Developing Therapeutics for the Treatment of Serious Rare and Life-Threatening Diseases with Significant Unmet Medical Needs

Corporate Presentation
November 19, 2021
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, timelines for clinical study enrollment or regulatory actions, or otherwise, future financial position, future revenues, projected expenses, regulatory submissions, actions or approvals, cash position, liquidity, prospects, plans and objectives of management are forward-looking statements. Examples of such statements include, but are not limited to, statements relating to the potential for our product candidates to safely and effectively treat diseases and to be approved for marketing; the commercial or market opportunity of any of our product candidates in any target indication and any territory; our ability to secure the additional capital necessary to fund our various product candidate development programs; the adequacy of our capital to support our future operations and our ability to successfully fund, initiate and complete clinical trials and regulatory submissions for ACER-801, EDSIVO™ or our other products; the ability to protect our intellectual property rights; our strategy and business focus; and the development, expected timeline and commercial potential of any of our product candidates. We 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 to fund our various product candidate development programs and to meet our 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 our intellectual property, risks related to the drug development and the regulatory approval process, including the timing and requirements of regulatory actions, and the impact of competitive products and technological changes. We disclaim 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.
Corporate Overview

Acer Therapeutics is a pharmaceutical company that acquires, develops and seeks to commercialize therapies for serious rare and life-threatening diseases with significant unmet medical needs.

- Offices: **Newton, MA** and **Bend, OR**
- Headcount: **33**
- Founded: **December 2013**
- Public: **September 2017**
- Cash as of September 30, 2021: **$14.2M**
  - Plus $10.0M Second Development Payment in two tranches following FDA acceptance for filing and review of an NDA for ACER-001 in a UCD per Collaboration Agreement with Relief
  - Expected to have sufficient capital to fund current operations into mid-2022$ 

$ Excluding the planned ACER-801 and EDSIVO™ clinical trials
## Leadership Team

<table>
<thead>
<tr>
<th>Name</th>
<th>Position</th>
<th>Years &amp; Experience</th>
<th>Company</th>
</tr>
</thead>
<tbody>
<tr>
<td>Chris Schelling</td>
<td>CEO &amp; Founder</td>
<td>22 years; strategic commercial dev. &amp; orphan</td>
<td>Biogen</td>
</tr>
<tr>
<td>Harry Palmin</td>
<td>COO &amp; CFO</td>
<td>25+ years; corporate &amp; finance experience</td>
<td>Novelos</td>
</tr>
<tr>
<td>Matt Seibt</td>
<td>Chief Commercial Officer</td>
<td>22 years; sales, market access &amp; product launch</td>
<td>Biogen</td>
</tr>
<tr>
<td>Jeff Davis</td>
<td>Chief Business Officer</td>
<td>25+ years; business &amp; corporate development</td>
<td>Genzyme</td>
</tr>
<tr>
<td>John Klopp</td>
<td>Chief Technical Officer</td>
<td>19 years; orphan manufacturing &amp; commercialization</td>
<td>Biomarin</td>
</tr>
<tr>
<td>Don Joseph, JD</td>
<td>Chief Legal Officer</td>
<td>25+ years; general counsel &amp; senior management</td>
<td>BVGH</td>
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<table>
<thead>
<tr>
<th>Name</th>
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<th>Company</th>
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<tbody>
<tr>
<td>Terrie Kellmeyer, Ph.D.</td>
<td>Group VP, Clinical Development</td>
<td>Intercept</td>
</tr>
<tr>
<td>Stacey Bain, Ph.D.</td>
<td>VP, Clinical Operations</td>
<td>WuXi Clinical</td>
</tr>
<tr>
<td>Renee Carroll</td>
<td>VP, Regulatory Affairs</td>
<td>Sunovion</td>
</tr>
<tr>
<td>Alka Chawla, M.D.</td>
<td>VP, Medical Director</td>
<td>FibroGen</td>
</tr>
<tr>
<td>William DeVincenzi</td>
<td>VP, Quality</td>
<td>Zogenix</td>
</tr>
<tr>
<td>Jeffrey Edwards, Ph.D.</td>
<td>VP, Clinical Sciences</td>
<td>Madrigal Pharmaceuticals</td>
</tr>
<tr>
<td>Tanya Hayden</td>
<td>VP, Program/Alliance Mgt</td>
<td>Lonza</td>
</tr>
<tr>
<td>John Hilton</td>
<td>VP, Marketing/Strategic Insights</td>
<td>Gilead</td>
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Investment Highlights

• Acer’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)
  • ACER-801 (osanetant) for the treatment of induced vasomotor symptoms (iVMS)
  • EDSIVO™ (celiprolol) for the treatment of vascular Ehlers-Danlos syndrome (vEDS) in patients with a confirmed type III collagen (COL3A1) mutation
  • ACER-2820 (emetine) a host-directed therapy against a variety of infectious diseases

• Product candidates are believed to present a comparatively de-risked profile, having one or more of:
  • Favorable safety profile; clinical proof-of-concept data; mechanistic differentiation
  • Potential expedited paths for development through specific FDA-established programs

• Multiple anticipated key milestones:
  ✓ ACER-801 IND submitted: Q4 2021
  • EDSIVO™ IND submission, work toward SPA, request BTD Q4 2021
  • ACER-801 Phase 2a clinical trial initiation*: Q1 2022
  • EDSIVO™ DiSCOVER clinical trial initiation**: Q1 2022
  • ACER-001 (UCDs) PDUFA target action date: June 5, 2022

* Subject to IND clearance
** Subject to successful IND submission and clearance, and agreement on SPA
$ Subject to additional capital
## Clinical Pipeline

<table>
<thead>
<tr>
<th>Program / Indication</th>
<th>Novel MOA / Unique Characteristics</th>
<th>Preclinical</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
<th>NDA</th>
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<tbody>
<tr>
<td>ACER-001 (sodium phenylbutyrate)</td>
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<tr>
<td>Urea Cycle Disorders</td>
<td>Nitrogen-binding agent</td>
<td></td>
<td></td>
<td></td>
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<td>PDUFA: June 5, 2022</td>
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<tr>
<td>Maple Syrup Urine Disease</td>
<td>Inhibition of BCKD kinase to increase BCAA metabolism</td>
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<tr>
<td>ACER-801 (osanetant)</td>
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<tr>
<td>Induced vasomotor symptoms (iVMS)</td>
<td>Neurokinin 3 receptor antagonist</td>
<td></td>
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<tr>
<td>EDSIVO™ (celiprolol)</td>
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<tr>
<td>Vascular Ehlers-Danlos syndrome (COL3A1+)</td>
<td>Induces vascular dilatation and smooth muscle relaxation</td>
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<tr>
<td>ACER-2820 (emetine)</td>
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<tr>
<td>Broad-spectrum antiviral</td>
<td>Host-directed therapy</td>
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$ Additional capital required to support planned ACER-801 and EDSIVO™ clinical trials, and to fund emetine program going forward
**Overview**

- **UCDs**: A group of metabolic genetic diseases that lead to toxic build-up of NH$_4^+$
- **UCDs**: Currently treated with RAVICTI®, BUPHENYL®, AMMONUL®, and a highly-restricted diet
- **MSUD**: A metabolic genetic disease that leads to toxic build-up of leucine and other branched-chain amino acids
- **MSUD**: Currently managed with a highly-restricted diet; poor compliance

- **Small molecule with unique MOAs in various disorders**
- **UCDs**: NaPB is a prodrug of phenylacetate, a NH$_4^+$ scavenger
- **MSUD**: NaPB is an allosteric inhibitor of BCKD kinase

- **Nitrogen-binding agent for use as adjunctive therapy in the chronic management of patients with UCDs**
- **UCDs**: Bioequivalence trials showed ACER-001 has similar relative bioavailability to BUPHENYL® in healthy volunteers under both fasted and fed conditions
- **ACER-001** under fasted conditions achieved >2x $C_{\text{max}}$ of PBA vs. fed conditions
- **MSUD**: POC study$^1$ suggests ~60% of patients have 30% reduction in Leucine

- **NDA filing accepted for FDA review; PDUFA target action date June 5, 2022**
- **UCDs**: ~700 patients treated with sodium / glycerol phenylbutyrate.
- **MSUD**: ~800 treatment-eligible patients in the U.S.; 3,000 patients worldwide
- Advantageous orphan pricing likely with robust program to support patient access and reimbursement
- Collaboration and License Agreement with Relief Therapeutics for development and commercialization of ACER-001

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Newborns with severe urea cycle disorders become significantly ill with symptoms that mimic sepsis -- failure to feed, lethargy, respiratory distress, seizures and ultimately coma.

Children and adults with milder (or partial) urea cycle enzyme deficiencies may go years without a diagnosis, until a trigger -- a high protein meal, viral illness, excessive exercise or calorie deficiency -- causes excessive ammonia to be produced in the body, resulting in critical elevations of blood ammonia levels.

For individuals with an ornithine transcarbamylase (OTC) deficiency, typical neuropsychological complications include developmental delay, learning disabilities, intellectual disability, attention deficit hyperactivity disorder (ADHD), and executive function deficits.

Symptoms of Hyperammonemia:
- **General**
  - Growth retardation
  - Hypothermia
- **Muscular/Neurologic**
  - Poor coordination
  - Dysdiadochokinesia
  - Hypotonia or hypertonia
  - Ataxia
  - Tremor
  - Seizures
  - Decorticate or decerebrate posturing
- **Central**
  - Combativeness
  - Lethargy
  - Coma
- **Eyes**
  - Papilledema
- **Pulmonary**
  - Shortness of breath
- **Liver**
  - Enlargement

Reproduced from:
Mechanism of Action

Ammonia-scavenger therapy

- Alternative pathway treatment diverts nitrogen from the urea cycle to alternate routes of excretion
- Both RAVICTI® and BUPHENYL® metabolize to phenylbutyrate (PBA), a prodrug of phenylacetate (PAA)
- PAA is the active moiety – it combines with glutamine, producing phenylacetylglutamine
- Phenylacetylglutamine (PAGN) is excreted by the kidneys

Fig. 1. Metabolizing pathway and mechanism of action of GPB. GPB (glycerol phenylbutyrate); PAA (phenylacetic acid); PBA (phenylbutyric acid); PAGN (phenylacetylglutamine).
Unmet Need

• **BUPHENYL®**: Foul odor and foul/bitter taste; considered unpalatable
  • 64% of patients reported it is difficult to take because of taste
  • Physicians reported that 25-33% of patients took less than target dose due to tolerability
  • Only 25% of patients indicated that they never miss a dose
  • 46% of patients reported taste as the reason for discontinuation

• **RAVICTI®**: Tasteless/Odorless
  • 75% of BUPHENYL® patients switched to RAVICTI®
  • Pricing has risen to levels considered challenging
  • Reports of difficult access, unaffordability, and forced switches back to sodium phenylbutyrate
    • Example: BUPHENYL® and RAVICTI® blocked on JPMorgan Chase plan Rx formulary
  • Some patients are not meeting the treatment goal of <0.5 ULN (~17.5 umol/L)

• Patients and physicians desire a taste-masked, effective, and affordable treatment option

1 Shchelochkov et al., Molecular Genetics and Metabolism Reports 8 (2016) 43-47.
2 Acer Market Research
3 https://www.caremark.com/portal/asset/Formulary_Drug_Removals_JPMC.pdf
### Phenylbutyrate Formulations

<table>
<thead>
<tr>
<th></th>
<th>ACER-001*</th>
<th>RAVICTI®</th>
<th>BUPHENYL®</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Efficacy / Safety in UCDs</strong></td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td><strong>Palatability / Compliance</strong></td>
<td>✓</td>
<td>✓</td>
<td>✘**</td>
</tr>
<tr>
<td><strong>Pricing (Per Patient Per Year)</strong></td>
<td>TBD, likely near BUPHENYL®</td>
<td>$174K-$1.3M*** (avg $950K)</td>
<td>$204K-$402K*** (avg $300K)</td>
</tr>
<tr>
<td><strong>Formulation</strong></td>
<td>Multi-Particulate (Sachet)</td>
<td>Oil (Tablespoons)</td>
<td>Powder/Tablets (up to 40 tablets/day)</td>
</tr>
</tbody>
</table>

* Subject to FDA approval
** Shchelochkov et al., Molecular Genetics and Metabolism Reports 8 (2016) 43-47
*** RAVICTI® and BUPHENYL® pppy is based on patient weight and WAC price
BE trial under fasted conditions completed in Q1 2020

BE trial under fed conditions completed in Q1 2021

Type B (pre-NDA) meeting with FDA conducted in Q2 2021

Reached agreement with FDA on initial Pediatric Study Plan (iPSP) in Q3 2021

505(b)(2) NDA for UCDs: NDA filing accepted for review by FDA in Oct. 2021

NDA for UCDs: PDUFA target action date June 5, 2022

**Lifecyle Opportunities**

- Evaluate after initial potential FDA approval for UCDs (under fed conditions)$:
  - Pre-meal administration of ACER-001, which would require additional nonclinical and clinical studies$ to demonstrate efficacy and safety in UCDs
  - MSUD
  - Other potential indications

$ Subject to additional capital
Food Effect

- Maximum concentration ($C_{\text{max}}$) ~2x higher under fasted (pre-meal) conditions
- Comparable PK between ACER-001 and NaPB under fed conditions*

* 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
Food Effect: In Silico Model

**Adult Virtual Patient**

- **PAA (Safety)**
  - Model predicted ACER-001 in a fasted state required ~30% less PBA to achieve comparable therapeutic benefit in a fed state

- **uPAGN (Efficacy)**
  - Model predicted 43% increase in urinary PAGN levels (inverse correlation with blood ammonia AUC)

**Child Virtual Patient**

Dosing and Administration for BUPHENYL®, RAVICTI® and Pheburane® are all instructed to be given with food\(^1\)

- There is a significant food effect with NaPB
- The pharmacokinetic (PK) and pharmacodynamic (PD) properties of RAVICTI® are indistinguishable in the fed or fasted states\(^2\)

Dosing in a pre-meal setting should increase exposure, and theoretically improve ammonia control / outcomes in UCDs patients

- Pre-meal administration of ACER-001 in UCDs will require additional nonclinical and clinical studies to demonstrate efficacy and safety and are subject to additional capital

- \(2\times \text{C}_{\text{max}}\) of PBA may also improve efficacy in other disorders (where PBA is the active moiety), such as MSUD and PFIC\(^3\)

- ACER-001’s formulation may improve tolerability of the drug when administered under fasted (pre-meal) conditions

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1 Initial ACER-001 proposed UCD instructions to include administration with food
2 United States Patent number US8642012B2
ACER-001 Value Proposition

- Taste-masked formulation designed to improve palatability of NaPB
- Bioequivalence trials showed ACER-001 has similar relative bioavailability to BUPHENYL® under both fasted and fed conditions
- Fasted (pre-meal) dosing data suggests ability to subsequently optimize Rx dosing approach¹
- Pricing projected to be significantly lower than current RAVICTI® price
- Robust patient support services program to remove barriers to care
- Payer engagement strategy to alleviate insurance paperwork and support switching
- Acer’s commitment to support the UCD community and on-going IEM research

¹ Intend to seek FDA approval in the U.S. to market ACER-001 for administration initially under fed conditions for the treatment of UCDs. Pre-meal administration of ACER-001 in UCDs will require additional nonclinical and clinical studies to demonstrate efficacy and safety and is subject to additional capital

² Payer Claims Data on File

- ~2,100 UCD Patients in U.S.
- ~1,100 UCD Pts Actively Managed
- ~700 Drug Treated Pts²
- ~400 Rx Status “unknown”

- BUPHENYL®/Generic Patients²
  - ~20%-30% of market
  - ~140-200 pts
- RAVICTI® Patients²
  - ~70%-80% of market
  - ~500-560 pts
IP / Exclusivities

• IP:
  • Issued patents (US/EP):
    • “Palatable compositions including sodium phenylbutyrate and uses thereof” (US PATENT NO. 11,154,521), including pharmaceutical composition claims covering ACER-001’s taste-masked, multi-particulate dosage formulation for oral administration
      • Proprietary position in U.S. until 2036
    • “Methods of modulation of branched chain acids and uses thereof” (US PATENT NO. 10,092,532), licensed from Baylor College of Medicine relating to MSUD
      • In addition, we continue to pursue new patents and exclusivity possibilities, based on our development plans and product attributes

• Regulatory Exclusivities:
  • MSUD: Granted U.S. Orphan Drug Designation: 7 years market exclusivity from FDA approval
  • MSUD: Pediatric exclusivity: +6 months added (if pediatric indication study approved)
Agreement with Relief Therapeutics

✓ Signed Collaboration and License Agreement March 19, 2021

✓ Acer received $30.0M to date ($1.0M Option Fee + $14.0M Reimbursement Payment + $10.0M First Development Payment + $5.0M 1st tranche of Second Development Payment)

• Relief to pay Acer additional $5.0M 2nd tranche of Second Development Payment upon the earlier of January 14, 2022 or 15 business days following Acer written notice

✓ Acer retained development and commercialization rights in the U.S., Canada, Brazil, Turkey and Japan
  • Split net profits from Acer’s territories 60%:40% in favor of Relief

✓ Relief licensed rights for the rest of the world
  • Acer will receive 15% royalty on all Net Sales in Relief’s territories
  • Acer could also receive up to $6.0M for UCD and MSUD approvals in EU
ACER-801 (osanetant) is a 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 and KNDy neurons. NK3R antagonism is an alternative to hormone replacement therapy for the treatment of VMS by mimicking the negative feedback of estrogen on KNDy neurons.

ACER-801 (osanetant) is a selective, non-peptide tachykinin NK3 receptor antagonist

Believed to have largest body of clinical safety data for any NK3R antagonist

Clinical and laboratory safety results are available from 23 completed Phase 1 and 2 studies (387 healthy subjects and 822 patients were treated with osanetant)*

Oral bioavailability, readily crosses the blood-brain barrier

Acer licensed worldwide rights to ACER-801 from Sanofi in Dec. 2018

IND submitted in Q4 2021

Plan to initiate Phase 2a clinical trial in Q1 2022**$

Currently no other NK3R antagonists in development in iVMS space

** Subject to IND clearance
$ Subject to additional capital
• Acer acquired worldwide rights to osanetant from Sanofi in December 2018

• Osanetant was the first selective non-peptide tachykinin NK3 receptor antagonist evaluated as a potential treatment for schizophrenia

• Clinical and laboratory safety results are available from 23 completed Phase 1 and 2 studies in which 387 healthy subjects and 822 patients (schizophrenia, depression, others) were treated with osanetant

• No major safety concerns identified from these studies after single-dose and repeated-dose administration of up to 400 mg QD for up to 21 days, and 200 mg QD for up to 6 weeks for schizophrenia

• In March 2005, Sanofi-Aventis discontinued the development of osanetant for schizophrenia citing ‘lack of efficacy compared with placebo’ in this indication as a major reason for this decision
Vasomotor Symptoms (VMS)

- VMS, typically comprised of hot flashes and night sweats, are associated with decreases in reproductive hormones commonly associated with menopause (e.g. MR-VMS)

A diminished amount of hormones, such as estrogen, affects the hypothalamus. This confuses the hypothalamus and makes it read “too hot.” The brain responds by relaying an alert to cool off. The body then tries to cool off by beginning to perspire.

# Induced Vasomotor Symptoms (iVMS)

## Women who are BRCA+ and have prophylactic bilateral salpingo-oophorectomy (PBSO)

- 67% of women have symptoms of menopause such as hot flashes[^5]
- Up to 35% complain of “extremely bothersome” symptoms up to two years after their surgery[^6]

## Men with HR+ Prostate Cancer (CaP) receiving Leuprolide

- 80% of men experience hot flashes[^3]
- 15-27% of patients consider hot flashes the most distressing side effect
- 30-40% experienced moderate-to-severe symptoms
- 20% discontinued or disrupted treatment

## Women with HR+ Breast Cancer (CaB) receiving Tamoxifen

- 84% of women experienced hot flashes[^1]
- 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[^2]

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Neurokinin B (NKB) belongs to a group of neuropeptides, called tachykinins or neurokinins, that includes substance-P (SP), neurokinin A (NKA), and two N-terminally extended forms of NKA, neuropeptide g and neuropeptide K.

The biological effects of tachykinins are mediated through specific receptors denoted NK1, NK2, and NK3.

NKB is the preferred endogenous ligand of tachykinin NK3 receptors.

The tachykinin NK3 receptors are located primarily in the brain, while a few receptors are also present in the peripheral nervous system (intestines, placenta).
**Clinical POC in VMS: NK3R Antagonist**

- Fezolinetant is a NK3R antagonist being developed by Astellas for moderate-to-severe VMS

**Average Daily Hot Flash Frequency Reported as per FDA Guidance**

- **At Week 4:**
  - fezolinetant group: 14/40 patients have ZERO hot flash
  - placebo group: 2/40 patients have ZERO hot flash

*: % decrease from the baseline
Pavinetant (MLE4901) was a NK3R antagonist that was discontinued by Millendo for the treatment of polycystic ovary syndrome and menopausal hot flushes.

FIG. 2. Hot flash frequency (A), severity (B), bother (C), and interference (D) outcomes: results are presented as percentage change with 95% CIs from baseline at each time point during the treatment period (ie, on day 3 of treatment, and then weekly mean total for each week (wk) of the 4-week treatment period for both placebo (white) and MLE4901 (gray). Minimum n = 33; maximum n = 37. *P < 0.0001, #P = 0.0006, ^P = 0.0011, +P = 0.0001. Week 4 data adapted from Prague et al, Lancet, 2017.18.
Acer is partnering with leading institutions to design and conduct clinical trials to evaluate ACER-801 in various patient populations with iVMS

These include patients with medically or surgically iVMS (may include any/all of the following):
- Women who are BRCA+ and have had a PBSO
- Men with HR+ Prostate Cancer receiving leuprolide
- Women with HR+ Breast Cancer receiving tamoxifen

Planning to initiate Phase 2a clinical trial in Q1 2022$: 
- Evaluate PK, efficacy and safety in postmenopausal women with VMS
- Identify the optimal dosing strategy to advance into subsequent efficacy studies in cancer patients with iVMS

$ Subject to IND clearance and additional capital
EDSIVO™ has a unique pharmacological profile
- β2 and β3 adrenergic receptor agonist; selective β1 and α2 adrenergic receptor antagonist; activates endothelial Nitric Oxide Synthase (eNOS)
- EDSIVO’s™ potential beneficial effects in vEDS thought to be through vascular dilatation and smooth muscle relaxation, thereby reducing the mechanical stress on collagen fibers within the arterial wall

BBEST Clinical Trial: 76% reduction in risk of arterial events observed in COL3A1+ subpopulation
- Statistically-significant improvement in event-free survival (EFS) compared to control in vEDS patients (n=53)

Planning pivotal clinical trial following FDA Type B meeting feedback
- Proposed DISCOVER Phase 3 decentralized (virtual) clinical trial expected to take ~3.5 years to complete once enrolled
- FDA indicated agreement w/Acer plan to submit proposed protocol under a Special Protocol Assessment (SPA) w/possible breakthrough therapy designation (BTD)

* There can be no assurance that the clinical trial will be successful, or that any resubmission of the original NDA will be approved
$ Subject to additional capital to conduct and complete the trial
Vascular Ehlers-Danlos Syndrome (vEDS)

- 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
    - Hollow Organs (e.g. 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, and 90% by the age of 40
  - Median age of death is estimated to be 51 years

- No approved 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™ has a unique pharmacological profile:
- β2 and β3 adrenergic receptor agonist
- Selective β1 and α2 adrenergic receptor antagonist
- Intrinsic sympathomimetic activity (ISA+)
- Lacks non-specific membrane effects
- Activates endothelial Nitric Oxide Synthase (eNOS)*

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

EDSIVO’s™ mechanism of action in vEDS patients is thought to be through vascular dilatation and smooth muscle relaxation, thereby reducing the mechanical stress on collagen fibers within the arterial wall

Efficacy:

- 76% reduction in the risk of fatal or nonfatal cardiac or arterial events in COL3A1+ EDSIVO™ patients vs. control group over mean follow-up of 47 months

- 75% reduction in risk of primary (cardiac or arterial events) and secondary (intestinal or uterine rupture) events in COL3A1+ EDSIVO™ patients vs. control group

Figure 3: Kaplan-Meier curves of event-free survival in 33 patients with positive COL3A1 mutation
Primary endpoint (A). Primary and secondary endpoints (B).

• Proposed DiSCOVER trial design* following Type B FDA meeting feedback:
  • DiSCOVER: Decentralized Study of Celiprolol on vEDS-related Event Reduction
  • A Phase 3, U.S.-based prospective, randomized, double-blind, placebo-controlled decentralized (virtual) clinical trial
  • Approximately 3.5 years to complete once fully enrolled*
  • Acer to coordinate with FDA to reach agreement on Special Protocol Assessment (SPA)
    • Primary endpoint: vEDS-related clinical events in patients treated with celiprolol vs. placebo
    • Statistical plan that considers the rare disease classification of vEDS
    • Enrollment: approximately 200 COL3A1+ U.S. vEDS patients
    • Modest safety data collection required based on celiprolol’s known safety profile 1,2,3,4

* Subject to successful IND submission and clearance, agreement on SPA, and additional capital to conduct and complete the trial
Next Steps

Q4 2021:
- Submit IND and SPA request to FDA for DiSCOVER clinical trial
- Identify ~200 COL3A1+ patients in the U.S. for trial participation
  - ~1,000 U.S. patients potentially available following evaluation of vEDS diagnosis code (ICD10: 79.6)
  - Launched www.discoverceliprolol.com as an outreach tool for interested parties

- Obtain up to ~$15.0M to conduct and complete pivotal trial – from potential current and future funding sources, including but not limited to:
  - Current cash
  - Strategic partnership
  - Other non-dilutive sources
  - Potential ACER-001 revenue

- Request breakthrough therapy designation (BTD)

- Planning to initiate pivotal DiSCOVER trial in Q1 2022*

* Subject to successful IND submission and clearance, agreement on SPA, and additional capital to conduct and complete the trial
Host-directed Therapy
- Restores cellular stress response by interrupting the viral-induced MDM2-P53 loop, by shepherding RPS14 from cytosol into the nucleus, thus inhibiting viral replication
- Few drugs in development that are host-directed; believed to be only drug exploiting this particular MOA

Broad-spectrum Antiviral
- In vitro +/- in vivo data across multiple virus families, including coronavirus, filovirus, flavivirus, herpesvirus, togavirus, arenavirus, HIV, influenza

Small molecule (SQ) used previously in tens of thousands of humans as an antiprotozoal, emetic, and antiviral agent
- Toxicity is related to cumulative dose; reasonable safety profile administered as subcutaneous injection in humans up to 10 mg/kg cumulative dose

Pursuing multiple non-dilutive funding options for emetine development in a variety of infectious diseases, including COVID-19
- Evaluating development pathways against viruses utilizing animal rule / Medical Countermeasure (MCM) pathway
  - Applied for MCDC funding for Ebola; received positive feedback and placed in “basket” if additional funding becomes available
  - Application for BARDA funding for COVID-19 remains active
Emetine is one of the main alkaloids found in ipecacuanha (ipecac) root.

Clinically, emetine hydrochloride was originally marketed in the U.S. as a topical anti-infective in dental applications (ca 1890s by Eli Lilly and Company).

Later, emetine hydrochloride for injection gained market adoption as a specific treatment for amebic infections and was used for this purpose through the 1980s in the U.S. until its market displacement by metronidazole.

An oral formulation, syrup of ipecac, also contains emetine as one of its active ingredients.

Substantial clinical experience with emetine and emetine-containing products exists because of their introduction in the U.S. prior to the 1938 Food, Drug, and Cosmetic Act, and especially the development of an over-the-counter monograph for syrup of ipecac.

Its broad antiviral activity has only been discovered in the past decade.

Clinically, emetine has been used to treat approximately 700 patients (including pediatrics) with viral hepatitis\(^1\) and varicella-zoster virus\(^2\).

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Mechanism of Action

- Viral infections have developed evolutionary mechanisms for inhibiting the cellular stress response and promote ribosome biogenesis to facilitate viral replication

- **Host-directed Therapy:** Binding of emetine with RPS14 restores the cellular stress response, which results in blocking ribosome biogenesis and translation-elongation of viral mRNA in infected cells

- Emetine does not inhibit viral replication in null RPS14 cells

- Viral resistance believed to be extremely unlikely given unique MOA

In high-density infected cells (A) emetine induces (1) nuclear translocation of RPS14 (2) followed by RPS14 binding to MDM2 (3 & 4) resulting in disruption of the interaction between MDM2-p53 (6) and MDM2- viral IE2 (5 & 7), and by RPS14 ubiquitination and degradation (8). In low-density infected cells (B) although emetine induces (1) nuclear translocation of RPS14 (2), it is unable to interact with MDM2 (4) which is already bound to p53 to facilitate virus replication (3).
Broad-spectrum, Antiviral Activity

Emetine

- Coronaviridae
- Hantaviridae
- Flaviviridae
- Herpesviridae
- Filoviridae
- Paramyxoviridae
- Arenaviridae
- Retroviridae
- Togaviridae
- Picornaviridae
- Bunyaviridae
- Poxviridae

Emetine has broad-spectrum antiviral activity against various virus families.
## Nanomolar Potency In Vitro

<table>
<thead>
<tr>
<th>Virus Type</th>
<th>Antiviral Activity</th>
<th>Reference</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Coronaviridae</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>SARS-CoV-2 (Caco-2)</td>
<td>IC\textsubscript{50} = 0.47</td>
<td>Bojkova et al. Proteomics of SARS-CoV-2 infected host cells reveals therapy targets. Nature <a href="https://doi.org/10.1038/s41586-020-2332-7">https://doi.org/10.1038/s41586-020-2332-7</a> (2020).</td>
</tr>
<tr>
<td>SARS-CoV-2 (Vero-E6)</td>
<td>EC\textsubscript{50} = 0.46</td>
<td>Choy et al. Antiviral Research. 2020 Apr 3; pre-proof <a href="https://doi.org/10.1016/j.antiviral.2020.104786">https://doi.org/10.1016/j.antiviral.2020.104786</a></td>
</tr>
<tr>
<td>SARS-CoV-2 (Vero-E6)</td>
<td>EC\textsubscript{50} &lt; 0.01</td>
<td>Janjic et al. 2020 May. Antiviral options against SARS-CoV-2 infection. <a href="https://doi.org/10.1101/2020.05.12.091165">https://doi.org/10.1101/2020.05.12.091165</a></td>
</tr>
<tr>
<td>MERS-CoV</td>
<td>EC\textsubscript{50} = 0.014</td>
<td></td>
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<tr>
<td>MERS-CoV</td>
<td>IC\textsubscript{50} = 0.34 / CC\textsubscript{50} = 3.08</td>
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<tr>
<td>HCoV-NL63</td>
<td>EC\textsubscript{50} = 1.43 / CC\textsubscript{50} = 3.63</td>
<td></td>
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<tr>
<td>HCoV-OC43</td>
<td>EC\textsubscript{50} = 0.30 / CC\textsubscript{50} = 2.69</td>
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<tr>
<td>MHV-A59</td>
<td>EC\textsubscript{50} = 0.12 / CC\textsubscript{50} = 3.51</td>
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<td><strong>Flaviviridae</strong></td>
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<tr>
<td>WN</td>
<td>IC\textsubscript{50} = 0.148</td>
<td>Unpublished Data on File (USAMRIID)</td>
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<tr>
<td>DENV</td>
<td>IC\textsubscript{50} = 0.023</td>
<td>Unpublished Data on File (USAMRIID)</td>
</tr>
<tr>
<td>DENV1, 3 &amp; 4 (Huh-7)</td>
<td>IC\textsubscript{50} = 0.5</td>
<td>Low et al. J AntiViral And RetroViral 1: 062-071.</td>
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<tr>
<td>Zika-FSS13025</td>
<td>IC\textsubscript{50} = 1.072e-008</td>
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<tr>
<td>Zika-PRVABC59</td>
<td>IC\textsubscript{50} = 9.591e-009</td>
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<tr>
<td><strong>FiloVirdae</strong></td>
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<tr>
<td>EBOV</td>
<td>IC\textsubscript{50} = 0.222</td>
<td>Unpublished Data on File (USAMRIID)</td>
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<tr>
<td><strong>Arenaviridae</strong></td>
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<tr>
<td>LASV</td>
<td>IC\textsubscript{50} = 0.055</td>
<td>Unpublished Data on File (USAMRIID)</td>
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<td><strong>Togaviridae</strong></td>
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<tr>
<td>VEEV</td>
<td>IC\textsubscript{50} = 0.133</td>
<td>Unpublished Data on File (USAMRIID)</td>
</tr>
<tr>
<td>CHIKV</td>
<td>IC\textsubscript{50} = 0.029</td>
<td>Unpublished Data on File (USAMRIID)</td>
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<tr>
<td><strong>Bunyaviridae</strong></td>
<td></td>
<td></td>
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<tr>
<td>RVFV</td>
<td>IC\textsubscript{50} = 0.093</td>
<td>Unpublished Data on File (USAMRIID)</td>
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<tr>
<td><strong>Hantaviridae</strong></td>
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<td></td>
</tr>
<tr>
<td>HTNV-G</td>
<td>ED\textsubscript{50} = 9.9*10^{-6}</td>
<td>Mayor et al. Viruses 2021, 13, 685.</td>
</tr>
<tr>
<td><strong>Herpesviridae</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>HCMV</td>
<td>EC\textsubscript{50} = 0.040 / CC\textsubscript{50} = 8</td>
<td>Mukhopadhyay et al. J Virology, Dec. 2007, p. 13392-13402.</td>
</tr>
<tr>
<td>HSV-2</td>
<td>EC\textsubscript{50} = 0.03 / CC\textsubscript{50} = 1.12</td>
<td>Andersen et al. Viruses, 2019 Oct 18;11(10). pii: E964. doi: 10.3390/v11100964.</td>
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<tr>
<td><strong>Paramyxoviridae</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>HMPV</td>
<td>EC\textsubscript{50} = 0.14 / CC\textsubscript{50} = 1</td>
<td>Andersen et al. Viruses, 2019 Oct 18;11(10). pii: E964. doi: 10.3390/v11100964.</td>
</tr>
<tr>
<td>NDV</td>
<td>ED\textsubscript{50} = 0.053 HA unit</td>
<td>Khandelwal et al. Antiviral Research 144 (2017) 196-204.</td>
</tr>
<tr>
<td><strong>Retroviridae</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>HIV M184V</td>
<td>EC\textsubscript{50} = 0.012 – 0.03</td>
<td>Chaves Valadao et al. Molecules, 2015 Jun 22;20(6):11474-89. doi: 10.3390/molecules200611474.</td>
</tr>
<tr>
<td><strong>Picornaviridae</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>EV-A71 (Vero)</td>
<td>EC\textsubscript{50} = 0.049 / CC\textsubscript{50} = 10</td>
<td>Tang et al. Antiviral Research 173 (2020) 104650. <a href="https://doi.org/10.1016/j.antiviral.2019.104650">https://doi.org/10.1016/j.antiviral.2019.104650</a>.</td>
</tr>
<tr>
<td>EV-D68</td>
<td>EC\textsubscript{50} = 0.019</td>
<td></td>
</tr>
<tr>
<td>Echod-6</td>
<td>EC\textsubscript{50} = 0.045</td>
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<tr>
<td>CV-A16</td>
<td>EC\textsubscript{50} = 0.083</td>
<td></td>
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<tr>
<td>CV-B1</td>
<td>EC\textsubscript{50} = 0.051</td>
<td></td>
</tr>
<tr>
<td><strong>Poxviridae</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>NP-S-EGFP (BSC40)</td>
<td>IC\textsubscript{50} = 0.100</td>
<td>Deng et al. Journal of Virology, Dec. 2007, p. 13392-13402.</td>
</tr>
</tbody>
</table>

EC\textsubscript{50} = concentration of a drug that gives half-maximal response. IC\textsubscript{50} = concentration of an inhibitor where the response is reduced by half
CC\textsubscript{50} = 50% cytotoxic concentration

*EC\textsubscript{50} / CC\textsubscript{50} values = μM (unless otherwise noted) **For reference, the EC\textsubscript{50} of remdesivir is 23.15 μM at MOI 0.02; paper demonstrates that emetine is synergistic with remdesivir
## In Vivo Efficacy Data

### Zika virus

- **Emetine suppresses ZIKV virus load in vivo.**
  - Three-month-old SJL male mice were infected retro-orbitally with ZIKV BR followed by IP administration of emetine (1 mg/kg/day) for the next 6 days (N = 4 mice per group). Two groups of SJL mice (N = 4) received the same volume of vehicle buffers. Statistical analysis by two tailed t-test. **p= 0.0014, ***p = 0.0005.

### Ebola virus

- **Emetine inhibits EBOV infection in vivo.**
  - The survival curve of MA-EBOV infected mouse treated with 1 mg/kg emetine every day. Six to eight week-old female BALB/c mice were randomly assigned into groups (N = 6 animals). All the mice were challenged with a lethal dose of 1000 times the LD50 mouse adapted EBOV via IP treatments with either emetine (1 mg/kg/day) or PBS (same volume for the control group) were initiated at 3 h before the challenge and continued for up to 6 days post infection. Survival was monitored for 28 days post infection.

### Cytomegalovirus

- **Emetine achieves high tissue concentrations and is efficacious against MCMV replication.**
  - Quantitative real-time PCR of viral gB was performed on DNA extracted from blood at day 14 post infection. Emetine was administered orally starting 24 hpi or 72 hpi at 0.1 or 1.0 mg/kg every 3 days. GCV dose was 10 mg/kg/dose administered intraperitoneally twice daily.

### Enterovirus

- Diagram of the mice infection model (A), survival rates (B) and clinical scores (C) of two-week KM mice infected with EV-A71 GZCII strain and treated with emetine. The treated mice were monitored for two weeks after infection. Disease score definition as followings: Healthy, 0 point; Lethargy and inactivity, limb weakness, 1 point; Less exercise, limb paralysis, 2 points; Quadriplegic, moribund, 3 points; Death, 4 points.

PK & Tox Data

- Non-clinical pharmacokinetic studies show that emetine rapidly concentrates in the tissues, reaching levels that are 1,000- to 3,000-fold greater than in the plasma.

- Emetine has been detected in a variety of tissues, including the heart, liver, lungs, intestines, kidney, spleen, stomach, adrenals, and brain, with a long (days/weeks) tissue half-life.

- However, the key target organs of toxicity identified in the literature are muscle and cardiac tissue. All identified studies (animals, in vitro, and humans) focused on muscle and cardiac effects.

- Acute toxicity with emetine appears to be related to cumulative dose exposure, but also appears completely reversible ~3 weeks after cessation of therapy.

<table>
<thead>
<tr>
<th>Species</th>
<th>Fatal Cumulative Dose</th>
<th>ROA</th>
</tr>
</thead>
<tbody>
<tr>
<td>Mice</td>
<td>50-100 mg/kg</td>
<td>IP</td>
</tr>
<tr>
<td>Rats</td>
<td>10-25 mg/kg</td>
<td>IP</td>
</tr>
<tr>
<td>Rabbits</td>
<td>10-30 mg/kg</td>
<td>PO</td>
</tr>
<tr>
<td>Cats</td>
<td>10-25 mg/kg</td>
<td>PO</td>
</tr>
<tr>
<td>Dogs</td>
<td>6 mg/kg</td>
<td>IP</td>
</tr>
<tr>
<td>Humans</td>
<td>15-20 mg/kg</td>
<td>SQ</td>
</tr>
</tbody>
</table>
Patients with solid tumors treated with 1 mg/kg/day emetine daily via SC injection for 10 days (cumulative dose 650 mg) did not experience any notable toxicity\(^1\)

Electrocardiographic abnormalities were observed, but not often associated with significant cardiac symptoms\(^2,3,4\):
- T wave inversion (TWI) is the first to appear and the last to disappear
- Q-T interval prolongation
- The average time required for complete return of the tracing to normal is ~six weeks

At higher cumulative doses (e.g. ≥ 650 mg): hypotension, tachycardia, cardiomyopathy, myocarditis, precordial pain, gallop rhythm (on auscultation), dyspnea, cardiac dilatation, congestive failure, and death have been reported\(^5\)

Toxicity with emetine appears to be cumulative-dose related and independent of schedule\(^1,6\)

Complete reversibility of cardiac adverse effects\(^6\)

5 Bleasel M, et al. Pharmaceuticals 2020, 13, 51
Financial Overview

• Cash as of September 30, 2021: **$14.2M**
  - Plus $10.0M Second Development Payment in two tranches following FDA acceptance for filing and review of an NDA for ACER-001 in a UCD per Collaboration Agreement with Relief
  - Expected to have sufficient capital to fund current operations into mid-2022$

• Capitalization as of November 19, 2021:
  - 14.3M shares of common stock outstanding
  - 16.3M shares of common stock fully diluted

• $100.0M invested through November 19, 2021, excluding Relief collaboration

• No debt, no warrants

$ Excluding the planned ACER-801 and EDSIVO™ clinical trials
ACER

Summary

- Acer’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)
  - **ACER-801** (osanetant) for the treatment of induced vasomotor symptoms (iVMS)
  - **EDSIVO™** (celiprolol) for the treatment of vascular Ehlers-Danlos syndrome (vEDS) in patients with a confirmed type III collagen (COL3A1) mutation
  - **ACER-2820** (emetine) a host-directed therapy against a variety of infectious diseases

- Product candidates are believed to present a comparatively de-risked profile, having one or more of:
  - Favorable safety profile; clinical proof-of-concept data; mechanistic differentiation
  - Potential expedited paths for development through specific FDA-established programs

- Multiple anticipated key milestones:
  - ✓ **ACER-801 IND submitted:** Q4 2021
  - **EDSIVO™ IND submission, work toward SPA, request BTD:** Q4 2021
  - **ACER-801 Phase 2a clinical trial initiation**: Q1 2022
  - **EDSIVO™ DiSCOVER clinical trial initiation**: Q1 2022
  - **ACER-001 (UCDs) PDUFA target action date:** June 5, 2022

* Subject to IND clearance
** Subject to successful IND submission and clearance, and agreement on SPA
$ Subject to additional capital
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 E, et al. 2011 Neuropsychiatric and Neurochemical Sequelae of MSUD
ACER-001 MSUD: Clinical POC Study

**Design:** Open label pilot study\(^1\) at Baylor College of Medicine – 3 healthy and 5 MSUD subjects with late onset disease

- 3 days of steady-state protein diet\(^*\); then 3 days of NaPB + diet\(^*\)
- BCAAs and BCKAs determined at day 3 of each study period (4 time points)

**Results:** NaPB showed a statistically significant reduction of leucine in all 3 healthy subjects (p< 0.05) and 3 out of 5 MSUD patients (p< 0.05 in responders)

- ~30% reduction (28-34%) in leucine in MSUD responders
- Clinicians view >20-30% ↓ as clinically meaningful\(^\star\star\)

**Comments:** 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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\(^\star\) All subjects received a constant protein intake of 0.6 g/kg/day as combination of BCAA-free formula and whole protein

\(^\star\star\) Acer commissioned market research
MSUD: Market Opportunity

• About 1,000 MSUD patients in the U.S., ~3,000 WW*
  • 20-25% MSUD patients in U.S. are Mennonite; incidence up to 1/380
  • Ashkenazi Jewish population; incidence of 1/26,000

• No treatments currently approved for MSUD

• Early treatment may help reduce the rate of neuropsychological comorbidities and optimize growth**

• MSUD specialists recognize NaPB’s potential effectiveness, yet tolerability is a concern***

• Anticipate initiation of clinical studies in MSUD in 2022

* https://www.ncbi.nlm.nih.gov/books/NBK1319/