



Corporate Presentation

February 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 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 investigational product candidates to safely and effectively treat diseases and to be approved for marketing; our ability to close upon and obtain the proceeds of any identified financing arrangements as well as to satisfy the ongoing conditions and requirements for maintaining the financing facilities and avoiding default or an accelerated payment requirement; the commercial or market opportunity and potential of OLPRUVA™ for the treatment of patients with UCDs, including the opportunity for approval in territories outside of the United States; the commercial or market opportunity of any of our product candidates in any target indication and any territory; our ability, in addition to the currently identified financings, 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 OLPRUVA™ in MSUD, ACER-801, EDSIVO™ or our other investigational product candidates; 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. Our pipeline product candidates are under investigation, 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, risks and uncertainties associated with the ability to project future cash utilization and reserves needed for contingent future liabilities and business operations, the ability to launch successfully and sustain commercial viability of OLPRUVA™ for the treatment of patients with UCDs in the United States, 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 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>.



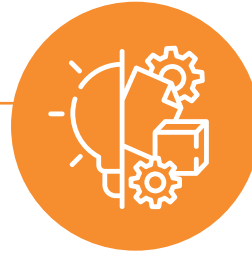
THE ACER STORY

Acer 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
B:OMARIN



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HARRY PALMIN
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JOHN KLOPP
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DON JOSEPH, JD
CHIEF LEGAL
OFFICER
bvgh
BIO Ventures for Global Health



BERNIE PAUL
CHIEF PEOPLE
OFFICER
clarisonic



PIPELINE

ACER

Program	Indication	Phase 1	Phase 2	Phase 3	Approved	Expected Milestones
OLPRUVA™ (sodium phenylbutyrate) for oral suspension	Urea Cycle Disorders ¹					Now Approved in US 12/22/2022
ACER-801 (osanetant)	Vasomotor Symptoms					Q1 2023: Topline Phase 2a trial data in VMS
	Prostate Cancer					Q1 2023: POSH & PORT trials initiated ²
	Acute Stress/PTSD					Q2 2023: OASIS trial initiation ³
EDSIVO™ (celiprolol)	Vascular Ehlers-Danlos Syndrome (COL3A1+)					Q4 2023: Full trial enrollment ^{\$}

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™

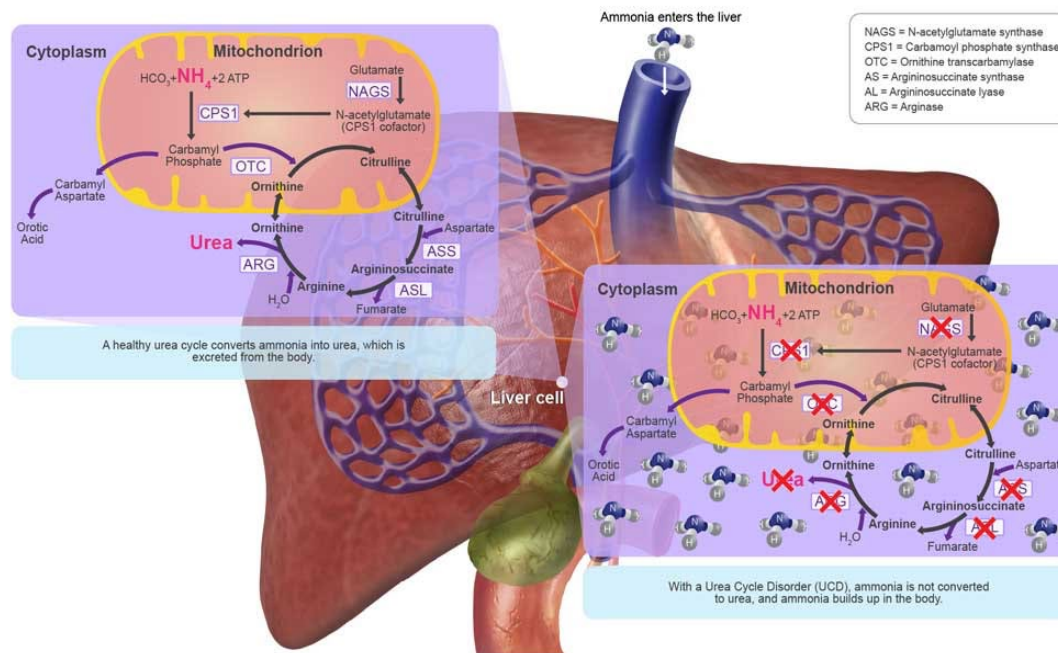
(sodium phenylbutyrate)
for oral suspension

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



UCDs: DISEASE OVERVIEW

- 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

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

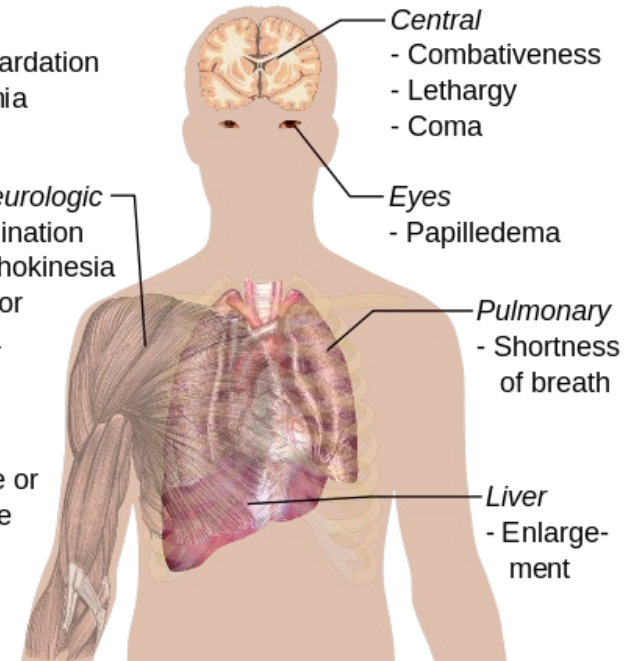
- Papilledema

Pulmonary

- Shortness of breath

Liver

- Enlargement



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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

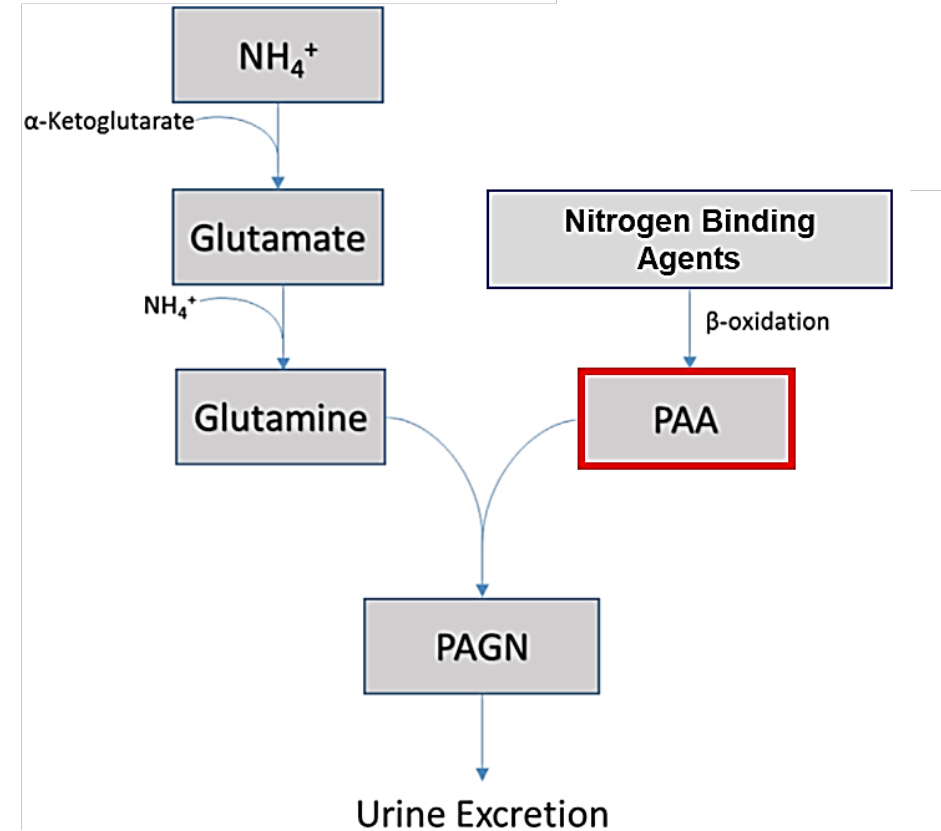
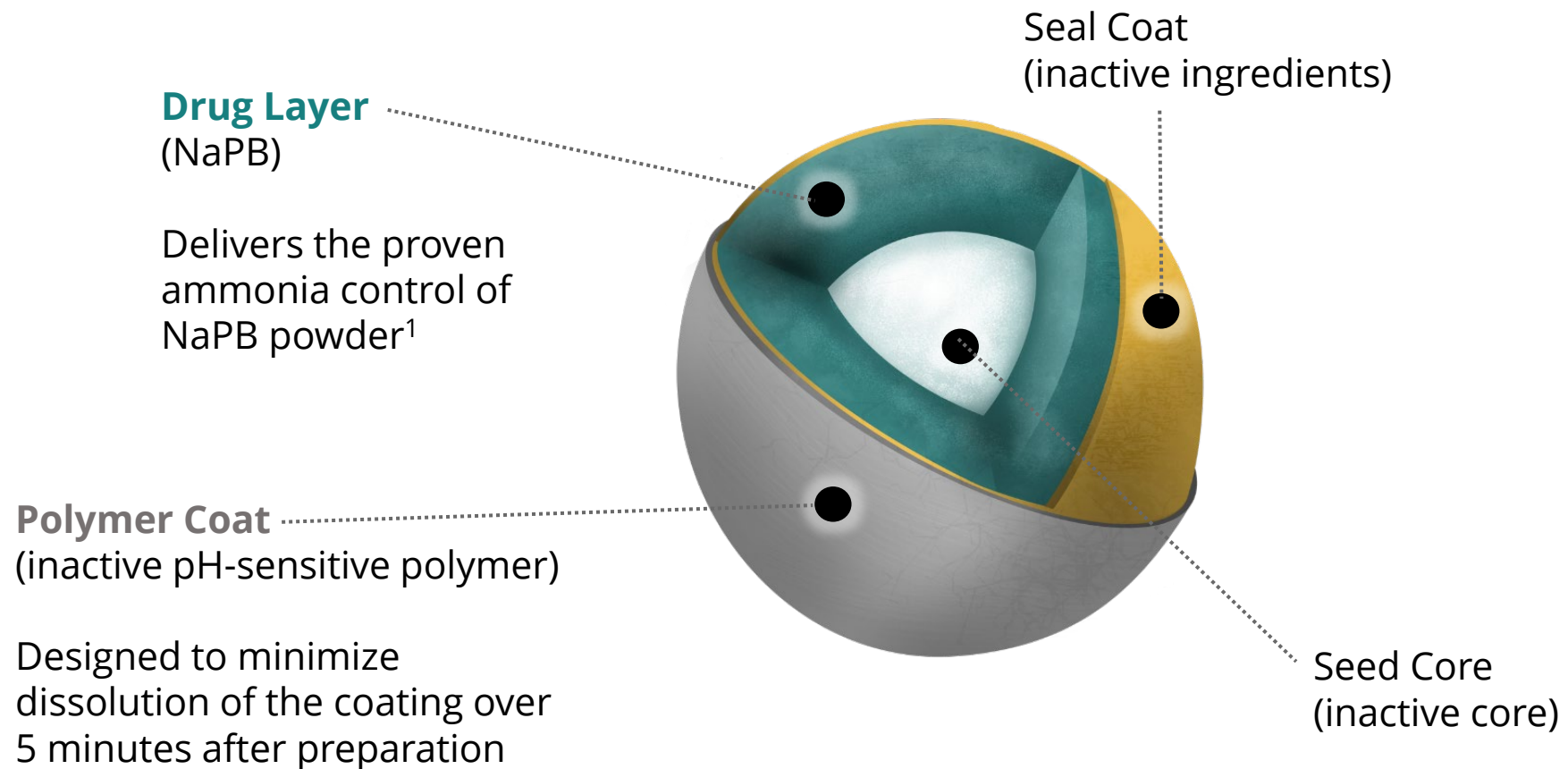


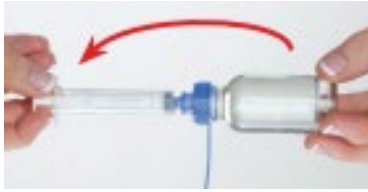
Fig. 1. Metabolizing pathway and mechanism of action of GPB. GPB (glycerol phenylbutyrate); PAA (phenylacetic acid); PBA (phenylbutyric acid); PAGN (phenylacetylglutamine).

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



NITROGEN SCAVENGER DIFFERENTIATION¹

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

OLPRUVA™, RAVICTI®, BUPHENYL®, and PHEBURANE® are the registered trademarks of their respective owners



COMMERCIAL STRATEGY

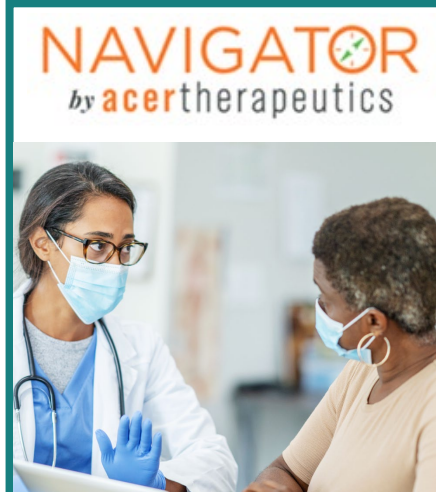
OLPRUVA™



Significant unmet need and desire for new treatment options



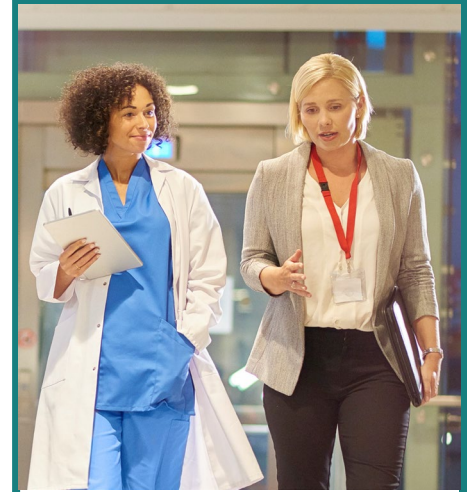
OLPRUVA™ is differentiated, dual coated, packaged in portable single dose envelopes



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.

Projected Prevalence¹	~ 2,100
Diagnosed Patients²	~ 1,100
Patients Treated with Phenylbutyrate² (Ravicti®, Buphenyl®, generics)	~ 800
Market share for nitrogen binding agents²	~ 80% Ravicti® ~ 20% Buphenyl® or generic NaPB
2021 U.S. Net Revenue³ (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 (IP)

- Issued patents:
 - US Patent 11,154,521 covering claims re: formulation compositions of matter (expires in 2036)
 - US Patent 11,202,767 covering claims re: certain methods of use (expires in 2036)
 - US Patent 10,092,532 covering claims re: methods of modulation of branched chain acids
 - Licensed from Baylor College of Medicine relating to MSUD
- Notice of Allowance:
 - US patent application No. 16,624,834 for claims re: a kit comprising a combination therapeutic product composed of sodium phenylbutyrate or glycerol phenylbutyrate and sodium benzoate
 - Licensed from Baylor College of Medicine
- 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

ACER-801

(osanetant)

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

Induced
Vasomotor
Symptoms

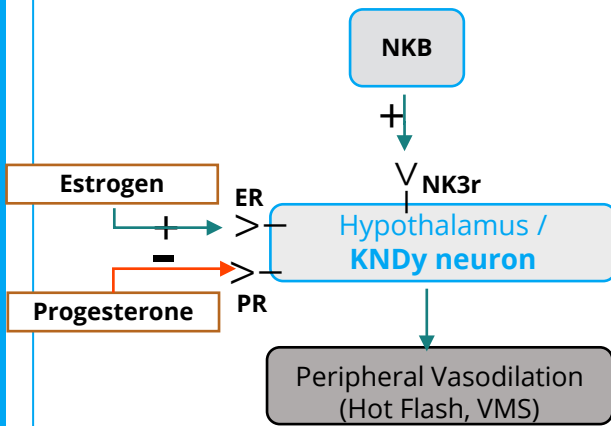
Prostate
Cancer

Acute
Stress &
PTSD



TARGETING KEY PATHWAYS OF NKB/NK3R SIGNALING

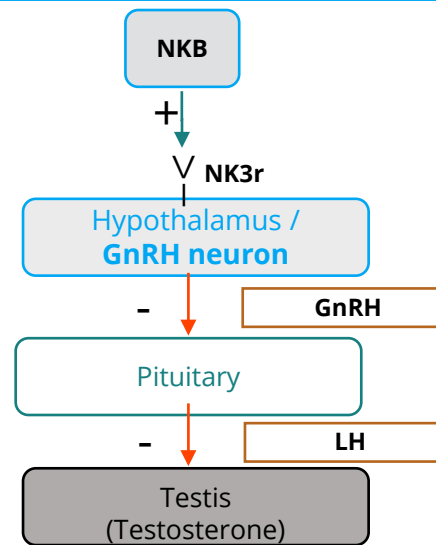
Vasomotor Symptoms



- Low Estrogen/High progesterone dysregulates NKB signaling in the hypothalamus leading to VMS
- Blocking NK3R prevents this dysregulation

- ACER-802-201 Trial
- POSH-MAP Trial (KUMC)

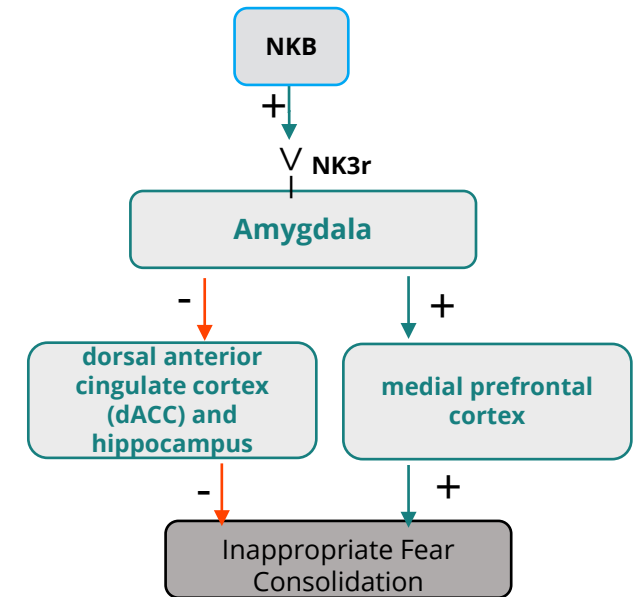
Testosterone Lowering



- Testosterone production is regulated through the Hypothalamus/Pituitary/Testis axis.
- GnRH production in the Hypothalamus is dependent on NK3R stimulation
- Blocking NK3R blocks testosterone production in the testes

- PORT-MAP Trial (KUMC)

PTSD / Fear Consolidation



- NK3R over-expression in the amygdala increases fear sensation signaling to dACC and hippocampus.
- This dysregulation causes inappropriate and exaggerated fear consolidation and PTSD

- OASIS Trial (UNC)



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}



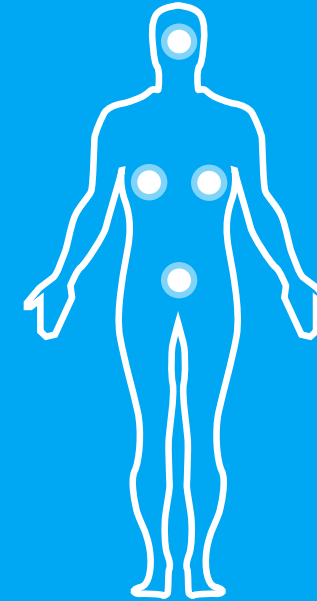
MECHANISM OF ACTION

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



OPPORTUNITY

- Potential for treatment with osanetant prior to removal of estrogen aims to control KNDy neuron hypertrophy and prevent vasomotor symptoms**
- Clinical and laboratory safety results are available from 23 completed Phase 1 and 2 studies (387 healthy subjects and 821 patients)¹ in various patient populations
- Phase 2a proof of concept trial topline data in menopause-related VMS anticipated in Q1 2023**



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 PBSO²

120,000 women in US w/HR+ breast cancer receiving Tamoxifen²

1 Johnson L, et al. American Society for Reproductive Medicine, 2014 Vol 102 No. 3, Supplement, e249

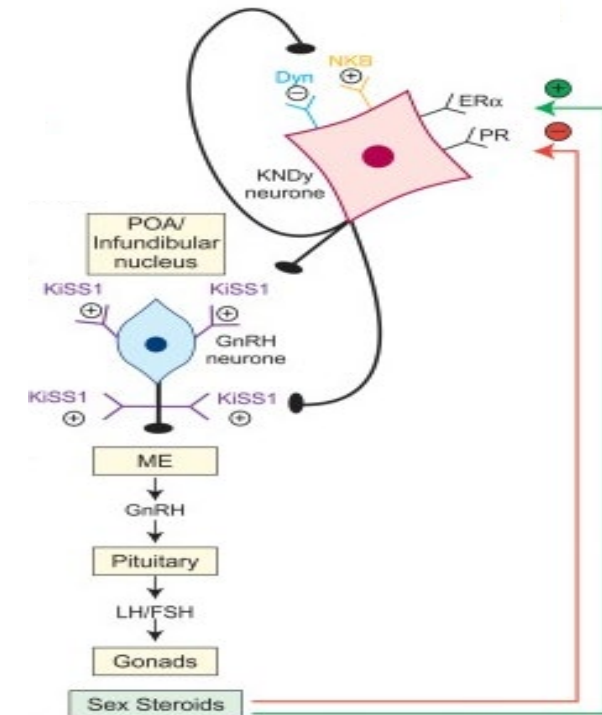
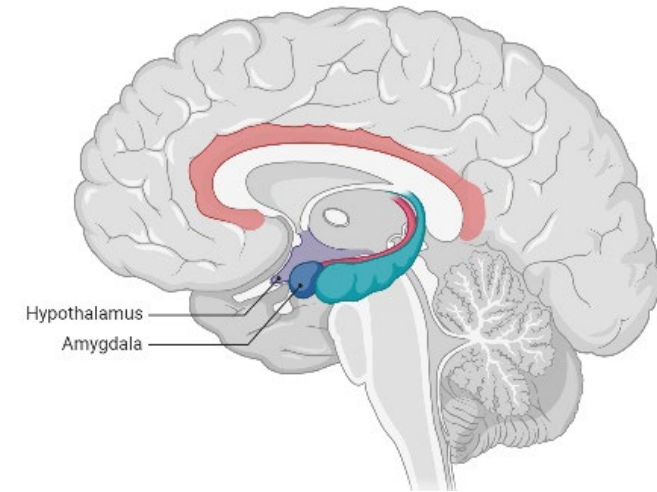
2 Nichols H, et al., JNCI J Natl Cancer Inst, 2015, 1-8

3 Challapalli A, et al., Clinical and Translational Radiation Oncology 10 (2018) 29-35



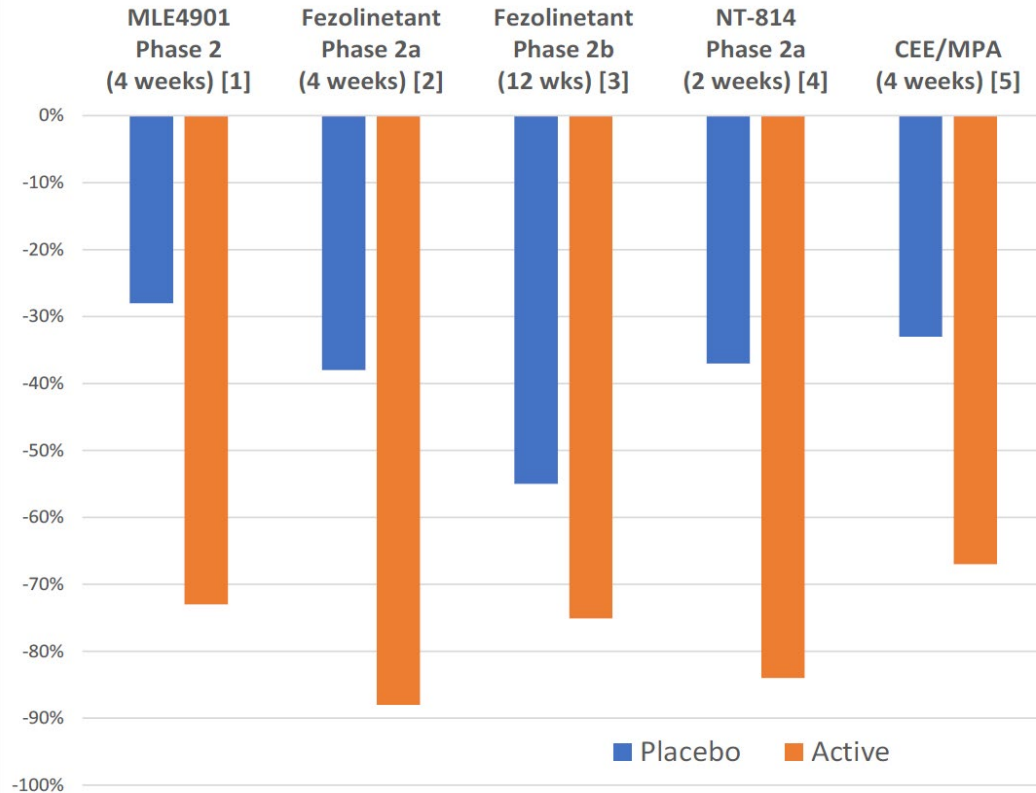
MOA: NEUROKININ-B (NKB) AND NK3 RECEPTOR

- Neurokinin B (NKB;TAC2 gene) is stimulatory molecule that activates the neurokinin 3 receptor (NK3R) found in the arcuate nucleus (ARC) of the hypothalamus and the central amygdala regions of the brain
- The hypothalamus controls body homeostasis and the kisspeptin/ neurokinin B/dynorphin (KNDy) neurons in the ARC are responsible for thermoregulation
- The central amygdala is critical for the perception of emotions, including fear memory consolidation
- Thermoregulation (hot flashes) is mediated by a balance between estrogen (inhibitory/protective) and neurokinin B (stimulatory) signaling in KNDy neurons



VMS REDUCTION ACROSS NK3R ANTAGONIST CLASS

% Change in Hot Flash Frequency From Baseline Across Individual Studies of NK3R Antagonists



- Three different NK3R antagonists have been associated with reductions of hot flashes in clinical studies in menopausal related vasomotor symptoms (MR-VMS)
 - MLE4901 (Millendo), fezolinetant (Astellas) and NT 814 (KaNDy Therapeutics)
- Effects appear similar across the class, and similar to estrogen, but with more rapid onset of action–
 - Reductions in both hot flash frequency and severity observed by day 3 in MLE4901 treated patients vs. placebo⁶

1: 4-week study; 40mg BID. Prague et al., 2017

2: 4-week study; 90mg BID. Depypere et al., 2019

3: 12-week study; 30mg QD. Fraser et al., 2020

4: 2-week study; 150mg QD. Trower et al., 2020

5: 4-week study; low dose (all doses similar). Utian, 2001

6: Prague et al., 2018

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 flashes¹
- Up to 35% complain of “extremely bothersome” symptoms up to two years after their surgery²

Women with HR+ Breast Cancer (CaB) receiving Tamoxifen

- **84% of women experienced hot flashes**³
- 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**⁴

Men with Prostate Cancer (CaP) receiving Leuprolide

- **80% of men experience hot flashes**⁵
- 15-27% of patients consider hot flashes the most distressing side effect
- 30-40% experienced moderate-to-severe symptoms
- **20% discontinued or disrupted treatment**

1 L. Johnson, et al. American Society for Reproductive Medicine, 2014 Vol 102 No. 3, Supplement, e249.

2 Griffiths, E, et al: The Obstetrician & Gynaecologist, 2005: 7:23-27.

3 Moon, Z. et al., JOURNAL OF PSYCHOSOMATIC OBSTETRICS & GYNECOLOGY, 2017 VOL. 38, NO. 3, 226-235

4 Nichols, H, et al., JNCI J Natl Cancer Inst, 2015, 1-8.

5 Challapalli, A, et al., Clinical and Translational Radiation Oncology 10 (2018) 29-35.



iVMS Unmet Need

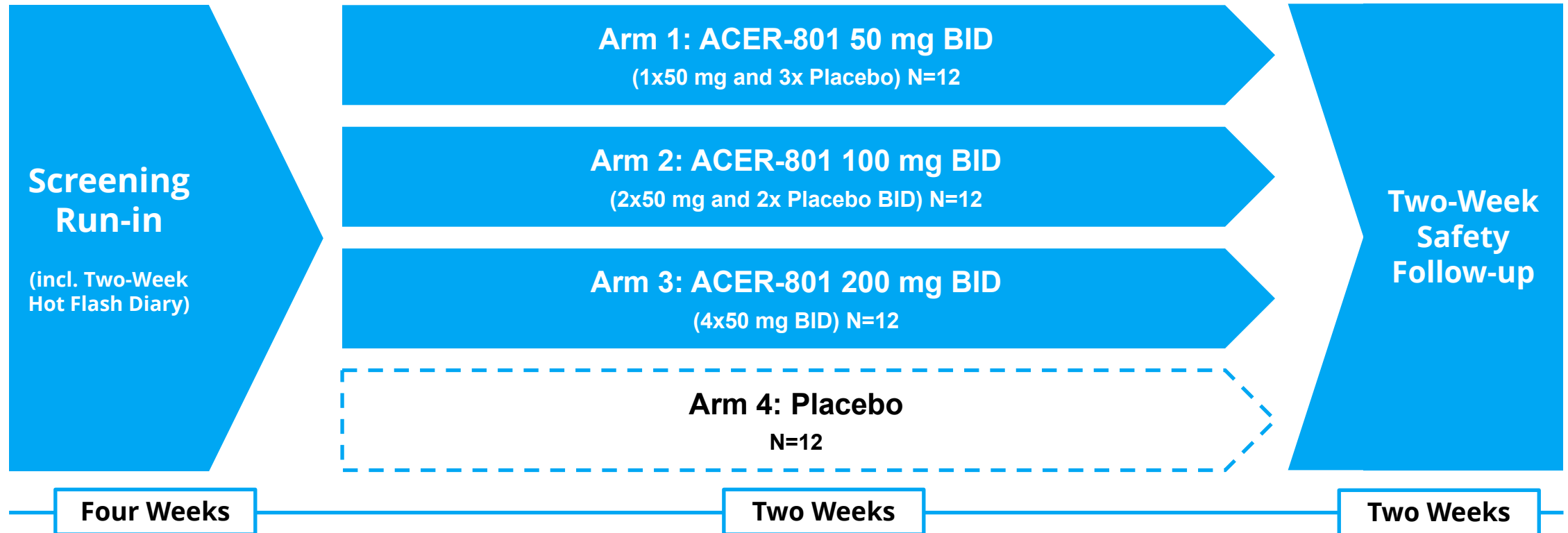
- iVMS are well documented with the use of cancer therapies and certain surgical procedures and symptoms appear immediately and can severely impact quality of life (QoL)
- Hormone Replacement Therapy (HRT) is contraindicated for the management of VMS in patients with hormone positive (HR+) tumors, including breast and prostate tumors
- A non-hormonal treatment for the management of moderate/severe iVMS is needed to help cancer patients start and stay on critical cancer therapy
- Non-adherence to cancer therapy can significantly shorten the time to recurrence and increase the mortality risk



VMS: PHASE 2 TRIAL

ACER-801

Ongoing ACER-801-201 Study: Topline Results Expected Q1 2023



Study Population

48 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

ACER-801

(osanetant)

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

Induced
Vasomotor
Symptoms

Prostate
Cancer

Acute
Stress &
PTSD



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



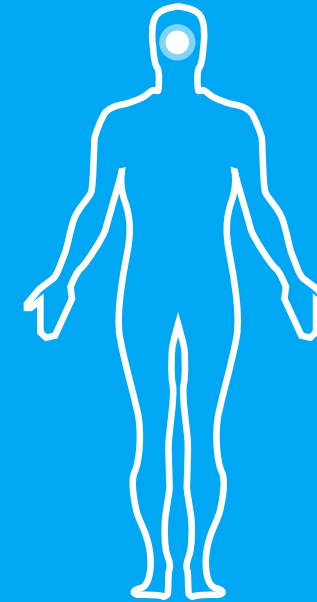
MECHANISM OF ACTION

- 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}



OPPORTUNITY

- A **non-hormonal treatment to lower testosterone levels and manage induced VMS** is needed as hormone replacement therapy (HRT) is contraindicated for the management of VMS in patients with hormone-positive prostate tumors



EPIDEMIOLOGY

Prostate Treatment:

3.25 million men in US³

268,000 newly diagnosed patients with prostate cancer³

Of men who received a diagnosis of prostate cancer from 2003-2005, a total of 43.2% received ADT within 6 months after diagnosis⁴

Prostate iVMS:

75,000 men in the US w/HR+ prostate cancer receiving Leuprolide⁵

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

PROSTATE CANCER: PILOT PHASE 2 TRIALS

2 Investigator-Sponsored Trials Ongoing at The University of Kansas Cancer Center:

PORT-MAP Trial (N=10)

Male prostate cancer patients undergoing curative intent surgery with testosterone >150ng/ml

Screening

ACER-801 (200 mg BID Twice Daily)
N=10

SOC
Surgery

Follow-up

Day -30

Day 7

Day 14

Day 28

Day 35

Day 56

Day 77

POSH-MAP Trial (N=10)

Male prostate cancer patients undergoing active ADT treatment with low testosterone levels ≤ 50 ng/dL and moderate to severe hot flashes

Screening

ACER-801 (200 mg BID Twice Daily)
N=10

Follow-up

ACER-801

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Cancer

Acute
Stress &
PTSD



ACUTE STRESS & PTSD



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



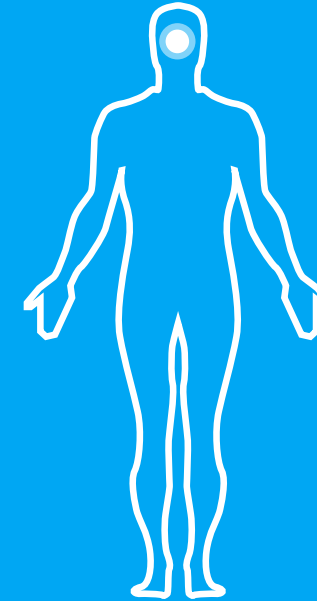
MECHANISM OF ACTION

- **ACER-801 targets region of the brain that controls the formation and consolidation of fear memories**, which may aid in preventing PTSD¹
- 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 fear¹



OPPORTUNITY

- **Treatment with osanetant has been shown to block a critical fear/stress sensitization step in the brain**^{1,2}
- UNC investigator-sponsored study planned in Q2 2023 to evaluate ACER-801's ability to reduce frequency and severity of ASD and PTSD



EPIDEMIOLOGY

PTSD Prevalence:

~12 million adults in US³

~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 PTSD⁴

Emergency Dept. Visits:

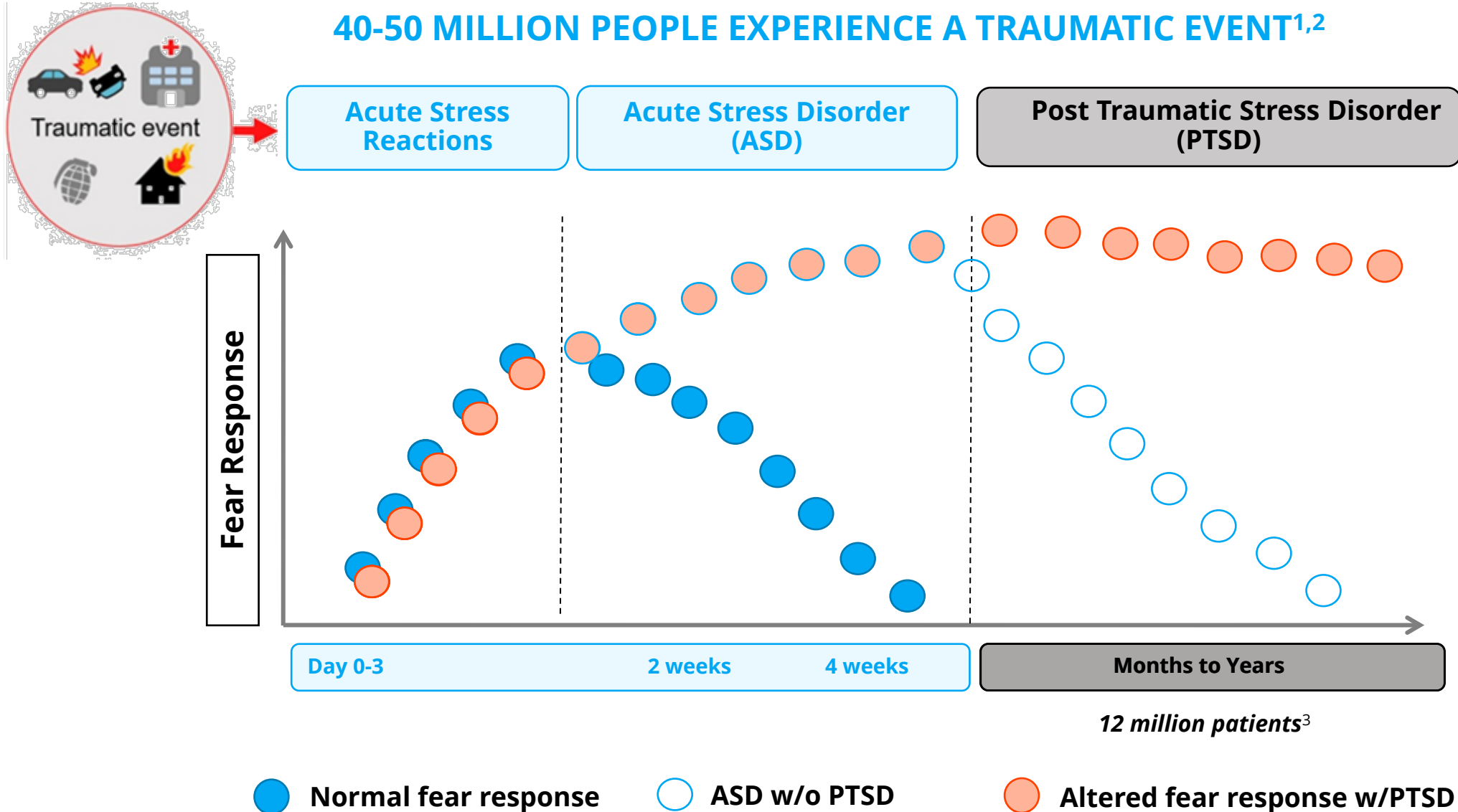
~1/3 of ED visits are for trauma exposure⁴

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.

DEVELOPMENT OF ASD & PTSD



¹ McLean et. al., The AURORA Study: a longitudinal, multimodal library of brain biology and function after traumatic stress exposure. Mol Psychiatry. 2020 Feb;25(2):283-296

² National Hospital Ambulatory Medical Care Survey: 2011 emergency department summary tables. 2011. (Accessed October 28, 2015, at http://www.cdc.gov/nchs/data/ahcd/nhamcs_emergency/2011_ed_web_tables.pdf.)

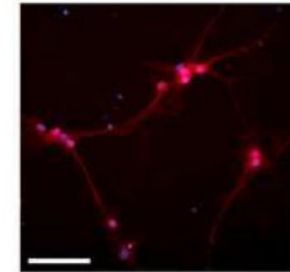
³ https://www.ptsd.va.gov/understand/common/common_adults.asp

MOA: OSANETANT CAN BLOCK FEAR CONSOLIDATION

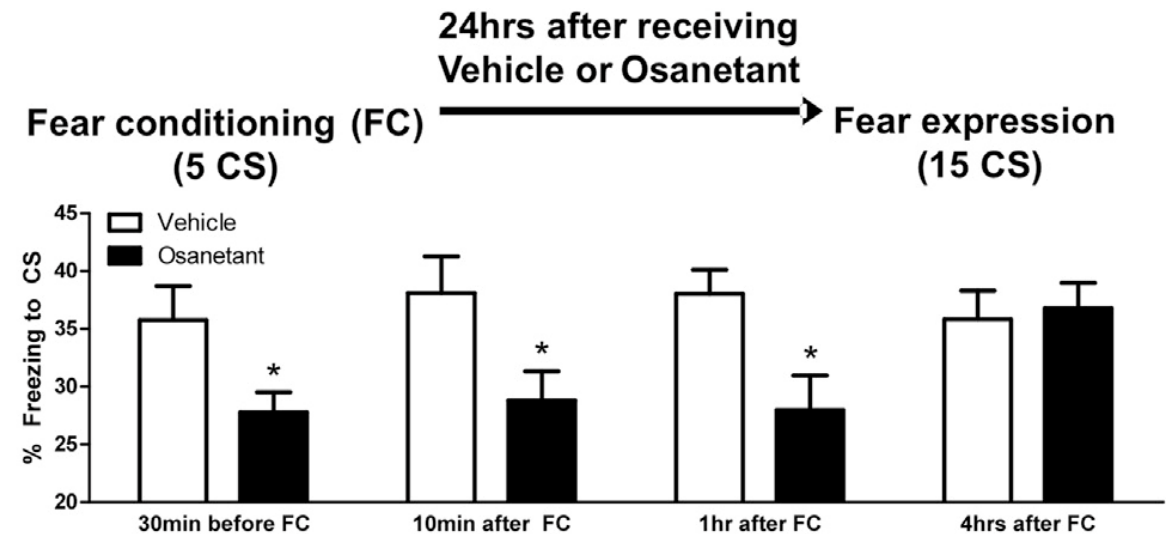
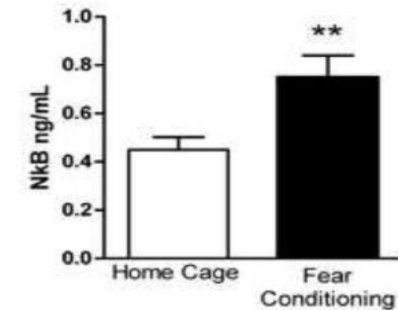
- NK3R activation is required for normal consolidation of fear memory formation
- Increased expression of the Tac2 gene (NK3 peptide) and activation of NK3R involved in stress sensitization and over consolidation of fear
- Genetic silencing of Tac2-expressing neurons impairs fear consolidation and blockade of this pathway
- Osanetant targets the NK3R in the amygdala and in vivo data has shown impairment of fear memory consolidation

Tac2 detected by immunocytochemistry in mouse amygdala cell culture

NkB in the Amygdala



NkB



PTSD: PARTNERSHIPS

ACER-801

Emory Agreement

- **Exclusive WW license to US patents and applications** covering certain methods of treating or preventing PTSD with osanetant
- Acer paid Emory a fee in exchange for exclusive license and will pay Emory certain development milestones and low single digit royalties on future product sales in these indications

University of North Carolina Partnership

- **Leading academic institution in trauma recovery**
- UNC awarded \$3 million grant from Department of Defense to investigate potential of ACER-801 (osanetant) to reduce frequency and severity of acute stress disorder and PTSD
- **UNC to conduct investigator-led trial in Q2 2023**

Osanetant After Stress to Increase Recovery Success (OASIS) Trial

- **Objective:** examine the safety / efficacy of ACER-801 to reduce acute stress response symptoms, PTSD symptoms & behavioral changes among emergency department patients after a motor vehicle collision
- **Trial to enroll 180 subjects who will be randomized in the emergency department**
- Patients to receive a low or high dose of ACER-801 or placebo and be discharged with a two-week supply of study drug

Participating OASIS Trial Sites

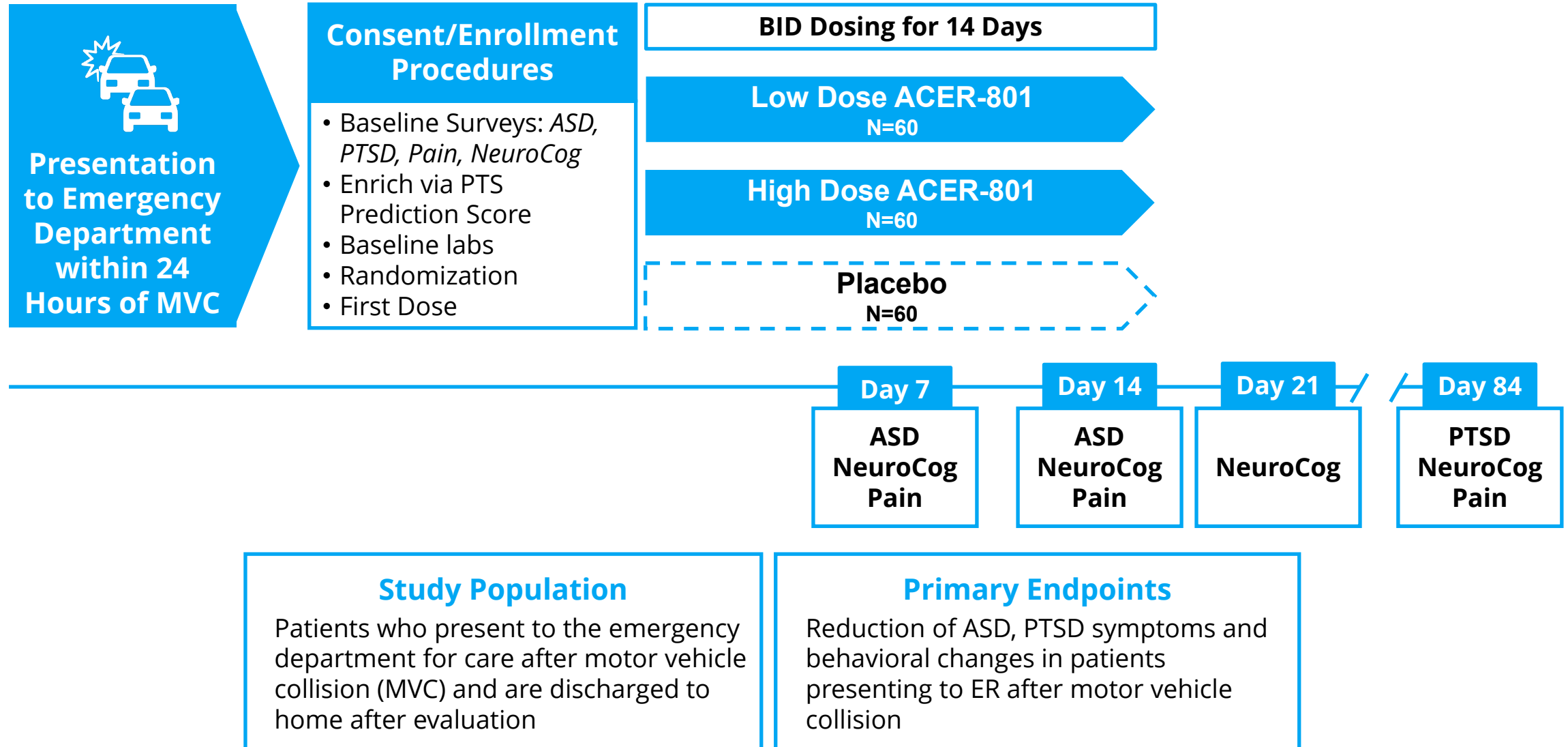
Washington University in St. Louis
University of Massachusetts Chan Medical School
Rhode Island Hospital
University of Florida College of Medicine – Jacksonville
Indiana University School of Medicine



PTSD: PHASE 2 TRIAL

ACER-801

Osanetant **A**fter **S**tress to **I**ncrease recovery **S**uccess (**OASIS Study**) (N=180)



VASCULAR EHLERS-DANLOS SYNDROME



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.)



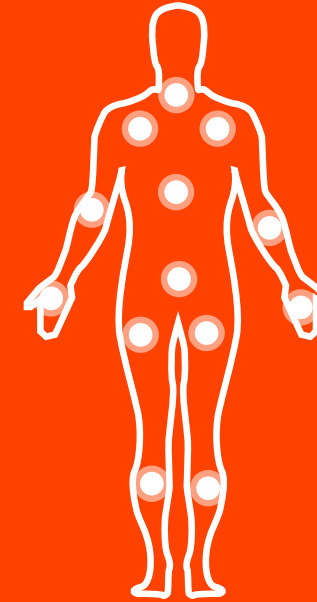
UNMET NEED

- 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 countries²



OPPORTUNITY

- Efficacy data from BBEST clinical trial showing reduction in risk of arterial events observed in COL3A1+ subpopulation³
- Additional data from long-term observational study in France⁴
- 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 US⁵

Median US Survival:

51 years of age¹

Risk:

Arterial rupture or dissection events occur in ~25% of patients before the age of 20, but **increase to ~90%** of patients by age 40¹

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

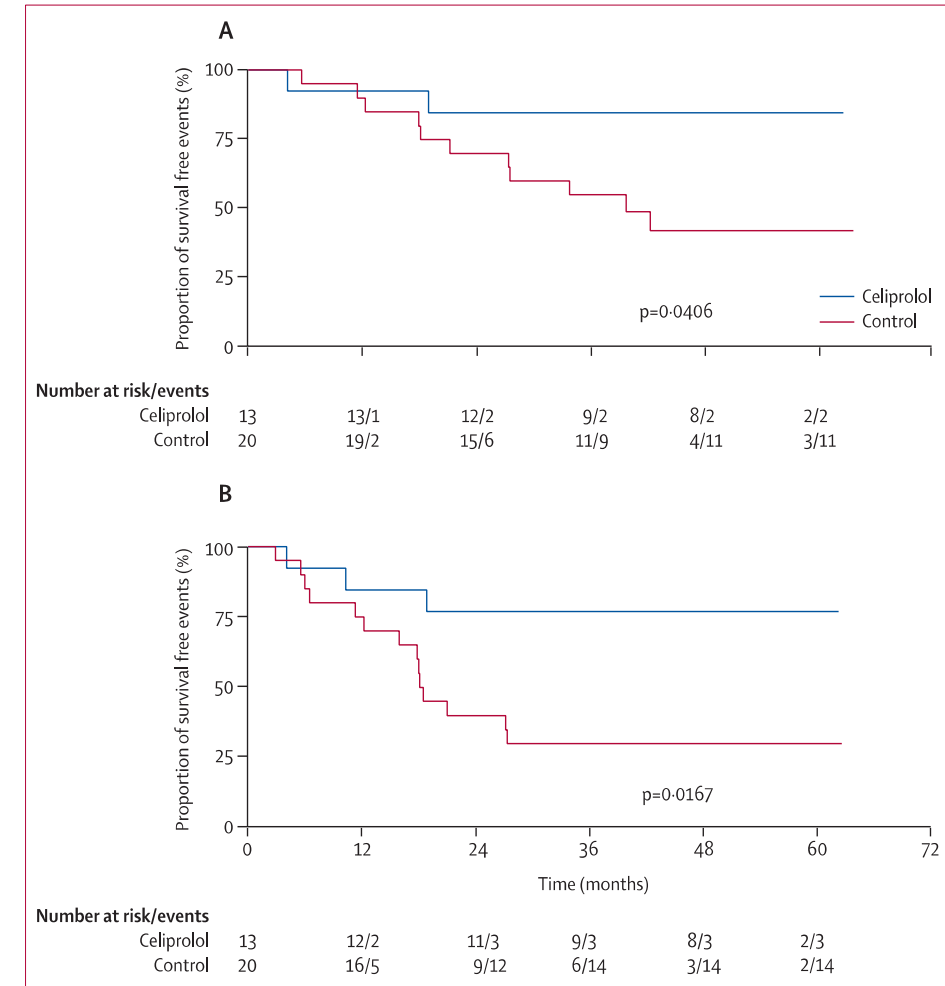


Figure 3: Kaplan-Meier curves of event-free survival in 33 patients with positive COL3A1 mutation. Primary endpoint (A). Primary and secondary endpoints (B).

PIVOTAL PHASE 3 TRIAL

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

FINANCIAL OVERVIEW

CASH POSITION

AS OF SEPTEMBER 30, 2022

\$6.4M

- +\$1.5M private placement w/Acer CEO and Chairman in Q4 2022
- +\$3.0M gross proceeds from ATM and equity line sales settled in Q4 2022
- +\$3.9M gross proceeds from ATM sales settled in January 2023
- +\$7.0M of SWK loan* proceeds in January 2023
- **Extends cash runway into early Q2 2023**

CAPITALIZATION

AS OF JANUARY 30, 2023

21.0M

Shares of common stock outstanding

26.7M

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

HISTORICAL GROSS PROCEEDS

THROUGH JANUARY 30, 2023

\$113.3M equity financings

\$35.0M from Relief Collaboration

\$19.5M from debt financings*

\$167.8M

* 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>



Thank You

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