Corporate Presentation

June 26, 2023

FORWARD-LOOKING STATEMENTS

ACER

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

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

Our Goal

Develop and deliver life - changing therapies to patients quickly and efficiently

Our Difference

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

COMMERCIAL AND LATE-STAGE PIPELINE

ACER

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 availability anticipated early July 2023[$]
EDSIVO™ (celiprolol)	Vascular Ehlers-Danlos Syndrome (COL3A1+)					**H1 2024**: Full trial enrollment[$]

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

OLPRUVA™

OLPRUVA™
(sodium phenylbutyrate) for oral suspension

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

HIGHLIGHTS

OLPRUVA™ is a commercial-ready, novel formulation of phenylbutyrate with convenient, single-dose packaging

- FDA approved in December 2022, patent protection through 2036
- Dual-coated formulation delays release in water for up to 5-minutes, rapidly dissolves in acidic environment (e.g., stomach)

- Multiple opportunities for potential indication expansion for OLPRUVA™ franchise:[$]
  - UCD investigations to determine whether it's possible to: enhance administration flexibility options; improve bioavailability via pre-meal administration
  - Other investigational indications: MSUD, liver disorders

Area of High Unmet Need (Urea Cycle Disorders)

- Currently, phenylbutyrate is prescribed for ~800 patients with UCDs
  - In real world setting, neither Ravicti® nor Buphenyl® maintain ammonia levels below ULN in long-term follow-up of UCD patients[1]
  - Palatability, odor, preparation, route of administration and packaging are listed as the most important attributes for UCD treatment adherence[2]
  - Adherence can also be affected when patients skip their mid-day dose, because existing treatments are inconvenient to take to work or school[2]

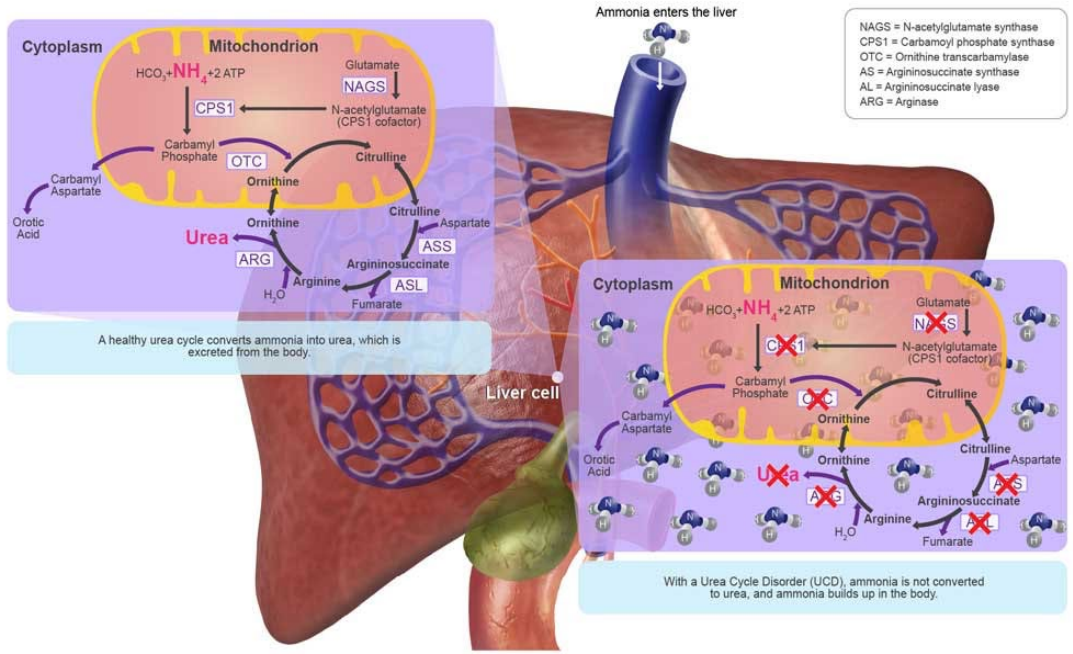
Commercial Opportunity

- Significant revenue opportunity for phenylbutyrate:
  - Net revenue of Ravicti® & Buphenyl®: $333M in FY 2022 and $91.7M in Q1 2023[3]
- Forecasted patient potential for OLPRUVA™: 250-350 patients (base case)
- Convenient and cost-advantageous alternative to Ravicti®
  - OLPRUVA™'s wholesale acquisition cost (WAC) price (per gram) 50% lower than Ravicti®'s
  - Ravicti® will be moved to "Excluded Medications" list on Express Script's National Preferred Formulary starting 7/1/2023[4]

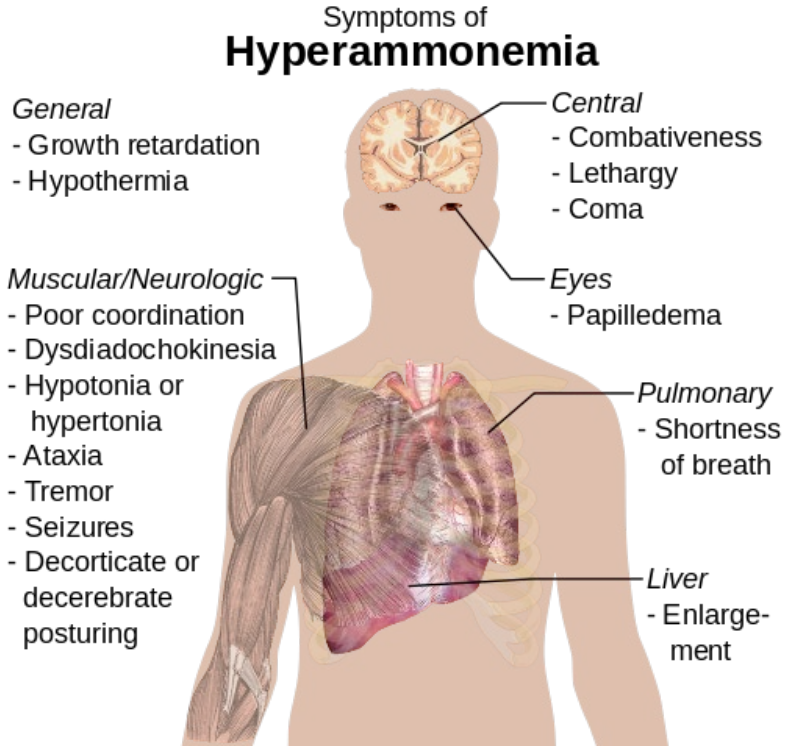
$ Subject to additional capital
1 https://www.clinicaltrials.gov/study/NCT01948427?term=thrive%20study%20horizon&rank=1
2 Data on file
3 https://ir.horizontherapeutics.com/news-releases/news-release-details/horizon-therapeutics-plc-reports-first-quarter-2023-financial
4 https://www.das.nh.gov/hr/documents/health_benefits/2023-Drug-List-Exclusion-Changes-Eff-07012023.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



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 (PAGN)

PAGN is then excreted by the kidneys in the urine

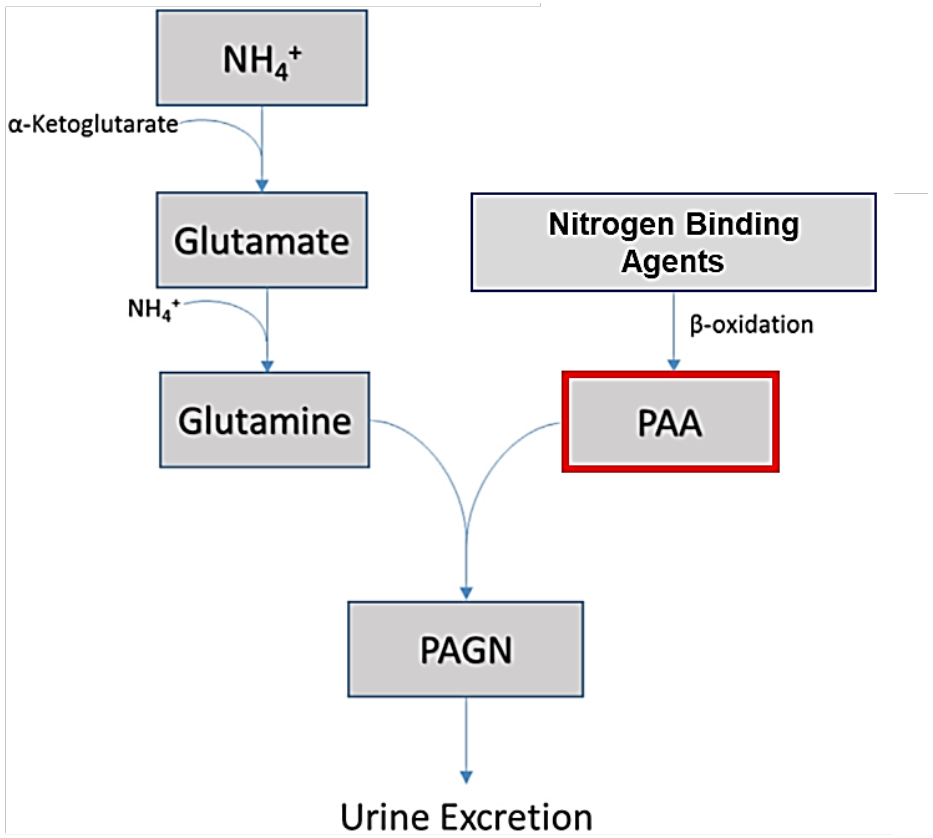
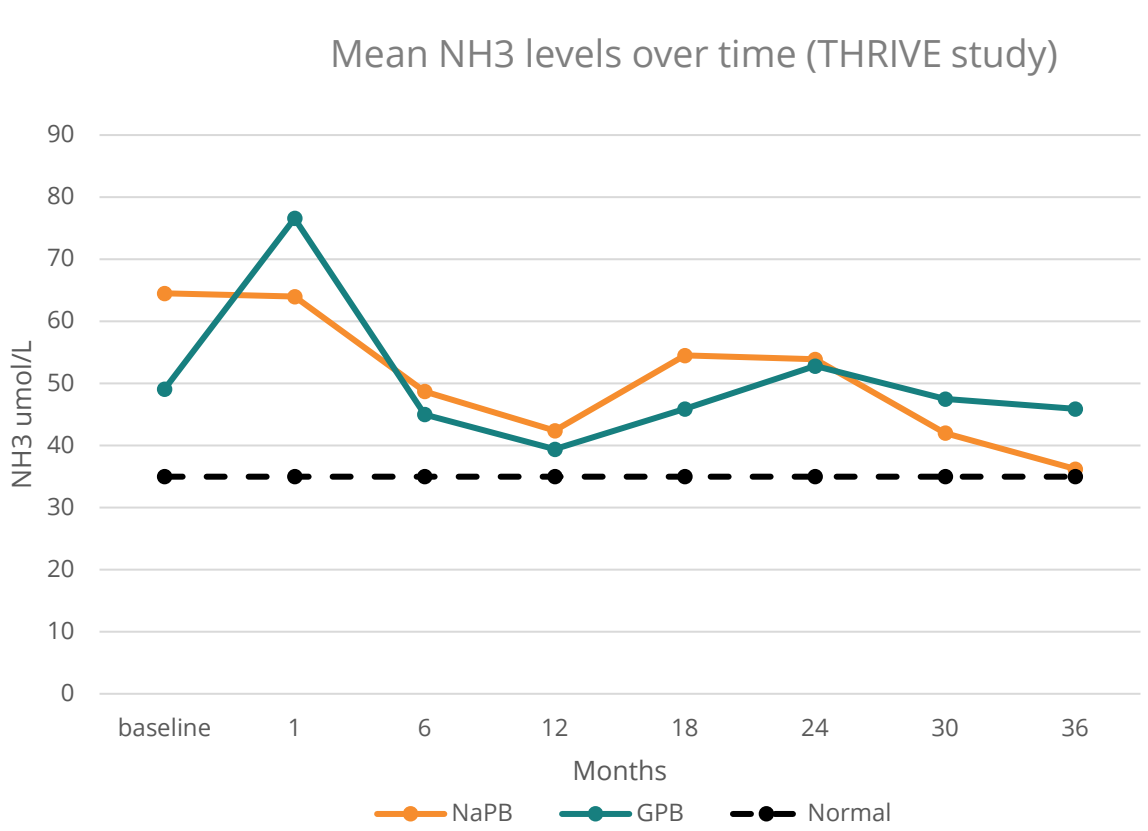


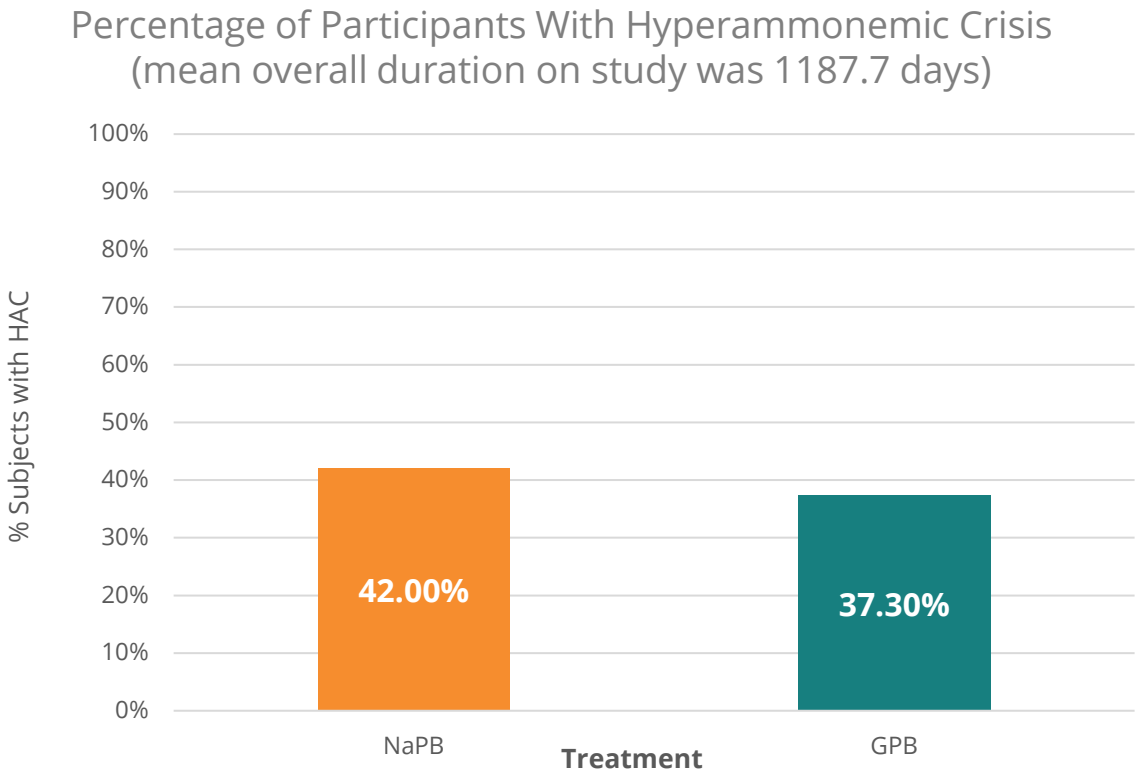
Fig. 1. Metabolizing pathway and mechanism of action of GPB.
GPB (glycerol phenylbutyrate); PAA (phenylacetic acid); PBA (phenylbutyric acid); PAGN (phenylacetylglutamine).

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

UNMET NEED – EFFICACY

- THRIVE Observational study[1] collected data on patients with UCDs
- Over first three years of follow-up, patients on both Ravicti® (GPB) and Buphenyl® (NaPB):
  - Had mean ammonia levels ≥ Upper Limit of Normal (ULN)
  - Comparable percentage of patients had a hyperammonemic crisis in both treatment groups

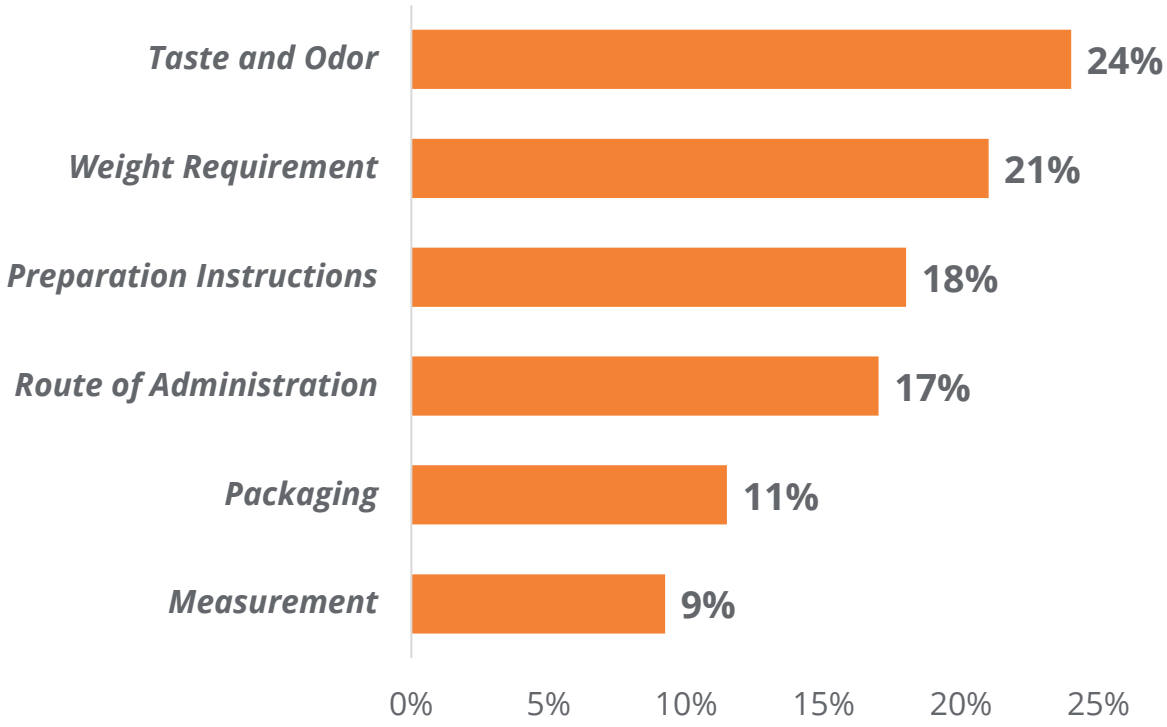




1 https://www.clinicaltrials.gov/study/NCT01948427?term=thrive%20study%20horizon&rank=1

UNMET NEED – TREATMENT ADHERENCE

Mean Relative Attribute Importance for UCD Treatment Adherence in a Discrete Choice Exercise[1,2]



Qualitative Interviews with Leading UCD Centers Uncovered the Mid-Day Dose as a Driver of Poor Adherence in UCD patients[2]

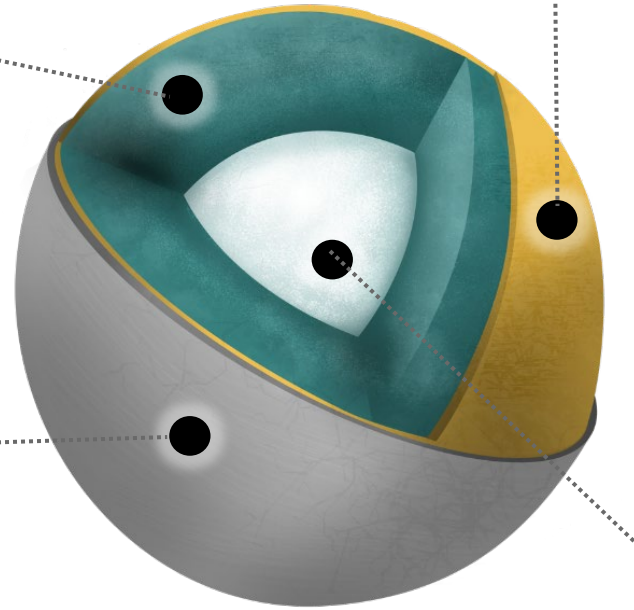
UCD Expert Insights:

- *"Skipping doses seems to increase with age, especially at mid-day"*
- *"Very common to skip dose"*
- *"Teenagers seem to struggle most"*
- *"Skipping mid-day dose happens all the time"*

1 ACER-sponsored, IRB-approved quantitative research study employing discrete choice study design.
2 Data on file.

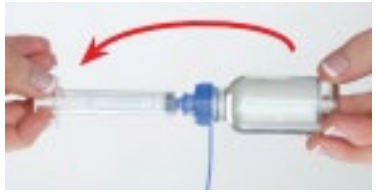
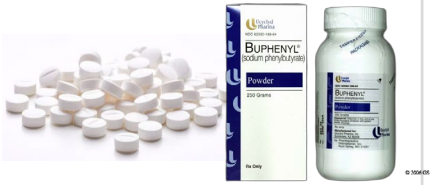
NOVEL FORMULATION

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]



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 with 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

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

$ Subject to additional capital

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]	**~ 85% Ravicti®** **~ 15% Buphenyl® or generic NaPB**
Ravicti® & Buphenyl® U.S. Net Revenue[3]	**FY 2022: $333M** **Q1 2023: $91.7M**

1 https://www.drugs.com/slideshow/top-10-most-expensive-drugs-1274
2 HealthVerity Payer claims data analysis
3 https://ir.horizontherapeutics.com

OLPRUVA™ PRICE & REIMBURSEMENT

- Acer believes pricing strategy will offer UCD patients a new treatment option at an approximately 50% discount to the current WAC price per gram of Ravicti®
- OLPRUVA™ pricing strategy informed by thorough market research and input received from advocacy organizations, focus groups, key opinion leaders and representatives from top U.S. commercial and government payers
- OLPRUVA™ wholesale acquisition cost (WAC) price submitted to the compendia
- WAC price now available in the major drug databases such as Redbook, Medispan, and First Data Bank

Acer is not currently providing guidance on anticipated number of patients treated, net revenue and operating expenses.

LIFECYCLE OPPORTUNITIES

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

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

$ Subject to additional capital

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

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

DISEASE OVERVIEW

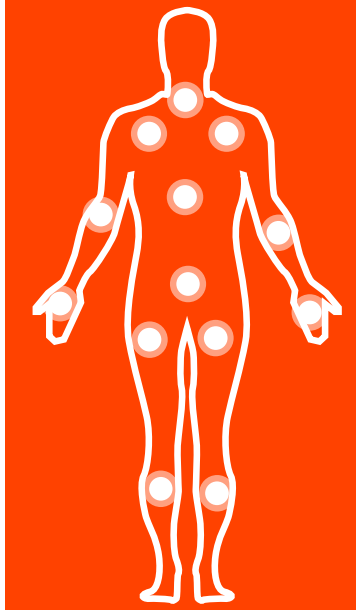
- Autosomal dominant connective tissue disorder of collagen synthesis caused by mutations in the COL3A1 gene for type III procollagen
- Characterized by arterial aneurysms, dissections and/or ruptures in arteries and hollow organs (intestines, uterus, lungs, etc.)

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]
- DiSCOVER Phase 3 decentralized (virtual) pivotal trial ongoing with Breakthrough Therapy Designation, Special Protocol Assessment
- New Chemical Entity w/Orphan Drug Designation
- Patent protection until 2038



COL3A1+ vEDS

Prevalence:

Between 5,000 to 10,000 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

BBEST TRIAL: COL3A1+ SUBPOPULATION

EDSIVO™

Efficacy:

- 18 months to reach dose > 300 mg
- 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

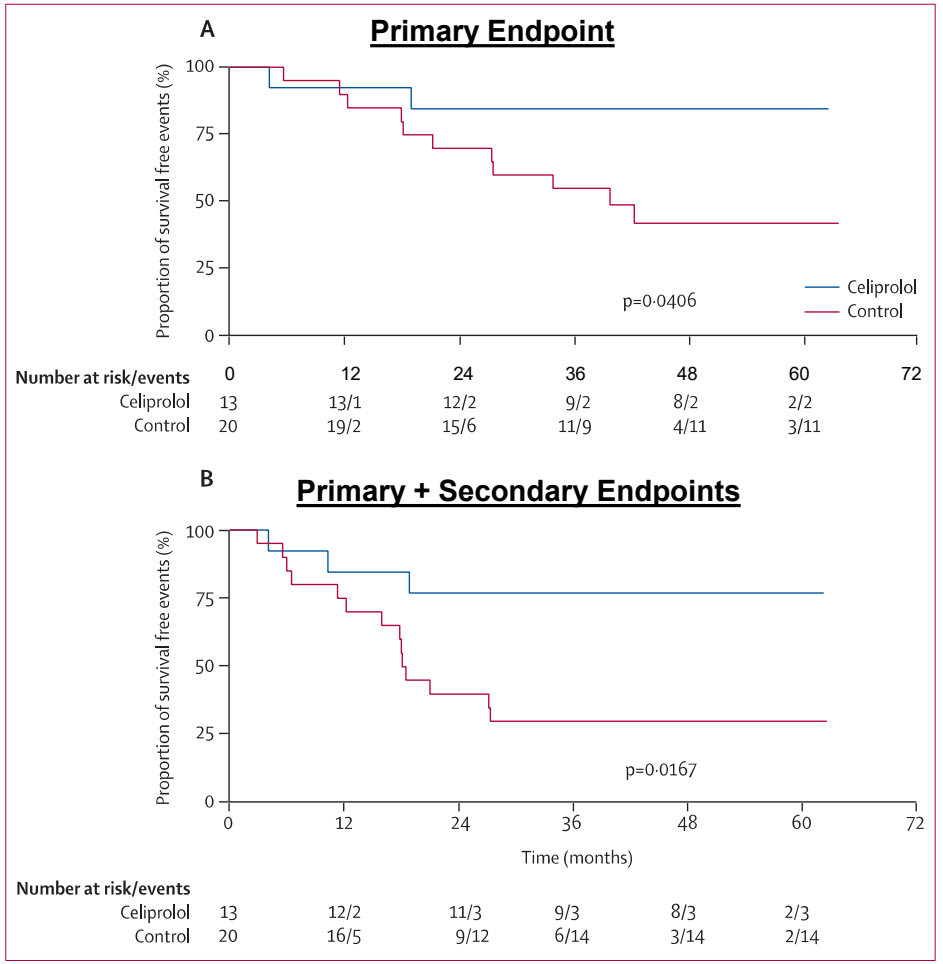


Figure 3: **Kaplan-Meier curves of event-free survival in 33 patients with positive *COL3A1* mutation**
Primary endpoint (A). Primary and secondary endpoints (B).

Ong Lancet 2010; 376: 1476-84.

LONG-TERM OBSERVATIONAL STUDY (FRENCH COHORT)

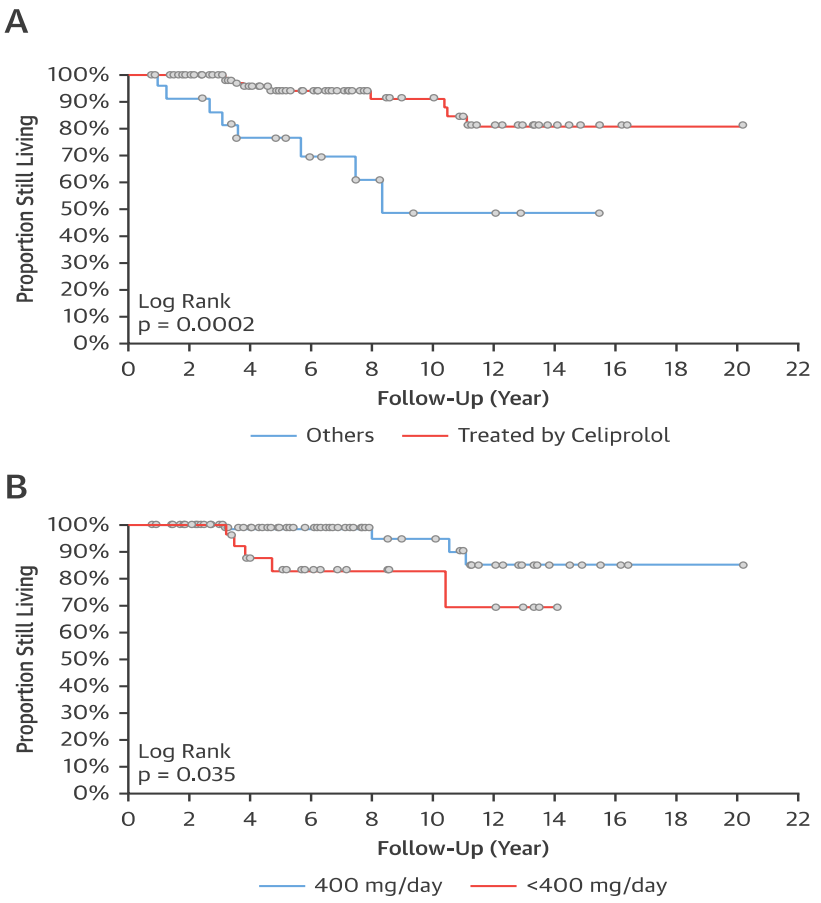
EDSIVO™

Statistically-significant Efficacy:

- Between 2000 and 2017, 144 patients (100% COL3A1+) were included in this study
- Patients were normotensive
- (A) Patients not treated with celiprolol had a significantly worse survival outcome than treated patients:
  - Overall survival was 80.7% (95% CI: 67.8% to 93.6%) in those treated with celiprolol (n = 110) versus 48.5% (95% CI: 19.7% to 77.4%) in those not treated (n = 22) after 11.1 years of follow-up: p = 0.0002
- (B) Survival was significantly improved in patients taking celiprolol 400 mg/day compared with patients taking lower doses, suggesting a dose effect and that 400 mg/day should be considered the optimal treatment dose:
  - At the end of follow-up, survival was 85% (95% CI: 70.5% to 99.5%) in those patients treated with celiprolol 400 mg/day and 69.2% (95% CI: 41.4% to 97.0%) in those taking celiprolol 100 to 300 mg/day: p = 0.035
- Statistically significant decreases in hospitalization rates on intra-patient basis pre-and post-celiprolol treatment

Baseline characteristics						
BMI, kg/m^2	21.2 (19.0-23.7)	21.0 (19.0-23.0)	22.9 (20.5-26.1)	22.6 (17.3-28.4)	20.6 (19.0-22.5)	0.2415
SBP, mm Hg	114.0 (106.0-123.0)	113.0 (105.0-121.0)	120.0 (112.0-126.0)	112.5 (107.0-131.0)	113.5 (104.0-120.0)	0.2145
DBP, mm Hg	70.0 (65.0-78.0)	70.0 (64.0-76.0)	74.0 (65.0-83.0)	75.0 (68.0-85.0)	69.0 (68.0-76.0)	0.2837

FIGURE 3 Kaplan-Meier Survival Analysis of vEDS Patients in Groups I and II *COL3A1* Pathogenic Variants, According to Celiprolol Treatment



Frank et al., J Am Coll Cardiol. 2019 Apr 23;73(15):1948-1957.

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)
- H1 2024: Full enrollment anticipated based on current enrollment rates[$]
- Double-blind portion of trial intended to end if statistical significance is reached at an interim analysis (occurs at 28 vEDS-related events)
  - Estimated to occur as early as approximately 18 months after completion of full enrollment, or after 46 vEDS-related clinical events[$]

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 with celiprolol vs. placebo

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

INTELLECTUAL PROPERTY AND EXCLUSIVITY

- Patents
  - Title: Method of Providing Celiprolol Therapy to a Patient
  - Issued patent #11,523,997 – approximate expiration 12/2038
  - Claims direct to methods of treating vEDS
  - Priority date is 12/21/2017
  - Assigned to AP-HP and Université Paris Descartes – Acer has exclusive license
  - Pending in AR, BR, CA, & MX
- Orphan Drug – 7 years
  - Bars FDA from approving any other application (ANDA, 505(b)(2) or "full" NDA or BLA) for the same drug for the same orphan disease or condition for 7 years, unless FDA finds that the applicant has shown clinical superiority
  - Covered under the Orphan Drug Act and 21 CFR 316.31
- New Chemical Entity – 5 years
  - 5 years from the date of approval of the first approved NDA
- Pediatric exclusivity
  - Additional 6 months to existing patents/exclusivity
  - Pediatric exclusivity takes on characteristics of five-year, three-year or orphan exclusivity when it attaches to those protections
  - Described in the Best Pharmaceuticals for Children Act (BCPA) and Section 505(A) of the Food and Drug Administration Modernization Act of 1997
- 1 pending trademark family
  - EDSIVO™
  - Registered in BR, CA
  - Pending in US

FINANCIAL OVERVIEW

ACER

CASH POSITION

AS OF MARCH 31, 2023

$6.4M

- +$0.4M gross proceeds from ATM sales in Q2 2023
- +$1.0M gross proceeds from promissory note with Chris Schelling, CEO & Founder*
- **Cash runway into early Q3 2023**

CAPITALIZATION

AS OF JUNE 22, 2023

24.5M Shares of common stock outstanding

33.9M Shares fully diluted (incl. stock options, convertible notes*, and warrants)

HISTORICAL GROSS PROCEEDS

THROUGH JUNE 22, 2023

$116.6M equity financings

$35.0M from Relief Collaboration

$20.5M from debt financings*

$172.1M

* June 26, 2023: See Form 8-K filed on June 26, 2023, at https://www.sec.gov/edgar/search/
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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Thank You
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