Developing Therapeutics for the Treatment of Serious Rare Diseases with Significant Unmet Medical Needs

October 2017

NASDAQ: ACER
Forward-looking Statements

This presentation contains "forward-looking statements" that involve substantial risks and uncertainties for purposes of the safe harbor provided by the Private Securities Litigation Reform Act of 1995. All statements, other than statements of historical facts, included in this presentation regarding strategy, future operations, future financial position, future revenue, projected expenses, prospects, plans and objectives of management are forward-looking statements. Examples of such statements include, but are not limited to, statements relating to Acer’s listing on the Nasdaq Capital Market; expectations regarding the capitalization, resources and ownership structure of the company; the potential for EDSIVO™ (celiprolol) and ACER-001 to safely and effectively target diseases; the adequacy of the company’s capital to support its future operations and its ability to successfully initiate and complete clinical trials and regulatory submissions; the nature, strategy and focus of the company; and the development, expected timeline and commercial potential of any product candidates of the company. Acer may not actually achieve the plans, carry out the intentions or meet the expectations or projections disclosed in the forward-looking statements and you should not place undue reliance on these forward-looking statements. Such statements are based on management’s current expectations and involve risks and uncertainties. Actual results and performance could differ materially from those projected in the forward-looking statements as a result of many factors, including, without limitation, risks and uncertainties associated with the ability to project future cash utilization and reserves needed for contingent future liabilities and business operations, the availability of sufficient resources of the company to meet its business objectives and operational requirements, the fact that the results of earlier studies and trials may not be predictive of future clinical trial results, the protection and market exclusivity provided by Acer’s intellectual property, risks related to the drug development and the regulatory approval process and the impact of competitive products and technological changes. Acer disclaims any intent or obligation to update these forward-looking statements to reflect events or circumstances that exist after the date on which they were made. You should review additional disclosures we make in our filings with the Securities and Exchange Commission, including our Quarterly Reports on Form 10-Q and our Annual Report on Form 10-K. You may access these documents for no charge at http://www.sec.gov.
Acer is a pharmaceutical company that acquires, develops and commercializes therapies for serious rare and ultra-rare diseases with significant unmet medical needs.

- Headquartered: Cambridge, MA
- Headcount: 7
- Founded: December 2013
- Public: September 2017
- Cash: $10M gross proceeds
  - From RTO financing in mid-September 2017
  - Cash expected to be sufficient through expected NDA filing for EDSIVO™ in 1H 2018
Management Team

Management has extensive expertise in orphan development & commercialization

- **Chris Schelling**, CEO and Founder
  - 18 years of biotech/pharma strategic and orphan drug expertise

- **Will Andrews, M.D.,** Chief Medical Officer
  - 17 years in both Clinical Development and Medical Affairs in multiple therapeutic areas, with a focus and extensive experience in rare diseases. Significant medical marketplace and product launch experience, having served as the medical lead for six product launches. He received his undergraduate degree in Biology from Harvard University and his M.D. degree from Yale University School of Medicine

- **Harry Palmin**, Chief Financial Officer
  - 20+ years of corporate experience

- **Pamela Williamson**, Acting Head, Regulatory Affairs
  - 30 years of orphan drug regulatory and CMC expertise

- **Jefferson Davis**, Acting Chief Business Officer
  - 20+ years of drug discovery, business and corporate development expertise

- **Philip Sager, M.D.,** Strategic Clinical Consultant – EDSIVO™
  - A senior healthcare executive (>25 years of working experience) with strategic and operating experience; extensive U.S. and international regulatory leadership, including Chair of the FDA Cardio-Renal Advisory Committee and the PhARMA Topic Leader to the ICH E14 Regulatory Guidance
Investment Highlights

• Lead Program (EDSIVO™) is a New Chemical Entity for treatment of Vascular Ehlers-Danlos Syndrome (vEDS) with a robust data package, including survival endpoints and long-term registry data at time of potential launch

• ACER-001 for treatment of Urea Cycle Disorders (UCD) and Maple Syrup Urine Disease (MSUD) is a proprietary, taste-masked, immediate release formulation of FDA/EMA approved products that are approaching $200M in revenue in the U.S.

• Both assets have been granted U.S. orphan status, are unencumbered and target indications with significant unmet medical needs

• Multiple key regulatory catalysts expected over next approximately 24 months
  • NDA filing for EDSIVO™ expected in 1H’18
  • NDA filing for ACER-001 anticipated in 1H’19

• Expected to have sufficient capital through NDA filing of EDSIVO™

• Experienced management team with significant orphan drug development expertise
## Clinical Pipeline

<table>
<thead>
<tr>
<th>Program / Indication</th>
<th>Novel MOA / Unique Characteristics</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
<th>NDA</th>
<th>Market</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>EDSIVO™ (celiprolol)</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>vEDS (COL3A1+)</td>
<td>Improves hemodynamic stability; decreases vascular resistance</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>ACER-001 (reformulated sodium phenylbutyrate)</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>UCD</td>
<td>Comparable to Buphenyl; taste-masked</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>MSUD</td>
<td>Inhibition of BCKD kinase to increase BCAA metabolism</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
Autosomal dominant connective tissue disorder of collagen synthesis caused by mutations in the COL3A1 gene for type III procollagen

Characterized by arterial aneurysms, dissections and/or ruptures

Median survival is 51 years of age

Identified in over 2,000 patients in the U.S.

Celiprolol is a New Chemical Entity (NCE) in the U.S.

EDSIVO™ (celiprolol) showed statistically-significant improvement in event-free survival (EFS) compared to control in vEDS patients

Clinical benefits seen in vEDS are via novel, disease-modifying MOA

Currently pre-NDA for treatment of vEDS

Significant unmet medical need with no direct competition

Expected NDA filing in 1H 2018

Orphan pricing with robust program to support reimbursement & access

Launch in 50-100 vEDS Centers of Excellence

Vascular Ehlers-Danlos syndrome (vEDS)

EDSIVO™ Product Profile

The Opportunity
Vascular Ehlers-Danlos Syndrome (vEDS)

- Ehlers-Danlos Syndrome (EDS) are a group of hereditary disorders of connective tissue

- Vascular EDS (vEDS, EDS type IV) is the severe subtype:
  - Characterized by aneurysms, dissections, ruptures
    - Vascular
    - Gastrointestinal
    - Uterine
  - Diagnosed by clinical symptoms and confirmed by presence of mutations in the COL3A1 gene
  - Events occur in 25% of patients before the age of 20, but 90% by the age of 40
  - Median age of death is estimated to be 51 years

- No therapeutic options for vEDS
  - Current treatment is focused on surgical intervention

Fig 1. Spectrum of facial features in individuals with vascular Ehlers-Danlos syndrome (vEDS) shows variability among patients. These four individuals died of vascular complications.

Fig 3. Distribution of 132 vascular complications in 24 patients with a clinical diagnosis of Ehlers-Danlos syndrome type IV.
“In vascular EDS the body lacks sufficient type III collagen, a molecule that contributes to the strength of the skin, intestines, uterus, and most importantly, the blood vessels.

“People with vascular EDS live with the knowledge that they will die from this condition...

“They are also told that there are no effective treatments. There are no medications that are known to strengthen the tissues or delay blood vessel rupture. Attempts at surgical repair are often delayed; there is confidence that the patient will die within hours if nothing is tried. This is because the tissues are so weak that they often simply fall apart during surgery – akin to trying to sew together wet tissue paper.

“Of all the conditions that I care for, I hate this one the most. It not only drastically shortens the length of life, but also robs people of any meaningful sense of hope and quality of life – always anticipating that the shoe will drop at any moment.”

vEDS KOL
Geneticist
January 2010
EDSIVO™ Unique Mechanism of Action

EDSIVO™ is the only agent to show clinical benefit in patients with vEDS

- EDSIVO™ has a very unique pharmacological profile:
  - Selective β1 and α2 adrenergic receptor antagonist
  - β2 and β3 adrenergic receptor agonist,
  - Intrinsic sympathomimetic activity (ISA+)
  - Lacks non-specific membrane effects

- Void of blood pressure lowering in normotensive people
  - Most vEDS patients are normotensive, thus the protective effect of celiprolol was unlikely to be through blood pressure lowering (β1 antagonism)

- EDSIVO™ positive effects in vEDS patients are thought to be through:
  - Providing more stable hemodynamic conditions that lead to a less fragile arterial wall
  - Upregulation of collagen synthesis via TGF-β, thus strengthening the arterial wall and reducing its susceptibility to rupture
# EDSIVO™ Pivotal Clinical Trial

<table>
<thead>
<tr>
<th><strong>Design:</strong></th>
<th>Multicenter, prospective, randomized, open trial with blinded endpoint assessment</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Location:</strong></td>
<td>9 sites: eight in France, one in Belgium</td>
</tr>
<tr>
<td><strong>Eligibility:</strong></td>
<td>Inclusion criteria adapted Villefranche diagnostic criteria: 1 major criterion and 2 minor criteria, or 4 minor criteria needed for enrollment</td>
</tr>
<tr>
<td><strong>Enrollment:</strong></td>
<td>53 patients with clinical vascular Ehlers-Danlos syndrome randomly assigned to 5 years treatment with 1) celiprolol or 2) no treatment</td>
</tr>
<tr>
<td><strong>Demographics:</strong></td>
<td>Ages 15 to 65 (mean 35), female/male ratio 2:1. Important phenotype characteristics equally balanced between celiprolol and control</td>
</tr>
<tr>
<td><strong>Dosing:</strong></td>
<td>Celiprolol administered twice daily and up-titrated every 6 mos. by 100 mg/day to max. 400 mg/day</td>
</tr>
</tbody>
</table>
| **Endpoints:** | Primary: arterial events (rupture/dissection, +/-fatal)  
Secondary: intestinal or uterine rupture |
| **Duration:** | Mean duration of follow-up 47 months; trial stopped early for treatment benefit |
| **Funding / PI:** | French Ministry of Health; PI: Prof. Pierre Boutouyrie |

ClinicalTrials.gov, number NCT00190411  
Ong Lancet 2010; 376: 1476-84.
Results & Analysis:

- Trial stopped early for treatment benefit (mean follow-up 47 months)
- The primary endpoint (arterial dissection or rupture) affected 5 (20%) celiprolol patients and 14 (50%) controls (hazard ratio [HR] 0.36; p=0.04)
- Primary and secondary endpoints (intestinal or uterine rupture) affected 6 (24%) celiprolol patients and 17 (61%) controls (HR 0.31; p=0.01)
- Post-hoc analysis of 33 patients with confirmed COL3A1 mutation indicated equal benefit for the primary (HR 0.24; p=0.04) and secondary endpoints (HR 0.25; p=0.02)
- Author’s Comments: “We suggest that celiprolol might be the treatment of choice for physicians aiming to prevent major complications in patients with vEDS”

Figure 2: Kaplan-Meier curves of event-free survival in 53 patients with vascular Ehlers-Danlos
Primary endpoint (A). Primary and secondary endpoints (B).
<table>
<thead>
<tr>
<th>Paris (AP-HP) Registry</th>
</tr>
</thead>
<tbody>
<tr>
<td>• PIs: Michael Frank, Xavier Jeunemaitre</td>
</tr>
<tr>
<td>• 148 patients enrolled in registry</td>
</tr>
<tr>
<td>• 100% of patients in registry have COL3A1 gene mutation</td>
</tr>
<tr>
<td>• &gt;80% of patients on celiprolol</td>
</tr>
<tr>
<td>• Study Period: Jan. 2000 to Dec. ‘14 (for initial referral patients), followed until 2017 (for follow-up visits)</td>
</tr>
<tr>
<td>• Median Age at Diagnosis: 35</td>
</tr>
<tr>
<td>• Median Duration F/U: 5.55 years [3.25 - 8.55 years]; max F/U: 20 years</td>
</tr>
</tbody>
</table>

Data should be e-published in 2H’2017
Acer will reference data in NDA
### EDSIVO™ Data Package

- **Pivotal Clinical Trial**
  - n = 53 patients (62% COL3A1+)
  - Statistically-significant improvement in 1º endpoint EFS (p=0.04)

- **Paris Registry:**
  - n = 148 patients (100% COL3A1+)
  - >80% patients on celiprolol

- **Sanofi MHRA Dossier**
  - >13,000 pages (220 studies)
  - Pharmacology, PK/PD, repro/tox, clinical safety

- **Updated CMC Package**
  - Current Analytical Methods
  - U.S. Manufacturing Standards

---

**Targeting NDA submission in 1H 2018**
vEDS Market Sizing

- vEDS Patient Population for basis of market sizing = \textbf{4,169 patients}
  - 2 instances of the EDS Dx Code 756.83 separated by \(\geq 2\) months
  - Includes at least one other Dx or CPT code relevant to vEDS -AND-
  - Excludes hypermobility syndrome

The raw patient counts are consistent with the larger population areas
# Accelerate Diagnosis

## Broad Familial Genetic Testing Program

- According to HVH, there are ~4,000 to 5,000 patients with vEDS in the U.S.
- According to genetic labs, ~2,000 patients have had a genetic test that confirms COL3A1 status
- A broad genetic testing program for COL3A1 (autosomal dominant) could substantially increase the number of confirmed patients
- Current test costs: $1,000 - $5,000 / test and aren’t always reimbursed
- Opportunity to centralize, reduce cost: $500 / test (target)
- Provide patients genetic counseling

## Facial Recognition Program

- Patients with vEDS have relatively similar / identifiable facial features
- Want a quick, cost-effective test to potentially help screen for vEDS patients in the clinic
- Acer is looking to collaborate with a leading AI firm to establish a phenotype - genotype database
- Facial recognition software is in the majority of genetic clinics in the U.S.
- May lead to earlier and more accurate diagnosis

[https://www.genetests.org/genes/?gene=COL3A1](https://www.genetests.org/genes/?gene=COL3A1)
vEDS Patient and Payer Demographics

- There is a higher number of patients in the 18-34 age group which is consistent with literature (Pepin et al., 2000)
- Greater distribution of diagnosed females vs. males which is consistent with literature (Castori et al., 2010)
- Highest claim volume found in the commercial payer system

Note 1: Payer distribution represents raw counts and is not normalized to the US population
EDSIVO™ Proposed Commercial Strategy

**vEDS Centers of Excellence:**
- 50 COEs at launch
- Grow to 100 COEs in 3 years
- ~20-50 vEDS patients / COE

**Major Genetic Labs (COL3A1+):**
- UW: 1,250 pts
- CTGT/GeneDx/ARUP/Ambry
- BCM/Mayo/MGH/Emory

**Acer Support:**
- Primarily MSLs (n=5-10)
- Commercial Operations
• Precision Health Economics is developing a health economic (cost-consequence) model demonstrating the economic and clinical value of vEDS Centers of Excellence, and the potential impact of EDSIVO™ on patient outcomes

• These models will help quantify and communicate cost offsets associated with the COE model for hospital systems and payers (if needed)

• It will also help support orphan pricing for EDSIVO™ with key stakeholders
EDSIVO™ Market Opportunity

- If approved, EDSIVO™ will be the *only* FDA-approved therapy to treat vEDS patients

- 2,000 to 5,000 vEDS patients in the U.S.

- Orphan pricing well supported by initial payer research, with additional validation from HEOR models

- Provide a robust patient assistance program (PAP) to help offset costs (so there will be little/no incentive for vEDS patients in the U.S. to attempt to obtain celiprolol elsewhere)

- Granted U.S. Orphan Drug Designation for vEDS (January 2015)
  - 5 years NCE exclusivity
  - 7 years orphan exclusivity
  - Potential for +0.5 years pediatric exclusivity

- 50-100 Centers of Excellence proposed
  - Small, dedicated commercial group
ACER-001 Overview

**Urea Cycle Disorders (UCD)**
- A group of metabolic genetic diseases that lead to toxic build-up of NH4+
- Currently treated with Ravicti, Buphenyl, Ammonul, and a highly-restricted diet
- >2,000 patients with UCD in the U.S.; ~600 patients treated with sodium / glycerol phenylbutyrate

**Maple Syrup Urine Disease (MSUD)**
- A metabolic genetic disease that leads to toxic build-up of leucine and other branched-chain amino acids
- Currently managed with a highly-restricted diet; poor compliance
- Well-defined patient population with ~800 eligible patients in the U.S.

**ACER-001 Product Profile**
- A taste-masked, immediate release formulation of sodium phenylbutyrate
- First office action (USPTO) on formulation patent
- PK/BE study to show equivalence to sodium phenylbutyrate

**The Opportunity**
- Expected NDA filing in 1H’2019
- Issued U.S. / EU patents covering methods of use in MSUD
- Orphan drug designation in MSUD
- Advantageous orphan pricing with robust program to support reimbursement and patient access
ACER-001 Taste-Masked, Immediate Release

**Mouth → Stomach**

Excellent protection for several minutes at mouth pH followed by rapid release at stomach pH

---

<table>
<thead>
<tr>
<th></th>
<th>ACER-001</th>
<th>RAVICTI</th>
<th>BUPHENYL</th>
</tr>
</thead>
<tbody>
<tr>
<td>Efficacy/Safety in UCD</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td>Efficacy/Safety in MSUD</td>
<td>✓</td>
<td>✘</td>
<td>✓</td>
</tr>
<tr>
<td>Palatability / Compliance</td>
<td>✓</td>
<td>✓</td>
<td>✘</td>
</tr>
<tr>
<td>Relatively Reasonable Orphan Pricing</td>
<td>✓</td>
<td>✘</td>
<td>✓</td>
</tr>
</tbody>
</table>

- Acer is working closely with KOLs & patient advocacy groups to provide a compelling alternative treatment option for patients with UCD
- ACER-001 provides significant differentiation from other approved formulations of phenylbutyrate
- Ravicti / Buphenyl reported 2016 revenue = $170M (U.S. UCD only)
  - Ravicti annual price: >$500K pppy
  - Buphenyl annual price: ~$120K pppy
ACER-001 Market Opportunity

- Cannibalize existing sodium phenylbutyrate market share in UCD
  - Taste-masked, immediate-release formulation
  - Competitively priced

- Life cycle expansion opportunity in MSUD

- Barriers to entry:
  - Filed formulation patent application (January 2016)
  - 505(b)(2) Exclusivity: 3 years in U.S. for UCD
  - Obtained U.S. Orphan Drug Designation for MSUD (August 2014)
  - Pediatric Exclusivity: 6 months

- Provide a robust PAP to help offset costs
Acer Financial Overview

• Expected to have sufficient funding through NDA filing for EDSIVO™
  • $10M gross proceeds from RTO financing in September 2017

• Capitalization at mid-September 2017
  • 6.5M shares of common stock
  • 6.9M shares of common stock fully diluted*

• $28M invested to date through mid-September 2017
  • TVM Capital has been the lead investor

*Excluding 300K of Opexa legacy warrants with exercise price >$100 and expiry within 1 year
Acer Summary

• Lead Program (EDSIVO™) is a New Chemical Entity for treatment of Vascular Ehlers-Danlos Syndrome (vEDS) with a robust data package, including survival endpoints and long-term registry data at time of launch

• ACER-001 for treatment of Urea Cycle Disorders (UCD) and Maple Syrup Urine Disease (MSUD) is a proprietary, taste-masked, immediate release formulation of FDA/EMA approved products that are approaching $200M in revenue in the U.S.

• Both assets have been granted U.S. orphan status, are unencumbered and target indications with significant unmet medical needs

• Multiple key regulatory catalysts expected over next approximately 24 months
  • NDA filing for EDSIVO™ expected in 1H’18
  • NDA filing for ACER-001 anticipated in 1H’19

• Expected to have sufficient capital through NDA filing of EDSIVO™

• Experienced management team with significant orphan drug development expertise