Corporate Presentation

March 27, 2023

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 press release are forward-looking statements. Examples of such statements include, but are not limited to, statements about plans for the commercialization of OLPRUVA™ for oral suspension in the U.S. for the treatment of certain patients with UCDs involving deficiencies of CPS, OTC, or AS, including negotiations with commercial payers and Medicaid organizations regarding access as well as the timing of drug availability and the expected commercial launch, statements about plans and potential milestones for the continued clinical development of OLPRUVA™ in other indications, statements about plans and potential milestones for the continued clinical development of EDSIVO™ for treatment of vEDS in patients with a confirmed type III collagen (COL3A1) mutation, statements about plans for the development of ACER-801, statements about our anticipated 2023 milestones, and statements about our capital requirements and sufficiency and duration of our current cash and cash equivalents. Our efforts to commercialize OLPRUVA™ for oral suspension in the U.S. for the treatment of certain patients with UCDs involving deficiencies of CPS, OTC, or AS are at an early stage, we currently do not have fully developed marketing, sales or distribution capabilities, and there is no guarantee that we will be successful in our commercialization efforts. Our pipeline products (including OLPRUVA™ for indications other than UCDs as well as EDSIVO™ and ACER-801) are under investigation and their safety and efficacy have not been established and there is no guarantee that any of our investigational products in development will receive health authority approval or become commercially available for the uses being investigated. 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, the availability of financing to fund our commercialization efforts, our pipeline product development programs and our general corporate operations as well as risks related to drug development and the regulatory approval process, including the timing and requirements of regulatory actions. 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 Annual Report on Form 10-K and Quarterly Reports on Form 10-Q. You may access these documents for no charge at http://www.sec.gov.

THE ACER STORY

ACER

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

Our Mission

To provide transformative treatments with a human touch to underserved or overlooked patients with rare and life-threatening diseases

Our Goal

To develop treatments quickly and more efficiently and deliver them to patients as fast as possible

Our Difference

We identify and develop treatments where science can be applied in new ways for use in diseases with high unmet need

ACER MANAGEMENT TEAM

CHRIS SCHELLING
CHIEF EXECUTIVE OFFICER & FOUNDER
BIOMARIN

ADRIAN QUARTEL, MD
CHIEF MEDICAL OFFICER
BIOMARIN

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genzyme

JOHN KLOPP
CHIEF TECHNICAL OFFICER
BIOMARIN

DON JOSEPH, JD
CHIEF LEGAL OFFICER
bvgh
BIO Ventures for Global Health

BERNIE PAUL
CHIEF PEOPLE OFFICER
clarisonic

PIPELINE

Program	Indication	Phase 1	Phase 2	Phase 3	Approved	Expected Milestones
OLPRUVA™ (sodium phenylbutyrate) for oral suspension	Urea Cycle Disorders[1]					**Approved** in US; drug in channel anticipated July 2023[$]
EDSIVO™ (celiprolol)	Vascular Ehlers-Danlos Syndrome (COL3A1+)					**Q4 2023**: Full trial enrollment[$]
ACER-801 (osanetant)	Menopausal-related Vasomotor Symptoms					**Q1 2023**: Topline P2a VMS data announced; program paused
	Prostate Cancer					**Q1 2023**: POSH & PORT trials initiated[2]; program paused
	Acute Stress/PTSD					Program paused[3]

1 OLPRUVA™ (sodium phenylbutyrate) for oral suspension approved in the U.S. for the treatment of certain patients living with urea cycle disorders (UCDs) involving deficiencies of carbamylphosphate synthetase (CPS), ornithine transcarbamylase (OTC), or argininosuccinic acid synthetase (AS)
$ Subject to additional capital
2 University of Kansas investigator-sponsored trial
3 University of North Carolina investigator-sponsored trial

OLPRUVA™

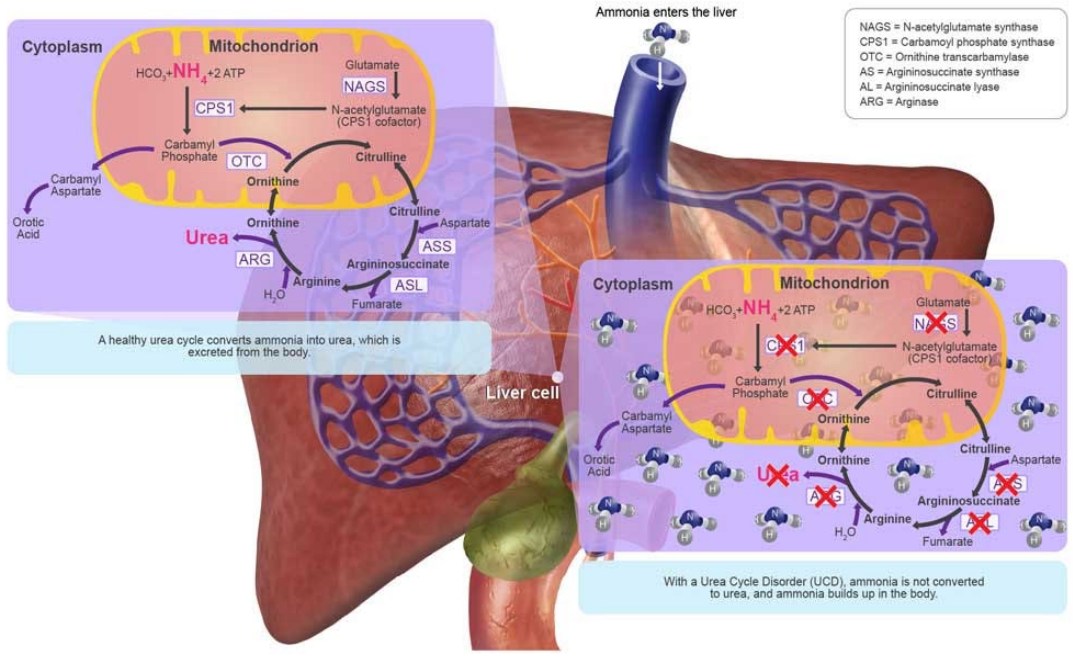
OLPRUVA™
(sodium phenylbutyrate) for oral suspension

Full Prescribing Information:
https://www.acertx.com/OLPRUVA/PI.pdf

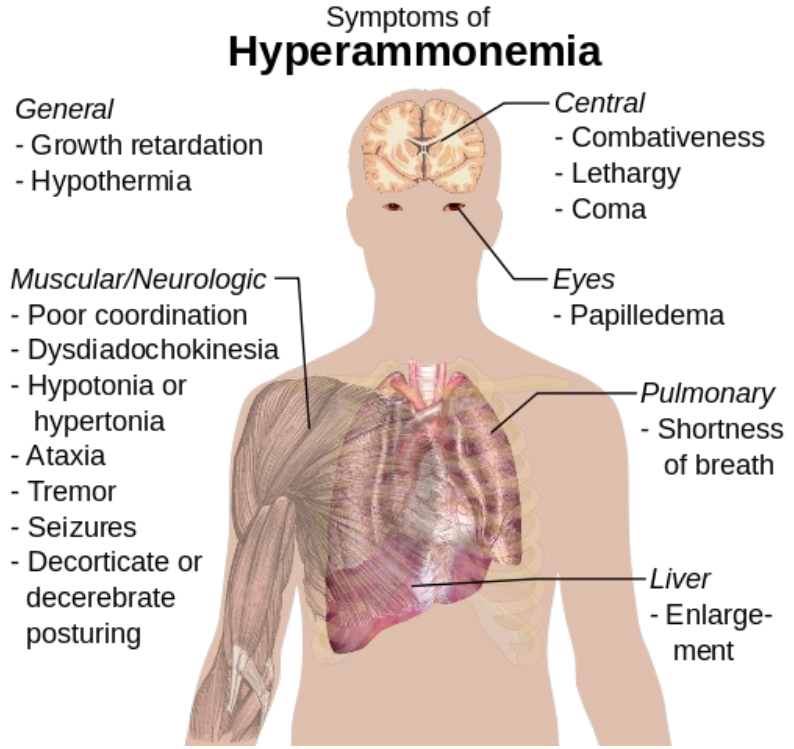
UCDs: DISEASE OVERVIEW

OLPRUVA™

- Urea Cycle Disorders (UCDs) are a group of rare, genetic disorders caused by mutations that result in a deficiency of one of the six enzymes or two transporters of the urea cycle
- These enzymes are responsible for removing ammonia from the bloodstream



- Elevated ammonia levels in both symptomatic and asymptomatic patients can be neurotoxic leading to neurocognitive damage, among other symptoms



Reproduced from: http://upload.wikimedia.org/wikipedia/commons/7/76/Symptoms_of_hyperammonemia.svg.

UCDs: MECHANISM OF ACTION

Nitrogen Binding Agents

Alternative pathway treatment diverts nitrogen from the urea cycle to alternate routes of excretion

Nitrogen binding agents, containing phenylbutyrate, are all metabolized to phenylacetate (PAA)

PAA is the active moiety – it combines with glutamine, producing phenylacetylglutamine

Phenylacetylglutamine (PAGN) is then excreted by the kidneys in the urine

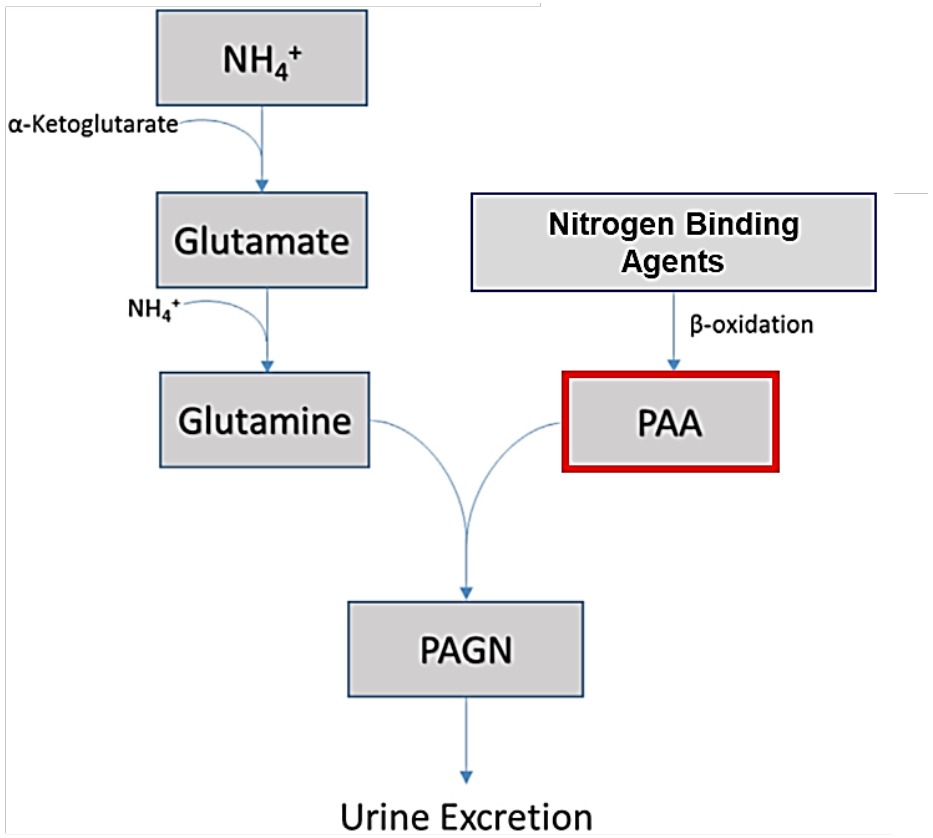
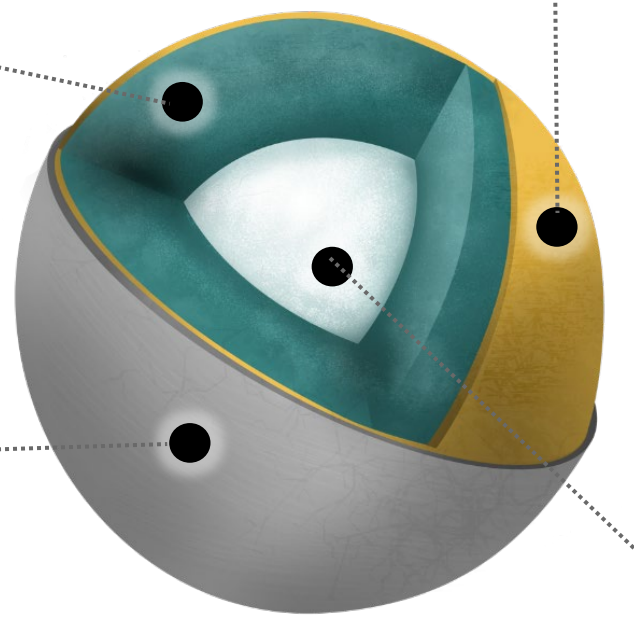


Fig. 1. Metabolizing pathway and mechanism of action of GPB. GPB (glycerol phenylbutyrate); PAA (phenylacetic acid); PBA (phenylbutyric acid); PAGN (phenylacetylglutamine).

Berry S, et al. Molecular Genetics and Metabolism 125 (2018) 251–257.

NOVEL FORMULATION

OLPRUVA™ is a proprietary and **novel formulation of NaPB powder** that has shown bioequivalence to existing NaPB powder but with a pH-sensitive polymer coating that is **designed to minimize dissolution of the coating for up to 5 minutes after preparation**[1]



Drug Layer
(NaPB)

Delivers the proven ammonia control of NaPB powder[1]

Seal Coat
(inactive ingredients)

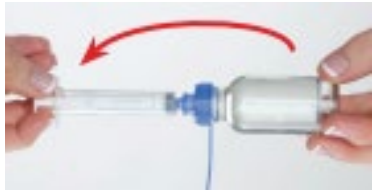
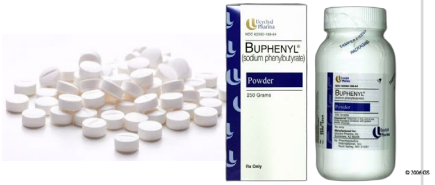
Polymer Coat
(inactive pH-sensitive polymer)

Designed to minimize dissolution of the coating over 5 minutes after preparation

Seed Core
(inactive core)

1 Acer Therapeutics. Data on File. 2021.

NITROGEN SCAVENGER DIFFERENTIATION[1]

Phenylbutyrate Formulations				
	OLPRUVA™	**RAVICTI®**	**BUPHENYL®**	**PHEBURANE®**
Efficacy / Safety in UCDs	✓	✓	✓	✓
Formulation	Dual-coated oral pellets	Clear Oily Liquid	Powder or Tablets	Single-coated oral pellets
Palatability	Up to 5 minutes	Tasteless	Bitter Taste	Up to 10 seconds
Packaging	Single-dose Envelopes	Glass vials with syringe for each dose	Tub of powder or pills	Large bottle of pellets
Portability[2]	+ +	+	-	+
Administration	Mix w/ water and Mix-Aid 	Meter dose into syringe from glass vial 	Measure powder and mix with water or take up to 40 tablets per day 	Pour directly into mouth or sprinkle on each bite of apple sauce or carrot puree 

1 No head-to-head studies have been conducted with OLPRUVA™ and any of the other products named, other than the bioequivalence study vs NaPB powder undertaken for 505(b)(2) pathway
2 Trinity Health Partners June 2022
RAVICTI®, BUPHENYL®, PHEBURANE® information sourced from prescribing information

COMMERCIAL STRATEGY

Significant unmet need and desire for new treatment options

OLPRUVA™ is differentiated, dual coated, packaged in portable single dose envelopes

Robust patient services program to support patients & caregivers

Customized exclusive pharmacy approach to facilitate patient access to care

Best in class team of rare disease professionals with deep relationships in UCD centers

Commercial Launch Strategy = Positive Patient Experience + Exceptional Support

DISRUPTING AN ESTABLISHED UCD MARKET IN U.S.

OLPRUVA™

Projected Prevalence[1]	**~ 2,100**
Diagnosed Patients[2]	**~ 1,100**
Patients Treated with Phenylbutyrate[2] (Ravicti®, Buphenyl®, generics)	**~ 800**
Market share for nitrogen binding agents[2]	**~ 80% Ravicti®** **~ 20% Buphenyl® or generic NaPB**
2021 U.S. Net Revenue[3] (Ravicti®, Buphenyl®)	**~ $300M**

1 https://www.drugs.com/slideshow/top-10-most-expensive-drugs-1274
2 HealthVerity Payer claims data analysis
3 https://ir.horizontherapeutics.com/static-files/47f395cb-4d8e-47a7-ba20-2f3c6f433e62; Represent North American sales

INTELLECTUAL PROPERTY AND EXCLUSIVITY

- Orange Book Listed Patents:
  - US Pat. Nos. 11,154,521 and 11,433,041 directed to formulations/compositions of matter
  - US Pat. No. 11,202,767 directed to methods of use (UCD)
    - Expiration date for OB patents is 10/17/2036
- MSUD
  - US Pat. Nos. 9,078,865 and 10,092,532 directed to methods of decreasing branched chain acids or MSUD
    - Licensed from Baylor College of Medicine
    - Expiration date is 7/26/2030
- Combination with Benzoate
  - US Pat. No. 11,517,547 directed to a kit comprising a combination therapeutic product composed of sodium phenylbutyrate or glycerol phenylbutyrate and sodium benzoate
    - Licensed from Baylor College of Medicine
    - Expiration date is 6/28/2038
- Continuing to pursue new patents and exclusivity possibilities, based on development plans and product attributes

LIFECYCLE OPPORTUNITIES

Acer intends to explore additional lifecycle opportunities for OLPRUVA™ (sodium phenylbutyrate) in various disorders where proof of concept data exists:

- Maple Syrup Urine Disease (MSUD)
- Pyruvate Dehydrogenase Complex Deficiency (PCDC)
- Rare pediatric epilepsies
- Various liver disorders

EDSIVO™

(celiprolol)

A selective adrenergic modulator (SAM) for the potential treatment of patients with COL3A1-positive vascular Ehlers-Danlos Syndrome (vEDS)

Vascular Ehlers-Danlos Syndrome (vEDS)

VASCULAR EHLERS-DANLOS SYNDROME

EDSIVO™

DISEASE OVERVIEW

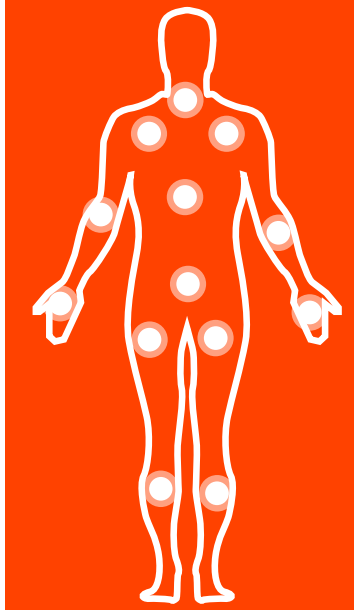
- Autosomal dominant connective tissue disorder of collagen synthesis caused by mutations in the COL3A1 gene for type III procollagen
- Characterized by arterial aneurysms, dissections and/or ruptures in arteries and hollow organs (intestines, uterus, lungs, etc.)

UNMET NEED

- No approved therapeutic options for vascular Ehlers-Danlos Syndrome (vEDS) patients
- Following the publication of the BBEST trial, celiprolol has become the primary treatment for vEDS patients in several European countries[2]

OPPORTUNITY

- Efficacy data from BBEST clinical trial showing reduction in risk of arterial events observed in COL3A1+ subpopulation[3]
- Additional data from long-term observational study in France[4]
- New Chemical Entity w/Orphan Drug Designation
- Issued patents providing protection until 2038
- DiSCOVER Phase 3 decentralized (virtual) pivotal trial ongoing w/Breakthrough Therapy Designation, Special Protocol Assessment



EPIDEMIOLOGY

Prevalence:

Up to 7,500 COL3A1+ vEDS patients in US[5]

Median US Survival:

51 years of age[1]

Risk:

Arterial rupture or dissection events occur in ~25% of patients before the age of 20, but **increase to ~90%** of patients by age 40[1]

1 Pepin, et al. Survival is affected by mutation type and molecular mechanism in vascular Ehlers–Danlos syndrome (EDS type IV). Genet Med. 2014 Dec;16(12):881-8.
2 https://www.ehlers-danlos.com/celiprolol-and-veds/
3 Ong K, et al. Lancet 2010; 376: 1476-84.
4 Frank M, et al. Vascular Ehlers-Danlos Syndrome: Long-Term Observational Study. J Am Coll Cardiol. 2019 Apr, 73 (15) 1948–1957
5 Truven MarketScan database and U.S> population data
$ Subject to additional capital

BBEST TRIAL: COL3A1+ SUBPOPULATION

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

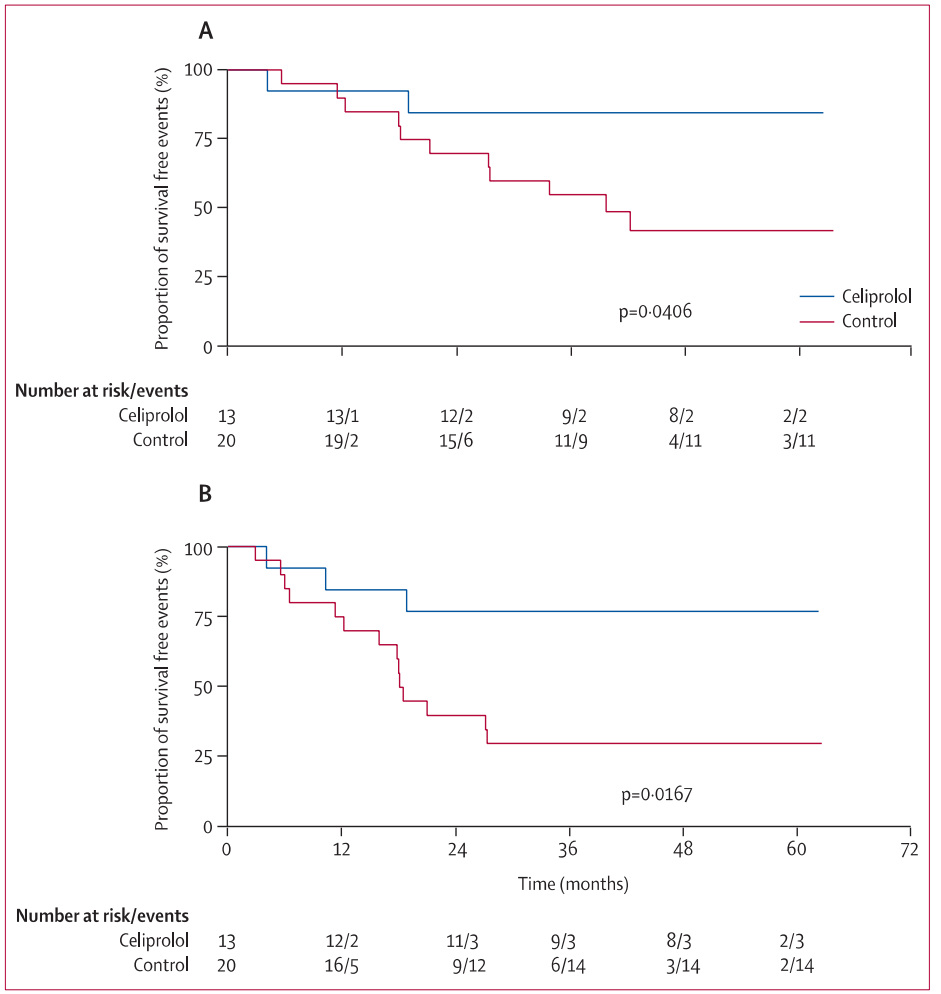


***Figure 3*: Kaplan-Meier curves of event-free survival in 33 patients with positive *COL3A1* mutation**
Primary endpoint (A). Primary and secondary endpoints (B).

Source: Ong K, et al. Lancet 2010; 376: 1476-84.

PIVOTAL PHASE 3 TRIAL: ENROLLING PATIENTS

EDSIVO™ Program Status

- ✓ Granted Breakthrough Therapy designation (BTD) by FDA
- ✓ Reached agreement with FDA on critical elements of protocol design under a Special Protocol Assessment (SPA)
- ✓ Launched discoverceliprolol.com as an educational tool for interested parties
- ✓ Initiated pivotal DiSCOVER trial (study NCT05432466)
- Q4 2023: Full enrollment anticipated by EOY 2023 based on current enrollment rates[$]
- Double-blind portion of trial intended to end if statistical significance is reached at an interim analysis (occurs at accrual of 28 vEDS-related events)
  - Estimated to occur as early as approximately 18 months after completion of full enrollment, or after accrual of 46 vEDS-related clinical events[$]

Decentralized Study of Celiprolol on vEDS-related Event Reduction (DiSCOVER) Trial

- **A Phase 3**, U.S.-based, randomized, double-blind, decentralized (virtual) clinical trial to compare the efficacy of celiprolol to placebo in the treatment of patients with COL3A1-positive vEDS
- **Primary objective**: compare time to first occurrence of a confirmed clinical event between celiprolol group and placebo group among confirmed COL3A1-positive vEDS patients
- **Secondary objectives**:
  - Safety and tolerability of celiprolol
  - Incidence rate of composite endpoint among vEDS patients treated w/ celiprolol vs. placebo

$ Additional capital required to conduct and complete the planned pivotal Phase 3 trial of EDSIVO™ beyond Q4 2022

ACER-801
(osanetant)

A novel, non-hormonal,
Neurokinin 3 receptor (NK3R) antagonist

- Vasomotor Symptoms
- Prostate Cancer
- Acute Stress & PTSD

VMS: PHASE 2a TRIAL

Endpoints

Study Population

49 women aged 40-65, who experience moderate to severe hot flashes

Primary Endpoints

1) Evaluate safety profile of ACER-801 at different doses
2) Evaluate PK profile of ACER-801 across dose levels

Key Secondary Endpoint

Evaluate the effect of ACER-801 at different doses on the frequency and severity of VMS associated with menopause versus placebo

Topline Results

- March 2023: ACER-801 was safe and well-tolerated but did not achieve statistical significance when evaluating ACER-801's ability to decrease the frequency or severity of hot flashes in postmenopausal women

Program Status

- March 2023: ACER-801 program paused in all indications until a thorough review of the full data set has been conducted

$ Subject to additional capital

VMS AND iVMS

ACER-801

DISEASE OVERVIEW

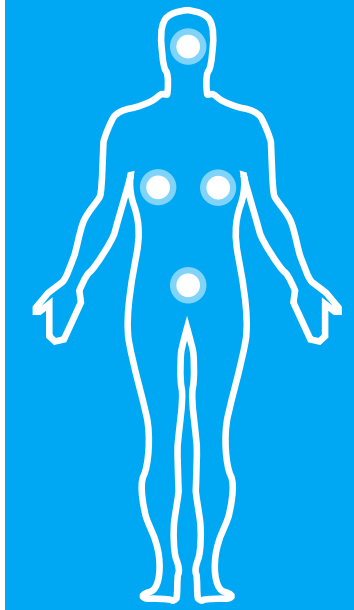
- Vasomotor symptoms are caused by a disruption in sex hormone signaling in the brain, **resulting in menopausal-like symptoms** (hot flashes, night sweats, etc.)

UNMET NEED

- 50% of women during menopause transition experience mod-severe hot flashes
- iVMS is a prevalent side-effect of various cancer treatments, with 60-80% reporting hot flashes[1,2,3]

MECHANISM OF ACTION

- A **novel, non-hormonal, neurokinin 3 receptor (NK3R) antagonist**
- Block stimulatory signaling of neurokinin B (NKB) through KNDy neurons with potential to control thermoregulation



EPIDEMIOLOGY

Moderate to Severe VMS Eligible Patients:

5.3M-7.1M in US (moderate-severe)[2]

iVMS Eligible Patients:

47,000 BRCA+ women in US that had PBSO[2]

120,000 women in US w/HR+ breast cancer receiving Tamoxifen[2]

1 Johnson L, et al. American Society for Reproductive Medicine, 2014 Vol 102 No. 3, Supplement, e249
2 Nichols H, et al., JNCI J Natl Cancer Inst, 2015, 1–8
3 Challapalli A, et al., Clinical and Translational Radiation Oncology 10 (2018) 29–35

ACER-801: PROSTATE CANCER

DISEASE OVERVIEW

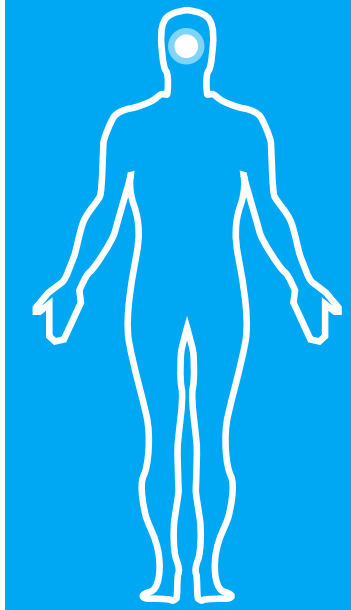
- A hormonally driven cancer - management for many men through suppression of testosterone production called androgen deprivation therapy (ADT)

UNMET NEED

- Most men on ADT treated with medications that suppress hormone production
- ADT treatment can cause dysfunctional thermoregulation and development of vasomotor symptoms (VMS) [1]

MECHANISM OF ACTION

- Early pharmacokinetic studies in men and women with various NK3R antagonists have shown an inhibitory effect on the levels of luteinizing hormone & testosterone[2,3]



EPIDEMIOLOGY

Prostate Treatment:

3.25 million men in US[3]

268,000 newly diagnosed patients with prostate cancer[3]

Of men who received a diagnosis of prostate cancer from 2003-2005, a total of 43.2% received ADT within 6 months after diagnosis[4]

Prostate iVMS:

75,000 men in the US w/HR+ prostate cancer receiving Leuprolide[5]

1 Trinity Partners 2020
2 Challapalli, Amarnath, et al. "Evaluating the Prevalence and Predictive Factors of Vasomotor and Psychological Symptoms in Prostate Cancer Patients Receiving Hormonal Therapy: Results from a Single Institution Experience." Clinical and Translational Radiation Oncology, Elsevier, 21 Mar. 2018
3 Prague J. et al. Neurokinin 3 receptor antagonism rapidly improves vasomotor symptoms with sustained duration of action. Menopause. 2018 Aug; 25(8): 862–869.

3 https://seer.cancer.gov/statfacts/html/prost.html
4 N Engl J Med 2010; 363:1822-1832
5 Nichols H, et al., JNCI J Natl Cancer Inst, 2015, 1–8

ACUTE STRESS & PTSD

ACER-801

DISEASE OVERVIEW

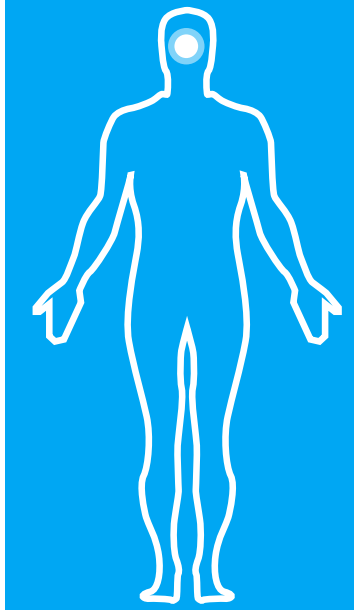
- **Acute Stress Disorder:** the body's immediate response to trauma
- **Post-traumatic Stress Disorder:** the long-term effects of trauma

UNMET NEED

- Existing approved therapies (sertraline, paroxetine) have limited efficacy

MECHANISM OF ACTION

- **ACER-801 targets region of the brain that controls the formation and consolidation of fear memories**, which may aid in preventing PTSD[1]
- Increased expression of Tac2 gene – NKB peptide – and activation of NK3R are involved in stress sensitization and over consolidation of fear
- Silencing of Tac2-expressing neurons impairs fear[1]



EPIDEMIOLOGY

PTSD Prevalence:

~12 million adults in US[3]

~60% of men and 50% of women experience at least 1 trauma in their lifetime

Up to 20% of those who experienced at least one traumatic event will develop PTSD[4]

Emergency Dept. Visits:

~1/3 of ED visits are for trauma exposure[4]

1 Andero R, Dias BG, Ressler KJ. A role for Tac2, NkB, and Nk3 receptor in normal and dysregulated fear memory consolidation. Neuron. 2014;83(2):444-454
2 Andero R, Daniel S, Guo JD, et al. Amygdala-Dependent Molecular Mechanisms of the Tac2 Pathway in Fear Learning. Neuropsychopharmacology. 2016;41(11):2714-2722
3 National Center for PTSD. How Common is PTSD in Adults?
4 Sidran Institute. Traumatic Stress Education & Advocacy Fact Sheet.

FINANCIAL OVERVIEW

ACER

CASH POSITION

AS OF DECEMBER 31, 2022

$2.3M

- +$4.1M gross proceeds from ATM sales in Q1 2023
- +$7.0M of SWK loan* gross proceeds in January 2023
- +$2.7M of registered direct offering** gross proceeds in March 2023
- **Extends cash runway into middle of Q2 2023**

CAPITALIZATION

AS OF MARCH 24, 2023

23.4M Shares of common stock outstanding

33.1M Shares fully diluted (incl. stock options, convertible notes*, and warrants)

HISTORICAL GROSS PROCEEDS

THROUGH MARCH 24, 2023

$116.2M equity financings

$35.0M from Relief Collaboration

$19.5M from debt financings*

$170.7M

* January 2023: https://www.sec.gov/ix?doc=/Archives/edgar/data/0001069308/000119312523019802/d427052d8k.htm
March 2022: https://www.sec.gov/ix?doc=/Archives/edgar/data/0001069308/000119312522066842/d279077d8k.htm
** March 2023: https://www.sec.gov/ix?doc=/Archives/edgar/data/0001069308/000119312523076670/d450883d8k.htm


acertherapeutics
Thank You
www.acertx.com