 1, 2021. Our financial statements do not include any adjustments or classifications that may result from our possible inability to continue as a going concern.

Restructuring

In June 2019, we received a Complete Response Letter from the FDA regarding our New Drug Application ("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. 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 $1.5 million associated with the workforce reduction in the quarter ended June 30, 2019.

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 or similar 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. 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 $54.2 million in research and development expenses through December 31, 2020. Of that amount, $31.9 million was directly related to EDSIVO™, $14.4 million was directly related to ACER-001, $4.3 million was directly related to emetine, and $3.3 million was directly related to osanetant.

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; 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 (Expense) Income, Net

Other (expense) income, net consists primarily of interest income and of gains and losses resulting from the revaluation of assets and liabilities denominated in foreign currencies. We earn interest income from interest-bearing accounts and money market funds, which we classify as cash and cash equivalents. We record as part of other (expense) income, net, transaction 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 financial statements, which have been prepared in accordance with GAAP. The preparation of these financial statements requires our management to make estimates and judgments that affect the reported amounts of assets, liabilities and expenses. On an ongoing basis, we evaluate these estimates and judgments. We base our estimates on historical experience and on various assumptions that we believe to be reasonable under the circumstances. These estimates and assumptions form the basis for making judgments about the carrying values of assets and liabilities and the recording of expenses that are not readily apparent from other sources. Actual results may differ materially from these estimates. We believe that the accounting policies discussed below are critical to understanding our historical and future performance, as these policies relate to the more significant areas involving our judgments and estimates.

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 the recoverability of goodwill according to Accounting Standards Update No. 2017-04, *Intangibles – Goodwill and Other* (Topic 350), which we 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. We may opt to perform a qualitative assessment or a quantitative impairment test to determine whether goodwill is impaired. Our goodwill is allocated to a single reporting unit. 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.

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. We determined that the asset was impaired as of December 31, 2020 and wrote off the value of the IPRD accordingly.

Stock-Based Compensation

We account for stock-based compensation expense related to stock options 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, which involves making the selection of inputs such as expected volatility of our stock, the anticipated term of the option, and a risk-free interest rate. The establishment of these inputs inherently require judgment and estimates and can change from time to time depending on market factors and actual experience. 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

Research and development costs are expensed as incurred and include compensation and related benefits, license fees and outside contracted research and manufacturing consultants. We sometimes 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 financial statements based on certain facts and circumstances at that time. Our accrued expenses for preclinical studies and clinical trials are based on estimates of costs incurred for services provided by contract research organizations ("CRO"), manufacturing organizations, and for other trial- 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 accruing for these activities, we obtain information from various sources and estimate the level of effort or expense allocated to each period. Adjustments to our research and development expenses may be necessary in future periods as our estimates change. As these activities are generally material to our overall financial statements, subsequent changes in estimates may result in a material change in our accruals. No material changes in estimates were recognized in the years ended December 31, 2020 and 2019. At December 31, 2020 and 2019, our accounts payable and accrued expenses included $1.8 million and $0.6 million, respectively, for costs associated with preclinical or clinical study expense.

Results of Operations

Comparison of the years ended December 31, 2020 and 2019

The following table summarizes our results of operations for the years ended December 31, 2020 and 2019:

	Years Ended December 31,			
	2020	2019	$ Change	% Change
Research and development	$ 11,847,902	$ 13,851,018	$ (2,003,116)	(14)%
General and administrative	10,954,923	16,046,423	(5,091,500)	(32)%
Loss from operations	(22,802,825)	(29,897,441)	7,094,616	(24)%
Total other (expense) income, net	(82,624)	479,472	(562,096)	(117)%
Net loss	$ (22,885,449)	$ (29,417,969)	$ 6,532,520	(22)%

Research and Development Expenses

Research and development expenses were $11.8 million for the year ended December 31, 2020, as compared to $13.9 million for the year ended December 31, 2019. This decrease of $2.1 million was primarily due to decreases in employee-related expenses and in spending related to clinical and other consulting services, partially offset by an increase in contract research expenses. This decrease in employee-related expenses resulted from a decrease in headcount as a result of the restructuring after we received a Complete Response Letter from the FDA in June 2019. Research and development expenses for the year ended December 31, 2020 were comprised of $4.6 million related to ACER-001, $4.3 million related to emetine, $2.1 million related to osanetant, and $0.8 million related to EDSIVOTM.

General and Administrative Expenses

General and administrative expenses were $11.0 million for the year ended December 31, 2020, as compared to $16.0 million for the year ended December 31, 2019. This decrease of $5.0 million was primarily due to decreases in employee-related expenses and precommercial activities, partially offset by an increase in legal expenses. This decrease in employee-related expenses resulted from a decrease in headcount as a result of the restructuring after we received a Complete Response Letter from the FDA in June 2019.

Other (Expense) Income, Net

Other (expense) income, net of $(0.1) million and $0.5 million during the years ended December 31, 2020 and 2019, respectively, was primarily attributable to interest income and to transactional gains and losses related to remeasurement of trade accounts payable and accruals denominated in foreign currencies.

Liquidity and Capital Resources

We have never been profitable and have incurred operating losses in each year since inception. From inception to December 31, 2020, we have raised net cash proceeds of $92.3 million, primarily from common stock offerings, private placements of convertible preferred stock, and debt financings. As of December 31, 2020, we had $5.8 million in cash and cash equivalents and current liabilities aggregating to $6.1 million. Our net loss for the years ended December 31, 2020 and 2019, was $22.9 million and $29.4 million, respectively. As of December 31, 2020, we had an accumulated deficit of $99.1 million. Substantially all of our operating losses resulted from expenses incurred in connection with our research and development programs and from general and administrative costs associated with our operations.

The following table summarizes our cash flows for the years ended December 31, 2020 and 2019:

	Years Ended December 31,	
	2020	2019
Net cash (used in) provided by:		
Operating activities	$ (17,027,415)	$ (29,506,949)
Investing activities	(10,902)	(178,967)
Financing activities	10,722,245	92,272
Net decrease in cash and cash equivalents	$ (6,316,072)	$ (29,593,644)

Operating Activities

Net cash used in operating activities was $17.0 million for the year ended December 31, 2020, as compared to $29.5 million for the year ended December 31, 2019. The decrease of $12.5 million was principally the result of a decrease in net loss as well as decreased use of cash due to the timing of accrued expenses and accounts payable.

Investing Activities

Cash used in investing activities during the years ended December 31, 2020 and 2019 relates to the purchase of property and equipment.

Financing Activities

Net cash provided by financing activities during the year ended December 31, 2020 consisted primarily of net proceeds of $10.2 million from the issuance of common stock, comprised of $6.9 million net proceeds from our ATM facility, $2.4 million proceeds from our equity line facility entered into with Lincoln Park, and $0.9 million proceeds from a private placement of shares of our common stock. 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.

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 in connection with our ongoing development and manufacturing activities, particularly as we continue the research, development, manufacture and clinical trials of, and seek regulatory approval for, our product candidates. In addition, subject to obtaining regulatory approval of any of our product candidates and thereafter successfully commercializing any such product candidates, we anticipate that we will need substantial additional funding in connection with our continuing operations.

As of December 31, 2020, we had $5.8 million in cash and cash equivalents and current liabilities of $6.1 million. Based on available resources, we believe that our cash and cash equivalents currently on hand, combined with the funds raised subsequent to December 31, 2020, are sufficient to fund our currently anticipated operating and capital requirements into the third quarter of 2021.

On January 25, 2021, we and Relief entered into an option agreement pursuant to which we granted Relief an exclusive option to pursue a potential collaboration and license agreement with us for the development, regulatory approval and commercialization for ACER-001 for the treatment of UCDs and MSUD. The option agreement provides a period of time up to June 30, 2021 for the parties to perform additional due diligence and to work toward negotiation and execution of a definitive agreement with respect to the potential collaboration for ACER-001. In consideration for the grant of the exclusivity option, (i) we received from Relief an upfront nonrefundable payment of $1.0 million, (ii) Relief provided to us a 12-month secured loan in the principal amount of $4.0 million, as evidenced by a promissory note we issued to Relief, and (iii) we granted to Relief a security interest in all of our assets to secure performance of the promissory note, as evidenced by a security agreement. The note is repayable in

one lump sum within 12 months from issuance and bears interest at a rate equal to 6% per annum. If a definitive agreement with respect to the potential collaboration is not executed by the parties on or before June 30, 2021, the exclusivity option will terminate and the note is repayable by us upon maturity. The note contains certain customary events of default (including, but not limited to, default in payment of principal or interest thereunder or a material breach of the security agreement). Under the terms of the proposed collaboration and license agreement, the key terms of which are set forth in the option agreement, if a definitive agreement is executed pursuant to these terms and closed by June 30, 2021, we will receive $14.0 million in cash (which can be offset at Relief's option by the outstanding balance of the $4.0 million loan from Relief to us). In addition, Relief will agree to pay up to $20.0 million in U.S. development and commercial launch costs for the UCDs and MSUD indications. Further, we will retain development and commercialization rights in the U.S., Canada, Brazil, Turkey and Japan. The companies will split net profits from Acer's territories 60%:40% in favor of Relief. Relief will also license the rights for the rest of the world, where we will receive from Relief a 15% net sales royalty on all revenues received in Relief's territories. We could also receive a total of $6.0 million in milestones based on the first European marketing approvals for UCDs and MSUD. There can be no assurance, however, that a definitive agreement will be successfully negotiated and executed between the parties on these terms, on other mutually acceptable terms, or at all. Except for the $1.0 million upfront payment to Acer and the $4.0 million 12-month secured loan from Relief to Acer, the remaining proposed terms of the collaboration are not binding and are subject to change as a result of further diligence by Relief and negotiation of a definitive collaboration and license agreement between the parties.

On July 24, 2020, we entered into a securities purchase agreement for the sale and issuance of an aggregate of 244,998 shares of our common stock, for an aggregate purchase price of $0.9 million, in a Private Placement transaction with certain directors, officers, and employees at a price per share of $3.50. The shares of common stock issued in the Private Placement constitute "restricted securities" under the federal securities laws and are subject to a minimum six-month holding period. The proceeds from the Private Placement will be used by us for working capital and general corporate purposes.

On April 30, 2020, we entered into a $15.0 million purchase agreement and registration rights agreement with Lincoln Park pursuant to which we have the right to sell to Lincoln Park an aggregate of up to $15.0 million in shares of our common stock, subject to certain conditions and limitations. Under the terms and subject to the conditions of the purchase agreement, Lincoln Park is obligated to purchase up to an aggregate of $15.0 million in shares of common stock (subject to certain limitations) from time to time over a 36-month period commencing on June 8, 2020. We may direct Lincoln Park, at our sole discretion and subject to certain conditions, to purchase up to 50,000 shares of common stock in regular purchases, increasing to amounts of up to 100,000 shares depending upon the closing sale price of our common stock. In addition, we may direct Lincoln Park to purchase additional amounts as accelerated purchases or as additional accelerated purchases. The purchase price of shares of common stock related to this future funding will be based upon the market price of our common stock preceding the time of sale as computed under purchase agreement. However, there can be no assurance that we will be able to receive all of the funds from Lincoln Park because the purchase agreement contains limitations, restrictions, requirements, events of default and other provisions that could limit our ability to cause Lincoln Park to buy common stock from us. As of December 31, 2020, we had sold 900,000 shares of common stock under the under the purchase agreement at an average gross sale price of $2.64 per share, for gross proceeds of $2.4 million. Proceeds, net of $0.2 million of offering costs, were $2.2 million. Subsequent to December 31, 2020 and through the date of this report, we have sold an additional 200,000 shares under the purchase agreement for additional proceeds of $0.5 million.

On March 18, 2020, we entered into an amended and restated sales agreement with JonesTrading and Roth Capital. 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 which aggregate no more than one-third of our public float using our shelf registration statement at this time. From May 19, 2020 through December 31, 2020, we sold an aggregate of 1,838,957 shares of common stock at an average gross sale price of $3.9228 per share, for gross proceeds of $7.2 million. Proceeds, net of $0.3 million of fees and offering costs, were $6.9 million. Subsequent to December 31, 2020 during multiple trading days through the date of this report, we sold an aggregate of 877,107 additional shares of common stock through the ATM facility, resulting in additional gross proceeds of $2.8 million and additional net proceeds of $2.7 million after an additional $0.1 million of fees and offering costs.

In June 2019, in order to reduce operating expenses and conserve cash resources following the 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 of EDSIVO™. We recorded a one-time severance-related charge of $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 us 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. We believe we have identified a plan to collect additional data that supports the results from the COL3A1-positive analysis from the BBEST trial and could help meet the standard set forth in the FDA Guidance document issued in December 2019. In February 2021, we submitted a meeting request to the FDA to discuss Acer's proposed plan to provide sufficient confirmatory evidence. If successful, data provided under our proposal could potentially satisfy the additional confirmatory evidence needed to support a resubmission of our NDA, assuming the additional data analysis is positive. There can be no assurance that FDA will accept our plan or, if accepted, that the resulting data would be adequate to support resubmission, filing or approval of our NDA. We may also conclude at any point that the cost, risk and uncertainty of obtaining that additional data does not justify continuing with the development of EDSIVO™.

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

- whether or not we are able to successfully negotiate and enter into a definitive agreement with Relief for the potential collaboration and license of ACER-001, which will impact our requirement to repay in cash the $4.0 million 12-month secured loan from Relief
- any development of emetine we may choose to pursue, depending on capital needs, scope of development, FDA conditions, the availability of non-dilutive funding, the cost of any such development, and other factors
- any continued development, including preparing for or initiation of a planned emetine Phase 2/3 clinical trial, which is also subject to ongoing discussions with the FDA
- any continued development, including the initiation of one or more clinical trials for osanetant
- 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 any new employees to support our business operations
- 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 conditions and requirements, 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 foreseeable future 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, non-dilutive funding, and strategic alliances. Other than the purchase agreement we entered into with Lincoln Park, which is subject to certain limitations and conditions, our ATM facility, and our option agreement and related $4.0 million 12-month secured loan from Relief, we do not maintain any lines of credit or have any sources of debt or equity capital committed for funding.

We continue to explore potential opportunities and alternatives to obtain the additional resources that will be necessary to support our ongoing operations through and beyond the next 12 months, including raising additional capital through either private or public equity or debt financing, or non-dilutive funding, as well as using our ATM facility and/or our equity line facility. 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 public or private equity or debt financings or other sources, such as non-dilutive funding or strategic collaborations, when needed, we may be required to delay, limit, reduce or terminate our product development or pursuit of regulatory approval efforts or provide rights to develop and market product candidates to third parties that we would otherwise prefer to develop and, if applicable, market ourselves. Further, if we are unable to obtain additional funding to support our current or proposed activities and operations, we may not be able to continue our operations as proposed, which may require us to suspend or terminate any ongoing development activities, modify our business plan, curtail various aspects of our operations, cease operations, or seek relief under applicable bankruptcy laws. In such event, our stockholders may lose a substantial portion or even all of their investment.

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 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 optimal dose of celiprolol in treating vascular Ehlers-Danlos syndrome ("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 subsequently filed three U.S. patent applications on this subject matter in October 2018. We may choose to limit our pursuit of patent applications to specific territories, in which case AP-HP would have the right to revise our territorial license rights accordingly.

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.

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, 2020 and 2019 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 Financial Statements

Report of Independent Registered Public Accounting Firm

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

Opinion on the Financial Statements

We have audited the accompanying balance sheets of Acer Therapeutics Inc. (the "Company") as of December 31, 2020 and 2019, the related statements of operations, changes in stockholders' equity, and cash flows for each of the years in the two-year period ended December 31, 2020, 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 at December 31, 2020 and 2019, and the results of its operations and its cash flows for each of the years in the two-year period ended December 31, 2020, in conformity with accounting principles generally accepted in the United States of America.

Going Concern Uncertainty

The accompanying financial statements have been prepared assuming that the Company will continue as a going concern. As described in Note 1 to the 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 financial statements do not include any adjustments that might result from the outcome of this uncertainty.

Basis for Opinion

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

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

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

Critical Audit Matter

The critical audit matter communicated below is a matter arising from the current period audit of the financial statements that was communicated or required to be communicated to the audit committee and that: (1) relates to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective, or complex judgments. The communication of the critical audit matter does not alter in any way our opinion on the financial statements, taken as a whole, and we are not, by communicating the critical audit matter below, providing separate opinions on the critical audit matter or on the accounts or disclosures to which it relates.

Common Stock Purchase Agreement

As described in Notes 1 and 7 to the financial statements, the Company entered into a purchase agreement and registration rights agreement with Lincoln Park pursuant to which it has the right to sell to Lincoln Park and Lincoln Park is obligated to purchase up to $15.0 million in amounts of shares of the common stock over the 36-month period commencing on June 8, 2020. The Company issued 148,148 shares of common stock to Lincoln Park as a commitment fee in connection with entering into the purchase agreement and the $0.4 million fair value of the commitment fee shares was recorded to general and administrative expense.

We identified the accounting for the purchase agreement and registration rights agreement with Lincoln Park as a critical audit matter. Our principal considerations included the determination of balance sheet classification of the put option and the accounting for the related transactions costs which requires management to make significant judgments in the determination of the appropriate application of complex accounting literature including the possible identification of embedded derivatives. Auditing these elements involved especially challenging, subjective or complex auditor judgment due to the nature and extent of audit effort required to address these matters, including the extent of specialized skills or knowledge needed.

The primary procedures we performed to address this critical audit matter included:

- Reviewing the purchase and registration rights agreement and gaining an understanding of the business purpose of the transaction.

- Utilizing personnel with specialized knowledge and skill in technical accounting to assist in: (i) evaluating the relevant terms and conditions of the purchase agreement and (ii) assessing the appropriateness of conclusions reached by the Company with respect to the accounting for the purchase agreement and identification, assessment and accounting for potential derivatives.

/s/ BDO USA, LLP

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

Boston, Massachusetts

March 1, 2021

ACER THERAPEUTICS INC.
BALANCE SHEETS
AS OF DECEMBER 31, 2020 AND 2019

	2020	2019
Assets		
Current assets:		
Cash and cash equivalents	$ 5,761,568	$ 12,077,640
Prepaid expenses and other current assets	679,461	807,356
Total current assets	6,441,029	12,884,996
Property and equipment, net	130,081	193,974
Other assets:		
Goodwill	7,647,267	7,647,267
In-process research and development	—	118,600
Other non-current assets	395,311	620,674
Total assets	$ 14,613,688	$ 21,465,511
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 1,672,109	$ 561,090
Accrued expenses	3,781,101	1,944,431
Other current liabilities	692,336	263,392
Total current liabilities	6,145,546	2,768,913
Other non-current liabilities	243,808	326,282
Total liabilities	6,389,354	3,095,195
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; 13,233,137 and 10,095,176 shares issued and outstanding at December 31, 2020 and 2019, respectively	1,324	1,010
Additional paid-in capital	107,358,971	94,619,818
Accumulated deficit	(99,135,961)	(76,250,512)
Total stockholders' equity	8,224,334	18,370,316
Total liabilities and stockholders' equity	$ 14,613,688	$ 21,465,511

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

ACER THERAPEUTICS INC.
STATEMENTS OF OPERATIONS
FOR THE YEARS ENDED DECEMBER 31, 2020 AND 2019

	2020	2019
Operating expenses:		
Research and development	$ 11,847,902	$ 13,851,018
General and administrative	10,954,923	16,046,423
Total operating expenses	22,802,825	29,897,441
Loss from operations	(22,802,825)	(29,897,441)
Other (expense) income, net:		
Interest and other income (expense), net	13,578	471,267
Foreign currency transaction (loss) gain	(96,202)	8,205
Total other (expense) income, net	(82,624)	479,472
Net loss	$ (22,885,449)	$ (29,417,969)
Net loss per share - basic and diluted	$ (2.06)	$ (2.91)
Weighted average common shares outstanding - basic and diluted	11,121,039	10,092,179

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

ACER THERAPEUTICS INC.
STATEMENTS OF CHANGES IN
STOCKHOLDERS' EQUITY
FOR THE YEARS ENDED DECEMBER 31, 2020 AND 2019

	Stockholders' Equity				
	Common stock		Additional Paid-in Capital	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount			
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
Issuance of common stock, net of issuance costs	2,983,955	298	10,159,468	—	10,159,766
Issuance of common stock for commitment fee	148,148	15	355,540	—	355,555
Issuance of common stock in connection with restricted stock unit vesting	5,858	1	(1)	—	—
Stock-based compensation	—	—	2,224,146	—	2,224,146
Net loss	—	—	—	(22,885,449)	(22,885,449)
Balance as of December 31, 2020	13,233,137	$ 1,324	$ 107,358,971	$ (99,135,961)	$ 8,224,334

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

ACER THERAPEUTICS INC.
STATEMENTS OF CASH FLOWS
FOR THE YEARS ENDED DECEMBER 31, 2020 AND 2019

	2020	2019
Cash flows from operating activities:		
Net loss	$ (22,885,449)	$ (29,417,969)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation	2,224,146	2,612,855
Depreciation	70,814	58,282
Fair value of shares issued for commitment fee	355,555	—
Loss on disposal of property and equipment	3,981	57,578
Non-cash interest expense	3,674	—
Impairment of in-process research and development	118,600	—
Changes in operating assets and liabilities		
Prepaid expenses and other current assets	127,895	267,665
Accounts payable	1,111,019	(472,739)
Accrued expenses and other current liabilities	1,842,350	(2,602,001)
Other noncurrent assets	—	(10,620)
Net cash used in operating activities	(17,027,415)	(29,506,949)
Cash flows from investing activities:		
Purchase of property and equipment	(10,902)	(178,967)
Net cash used in investing activities	(10,902)	(178,967)
Cash flows from financing activities:		
Proceeds from issuance of common stock, net of issuance costs	10,159,766	—
Proceeds from Paycheck Protection Program loan	562,479	—
Proceeds from exercise of stock options	—	92,272
Net cash provided by financing activities	10,722,245	92,272
Net decrease in cash and cash equivalents	(6,316,072)	(29,593,644)
Cash and cash equivalents, beginning of the year	12,077,640	41,671,284
Cash and cash equivalents, end of the year	$ 5,761,568	$ 12,077,640

The accompanying notes are an integral part of these 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 four programs: ACER-001 (sodium phenylbutyrate) for the treatment of various inborn errors of metabolism, including urea cycle disorders ("UCDs") and Maple Syrup Urine Disease ("MSUD"); EDSIVO™ (celiprolol) for the treatment of vascular Ehlers-Danlos syndrome ("vEDS") in patients with a confirmed type III collagen (COL3A1) mutation; ACER-801 (osanetant) for the treatment of induced Vasomotor Symptoms ("iVMS"); and ACER-2820 (emetine), a host-directed therapy against a variety of infectious diseases, including COVID-19.

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 Food and Drug Administration ("FDA") regarding its new drug application ("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. 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 EDSIVOTM. 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. In February 2021, the Company submitted a meeting request to the FDA to discuss Acer's proposed plan to provide sufficient confirmatory evidence. If successful, data provided under the Company's proposal could potentially satisfy the additional confirmatory evidence needed to support a resubmission of the Company's NDA, assuming the additional data analysis is positive. There can be no assurance that FDA will accept the Company's plan or, if accepted, that the resulting data would be adequate to support resubmission, filing or approval of the Company's NDA. The Company may also conclude at any point that the cost, risk and uncertainty of obtaining that additional data does not justify continuing with the development of EDSIVO™.

Liquidity

The Company had an accumulated deficit of $99.1 million and cash and cash equivalents of $5.8 million as of December 31, 2020. Net cash used in operating activities was $17.0 million and $29.5 million for the years ended December 31, 2020 and 2019, 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 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 ("ATM") offering. 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. From May 19, 2020 through December 31, 2020, during multiple trading days, the Company sold an aggregate of 1,838,957 shares of common stock at an average gross sale price of $3.9228 per share, resulting in gross proceeds of $7.2 million. Proceeds, net of $0.3 million of fees and offering costs were $6.9 million. See Note 11, "Subsequent Events," regarding additional sales made subsequent to December 31, 2020.

On April 30, 2020, the Company entered into a purchase agreement and registration rights agreement pursuant to which Lincoln Park Capital Fund, LLC ("Lincoln Park") has committed to purchase up to $15.0 million of the Company's common stock. Under the terms and subject to the conditions of the purchase agreement, the Company has the right, but not the obligation, to sell to Lincoln Park, and Lincoln Park is obligated to purchase up to $15.0 million of the Company's common stock. Such sales of common stock by the Company will be subject to certain limitations, and may occur from time to time, at the Company's sole discretion, over the 36-month period commencing on June 8, 2020. The number of shares the Company may sell to Lincoln Park on any single business day in a regular purchase is 50,000, but that amount may be increased up to 100,000 shares, depending upon the market price of the Company's common stock at the time of sale and subject to a maximum limit of $1.0 million per regular purchase. The purchase price per share for each such regular purchase will be based on prevailing market prices of the Company's common stock immediately preceding the time of sale as computed under the purchase agreement. In addition to regular purchases, the Company may also direct Lincoln Park to purchase other amounts as accelerated purchases or as additional accelerated purchases if the closing sale price of the common stock exceeds certain threshold prices as set forth in the purchase agreement. The Company issued 148,148 shares of common stock to Lincoln Park as a commitment fee in connection with entering into the purchase agreement. The $0.4 million fair value of the commitment fee shares was recorded to General and administrative expense along with other costs incurred in connection with entering into the purchase agreement. As of December 31, 2020, the Company had sold 900,000 shares of common stock under its purchase agreement with Lincoln Park at a weighted average price of $2.64 per share, resulting in gross proceeds of $2.4 million. Proceeds, net of $0.2 million of offering costs, were $2.2 million. See Note 11, "Subsequent Events," regarding additional sales made subsequent to December 31, 2020.

On July 24, 2020, the Company entered into a securities purchase agreement for the sale and issuance of an aggregate of 244,998 shares of the Company's common stock, for an aggregate purchase price of $0.9 million, in a private placement transaction ("Private Placement") with certain directors, officers, and employees at a price per share of $3.50. The shares of common stock issued in the Private Placement constitute "restricted securities" under the federal securities laws and are subject to a minimum six-month holding period.

On January 25, 2021, the Company and Relief Therapeutics Holding AG ("Relief") entered into an option agreement pursuant to which the Company granted Relief an exclusive option to pursue a potential collaboration and license agreement with the Company for development, regulatory approval, and commercialization for ACER-001 for the treatment of UCDs and MSUD. The option agreement provides for a period of time up to June 30, 2021 for the parties to perform additional due diligence and to work toward negotiation and execution of a definitive agreement with respect to the potential collaboration for ACER-001. In consideration for the grant of the exclusivity option, (i) the Company received from Relief an upfront non-refundable payment of $1.0 million, (ii) Relief provided to the Company a 12-month secured loan in the principal amount of $4.0 million, as evidenced by a promissory note the Company issued to Relief, and (iii) the Company granted Relief a security interest in all of its assets to secure performance of the promissory note, as evidenced by a security agreement. The note is repayable in one lump sum within 12 months from issuance and bears interest at a rate equal to 6% per annum. At Relief's option, the outstanding balance of the $4.0 million loan can be used to offset the $14.0 million payment that may otherwise be payable to the Company from Relief if a definitive agreement is executed. If a definitive agreement with respect to the potential collaboration is not executed by the parties on or before June 30, 2021, the exclusivity option will terminate and the note is repayable by the Company upon maturity. The note contains certain customary events of default (including, but not limited to, default in payment of principal or interest thereunder or a material breach of the security agreement). See Note 11, "Subsequent Events," regarding the option agreement and $4.0 million 12-month secured loan arrangement with Relief.

The Company's existing cash and cash equivalents available at December 31, 2020, combined with the funds raised subsequent to December 31, 2020, are expected to fund operations into the third quarter of 2021.

Management expects to continue to finance operations through the issuance of additional equity or debt securities, non-dilutive funding, 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 accounting principles generally accepted in the U.S. ("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 has an accumulated deficit of $99.1 million as of December 31, 2020 and expects to incur further losses over the foreseeable future 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, 2020, the Company had cash and cash equivalents of $5.8 million and current liabilities of $6.1 million. The Company's cash and cash equivalents available at December 31, 2020, combined with the funds raised subsequent to December 31, 2020, are expected to fund operations into the third quarter of 2021.

The Company will need to raise additional capital to fund continued operations in 2021. The Company may not be successful in its efforts to raise additional funds or achieve profitable operations. The Company continues to explore potential opportunities and alternatives to obtain the additional resources that will be necessary to support its ongoing operations through and beyond the next 12 months, including raising additional capital through either private or public equity or debt financing or non-dilutive funding, as well as using its ATM facility and/or its $15.0 million equity line facility entered into on April 30, 2020 with Lincoln Park, which is subject to certain limitations and conditions. The Company has no commitments for any additional financing except for the agreement with Lincoln Park. As noted above, the Company received a 12-month $4.0 million secured loan from Relief in connection with granting Relief an exclusivity option to pursue a potential collaboration and license agreement with the Company for ACER-001. At Relief's option, the outstanding balance of the $4.0 million loan can be used to offset the payment that may otherwise be payable to the Company from Relief if a definitive agreement is executed. If a definitive agreement is not executed by the parties on or before June 30, 2021, the exclusivity option will terminate and the note will be repayable by the Company upon maturity at January 25, 2022. Any amounts raised will be used for further development of its product candidates, precommercial activities, potential acquisitions of additional product candidates and for other working capital purposes.

If the Company is unable to obtain additional funding to support its current or proposed activities and operations, it may not be able to continue its operations as proposed, which may require it to suspend or terminate any ongoing development activities, modify its business plan, curtail various aspects of its operations, cease operations, or seek relief under applicable bankruptcy laws. In such event, the Company's stockholders may lose a substantial portion or even all of their investment.

These factors individually and collectively raise substantial doubt about the Company's ability to continue as a going concern for twelve months from the date these financial statements are available, or March 1, 2021. 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.

Basis of Presentation

Any reference in these notes to applicable guidance is meant to refer to the authoritative accounting principles generally accepted in the United States ("U.S."), as found in the Accounting Standards Codification ("ASC") and Accounting Standards Update ("ASU") of the Financial Accounting Standards Board ("FASB").

2. SIGNIFICANT ACCOUNTING POLICIES

The preparation of these financial statements and related disclosures is in conformity with GAAP. A summary of the significant accounting policies followed by the Company in the preparation of the accompanying 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 $5.3 million and $11.6 million as of December 31, 2020 and 2019, 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 loans payable recorded in Other current liabilities and Other non-current liabilities approximates their carrying value, based upon their short-term maturities or prevailing interest rates.

Research and Development Expenses

Research and development costs are expensed as incurred and include compensation and related benefits, license fees, and third-party contracted research and manufacturing consultants. The Company will sometimes 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 the goods are received or that the services are performed.

Clinical Trial and Preclinical Study Expenses

The Company makes estimates of prepaid and/or accrued expenses as of each balance sheet date in its financial statements based on certain facts and circumstances at that time. The Company's accrued expenses for preclinical studies and clinical trials are based on estimates of costs incurred for services provided by contract research organizations ("CRO"), manufacturing organizations, and for other trial- and study-related activities. Payments under the Company's agreements with external service providers depend on a number of factors such as site initiation, patient screening, enrollment, delivery of reports, and other events. In accruing for these activities, the

Company obtains information from various sources and estimate the level of effort or expense allocated to each period. Adjustments to research and development expenses may be necessary in future periods as the Company's estimates change. As these activities are generally material to the financial statements, subsequent changes in estimates may result in a material change in the Company's accruals.

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:

	2020	2019
Risk-free interest rate	0.36% - 1.61%	1.68% - 2.57%
Expected life (years)	6.25	6.25
Expected 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 at least 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. As part of our annual impairment analysis as of December 31, 2020, the Company evaluated the potential future cash flows of the IPRD asset, and it was determined that the fair value was at or near zero due to the limited time remaining for the asset to be developed before the expiration of its intellectual property exclusivity. As a result, the Company determined that IPRD was impaired as of December 31, 2020 and wrote off the value of the IPRD accordingly. The Company recorded the impairment charge of $0.1 million in research and development expense during the period.

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. The COVID-19 pandemic involving a respiratory illness caused by a novel coronavirus affected the worldwide economy and triggered decline in the stock markets. The Company considered potential triggering events related to COVID-19 and concluded that there was not a triggering event that would require the Company to perform further impairment analysis. The Company performed a qualitative analysis of goodwill as of December 31, 2020 and 2019, 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 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 financial statements as of December 31, 2020 and 2019. The Company's policy is to recognize interest and penalties related to income tax, if any, in income tax expense. As of December 31, 2020 and 2019, the Company had no accruals for interest or penalties related to income tax matters.

The Coronavirus Aid, Relief and Economic Security Act (the "CARES Act") was enacted in the U.S. on March 27, 2020. The CARES Act, among other things, includes provisions relating to refundable payroll tax credits, deferment of employer side social security payments, net operating loss carryback periods, alternative minimum tax credit refunds, modifications to the net interest deduction limitations, increased limitations on qualified charitable contributions, and technical corrections to tax depreciation methods for qualified improvement property. The Company is required to recognize the effects of tax law changes in the period of enactment. The enactment of the CARES Act did not result in material adjustments for the income tax provision for the year ended December 31, 2020 or to the Company's assessment of the realizability of deferred tax assets as the carry back of net operating losses was used as a source of income. There were no other effects to the Company's tax provision as a result of the CARES Act as of December 31, 2020.

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 Issued Accounting Pronouncements

In August 2020, the FASB issued ASU No. 2020-06, *Debt – Debt with Conversion and Other Options* (Subtopic 470-20) and Derivatives and Hedging – Contracts in Entity's Own Equity (Subtopic 815-40), which simplifies the accounting for convertible instruments by removing, in certain cases, the need for models that required separate accounting for embedded conversion features and also amends the guidance for the derivatives scope exceptions for contracts in an entity's own equity. This ASU also requires expanded disclosures, including additional information related to the terms and features of convertible instruments and information about events or conditions that cause conversion contingencies to be met or conversion terms to be significantly changed. The amendments of this ASU are effective for fiscal years beginning after December 15, 2021 and must be applied using either a modified or full retrospective approach. Early adoption is permitted for interim or annual reporting periods beginning after December 15, 2020. The Company is currently evaluating the impact of the adoption of this ASU on its financial statements.

3. PROPERTY AND EQUIPMENT

Property and equipment consisted of the following at December 31, 2020 and 2019:

	December 31, 2020	December 31, 2019
Computer hardware and software	$ 58,903	$ 60,749
Leasehold improvements	60,535	60,535
Furniture and fixtures	145,487	145,487
Subtotal property and equipment, gross	264,925	266,771
Less accumulated depreciation	(134,844)	(72,797)
Property and equipment, net	$ 130,081	$ 193,974

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, 2020 and 2019:

	December 31, 2020	December 31, 2019
Accrued contract manufacturing	$ 1,479,771	$ 655,207
Accrued contract research and regulatory consulting	1,070,664	418,000
Accrued legal	350,517	152,340
Accrued payroll and payroll taxes	267,159	153,238
Accrued license fees	240,041	—
Accrued accounting, audit, and tax fees	181,200	147,850
Accrued miscellaneous expenses	102,999	56,598
Accrued consulting	88,750	109,073
Accrued precommercial costs	—	252,125
Total accrued expenses	$ 3,781,101	$ 1,944,431

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"). The term of the lease for the Bend Premises and the Additional Bend Premises expires on March 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. These options to extend were not included in the estimated term for the right of use asset or lease liability.

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 non-current 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% for each of the years ended December 31, 2020 and 2019, which corresponds to the Company's incremental borrowing rate. As of December 31, 2020, the weighted average remaining lease term was 1.4 years. For the years ended December 31, 2020 and 2019, the Company recorded expense of $0.3 million and $0.2 million, respectively, related to the leases. During the years ended December 31, 2020 and 2019, the Company made cash payments of $0.3 million and $0.2 million, respectively for amounts included in the measurement of lease liabilities. The Company is therefore reporting a right-of-use asset of $0.4 million in Other non-current assets and lease liabilities totaling $0.4 million in Other current liabilities and Other non-current liabilities as of December 31, 2020.

The following table reconciles the undiscounted lease liabilities to the total lease liabilities recognized on the balance sheet as of December 31, 2020:

Undiscounted lease liabilities for years ending December 31,		
2021	$	273,158
2022		115,951
Total undiscounted lease liabilities	$	389,109
Less effects of discounting		(24,798)
Total lease liabilities as of December 31, 2020	$	364,311

The Company's lease liabilities are reported on the consolidated balance sheets as follows:

	December 31,			
	2020		2019	
Other current liabilities	$	274,172	$	263,392
Other non-current liabilities		90,139		326,282
Total lease liabilities	$	364,311	$	589,674

6. COMMITMENTS AND CONTINGENCIES

License Agreements

In April 2014, the Company 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, the Company 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.

Paycheck Protection Program ("PPP") Loan

On April 11, 2020, the Company was advised that its principal bank, JPMorgan Chase Bank, N.A., had approved a $0.6 million loan under the PPP pursuant to the CARES Act that was signed into law on March 27, 2020.

As a U.S. small business, the Company has qualified for the PPP, which allows businesses and nonprofits with fewer than 500 employees to obtain loans of up to $10 million to incent companies to maintain their workers as they manage the business disruptions caused by the COVID-19 pandemic.

The loan, evidenced by a promissory note to JPMorgan Chase Bank, N.A. as lender, has a term of two years, is unsecured, and is guaranteed by the Small Business Administration. The loan bears interest at a fixed rate of one percent per annum, with the first six months of interest and principal deferred. Some or all of the loan may be forgiven if at least 75% of the loan proceeds are used by the Company to cover payroll costs, including benefits, and if the Company maintains its employment and compensation within certain parameters during the period following the loan origination date and complies with other relevant conditions. On June 5, 2020, the Payroll Protection Flexibility Act of 2020 was signed into law, adjusting certain terms of the loans issued under the PPP, including extending the initial deferral period from six to up to ten months, reducing from 75% to 60% the portion of loan proceeds required to be used to cover payroll costs, and allowing borrowers to elect a 24-week rather than an eight-week period related to employment and compensation provisions.

There can be no assurance that this PPP loan can be forgiven. The Company is reporting the liability associated with the loan as $0.4 million in Other current liabilities and $0.2 million in Other non-current liabilities and accounts for the loan according to ASC 470.

Litigation

Piper vs. Acer Therapeutics Inc.

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 $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 fact discovery has largely concluded. On February 22, 2019, Piper filed a motion for summary judgment. On March 26, 2020, the Court denied Piper's motion in part, as to Piper's claim and the Company's counterclaim for damages, and granted in part, as to certain counterclaims by the company. Discovery is ongoing in the case. Pursuant to the Court's directive, the parties have submitted a joint request for a pre-trial conference, which has not yet been scheduled. The Company has not recorded a liability as of December 31, 2020 because a potential loss is not probable or reasonably estimable given the status of the proceedings.

The Securities Class Action

On July 1, 2019, plaintiff Tyler Sell filed a putative class action lawsuit, *Sell v. Acer Therapeutics Inc. et al.*, No. 1:19-cv-06137GHW, 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 the Company violated federal securities laws by allegedly making material false and misleading statements regarding the likelihood of FDA approval for the EDSIVO™ NDA. With the selection of a lead plaintiff, the case is now captioned *Skiadas v. Acer Therapeutics Inc. et al*. The Lead Plaintiff filed a Second Amended Complaint on February 28, 2020 and the Company moved to dismiss the Second Amended Complaint on May 1, 2020. On June 16, 2020, the Court granted in part and denied in part the Company's motion to dismiss. The Company filed its answer to the Second Amended Complaint on August 7, 2020, and the Court held an initial conference on August 17, 2020. After obtaining leave from the Court to do so, the Lead Plaintiff filed his Third Amended Complaint on February 4, 2021. The Company has not recorded a liability as of December 31, 2020 because a potential loss is not probable or reasonably estimable given the preliminary nature of the proceedings.

On August 12, 2019, a stockholder's derivative action, *Gress v. Aselage, et al*., No. 1:19-cv-01505-MN,was filed in the U.S. District Court for the District of Delaware against certain of the Company's present and former officers and directors, asserting damages resulting from the alleged breach of their fiduciary duties, based on the same facts at issue in the *Skiadas* case. On March 17, 2020, a second stockholder's derivative action, *Giroux v. Amello et al.*, No. 1:20-cv-10537-GAO, was filed in the U.S. District Court for the District of Massachusetts against certain of the Company's present and former officers and directors, asserting claims based on the same facts at issue in the *Skiadas* and *Gress* cases. The parties in the *Gress* and *Giroux* cases have entered stipulations to stay the cases and the parties will meet and confer to propose case schedules to the Court in each of the respective cases. On June 23, 2020, a third stockholder's derivative action, *King v. Schelling*, *et al*., No. 1:20-cv-04779-GHW, was filed in the U.S. District Court for the Southern District of New York against certain of the Company's present and former officers and directors that arises from the same facts underlying the *Skiadas*, *Gress*, and *Giroux* cases. The parties have agreed to extend the deadline to respond to the Derivative Complaint to December 10, 2020. On July 6, 2020, a fourth stockholder derivative action, *Diaz v. Amello et al.*, No. 1:20-cv-00909-MN, was filed in the U.S. District Court for the District of Delaware. By Stipulation and Order dated August 7, 2020, the *Gress* and *Diaz* cases were consolidated under the caption *In re Acer Therapeutics Inc. Derivative Litigation*, Lead Case No. 1:19-cv-01505-MN. The parties recently reached an agreement to settle all of the derivative cases and on January 21, 2021, plaintiff Giroux filed a motion to approve that settlement in the District Court of Massachusetts, the Court which will administer the settlement. If fully and finally approved by the Court as proposed, the settlement would provide for, among other things, (i) implementation or continuation by the Company of an agreed set of corporate governance measures, (ii) payment by the Company's insurance carriers of a total of $500,000 to plaintiffs' counsels, and (iii) a full and final release of all claims by the plaintiffs and a dismissal with prejudice of all of the pending derivative cases. The Company has not recorded a liability as of December 31, 2020 because a potential loss is not probable or reasonably estimable given the nature of the proceedings.

7. STOCKHOLDERS' EQUITY

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" ("ATM") offering. 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. From May 19, 2020 through December 31, 2020, the Company sold an aggregate of 1,838,957 shares of common stock at an average gross sale price of $3.9228 per share, for gross proceeds of $7.2 million. Proceeds, net of $0.3 million fees and offering costs, were $6.9 million. See Note 11, "Subsequent Events," regarding additional sales made subsequent to year-end.

Private Placement

On July 24, 2020, the Company entered into a securities purchase agreement for the sale and issuance of an aggregate of 244,998 shares of the Company's common stock, for an aggregate purchase price of $0.9 million, in a Private Placement with certain directors, officers, and employees at a price per share of $3.50. The shares of common stock issued in the Private Placement constitute "restricted securities" under the federal securities laws and are subject to a minimum six-month holding period.

Common Stock Purchase Agreement

On April 30, 2020, the Company entered into a purchase agreement and a registration rights agreement pursuant to which Lincoln Park has committed to purchase up to $15.0 million of the Company's common stock. Under the terms and subject to the conditions of the purchase agreement, the Company has the right, but not the obligation, to sell to Lincoln Park, and Lincoln Park is obligated to purchase up to $15.0 million of the Company's common stock. Such sales of common stock by the Company will be subject to certain limitations, and may occur from time to time, at the Company's sole discretion, over the 36-month period commencing on June 8, 2020. The number of shares the Company may sell to Lincoln Park on any single business day in a regular purchase is 50,000, but that amount may be increased up to 100,000 shares, depending upon the market price of the Company's common stock at the time of sale and subject to a maximum limit of $1.0 million per regular purchase. The purchase price per share for each such regular purchase will be based on prevailing market prices of the Company's common stock immediately preceding the time of sale as computed under the purchase agreement. In addition to regular purchases, the Company may also direct Lincoln Park to purchase other amounts as accelerated purchases or as additional accelerated purchases if the closing sale price of the common stock exceeds certain threshold prices as set forth in the purchase agreement.

Under applicable rules of the Nasdaq Capital Market, in no event may the Company issue or sell to Lincoln Park under the purchase agreement more than 19.99% of the shares of the Company's common stock outstanding immediately prior to the execution of the purchase agreement, unless (i) the Company obtains stockholder approval to issue shares of common stock in excess of the Exchange Cap or (ii) the average price of all applicable sales of common stock to Lincoln Park under the purchase agreement equals or exceeds $2.1668, such that issuances and sales of the common stock to Lincoln Park under the purchase agreement would be exempt from the issuance limitation under applicable Nasdaq rules. The Company determined that the right to sell additional shares represents a freestanding put option under ASC 815 Derivatives and Hedging, but has a fair value of zero, and therefore no additional accounting was required.

Lincoln Park has no right to require the Company to sell any shares of common stock to Lincoln Park, but Lincoln Park is obligated to make purchases as the Company directs, subject to certain conditions. In all instances, the Company may not sell shares of its common stock to Lincoln Park under the purchase agreement if doing so would result in Lincoln Park beneficially owning more than 9.99% of its common stock.

Actual sales of shares of common stock to Lincoln Park under the purchase agreement will depend on a variety of factors to be determined by the Company from time to time, including, among others, market conditions, the trading price of the common stock and determinations by the Company as to the appropriate sources of funding for the Company and its operations.

The proceeds under the purchase agreement to the Company will depend on the frequency and prices at which the Company sells shares of its stock to Lincoln Park. The Company issued 148,148 shares of common stock to Lincoln Park as a commitment fee in connection with entering into the purchase agreement. The $0.4 million fair value of the commitment fee shares was recorded to General and administrative expense along with other costs incurred in connection with entering into the purchase agreement. As of December 31, 2020, the Company had sold 900,000 shares of common stock under its purchase agreement with Lincoln Park at a weighted average price of $2.64 per share, resulting in gross proceeds of $2.4 million. Proceeds, net of $0.2 million of offering costs, were $2.2 million. See Note 11, "Subsequent Events," regarding additional sales made subsequent to year-end.

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, 2020 and 2019, 403,807 and 403,495 additional shares, respectively, were authorized according to the evergreen provision. At December 31, 2020, 642,074 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

The Company's 2013 Plan 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, 2020, 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, 2020, all shares available under the 2010 Plan were subject to outstanding equity awards, and no new awards may be granted under the 2010 Plan.

Stock Plan Activity

A summary of option activity under the 2018 Plan, 2013 Plan, and 2010 Plan for the year ended December 31, 2020 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, 2019	1,313,475	$ 12.28	8.7	
Granted	141,000	$ 3.56		
Cancelled/forfeited	(214,121)	$ 13.04		
Options outstanding at December 31, 2020	1,240,354	$ 11.16	7.8	$ —
Options exercisable at December 31, 2020	490,345	$ 15.36	6.6	$ —

A summary of restricted stock unit activity under the 2018 Plan for the year ended December 31, 2020 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, 2019	9,000	$ 23.60	
Vested/settled	(5,858)		
Cancelled/forfeited	(3,142)		
Non-vested outstanding at December 31, 2020	—	$ —	$ —

At December 31, 2020, there was $2.8 million of unrecognized compensation expense related to the stock-based compensation arrangements granted under all plans. The average remaining vesting period for options was 1.3 years. The weighted average grant-date fair value of options granted during the years ended December 31, 2020 and 2019 was $1.99 and $8.03, respectively. The amount of stock-based compensation expense recorded to research and development expenses and to general and administrative expenses was $0.8 million and $1.5 million, respectively, for the year ended December 31, 2020. The amount of stock-based compensation expense recorded to research and development expenses and to general and administrative expenses was $1.0 million and $1.6 million, respectively, for the year ended December 31, 2019.

8. INCOME TAXES

There was no provision for income taxes for the years ended December 31, 2020 and 2019, 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,	
	2020	2019
Deferred tax assets:		
Net operating loss carry forwards	$ 11,979,869	$ 8,506,035
Capitalized research and development costs	18,650,538	17,477,585
Accrued liabilities	111,481	133,933
Tax credit carryforwards	7,755,146	7,174,516
Stock-based compensation	1,229,407	935,508
Operating lease	86,491	141,777
Total deferred tax assets	39,812,932	34,369,354
Valuation allowance	(39,726,441)	(34,221,451)
Net deferred tax assets	86,491	147,903
Deferred tax liabilities:		
Operating lease right of use asset	(86,491)	(141,777)
Other	—	(6,126)
Total deferred tax liabilities	(86,491)	(147,903)
	$ —	$ —

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

	December 31,	
	2020	2019
Federal statutory rate	21.0%	21.0%
R&D and Orphan Drug credits	2.5%	4.4%
State income tax, net of federal tax benefit	1.5%	6.6%
Valuation allowance	(24.3%)	(31.6%)
Share-based compensation	(0.8%)	(0.2%)
Other, net	0.1%	(0.2%)
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, 2020, for income tax reporting purposes, the Company had U.S. federal and state net operating loss carryforwards of $51.0 million and research and development credits and Orphan Drug credits of $7.8 million that can be carried forward and offset against taxable income. For state purposes, the Company had state net operating loss carryforwards of $1.6 million and research and development credits of $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, 2020 and 2019, the number of shares of common stock underlying potentially dilutive securities consist of:

	December 31,	
	2020	2019
Options to purchase common stock	1,240,354	1,313,475
Unvested, unsettled restricted stock units	—	9,000
Total	1,240,354	1,322,475

10. RESTRUCTURING

In June 2019, the Company received a Complete Response Letter from the FDA regarding its New Drug Application 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. 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 EDSIVOTM. In the second quarter of 2019, the Company recorded a one-time severance-related charge of $1.5 million associated with the workforce reduction in the quarter ended June 30, 2019, of which $1.0 million was included in general and administrative expenses and $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.

11. SUBSEQUENT EVENTS

On January 25, 2021, the Company and Relief entered into an option agreement pursuant to which the Company granted Relief an exclusive option to pursue a potential collaboration and license agreement with the Company for development, regulatory approval and commercialization for ACER-001 for the treatment of UCDs and MSUD. The option agreement provides for a period of time up to June 30, 2021 for the parties to perform additional due diligence and to work toward negotiation and execution of a definitive agreement with respect to the potential collaboration for ACER-001. In consideration for the grant of the exclusivity option, (i), the Company received from Relief an upfront non-refundable payment of $1.0 million , (ii) Relief provided to the Company a 12-month secured loan in the principal amount of $4.0 million, as evidenced by a promissory note the Company issued to Relief, and (iii) the Company granted Relief a security interest in all of its assets to secure performance of the promissory note, as evidenced by a security agreement. The note is repayable in one lump sum within 12 months from issuance and bears interest at a rate equal to 6% per annum. If a definitive agreement with respect to the potential collaboration is not executed by the parties on or before June 30, 2021, the exclusivity option will terminate and the note is repayable by the Company upon maturity. The note contains certain customary events of default (including, but not limited to, default in payment of principal or interest thereunder or a material breach of the security agreement). Under the terms of the proposed collaboration and license agreement, the key terms of which are set forth in the option agreement, if a definitive agreement is executed pursuant to these terms and closed by June 30, 2021, the Company will receive $14.0 million in cash (which can be offset at Relief's option by the outstanding balance of the $4.0 million loan from Relief to the Company). In addition, Relief will agree to pay up to $20.0 million in U.S. development and commercial launch costs for the UCDs and MSUD indications. Further, the Company will retain development and commercialization rights in the U.S., Canada, Brazil, Turkey and Japan. The companies will split net profits from the Company's territories 60%:40% in favor of Relief. Relief will also license the rights for the rest of the world, where the Company will receive from Relief a 15% net sales royalty on all revenues received in Relief's territories. The Company could also receive a total of $6.0 million in milestones based

on the first European (EU) marketing approvals for UCDs and MSUD. There can be no assurance, however, that a definitive agreement will be successfully negotiated and executed between the parties on these terms, on other mutually acceptable terms, or at all. Except for the $1.0 million upfront payment to the Company and the $4.0 million 12-month secured loan from Relief to the Company, the remaining proposed terms of the collaboration are not binding and are subject to change as a result of further diligence by Relief and negotiation of a definitive collaboration and license agreement between the parties.

Subsequent to December 31, 2020, during multiple trading days through the date of this report, the Company sold an aggregate of 877,107 shares of common stock under its ATM facility at an average gross sale price of $3.17 per share, resulting in gross proceeds of $2.8 million.

Subsequent to December 31, 2020, through the date of this report, the Company sold 200,000 shares of common stock under its purchase agreement with Lincoln Park at a price of $2.47 per share, resulting in gross proceeds of $0.5 million.

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, 2020 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, 2020 in providing reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles.

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

Changes in Internal Control over Financial Reporting

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

Item 9B. Other Information.

None.

PART III

Item 10. Directors, Executive Officers and Corporate Governance.

Executive Officers

The names of our executive officers and their ages as of February 15, 2021 are as follows:

Name	Age	Position
Chris Schelling	45	President, Chief Executive Officer and Director
Jefferson E. Davis	54	Chief Business Officer
Donald R. Joseph	66	Chief Legal Officer and Secretary
John M. Klopp	46	Chief Technical Officer
Harry S. Palmin	51	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 2017 merger of Opexa Therapeutics, Inc. and private Acer. Mr. Schelling founded private Acer Therapeutics Inc. in December 2013 and served as a director from that time until the 2017 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 served as a director until the acquisition by PTC Therapeutics Inc. in May 2020. 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.

Jefferson E. Davis was appointed as our Chief Business Officer in February 2021. He previously served as our head of corporate development from July 2020 to January 2021 and as Acting Chief Business Officer of private Acer and then public Acer from December 2013 to October 2018. He served as Vice President of Corporate Development for Censa Pharmaceuticals Inc. from November 2019 until its acquisition by PTC Therapeutics Inc. in May 2020, and as a consultant from May 2015 to November 2019. Mr. Davis was employed by Extera Partners from April 2011 to July 2020, having served as a Partner from October 2014. In addition, he held senior business and corporate development roles at Genzyme, Archemix, ImmunoGen, GenVec, and Eli Lilly during the period of 1996 to 2011. Mr. Davis earned a B.S. in biochemistry from North Carolina State University and an M.B.A. from Duke University.

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 M. 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 2017 merger of Opexa Therapeutics, Inc. and private Acer Therapeutics Inc., 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 February 15, 2021 are as follows:

Name	Age	Position
Stephen J. Aselage	69	Chairman of the Board
Jason Amello	52	Director
John M. Dunn	69	Director
Michelle Griffin	55	Director
Chris Schelling	45	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 2017 merger of Opexa Therapeutics Inc. and private Acer Therapeutics Inc. From October 2015 until the merger, Mr. Aselage served as the Chairman of private Acer's Board of Directors. Most recently, he was the Chief Executive Officer of Travere Therapeutics, Inc. (formerly known as 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 Travere Therapeutics, Inc. Prior to joining Travere Therapeutics, Inc., 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. Mr. Aselage currently serves on the Advisory Council for the Department of Science at the University of Notre Dame and also serves on the Board of Directors at BioCryst Pharmaceuticals, Inc.

Jason Amello has served as a director since the completion of the 2017 merger. Since September 2020, Mr. Amello has served as Chief Financial Officer of Saniona AB, a rare disease biopharmaceutical company listed on

Nasdaq Stockholm Small Cap. From September 2013 to August 2020, he 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, Mr. Amello served as Executive Vice President, Chief Financial Officer and Treasurer of ZIOPHARM Oncology, Inc., a Nasdaq-listed biopharmaceutical company. From April 2000 to June 2011, he held various positions at Genzyme Corporation, a then Nasdaq-listed 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 2017 merger. From October 2015 until the merger, Mr. Dunn served as a member of private Acer's Board of Directors. Since 2019, Mr. Dunn has worked as a consultant in the life sciences industry. 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 2017 merger. Ms. Griffin serves as a member of the Board of Directors and as Chair of the Audit Committee for publicly traded companies Chinook Therapeutics, Inc. since October 2020, Adaptive Biotechnologies Corporation since March 2019, and HTG Molecular Diagnostics, Inc. since August 2018. From 2013 to 2020, Ms. Griffin served as the Principal of Pacific Biotechnology Consulting Group, a firm providing consulting services to biotechnology companies and their Boards of Directors. 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. Ms. Griffin served from January 2011 to March 2013 as Executive Vice President, Operations and Chief Financial Officer of OncoGenex Pharmaceuticals, Inc. 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 five 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, 2020.

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, 2020 and 2019.

2020 Summary Compensation Table

Name and Principal Position	Year	Salary ($)	Option Awards ($)(1)		All Other Compensation ($)	Total ($)
Chris Schelling	2020	$ 436,000	$ —		$ —	$ 436,000
President and Chief Executive Officer	2019	$ 436,000	$ 1,031,211	(2)	$ —	$ 1,467,211
Harry S. Palmin	2020	$ 382,400	$ —		$ —	$ 382,400
Chief Operating Officer and Chief Financial Officer	2019	$ 382,400	$ 462,041	(2)	$ —	$ 844,441
Donald R. Joseph	2020	$ 350,200	$ —		$ —	$ 350,200
Chief Legal Officer and Secretary(3)						

(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 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 Messrs. Schelling and Palmin and (ii) on September 18, 2019 to 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 grant vests 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) Mr. Joseph has served as Chief Legal Officer and Secretary since April 2018.

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 2020 and 2019 generally consisted of base salary and equity awards for options to purchase shares of our common stock. 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, we retained the services of Radford as an independent compensation consultant to (i) evaluate our executive compensation program and recommend a course of action for consideration in preparation for becoming a public company and (ii) assess our 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 us and implemented certain compensation adjustments for our executives and non-employee directors. In reviewing the reports prepared by Radford, 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.

On February 22, 2018, we entered into an employment agreement with each of Messrs. Schelling and Palmin, and on April 20, 2018, we entered into an employment agreement with Mr. Joseph. The terms and conditions of such employment agreements are described below and are identical for each executive officer, other than in respect of each individual's title, duties, salary and target bonus percentage as set forth below.

Pursuant to each individual's employment agreement, each executive is compensated at an annual base rate and is eligible to receive an annual discretionary cash bonus of up to a target bonus percentage of his base salary (i.e., 50% for Mr. Schelling and 35% for each of the other executive officers) per 12-month period, based upon the achievement of corporate objectives established from time to time by our Board of Directors. In addition, each executive receives our standard benefits and insurance coverage as generally provided to our employees. Each executive's employment is at-will and he serves as an executive officer at the discretion of our Board of Directors.

In the event the executive's employment is terminated by us without Cause (as defined in the agreement) or due to a Constructive Termination (as defined in the agreement), in each instance during the period commencing one month prior to a Change in Control (as defined in the agreement) and terminating 12 months after such Change in Control, the executive will be entitled to (i) a payment, less applicable taxes and withholdings, equal to his then-current base salary for a period of 12 months plus (ii) 1x times his annual discretionary target bonus calculated for such period. In the event the executive's employment is terminated by us without Cause or due to a Constructive Termination occurring outside of a Change in Control Period (as defined in the agreement), the executive will be entitled to a payment, less applicable taxes and withholdings, equal to his then-current base salary for a period of 12 months. The executive would receive any such payment in the form of a lump sum 60 days following such termination of employment. In addition, whether in the context of a Change in Control or otherwise, (x) if the executive elects to continue his health insurance coverage under COBRA, then we will reimburse the executive for the same portion of the executive's monthly premium over such 12-month period as we are then paying for health insurance coverage for active employees, and (y) to the extent not otherwise addressed by any equity-based compensation arrangements, the executive will be entitled to 12 months of credited vesting beyond the employment

termination date for any outstanding equity-based awards. The severance benefits are subject to the executive having been continuously employed through the termination event as well as executing and delivering a general release and waiver of claims in favor of us. The timing of any payments to the executive under the employment agreement are subject to applicable requirements of Section 409A of the Internal Revenue Code of 1986 and the related Treasury Regulations and may be delayed or reformed to comply with such provisions. In the event any payment or benefit the executive might be entitled to receive would constitute a "parachute payment" under Section 280G of the Internal Revenue Code, such payment or benefit will be reduced so as not to trigger excise tax under Section 4999 of such Code.

Outstanding Equity Awards at Fiscal Year-End

The following table presents information regarding outstanding equity awards at December 31, 2020 for each of the Named Executive Officers.

		Option Awards				
Name	Grant Date	Number of Securities Underlying Unexercised Options (#) Exercisable	Number of Securities Underlying Unexercised Options (#) Unexercisable		Option Exercise Price ($)	Option Expiration Date
Chris Schelling	2/1/19	31,718	40,782	(1)	$ 24.46	2/1/29
	10/4/17	34,500	11,500	(1)	$ 15.34	10/4/27
Harry S. Palmin	9/18/19	—	40,000	(2)	$ 3.42	9/18/29
	2/1/19	11,814	15,186	(1)	$ 24.46	2/1/29
	10/4/17	50,700	16,900	(1)	$ 15.34	10/4/27
Donald R. Joseph	9/18/19	—	40,000	(2)	$ 3.42	9/18/29
	2/1/19	11,814	15,186	(1)	$ 24.46	2/1/29
	4/20/18	37,500	22,500	(1)	$ 18.53	4/20/28

(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.

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, 2020.

Name	Fees Earned or Paid in Cash ($)	Option Awards ($) (1)(2)	All Other Compensation ($)	Total ($)
Jason Amello	42,500	19,417	—	61,917
Stephen J. Aselage	73,750	19,417	—	93,167
John M. Dunn	50,000	19,417	—	69,417
Michelle Griffin	55,000	19,417	—	74,417

(1) Amounts shown in this column represent the aggregate grant date fair value of stock option awards made during 2020, calculated in accordance with ASC Topic 718. See Note 2 to our financial statements appearing elsewhere in this report for a discussion of the relevant assumptions used in calculating these amounts.

(2) The aggregate number of shares underlying outstanding option awards as of December 31, 2020 was: Mr. Amello, 24,000 shares; Mr. Aselage, 42,000 shares; Mr. Dunn, 37,000 shares; and Ms. Griffin, 24,000 shares.

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 – 9,000 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 February 15, 2021, 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) our Named Executive Officers; and (d) all current directors and executive officers as a group. As of February 15, 2021, there were 14,310,244 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)	18.7%
Nantahala Capital Management, LLC	989,755	(3)	6.9%
AIGH Capital Management, LLC	982,446	(4)	6.9%
Executive Officers and Directors:			
Chris Schelling	1,969,356	(5)	13.7%
Harry S. Palmin	217,652	(6)	1.5%
Donald R. Joseph	89,037	(7)	*
Jason Amello	24,000	(8)	*
Stephen J. Aselage	105,905	(9)	*
John M. Dunn	64,380	(10)	*
Michelle Griffin	24,000	(11)	*
All current directors and executive officers as a group (10 persons)	2,664,487	(12)	18.0%

* 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, 2021. Pursuant to the Schedule 13G, 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 a Schedule 13G/A filed with the SEC on August 6, 2020. Pursuant to the Schedule 13G, AIGH Capital Management, LLC ("AIGH") and Orin Hirschman, as managing member of AIGH and president of AIGH Investment Partners LLC, each have sole voting and investment power with respect to the shares which are held directly by the persons named therein. The address for AIGH and Mr. Hirschman is 6006 Berkeley Avenue, Baltimore, Maryland 21209.

(5) Consisting of (i) 1,892,857 shares of common stock; and (ii) 76,499 shares of common stock underlying stock options exercisable within 60 days of February 15, 2021.

(6) Consisting of (i) 125,000 shares of common stock; and (ii) 92,652 shares of common stock underlying stock options exercisable within 60 days of February 15, 2021.

(7) Consisting of (i) 14,285 shares of common stock; and (ii) 74,752 shares of common stock underlying stock options exercisable within 60 days of February 15, 2021.

(8) Represents shares of common stock underlying stock options exercisable within 60 days of February 15, 2021.

(9) Consisting of (i) 63,905 shares of common stock; and (ii) 42,000 shares of common stock underlying stock options exercisable within 60 days of February 15, 2021.

(10) Consisting of (i) 27,380 shares of common stock; and (ii) 37,000 shares of common stock underlying stock options exercisable within 60 days of February 15, 2021.

(11) Represents shares of common stock underlying stock options exercisable within 60 days of February 15, 2021.

(12) Consisting of: (i) 2,195,072 shares of common stock; and (ii) 469,415 shares of common stock underlying stock options exercisable within 60 days of February 15, 2021.

Securities Authorized for Issuance Under Equity Compensation Plans

The following table sets forth information, as of December 31, 2020, 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	1,240,354	$ 11.16	642,074
Equity Compensation Plans Not Approved by Stockholders	—	—	—
Total	1,240,354	$ 11.16	642,074

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

Transactions with Related Persons

Since January 1, 2020, we have engaged in the following reportable transactions with our directors, executive officers, beneficial holders of more than 5% of our voting securities, and affiliates or their immediate family members.

On July 24, 2020, we entered into a securities purchase agreement for the sale and issuance of an aggregate of 244,998 shares of our common stock, for an aggregate purchase price of $857,493, in a private placement transaction at a price per share of $3.50, which represented a 5.7% premium to the $3.31 closing price of the common stock on that day. The shares were purchased by the following directors, executive officers and employees:

Name	Relationship	Purchase Price	Number of Shares Purchased
Chris Schelling	President, Chief Executive Officer and Director	$499,999.50	142,857
Stephen J. Aselage	Chairman of the Board of Directors	$157,500.00	45,000
John M. Dunn	Director	$ 74,998.00	21,428
Donald R. Joseph	Chief Legal Officer and Secretary	$ 49,997.50	14,285
Jefferson Davis	Chief Business Officer(1)	$ 74,998.00	21,428

(1) Mr. Davis was an employee at the time of the offering and was subsequently appointed as our Chief Business Officer on February 1, 2021.

The shares of common stock issued in the private placement constitute "restricted securities" under the federal securities laws and are subject to a minimum six-month holding period. The private placement closed on July 29, 2020.

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 the aggregate fees billed to us for the fiscal years ended December 31, 2020 and 2019 by BDO USA, LLP ("BDO"), who was appointed as our independent registered public accounting firm on March 7, 2019.

	Years Ended December 31,	
	2020	2019
Audit fees(1)	$ 322,064	$ 283,128
Audit-related fees(2)	—	—
Tax fees(3)	—	—
All other fees	—	—
Total fees	$ 322,064	$ 283,128

(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.

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. While the Audit Committee's practice is to pre-approve 100% of any audit-related services, tax services, or other services provided by our independent auditors, there were no such services rendered 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 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
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 (incorporated by reference to Exhibit 4.1 to the Company's Annual Report on Form 10-K for the year ended December 31, 2019).
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.
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).
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).

Exhibit No.	Description
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♦♦	License Agreement, dated as of December 21, 2018, by and between Acer Therapeutics Inc. and Sanofi (incorporated by reference to Exhibit 10.1 to the to the Company's Quarterly Report on Form 10-Q filed on August 13, 2020.)
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*+	Employment Agreement, dated February 1, 2021, by and between Acer Therapeutics Inc. and Jefferson E. Davis.
10.23	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).
10.24	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).

Exhibit No.	Description
10.25	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).
10.26+	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).
10.27	Amended and Restated Sales Agreement, dated March 18, 2020, by and among Acer Therapeutics Inc., Roth Capital Partners, LLC, and Jones Trading Institutional Services LLC (incorporated by reference to Exhibit 10.27 to the Company's Annual Report on Form 10-K for the year ended December 31, 2019).
10.28	Purchase Agreement, dated April 30, 2020, by and between Acer Therapeutics Inc. and Lincoln Park Capital Fund, LLC (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K filed on April 30, 2020).
10.29	Registration Rights Agreement, dated April 30, 2020, by and between Acer Therapeutics Inc. and Lincoln Park Capital Fund, LLC (incorporated by reference to Exhibit 10.2 to the Company's Current Report on Form 8-K filed on April 30, 2020).
10.30	Research Collaboration Agreement, dated March 26, 2020, by and between Acer Therapeutics Inc. and the National Center for Advancing Translational Sciences (incorporated by reference to Exhibit 10.30 to the Company's Registration Statement on Form S-1, as amended (File No. 333-238192) filed on June 1, 2020).
10.31	Option Agreement, dated January 25, 2021, by and between Acer Therapeutics Inc. and Relief Therapeutics Holding AG (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K filed on January 25, 2021).
10.32	Promissory Note, dated January 25, 2021, issued by Acer Therapeutics Inc. in favor of Relief Therapeutics Holding AG (incorporated by reference to Exhibit 10.2 to the Company's Current Report on Form 8-K filed on January 25, 2021).
10.33	Security Agreement, dated January 25, 2021, by and between Acer Therapeutics Inc. and Relief Therapeutics Holding AG (incorporated by reference to Exhibit 10.3 to the Company's Current Report on Form 8-K filed on January 25, 2021).
23.1*	Consent of Independent Registered Public Accounting Firm BDO USA, LLP.
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.

Exhibit No.	Description
101*	Financial statements from the Annual Report on Form 10-K of the Company as of and for the period ended December 31, 2020, formatted in Extensible Business Reporting Language (XBRL): (i) Balance Sheets; (ii) Statements of Operations; (iii) Statements of Changes in Redeemable Preferred Stock and Stockholders' Equity; (iv) Statements of Cash Flows; and (v) Notes to Financial Statements.

* Filed herewith.

\+ Management contract or compensatory plan or arrangement.

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

♦♦ Portions of the exhibit have been omitted for confidential treatment.

^ 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 1, 2021

By: /s/ Harry S. Palmin
Harry S. 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 S. 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 1, 2021
/s/ Harry S. Palmin Harry S. Palmin	Chief Operating Officer and Chief Financial Officer *(Principal Financial and Accounting Officer)*	March 1, 2021
/s/ Jason Amello Jason Amello	Director	March 1, 2021
/s/ Stephen J. Aselage Stephen J. Aselage	Chairman of the Board	March 1, 2021
/s/ John M. Dunn John M. Dunn	Director	March 1, 2021
/s/ Michelle Griffin Michelle Griffin	Director	March 1, 2021

ACER THERAPEUTICS INC.
2018 STOCK INCENTIVE PLAN
NOTICE OF STOCK OPTION GRANT

You have been granted the following Option (this "Option" or this "Award") to purchase shares of Common Stock ("Stock") of Acer Therapeutics Inc (the "Company") under the Acer Therapeutics Inc. 2018 Stock Incentive Plan (as may be amended from time to time, the "Plan"):

Participant:	[Name of Optionee]
ID:	[Optionee Identification Number]
Award Number:	[Award Number]
Type of Option:	[Incentive Stock Option or Nonstatutory Stock Option]
Date of Grant:	[Date of Grant]
Vesting Commencement Date:	[Vesting Commencement Date]
Number of Shares:	[Number of Shares]
Exercise Price Per Share:	$[Exercise Price]
Vesting Acceleration:	The Shares will become fully vested immediately prior to a "Change in Control," as described in the Stock Option Agreement.
Expiration Date:	[Expiration Date] This Option expires earlier if your Service terminates earlier, as described in the Stock Option Agreement.
Vesting Schedule:	The Shares subject to this Option become exercisable over a four-year period, with 25% vesting on the one-year anniversary of the Vesting Commencement Date and the remaining 75% vesting in equal increments quarterly thereafter (in arrears) over the remaining three years, subject to continuous Service from the Vesting Commencement Date.

By your electronic signature and the electronic signature of the Company's representative below, you and the Company agree that this Option is granted under and governed by the term and conditions of the Plan and the Stock Option Agreement (this "*Agreement*"), both of which are attached to and made a part of this document.

By your electronic signature, you further agree that the Company may deliver by e-mail all documents relating to the Plan or this Award (including without limitation, prospectuses required by the Securities and Exchange Commission) and all other documents that the Company is required to deliver to its security holders (including without limitation, annual reports and proxy statements). You also agree that the Company may deliver these documents by posting them on a website maintained by the Company or by a third party under contract with the Company. If the Company posts these documents on a website, it will notify you by e-mail. By your electronic signature, you

agree to the following: "This electronic contract contains my electronic signature, which I have executed with the intent to sign this Agreement."

ACER THERAPEUTICS INC.

By: _____
Name: _____
Title: _____

ACER THERAPEUTICS INC.
2018 STOCK INCENTIVE PLAN
STOCK OPTION AGREEMENT

The Plan and Other Agreements

The Option that you are receiving is granted pursuant and subject in all respects to the applicable provisions of the Plan, which is incorporated herein by reference. Capitalized terms not defined in this Agreement will have the meanings ascribed to them in the Plan.

The attached Notice, this Agreement and the Plan constitute the entire understanding between you and the Company regarding this Award. Any prior agreements, commitments or negotiations concerning this Option are superseded. This Agreement may be amended by the Committee without your consent; however, if any such amendment would materially impair your rights or obligations under this Agreement, this Agreement may be amended only by another written agreement, signed by you and the Company.

Tax Treatment

This Option is intended to be an incentive stock option under Section 422 of the Code or a nonstatutory option, as provided in the Notice of Stock Option Grant. Even if this Option is designated as an incentive stock option, it wi be deemed to be a nonstatutory option to the extent required by the $100,000 annual limitation under Section 422(d) of the Code.

Vesting

This Option becomes exercisable in installments, as shown in the Notice of Stock Option Grant. The Shares w become fully vested immediately prior to a "Change in Control" as defined in the Plan. This Option will in no even become exercisable for additional Shares after your Service as an Employee or a Consultant has terminated for any reason.

Term

This Option expires in any event at the close of business at Company headquarters on the tenth (10th) anniversary of the Grant Date, as shown on the Notice of Stock Option Grant (fifth (5th) anniversary for a more than te percent (10%) shareholder as provided under the Plan if this is an incentive stock option). This Option may expire earlier if your Service terminates, as described below.

Regular Termination

If your Service terminates for any reason except due to your death or Disability, then this Option will expire at the close of business at Company headquarters on the date three (3) months after the date your Service terminates (or, if earlier, the Expiration Date). The Company determines when your Service terminates for this purpose and al purposes under the Plan and its determinations are conclusive and binding on all persons.

Death

If your Service terminates because of your death, then this Option will expire at the close of business at Company headquarters on the date twelve (12) months after the date your Service terminates (or, if earlier, the Expiration Date). During that period of up to twelve (12) months, your estate or heirs may exercise this Option.

Disability

If your Service terminates because of your Disability, then this Option will expire at the close of business a Company headquarters on the date twelve (12) months after the date your Service terminates (or, if earlier, the Expiration Date).

Leaves of Absence

For purposes of this Option, your Service does not terminate when you go on a military leave, a sick leave o another *bona fide* leave of absence, if the leave of absence was approved by the Company in writing and i continued crediting of Service is required by the terms of the leave or by applicable law. But your Service terminates when the approved leave ends, unless you immediately return to active work.

If you go on a leave of absence, then the vesting schedule specified in the Notice of Stock Option Grant may b adjusted in accordance with the Company's leave of absence policy or the terms of your leave. If you commence working on a part-time basis, then the vesting schedule specified in the Notice of Stock Option Grant may b adjusted in accordance with the Company's part-time work policy or the terms of an agreement between you and the Company pertaining to your part-time schedule.

Restrictions on Exercise

The Company will not permit you to exercise this Option if the issuance of Shares at that time would violate any law or regulation. The inability of the Company to obtain approval from any regulatory body having authority deemed by the Company to be necessary to the lawful issuance and sale of the Stock pursuant to this Option will relieve the Company of any liability with respect to the non-issuance or sale of the Stock as to which such approval will not have been obtained.

Notice of Exercise

When you wish to exercise this Option you must provide a written or electronic notice of exercise form (substantially in the form attached to this Agreement as Exhibit A) in accordance with such procedures as are established by the Company and communicated to you from time to time. Any notice of exercise must specify how many Shares you wish to purchase and how your Shares should be registered. The notice of exercise will be effective when it is received by the Company. If someone else wants to exercise this Option after your death, that person must prove to the Company's satisfaction that he or she is entitled to do so.

Form of Payment

When you submit your notice of exercise, you must include payment of the Option exercise price for the Shares you are purchasing. Payment may be made in the following form(s):

- Your personal check, a cashier's check, a money order or a wire transfer.
- Certificates for Shares that you own, along with any forms needed to effect a transfer of those Shares to the Company. The value of the Shares, determined as of the effective date of the Option exercise, will be applied to the Option exercise price. Instead of surrendering Shares, you may attest to the ownership of those Shares on a form provided by the Company and have the same number of Shares subtracted from the Shares issued to you upon exercise of this Option. However, you may not surrender or attest to the ownership of Shares in payment of the exercise price if your action would cause the Company to recognize a compensation expense (or additional compensation expense) with respect to this Option for financial reporting purposes.
- By delivery on a form approved by the Company of an irrevocable direction to a securities broker approved by the Company to sell all or part of the Shares that are issued to you when you exercise this Option and to deliver to the Company from the sale proceeds an amount sufficient to pay the Option exercise price and any withholding taxes. The balance of the sale proceeds, if any, will be delivered to you. The directions must be given by providing a notice of exercise form approved by the Company.
- By delivery on a form approved by the Company of an irrevocable direction to a securities broker or lender approved by the Company to pledge Shares that are issued to you when you exercise this Option as security for a loan and to deliver to the Company from the loan proceeds an amount sufficient to pay the Option exercise price and any withholding taxes. The directions must be given by providing a notice of exercise form approved by the Company.
- If permitted by the Committee, by a "*net exercise*" arrangement pursuant to which the number of Shares issuable upon exercise of the Option will be reduced by the largest whole number of Shares having an aggregate Fair Market Value that does not exceed the aggregate exercise price (plus tax withholdings, if applicable) and any remaining balance of the aggregate exercise price (and/or applicable tax withholdings) not satisfied by such reduction in the number of whole Shares to be issued will be paid by you in cash other form of payment permitted under this Option. The directions must be given by providing a notice of exercise form approved by the Company.
- Any other form permitted by the Committee in its sole discretion.

Notwithstanding the foregoing, payment may not be made in any form that is unlawful, as determined by the Committee in its sole discretion.

Withholding Taxes and Stock Withholding

Regardless of any action the Company and/or the Subsidiary or Affiliate employing you ("*Employer*") takes with respect to any or all income tax, social insurance, payroll tax, payment on account or other tax-related withholding ("*Tax-Related Items*"), you acknowledge that the ultimate liability for all Tax-Related Items legally due by you is and remains your responsibility and that the Company and/or your Employer (1) make no representations or undertakings regarding the treatment of any Tax-Related Items in connection with any aspect of this Option grant, including the grant, vesting or exercise of this Option, the subsequent sale of Shares acquired pursuant to such exercise and the receipt of any dividends; and (2) do not commit to structure the terms of the grant or any aspect of this Option to reduce or eliminate your liability for Tax-Related Items.

Prior to exercise of this Option, you will pay or make adequate arrangements satisfactory to the Company and/or your Employer to satisfy all withholding and payment on account of obligations of the Company and/or your Employer. In this regard, you authorize the Company and/or your Employer to withhold all applicable Tax-Related Items legally payable by you from your wages or other cash compensation paid to you by the Company and/or your Employer. With the Company's consent, these arrangements may also include, if permissible under local law, (a) withholding Shares that otherwise would be issued to you when you exercise this Option, provided that the Company only withholds the amount of Shares necessary to satisfy the maximum legally required tax withholding, (b) having the Company withhold taxes from the proceeds of the sale of the Shares, either through a voluntary sale or through a mandatory sale arranged by the Company (on your behalf pursuant to this authorization), or (c) any other arrangement approved by the Company. The Fair Market Value of the Shares, determined as of the effective date of the Option exercise, will be applied as a credit against the withholding taxes. Finally, you will pay to the Company or your Employer any amount of Tax-Related Items that the Company or your Employer may be required to withhold as a result of your participation in the Plan or your purchase of Shares that cannot be satisfied by the means previously described. The Company may refuse to honor the exercise and refuse to deliver the Shares if you fail to comply with your obligations in connection with the Tax-Related Items as described in this section.

Restrictions on Resale

You agree not to sell any Shares at a time when applicable laws, Company policies or an agreement between the Company and its underwriters prohibit a sale. This restriction will apply as long as your Service continues and for such period of time after the termination of your Service as the Company may specify.

Transfer of Option

In general, only you can exercise this Option prior to your death. You may not sell, transfer, assign, pledge or otherwise dispose of this Option, other than as designated by you by will or by the laws of descent and distribution, except as provided below. For instance, you may not use this Option as security for a loan. If you attempt to do any of these things, this Option will immediately become invalid. You may in any event dispose of this Option in your will. Regardless of any marital property settlement agreement, the Company is not obligated to honor a notice of exercise from your former spouse, nor is the Company obligated to recognize your former spouse's interest in this Option in any other way.

However, if this Option is designated as a nonstatutory stock option in the Notice of Stock Option Grant, then the Committee may, in its sole discretion, allow you to transfer this Option as a gift to one or more family members. For purposes of this Agreement, "*family member*" means a child, stepchild, grandchild, parent, stepparent, grandparent, spouse, former spouse, sibling, niece, nephew, mother-in-law, father-in-law, son-in-law, daughter-in-law, brother-in-law, or sister-in-law (including adoptive relationships), any individual sharing your household (other than a tenant or employee), a trust in which one or more of these individuals have more than fifty percent (50%) of the beneficial interest, a foundation in which you or one or more of these persons control the management of assets, and any entity in which you or one or more of these persons own more than fifty percent (50%) of the voting interest.

In addition, if this Option is designated as a nonstatutory stock option in the Notice of Stock Option Grant, then the Committee may, in its sole discretion, allow you to transfer this Option to your spouse or former spouse pursuant to a domestic relations order in settlement of marital property rights.

The Committee will allow you to transfer this Option only if both you and the transferee(s) execute the forms prescribed by the Committee, which include the consent of the transferee(s) to be bound by this Agreement.

Retention Rights	Neither this Option nor this Agreement gives you the right to be employed or retained by the Company or any Subsidiary or Affiliate of the Company in any capacity. The Company and its Subsidiaries and Affiliates reserve the right to terminate your Service at any time, with or without cause.
Shareholder Rights	This Option carries neither voting rights nor rights to dividends. You, or your estate or heirs, have no rights as a shareholder of the Company unless and until you have exercised this Option by giving the required notice to the Company and paying the exercise price. No adjustments will be made for dividends or other rights if the applicable record date occurs before you exercise this Option, except as described in the Plan.
Adjustments	The number of Shares covered by this Option and the exercise price per Share will be subject to adjustment in the event of a stock split, a stock dividend or a similar change in Company Shares, and in other circumstances, as set forth in the Plan. The forfeiture provisions and restrictions described above will apply to all new, substitute or additional stock options or securities to which you are entitled by reason of this Award.

Successors and Assigns

Except as otherwise provided in the Plan or this Agreement, every term of this Agreement will be binding upon and inure to the benefit of the parties hereto and their respective heirs, legatees, legal representatives, successors, transferees and assigns.

Notice

Any notice required or permitted under this Agreement will be given in writing and will be deemed effectively given upon the earliest of personal delivery, receipt or the third (3rd) full day following mailing with postage and fees prepaid, addressed to the other party hereto at the address last known in the Company's records or at such other address as such party may designate by ten (10) days' advance written notice to the other party hereto.

Section 409A of the Code

To the extent this Agreement is subject to, and not exempt from, Section 409A of the Code, this Agreement is intended to comply with Section 409A, and its provisions will be interpreted in a manner consistent with such intent. You acknowledge and agree that changes may be made to this Agreement to avoid adverse tax consequences to you under Section 409A.

Applicable Law and Choice of Venue

This Agreement will be interpreted and enforced under the laws of the State of Delaware, without regard to the laws of any other jurisdiction that might be applied because of the conflicts of laws principles of any state.

For purposes of litigating any dispute that arises directly or indirectly from the relationship of the parties evidenced by this Award or this Agreement, the parties hereby submit to and consent to the exclusive jurisdiction of the State of Texas and agree that such litigation will be conducted only in the courts of Montgomery County, Texas, or the federal courts for that district, and no other courts, where this grant is made and/or to be performed.

Miscellaneous

You understand and acknowledge that (1) the Plan is entirely discretionary, (2) the Company and your Employer have reserved the right to amend, suspend or terminate the Plan at any time, (3) the grant of this Option does not in any way create any contractual or other right to receive additional grants of options (or benefits in lieu of options) at any time or in any amount and (4) all determinations with respect to any additional grants, including (without limitation) the times when options will be granted, the number of Shares subject to awards, the exercise price and the vesting schedule, will be at the sole discretion of the Company.

The value of this Option will be an extraordinary item of compensation outside the scope of your employment contract, if any, and will not be considered a part of your normal or expected compensation for purposes of calculating severance, resignation, redundancy or end-of-service payments, bonuses, long-service awards, pension or retirement benefits or similar payments.

You understand and acknowledge that participation in the Plan ceases upon termination of your Service for any reason, except as may explicitly be provided otherwise in the Plan or this Agreement.

You hereby authorize and direct your Employer to disclose to the Company or any Subsidiary or Affiliate any information regarding your employment, the nature and amount of your compensation and the fact and conditions of your participation in the Plan, as your Employer deems necessary or appropriate to facilitate the administration of the Plan.

You consent to the collection, use and transfer of personal data as described in this subsection. You understand and acknowledge that the Company, your Employer and the Company's other Subsidiaries and Affiliates hold certain personal information regarding you for the purpose of managing and administering the Plan, including (without limitation) your name, home address, telephone number, date of birth, social insurance or other government identification number, salary, nationality, job title, any Shares or directorships held in the Company and details of all options or any other entitlements to Shares awarded, canceled, exercised, vested, unvested or outstanding in your favor (the "*Data*"). You further understand and acknowledge that the Company, its Subsidiaries and/or its Affiliates will transfer Data among themselves as necessary for the purpose of implementation, administration and management of your participation in the Plan and that the Company and/or any Subsidiary may each further transfer Data to any third party assisting the Company in the implementation, administration and management of the Plan. You understand and acknowledge that the recipients of Data may be located in the United States or elsewhere, and that the laws of a recipient's country of operation (e.g., the United States) may not have equivalent privacy protections as local laws where you reside or work. You authorize such recipients to receive, possess, use, retain and transfer Data, in electronic or other form, for the purpose of administering your participation in the Plan, including a transfer to any broker or other third party with whom you elect to deposit Shares acquired under the Plan of such Data as may be required for the administration of the Plan and/or the subsequent holding of Shares on your behalf. You may, at any time, view the Data, require any necessary modifications of Data, make inquiries about the treatment of Data or withdraw the consents set forth in this subsection by contacting the Human Resources Department of the Company in writing.

BY ELECTRONICALLY SIGNING THE COVER SHEET OF THIS AGREEMENT, YOU AGREE TO ALL OF THE TERM CONDITIONS DESCRIBED ABOVE AND IN THE PLAN.

ACER THERAPEUTICS INC.
2018 STOCK INCENTIVE PLAN
NOTICE OF EXERCISE OF STOCK OPTION

OPTIONEE INFORMATION:

Name: _____
Social Security Number: _____
Employee Number: _____
Address: _____

OPTION INFORMATION:

Grant Date:

Exercise Price per Share:	**$**
Total Number of Shares of Acer Therapeutics Inc. (the "*Company*") Covered by Option:	
Type of Stock Option:	☐ Nonstatutory (NSO)
	☐ Incentive (ISO)
Number of Shares of the Company for which Option is Being Exercised Now:	("*Purchased Shares*")
Total Exercise Price for the Purchased Shares:	$
Form of Payment:	☐ Cash or Check for $ payable to "Acer Therapeutics Inc."
	☐ Cashless exercise
	☐ Net exercise
Name(s) in which the Purchased Shares should be Registered:	
The Certificate for the Purchased Shares (if any) should be sent to the Following Address:	

ACKNOWLEDGMENTS:

1. I understand that all sales of Purchased Shares are subject to compliance with the Company's policy on securities trades.

2. I hereby acknowledge that I received and read a copy of the prospectus describing the Acer Therapeutics Inc. 2018 Stock Incentive Plan and the ta consequences of an exercise.

3. In the case of a nonstatutory option, I understand that I must recognize ordinary income equal to the spread between the fair market value of the Purchased Shares on the date of exercise and the exercise price. I further understand that I am required to pay withholding taxes at the time of exercising a nonstatutory option.

4. In the case of an incentive stock option, I agree to notify the Company if I dispose of the Purchased Shares before I have met both of the tax holding period applicable to incentive stock options (that is,

if I dispose of the Purchased Shares prior to the date that is two (2) years after the Grant Date and one (1) year after the date the option was exercised).

SIGNATURE AND DATE:

_____ , 20

Exhibit 10.22

EMPLOYMENT AGREEMENT

This Employment Agreement (this "*Agreement*") is made and entered into effective as of February 1, 2021, by and between Acer Therapeutics Inc., a Delaware corporation (the "*Company*"), and Jefferson E. Davis (the "*Key Employee*"). The Company and Key Employee are hereinafter collectively referred to as the "*Parties*."

W I T N E S S E T H:

A. The Company desires assurance of the association and services of Key Employee in order to retain Key Employee's skills, abilities background and knowledge, and is willing to engage Key Employee's services on the terms and conditions set forth in this Agreement.

B. Key Employee desires to be in the employ of the Company and is willing to accept such employment on the terms and conditions set forth in this Agreement.

NOW, THEREFORE, for and in consideration of the employment by the Company, the compensation and other remuneration paid and to be paid by the Company and received and to be received by Key Employee for such employment, and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged by Key Employee, it is agreed by and between the Parties hereto as follows:

1. Duties. As the Company's Chief Business Officer, Key Employee will perform such duties as are ordinary, customary and necessary in Key Employee's role. Key Employee will report directly to the Chief Executive Officer who will be primarily responsible for evaluating Key Employee's performance. The Company may change Key Employee's duties, compensation, benefits and place of employment from time to time as it deems necessary. In addition, during Key Employee's employment with the Company, Key Employee shall devote Key Employee's best efforts and Key Employee's full business time, skill and attention to the performance of Key Employee's duties on behalf of the Company.

2. Salary and Bonus. Key Employee will be compensated for full-time service (pro-rated for any part-time service) at a base rate of $340,000 per year, less all deductions and withholdings, to be paid in accordance with the Company's standard payroll practices, as they may be changed from time to time. In addition, Key Employee shall be eligible to receive an annual discretionary bonus, starting with 2021, with a target (the "*Target Bonus*") of forty percent (40%) of Key Employee's base salary per 12-month period (pro-rated for any partial period of less than 12 months), based upon a determination by the CEO and, where applicable, the Company's Board of Directors (the "*Board*") of the achievement of objectives to be set from time to time by the Board, provided that Key Employee must remain employed through the payment date in order to earn the bonus. The measurement period for this purpose will end on approximately December 31 of each year. The annual discretionary bonus, if otherwise earned subject to continued employment through the payment date, will be paid as soon as practicable after the achievement of objectives for the measurement period has been determined, but in no event will such bonus be paid after March 15 following the last day of the measurement period. The Company may modify Key Employee's compensation and benefits from time to time at its sole discretion.

3. Other Benefits. The Company will provide Key Employee with participation in Company-sponsored employee benefits programs on the same basis as such benefits are generally available to its employees, as determined from time to time by the Board. The Company may, from time to time, change, amend, add to, or terminate these benefits at any time in its sole discretion.

4. Employee Nondisclosure and Developments Agreement. As a condition to and in consideration for employment, Key Employee has previously entered into the Employee Nondisclosure and Developments Agreement (the "*NDA*") in the form used by the Company, which remains in full force and effect.

5. At-Will Employment. Key Employee's employment with Company is "at-will." This means that either Key Employee or the Company may terminate Key Employee's employment at any time, with or without cause, and with or without notice. Any contrary representations or agreements which may have been made to Key Employee are superseded by this Agreement. The "at-will" nature of Key Employee's employment described in this Agreement shall constitute the entire agreement between Key Employee and the Company concerning the nature and duration of Key Employee's employment. Though Key Employee's duties, compensation, benefits and place of employment may change over time and Key Employee may be subject to incremental discipline that does not include a termination, none of these events change the agreement that Key Employee is an "at-will" employee. In addition, the fact that the rate of Key Employee's salary or other compensation is stated in units of years or months, and that Key Employee's vacation and sick leave accrue annually or monthly, does not alter the at-will nature of the employment, and does not mean and should not be interpreted to mean that Key Employee is guaranteed employment to the end of any period of time or for any period of time. The "at-will" term of Key Employee's employment with the Company can only be changed in a writing signed by Key Employee and an authorized officer of the Company.

6. Severance Payment. Without limiting the provisions of the foregoing Section, assuming Key Employee's employment with the Company shall have been continuous from Key Employee's start date through the occurrence of the applicable event, and provided Key Employee executes and delivers to the Company, within twenty-one (21) days (or, to the extent required by law, forty-five (45) days) following the termination date (with any revocation periods having expired without any revocations by Key Employee), a separation agreement that includes a general release of claims against the Company and persons affiliated with the Company substantially in the form attached hereto as Exhibit A (the "Release"), then:

a. In the event of any Termination without Cause (as defined below) or any Constructive Termination (as defined below) which occurs during the period commencing one (1) month prior to a Change in Control (as defined below) and terminating twelve (12) months after such Change in Control (the "*Change in Control Period*"), Key Employee will be entitled to the following:

i. a lump sum payment (paid on the sixtieth (60th) day following such termination of employment) equal to the sum of (A) Key Employee's then current base salary rate calculated for a period of twelve (12) months and (B) one (1) times the Target Bonus calculated for a period of twelve (12) months (*i.e.,* no proration); and

ii. if Key Employee elects to continue Key Employee's health insurance coverage under COBRA, then the Company will reimburse Key Employee for the same portion of Key Employee's monthly premiums over such twelve (12) month period under COBRA (or, if applicable, such lesser period as is available to Key Employee under COBRA) as the Company is then paying (relative to health insurancecoverage) for active employees; and

b. In the event of any Termination without Cause or any Constructive Termination which occurs outside of a Change in Control Period, Key Employee will be entitled to the following:

i. a lump sum payment (paid on the sixtieth (60th) day following such termination of employment) equal to Key Employee's then current base salary rate calculated for a period of twelve (12) months; and

ii. if Key Employee elects to continue Key Employee's health insurance coverage under COBRA, then the Company will reimburse Key Employee for the same portion of Key Employee's monthly premiums over such twelve (12) month period under COBRA as the Company is then paying (relative to health insurance coverage) for active employees; and

iii. the vesting arrangements with respect to any equity-based compensation (*e.g.*, any stock options and any shares of restricted stock) other than any equity-based incentive awards that are earned based upon achievement of performance measures during a performance period (which shall remain subject to the terms of the applicable award agreement), will thereupon accelerate such that Key Employee will be vested in an additional twelve (12) months' worth of vesting beyond the date of such Termination without Cause or Constructive Termination, with the Company's standard post-termination exercise period as set forth in such equity award.

The following definitions shall apply for purposes of this Section:

- *A "Change in Control"* has the meaning set forth in the Company's 2018 Stock Incentive Plan.

- "*Constructive Termination*" means Key Employee's election in a written notice to the Company to terminate any employment relationship where such notice is delivered within ninety (90) days after any of the following: (i) a material reduction in Key Employee's level of duties or responsibilities or the nature of Key Employee's functions; (ii) a material reduction in Key Employee's base salary or potential total cash compensation (consisting of base salary and target bonus); (iii) a relocation of Key Employee's principal place of employment by more than fifty (50) miles, if the new location is both (A) more than fifty (50) miles from Key Employee's principal residence and (B) farther from Key Employee's principal residence than Key Employee's principal place of employment immediately before such relocation; or (iv) any material breach of Key Employee's employment agreement by the Company; provided, that in all cases such action is not cured within thirty (30) days following written notice and, if the Company has not cured such action within the cure period, termination of employment occurs within thirty (30) days after the end of such cure period.

- "*Termination without Cause*" means the termination by the Company of any employment relationship with Key Employee for any reason other than (i) commission by Key Employee of any act of fraud or embezzlement with regard to the Company or one or more of its parent or subsidiary corporations; (ii) any material, intentional and unauthorized use or disclosure of material confidential information or trade secrets of the Company or one or more of its parent or subsidiary corporations by Key Employee (other than in the good-faith performance of Key Employee's duties); (iii) a violation of the NDA or any other agreement entered into with the Company;(iv) any other intentional misconduct by Key Employee with regard to the Company or one or more of its parent or subsidiary corporations (including severe absenteeism other than as a result of physical or mental incapacity) which adversely affects the business or affairs of the Company or one or more of its parent or subsidiary corporations in a material manner; or (v) Key Employee's failure to attempt in good faith to either perform duties consistent with Key Employee's position with the Company or one or more of its parent or subsidiary corporations or to follow the reasonable requests of the Company's Board, so long as Key Employee has been provided with an opportunity for a period of at least ten (10) business days following written notice to Key Employee to cure such failure (provided such conduct constituting Cause is capable of cure) and Key Employee fails to so cure, each as reasonably determined by the Company; provided, however, that clause (v) shall no longer apply following a Change in Control.

7. Separation for Any Other Reason. In the event that Key Employee's employment is terminated in any instance not addressed by Section 6 (including, without limitation, a termination by the Company other than a Termination without Cause, a resignation by Key Employee other than for a Constructive Termination, in the event of Key Employee's death, or in the event Key Employee is unable to perform the essential functions of Key Employee's job position, with or without accommodation, due to mental or physical disability), Key Employee shall not be entitled to any compensation or any other sum (other than accrued but unpaid base salary, accrued vacation pay and such other benefits if any as may be required by applicable law).

8. Code Section 409A. The intent of the Parties is that payments and benefits under this Agreement and any equity-based compensation (*e.g.*, any stock options and any shares of restricted stock) comply with, or be exempt from, Section 409A of the Internal Revenue Code (the "*Code*") and, accordingly, to the maximum extent permitted, this Agreement and any equity-based compensation shall be interpreted to be in compliance therewith or exempt therefrom. If Key Employee notifies the Company (with specificity as to the reason therefor) that Key Employee believes that any provision of this Agreement or any equity-based compensation (or of any award of compensation) would cause Key Employee to incur any additional tax or interest under Code Section 409A and the Company concurs with such belief or the Company independently makes such determination, the Company shall, after consulting with Key Employee, reform such provision to try to comply with Code Section 409A through good-faith modifications to the minimum extent reasonably appropriate to conform with Code Section 409A. To the extent that any provision hereof is modified in order to comply with Code Section 409A, such modification shall be made in good-faith and shall, to the maximum extent reasonably possible, maintain the original intent and economic benefit to Key Employee and the Company of the applicable provision without violating the provisions of Code Section 409A.

a. A termination of employment shall not be deemed to have occurred for purposes of any provision of this Agreement providing for the payment of any amounts or benefits upon or following a termination of employment that are considered "nonqualified deferred compensation" under Code Section 409A unless such termination is also a "separation from service" within the meaning of Code Section 409A and, for purposes of any such provision of this Agreement, references to a "termination," "termination of employment" or like terms shall mean "separation from service." If Key Employee is deemed on the date of termination to be a "specified employee" within the meaning of that term under Code Section 409A(a)(2)(B), then with regard to any payment that is considered nonqualified deferred compensation under Code Section 409A payable on account of a "separation from service," such payment or benefit shall be made or provided at the date which is the earlier of (A) the expiration of the six (6)-month period measured from the date of Key Employee's "separation from service" and (B) the date of Key Employee's death (the "*Delay Period*"). Upon the expiration of the Delay Period, all payments and benefits delayed pursuant to this Section 8 (whether they would have otherwise been payable in a single sum or in installments in the absence of such delay) shall be paid or reimbursed to Key Employee in a lump sum with interest at the prime rate as published in The Wall Street Journal on the first business day following the end of the Delay Period, and any remaining payments and benefits due under this letter shall be paid or provided in accordance with the normal payment dates specified for them herein.

b. With regard to any provision herein that provides for reimbursement of costs and expenses or in-kind benefits, except as permitted by Code Section 409A, (i) the right to reimbursement or in-kind benefits shall not be subject to liquidation or exchange for another benefit, (ii) the amount of expenses eligible for reimbursement, or in-kind benefits, provided during any taxable year shall not affect the expenses eligible for reimbursement, or in-kind benefits to be provided, in any other taxable year, provided that the foregoing clause (ii) shall not be violated with regard to expenses reimbursed under any arrangement covered by Section 105(b) of the Code solely because such expenses are subject to a limit related to the period the arrangement is in effect and (iii) such payments shall be made on or before the last day of Key Employee's taxable year following the taxable year in which the expense occurred.

c. For purposes of Code Section 409A, Key Employee's right to receive any installment payments pursuant to this Agreement shall be treated as a right to receive a series of separate and distinct payments. In no event may Key Employee, directly or indirectly, designate the calendar year of any payment to be made under this Agreement that is considered nonqualified deferred compensation.

9. Excess Parachute Payments and Limitations If any payment or distribution made to Key Employee in the nature of compensation (within the meaning of Section 280G(b)(2) of the Code) (a "*Payment*") or portion thereof would constitute a "parachute payment" within the meaning of Section 280G of the Code and, but for this sentence, would be subject to the excise tax imposed under Section 4999 of the Code (the "*Excise Tax*") (all such Payments (or portions thereof) being hereinafter referred to as the "*Total Payments*"), then such Total Payments shall be whichever of the following amounts, after taking into account all applicable federal, state and local employment taxes, income taxes and the Excise Tax, that results in Key Employee's receipt, on an after-tax basis, of the greater amount: (a) the net amount of the Total Payments that would result in no portion of the Total Payments being subject to the Excise Tax; or (b) the net amount of the

Total Payments without reduction (notwithstanding that all or some portion of the Total Payments may be subject to the Excise Tax). If a reduction in the Total Payments is necessary so that the Total Payments equal the amount described in clause (a) above, reduction shall occur in the following order: (i) the cancellation of acceleration of vesting of any equity awards for which the exercise price exceeds the then fair market value of the underlying equity (the "*GAP*") that have a ninety (90) day or less exercise period, starting with such equity awards with the largest amount of GAP, (ii) reduction of cash payments (in reverse order of the date otherwise due), (iii) reduction of employee benefits (in reverse order of the date otherwise due), and (iv) the cancellation of vesting of any equity awards not covered in clause (i) above, provided, that such cancellation will first apply to equity awards that are "fully valued" under Section 280G of the Code (including those subject to present value adjustments) and thereafter, to equity awards valued on an acceleration of vesting basis, and provided, further, within each category, the cancellation shall be in a manner as providing Key Employee with the highest net amount; provided, however, that to the extent permitted by Code Section 409A and Sections 280G and 4999 of the Code, if a different reduction procedure would be permitted without violating Code Section 409A or losing the benefit of the reduction under Sections 280G and 4999 of the Code, Key Employee may designate a different order of reduction. For purposes of determining whether and the extent to which the Total Payments will be subject to the Excise Tax, (x) no portion of the Total Payments the receipt or enjoyment of which Key Employee shall have waived at such time and in such manner as not to constitute a "payment" within the meaning of Section 280G(b) of the Code shall be taken into account; (y) no portion of the Total Payments shall be taken into account which, in the written opinion of Wolf & Company, P.C. or such accounting or consulting firm with particular expertise regarding excise taxes under Section 4999 of the Code selected by the Board in good faith prior to the applicable Change in Control (the "*Accounting Firm*"), does not constitute a "parachute payment" within the meaning of Section 280G(b)(2) of the Code (including by reason of Section 280G(b)(4)(A) of the Code) and, in calculating the Excise Tax, no portion of such Total Payments shall be taken into account which, in the opinion of the Accounting Firm, constitutes reasonable compensation for services actually rendered, within the meaning of Section 280G(b)(4)(B) of the Code, in excess of the "base amount" (as defined in Section 280G(b)(3) of the Code) allocable to such reasonable compensation; and (z) the value of any non-cash benefit or any deferred payment or benefit included in the Total Payments shall be determined by the Accounting Firm in accordance with the principles of Sections 280G(d)(3) and (4) of the Code.

10. **Miscellaneous.** Key Employee agrees to abide by all applicable laws and regulations and all Company policies and procedures as they are established. Violation of such laws, regulations, policies, procedures or the NDA may lead to immediate termination of employment. The terms of this Agreement and Key Employee's employment with the Company shall be governed in all aspects by the laws of the State of Delaware; provided, however, if Key Employee lives and works primarily in the State of California, the laws of the State of California shall govern. This Agreement may be executed in more than one counterpart, and signatures transmitted via facsimile or PDF shall be deemed equivalent to originals.

11. **Integrated Agreement.** This Agreement supersedes any prior agreements, representations or promises of any kind, whether written, oral, express or implied between the Parties with respect to the subject matters herein. Likewise, the terms of this Agreement and the NDA incorporated herein by reference shall constitute the full, complete and exclusive agreement between Key Employee and the Company with respect to the subject matters herein. This

Agreement may only be changed by a writing, signed by Key Employee and an authorized officer of the Company.

12. **Withholding.** Any payments or other compensation provided to Key Employee or for Key Employee's benefit will be subject to (and thus reduced by) all applicable deductions and withholdings.

13. **Severability.** If any term herein is held to be invalid, void or unenforceable, the remainder of the terms herein shall remain in full force and effect and shall in no way be affected, and the Parties shall use their best efforts to find an alternative way to achieve the same result.

14. **Successors.**

a. This Agreement is personal to Key Employee and, without the prior written consent of the Company, shall not be assignable by Key Employee otherwise than by will or the laws of descent and distribution. This Agreement shall inure to the benefit of and be enforceable by Key Employee's legal representatives.

b. This Agreement shall inure to the benefit of and be binding upon the Company and its successors and assigns. The Company will require any successor (whether direct or indirect, by purchase, merger, consolidation or otherwise) to all or substantially all of the business and/or assets of the Company to assume and agree to perform this Agreement in the same manner and to the same extent that the Company would be required to perform it if no such succession had taken place. As used in this Agreement, "Company" shall mean the Company as hereinbefore defined and any successor to its business and/or assets as aforesaid which assumes and agrees to perform this Agreement by operation of law, or otherwise.

15. **Amendment**. No amendment or other modification of this Agreement shall be effective unless made in writing and signed by the parties hereto.

IN WITNESS WHEREOF, the Parties have caused this Agreement to be duly executed as of the day and year first above written.

THE COMPANY:		**KEY EMPLOYEE:**	
ACER THERAPEUTICS INC.			
By:	/s/ Chris Schelling	By:	/s/ Jefferson E. Davis
	Chris Schelling CEO & Founder	Printed	Name: Jefferson E. Davis
Date:	2/3/2021	Date:	2/3/2021

Exhibit A

General Release of Claims

[attach]

Exhibit 23.1

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), S-1 (No. 333-238192) and Form S-8 (Nos. 333-237265, 333-230133, 333-224942, 333-221566) of Acer Therapeutics, Inc. of our report dated March 1, 2021, relating to the financial statements which appear in this Annual Report on 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 1, 2021

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 1, 2021

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 S. 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 1, 2021

By: /s/ Harry S. Palmin

Harry S. 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, 2020 (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 1, 2021

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, 2020 (the "Report"), as filed with the Securities and Exchange Commission on the date hereof, I, Harry S. 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 1, 2021

By: /s/ Harry S. Palmin

Harry S. Palmin
Chief Operating Officer and Chief Financial Officer
(Principal Financial and Accounting Officer)