Developing Therapeutics for the Treatment of Serious Rare Diseases with Significant Unmet Medical Needs
Forward-looking Statements

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Acer Corporate Overview

Acer is a pharmaceutical company that acquires, develops and commercializes therapies for serious rare and ultra-rare diseases with significant unmet medical needs.

- Headquartered: **Newton, MA**
- Headcount: **15**
- Founded: **December 2013**
- Public: **September 2017**
- Cash: **$12.4M** (3/31/18)
  - Expected to have sufficient capital through the end of 2018
## Senior Management

<table>
<thead>
<tr>
<th>Name</th>
<th>Position</th>
<th>Experience Details</th>
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<tbody>
<tr>
<td>Chris Schelling</td>
<td>CEO &amp; Founder</td>
<td>• 19 years; strategic commercial development &amp; orphan</td>
</tr>
<tr>
<td>Will Andrews, MD</td>
<td>Chief Medical Officer</td>
<td>• 18 years; clinical development, medical affairs &amp; orphan</td>
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<td>• M.D. Yale University School of Medicine</td>
</tr>
<tr>
<td>Harry Palmin</td>
<td>Chief Financial Officer</td>
<td>• 25 years; corporate &amp; finance experience</td>
</tr>
<tr>
<td>Don Joseph, JD</td>
<td>Chief Legal Officer &amp; Secretary</td>
<td>• 20+ years; general counsel &amp; senior management</td>
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<tr>
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<td></td>
<td>• J.D. University of Texas School of Law</td>
</tr>
<tr>
<td>Stacey Bain, PhD</td>
<td>VP, Clinical Development</td>
<td>• 20 years; international clinical operational &amp; drug development</td>
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<td>• Ph.D. Texas A&amp;M</td>
</tr>
<tr>
<td>Nancy Duarte-Lonnroth, MBA</td>
<td>VP, Quality</td>
<td>• 25 years; QA/QC, QMS, validation &amp; compliance</td>
</tr>
<tr>
<td>Terrie Kellmeyer, PhD</td>
<td>VP, Clinical Science</td>
<td>• 17 years; clinical development, medical writing &amp; medical affairs</td>
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<td>• Ph.D. SUNY Health Science Center – Syracuse</td>
</tr>
<tr>
<td>John Klopp</td>
<td>VP, Manufacturing</td>
<td>• 15 years; research, process development &amp; contract manufacturing</td>
</tr>
<tr>
<td>Jason Kneeland, CPA</td>
<td>VP, Finance &amp; Controller</td>
<td>• 20 years; corporate finance leadership</td>
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<tr>
<td>Kristin Mulready</td>
<td>VP, Program &amp; Alliance Management</td>
<td>• 20 years; executional strategy &amp; team leadership</td>
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<tr>
<td>Matt Seibt</td>
<td>VP, Market Access &amp; Reimbursement</td>
<td>• 22 years; managed care market access, reimbursement &amp; orphan</td>
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Investment Highlights

• Lead candidate (EDSIVO™) is a new chemical entity (NCE) 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 sodium phenylbutyrate
  • In 2017, Ravicti/Buphenyl generated $214.7M in revenue (U.S. / UCD only)¹

• Multiple key regulatory catalysts expected through 2019
  - EDSIVO™ Type C Meeting with FDA: Q2 2018
  - EDSIVO™ Type B (Pre-NDA) Meeting with FDA: Q2 2018
  - EDSIVO™ (vEDS) expected NDA submission: early Q4 2018²
  - ACER-001 (UCD) anticipated NDA submission: late Q4 2019³

• Expected to have sufficient capital through the end of 2018

² Acer intends to request Priority Review for EDSIVO, which if granted, could result in a PDUFA date of Q2 2019
³ Subject to Acer’s ability to obtain sufficient capital resources.
## 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>
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<tbody>
<tr>
<td><strong>EDSIVO™ (celiprolol)</strong></td>
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<tr>
<td>vEDS (COL3A1+)</td>
<td>Improves hemodynamic stability; decreases vascular resistance</td>
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<tr>
<td><strong>ACER-001 (reformulated sodium phenylbutyrate)</strong></td>
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<tr>
<td>UCD</td>
<td>Comparable to Buphenyl; taste-masked</td>
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<td>MSUD</td>
<td>Inhibition of BCKD kinase to increase BCAA metabolism</td>
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### Key Regulatory Milestones

- NDA submission for EDSIVO™ (vEDS) expected early Q4 2018
- NDA submission for ACER-001 (UCD) anticipated late Q4 2019*

* Subject to Acer’s ability to obtain sufficient capital resources.
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 COL3A1+ vEDS 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 (COL3A1+).

Significant unmet medical need with no direct competition. Expected NDA submission early Q4 2018. Orphan pricing with robust program to support reimbursement & access. Launch in 50 vEDS Centers of Excellence (COE).
Ehlers-Danlos syndrome (EDS) is a group of hereditary disorders of connective tissue.

vEDS (EDS type IV) is the severe subtype:
- Characterized by aneurysms, dissections and/or ruptures
  - Vascular
  - Gastrointestinal
  - Uterine
- Autosomal dominant (50%); spontaneous mutations (50%)
- 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 effective therapeutic options for vEDS
- Current treatment is focused on surgical intervention

Fig. 3 Distribution of 132 vascular complications in 24 patients with a clinical diagnosis of EDS type IV, J Vasc Surg 2005;42:98-106.
EDSIVO™ Unique Mechanism of Action

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

- EDSIVO™ has a unique pharmacological profile:
  - Selective $\beta_1$ and $\alpha_2$ adrenergic receptor antagonist
  - $\beta_2$ and $\beta_3$ 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 is unlikely to be through blood pressure lowering ($\beta_1$ 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**

| **Design:** | Multicenter, prospective, randomized, open trial with blinded endpoint assessment |
| **Location:** | 9 sites: eight in France, one in Belgium |
| **Eligibility:** | Inclusion criteria adapted Villefranche diagnostic criteria: 1 major criterion and 2 minor criteria, or 4 minor criteria needed for enrollment |
| **Enrollment:** | 53 patients with clinical vascular Ehlers-Danlos syndrome, Randomly assigned to 5 years treatment with 1) celiprolol or 2) no treatment |
| **Demographics:** | Ages 15 to 65 (mean 35), female/ male ratio 2:1, Important phenotype characteristics equally balanced between celiprolol and control |
| **Dosing:** | Celiprolol administered twice daily, Up-titrated every 6 mos. by 100 mg/ day to max. 400 mg/ day |
| **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”

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**Figure 2:** Kaplan-Meier curves of event-free survival in 53 patients with vascular Ehlers-Danlos

Primary endpoint (A). Primary and secondary endpoints (B).
Paris vEDS Patient Registry

**Assistance Publique - Hôpitaux de Paris (AP-HP) Registry**

- PIs: Michael Frank, Xavier Jeunemaitre
- 144 patients enrolled in registry
- 100% of patients in registry have COL3A1 gene mutation
- >80% of patients treated with celiprolol
- Study Period: Jan. 2000 to Dec. ‘14 (for initial referral patients), followed until 2017 (for follow-up visits)
- Median Age at Diagnosis: 35
- Median Duration F/U: 5.35 years [3.25 - 8.55 years]

Manuscript has been submitted & is being peer reviewed
1,231 patients (>90% COL3A1+; 630 index; 601 relatives)

Figure 1 Kaplan–Meier survival curve comparing vascular Ehlers–Danlos syndrome (vEDS) study population to 2008 US population.

Median Survival: 51 years (46 y.o. males; 54 y.o. females)
EDSIVO™ Data Package

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

• Paris Registry:
  • n = 144 patients (100% COL3A1+)
  • >80% patients on celiprolol
  • Compare with U.S. Natural History study (Pepin 2014)
  • Presented to FDA at Type C meeting in Q2 2018
  • Not rate-limiting to NDA submission – if published, to be included in support of NDA

• 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
vEDS Market Sizing*

- vEDS patient population for basis of market sizing = **4,169 patients**
  - 2 instances of the EDS Dx Code 756.83 separated by ≥ 2 months
  - Includes at least one other Dx or CPT code relevant to vEDS -AND-
  - Excludes hypermobility syndrome

* Study conducted on 06/2017; based on sampling of phenotype, rather than genotype
Accelerate Diagnosis

Broad Familial Genetic Testing Program

- According to HVH Patient Precision Analytics, 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 are not always reimbursed
- Opportunity to centralize, reduce cost: $500 / test (target)

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

* Based on sampling of phenotype, rather than genotype

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

1. Identify vascular medicine specialists / cardiologists who:
   - Can manage patients and their families
   - Are familiar with orphan therapies
   - Refer to CV surgeons
   - Work closely with a team of specialists

2. Will focus initially on cardiology/vascular medicine as “vEDS HOME”
   - Academic centers

3. vEDS COEs
   - 50 COEs at launch
   - Grow to 100 COEs in 3 years
   - ~20-50 vEDS patients/COE

4. Focused and dedicated medical & commercial effort to support launch (n~20)

*Commercialization of EDSIVO is subject to (a) Acer’s ability to obtain sufficient capital resources and (b) FDA approval.
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)
  - If approved, would grant 7 years market exclusivity in vEDS
  - Potential +0.5 years pediatric exclusivity
  - Use patents filed may provide additional exclusivity
- 50-100 Centers of Excellence
  - Focused, dedicated field support (n~20-25 people)
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**
- Anticipate NDA submission for UCD late Q4 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

* Subject to Acer’s ability to obtain sufficient capital resources.
Urea Cycle Disorders (UCD)

- Urea cycle disorders are a group of genetic disorders caused by mutations that result in a deficiency of one of the six enzymes in the urea cycle. These enzymes are responsible for removing ammonia from the bloodstream.

- The estimated incidence of urea cycle disorders is 1 in 8,500 births.

- Treatment options for UCD include:
  - Phenylbutyrate (Buphenyl, Ravicti)
  - IV Benzoate / Phenylacetate (Ammonul)
  - Sodium Benzoate
  - Restricted Diet
  - Liver Transplantation

The Urea Cycle

Mechanism of ammonia diversion from the urea cycle with the administration of sodium phenylacetate, sodium benzoate, or sodium phenylbutyrate (a prodrug of phenylacetate)
In addition to lowering levels of ammonia in patients with urea cycle disorders (UCD), sodium phenylbutyrate (NaPB) also significantly reduces branched-chain amino acids (BCAAs)¹

NaPB’s ability to lower leucine and other BCAAs could provide clinically-meaningful benefit in another genetic disease: Maple Syrup Urine Disease (MSUD)²

- Open label pilot study at BCM – 3 healthy and 5 MSUD subjects with late onset disease
- Despite the short treatment duration (3 days) NaPB showed statistically significant (intra-subject) reduction in leucine in 75% of the subjects

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¹Burrage et al., Mol. Genet. Metab. 2014
Maple Syrup Urine Disease (MSUD)

- MSUD is an inborn error of Branched-chain Amino Acid (BCAA) - leucine, isoleucine, valine - metabolism
  - Caused by deficiency of the mitochondrial Branched-chain Keto Acid Dehydrogenase complex (BCKDC)
  - ~800 patients in U.S., ~3,000 patients worldwide
  - MSUD Family Support Group has >500 patients
  - Part of newborn screening in U.S., UK, Germany

- High leucine levels lead to chronic and acute neurological damage
  - Lower IQ
  - Mental impairment (poor cognitive function)
  - Social impairment (poor executive function)
  - Metabolic decompensation (seizures and coma)

- A highly-restricted diet is the primary treatment
  - Consists of BCAA-free synthetic foods and formula
  - Very few foods have low BCAAs (fruits & vegetables)
  - Balancing act: enough BCAAs for growth & development

* Muelly 2011 Neuropsychiatric and Neurochemical Sequelae of MSUD
ACER-001 Taste-Masked, Immediate Release

Mouth → Stomach

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

Phenylbutyrate Formulations

<table>
<thead>
<tr>
<th></th>
<th>ACER-001</th>
<th>RAVICTI</th>
<th>BUPHENYL</th>
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<tbody>
<tr>
<td>Efficacy/Safety in UCD</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
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<tr>
<td>Efficacy/Safety in MSUD</td>
<td>✓</td>
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<td>✓</td>
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<tr>
<td>Palatability / Compliance</td>
<td>✓</td>
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<tr>
<td>Reasonable Orphan Pricing</td>
<td>✓</td>
<td>✗</td>
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- 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 2017 revenue $214.7M (U.S. / UCD only)*
  - Ravicti annual price: $794K pppy
  - Buphenyl annual price: $150K pppy
  - HZNP: 9.9% price increase on both products (12/29/17)

* Ravicti & Buphenyl pppy is based on patient weight
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)
  - Issued patent (US/EP) “Methods of modulation of branched chain acids and uses thereof”
  - 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

• Cash: $12.4M (3/31/18)
  • Expected to have sufficient capital through the end of 2018

• Capitalization at March 2018
  • 7.5M shares of common stock outstanding
  • 8M shares of common stock fully diluted

• $40.6M invested to date through December 2017
  • TVM Capital has been the lead investor
Acer Summary

- Lead candidate (EDSIVO™) is a new chemical entity (NCE) 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 sodium phenylbutyrate
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