ZEVRA THERAPEUTICS

Corporate Presentation

December 2024

A Rare Approach to Therapeutics

NasdaqGS: ZVRA

Cautionary Note Regarding Forward-Looking Statements

Presentation may contain forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements relate to future events or our future financial performance. We generally identify forward-looking statements by terminology such as “may,” “will,” “would,” “should,” “expects,” “plans,” “anticipates,” “could,” “intends,” “target,” “projects,” “contemplates,” “believes,” “estimates,” “predicts,” “assume,” “intend,” “potential,” “continue” or other similar words or the negative of these terms. We have based these forward-looking statements largely on our current expectations about future events and financial trends that we believe may affect our business, financial condition and results of operations. Forward-looking statements are not guarantees of future actions or performance. These forward-looking statements include statements regarding the promise and potential impact of our preclinical or clinical trial data, including without limitation the timing and results of any clinical trials or readouts, our anticipated financial performance, our industry, our intellectual property, business strategy, plans, goals and expectations concerning our market position, future operations, the timing or results of any Investigational New Drug Applications (NDA) and NDA submissions, communications with the U.S. Food and Drug Administration (FDA), the potential uses or benefits of MIPLYFFA, KP1077, SDX or any other product candidates for any specific disease indication or at any dosage, the potential benefits of any of Zevra’s product candidates, the success or timing of the launch or commercialization of any products or related sales milestones, and our strategic and product development objectives. These forward-looking statements are based on information currently available to Zevra and its current plans or expectations and are subject to a number of known and unknown uncertainties, risks and other important factors including those discussed under the caption “Risk Factors” in our Annual Report for the year ended December 31, 2023, on Form 10-K and filed with the Securities and Exchange Commission (SEC) on September 30, 2024, and in our other filings with the SEC could cause actual results, performance, or achievements to differ materially from those indicated by the forward-looking statements made herein.

While we may elect to update such forward-looking statements at some point in the future, except as required by law, we disclaim any obligation to do so, even if subsequent events cause our views to change. Although we believe the expectations reflected in such forward-looking statements are reasonable, we can give no assurance that such expectations will prove to be correct. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to this presentation.

This presentation also may contain estimates and other statistical data made by independent parties and by us relating to market size and other data about our industry. This data involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. In addition, projections, assumptions and estimates of our future performance and the future performance of the markets in which we operate are necessarily subject to a high degree of uncertainty and risk.

Becoming a Leading Rare Disease Company

Key Pillars for Strategic Growth

Our Mission:

Bringing life-changing therapeutics to people living with rare diseases

Experienced team with extensive rare disease expertise

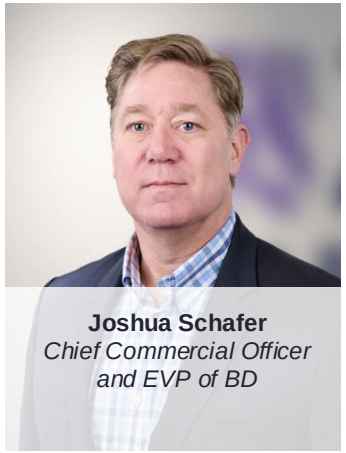
Commercial excellence to enable patient access to therapeutics

Growing pipeline with potential to bring new products and deliver value for patients

Experienced Team with Rare Disease Expertise

Neil F. McFarlane
Chief Executive Officer and President

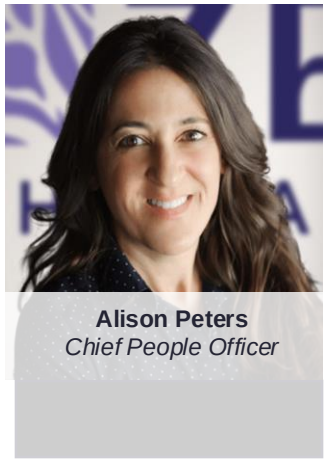
R. LaDuane Clifton, CPA
Chief Financial Officer and Treasurer



Joshua Schafer
Chief Commercial Officer and EVP of BD

Adrian W. Quartel, M.D., FFPM
Chief Medical Officer

Rahsaan W. Thompson
Chief Legal Officer, Secretary, and Compliance Officer



Alison Peters
Chief People Officer

RARE DISEASE EXPERIENCE

ADAMAS · genzyme · BIOMARIN · Mallinckrodt Pharmaceuticals · astellas · Retrophin · Takeda · Lyell™ · ACTELION · janssen · gritstone bio

PRODUCT LAUNCH EXPERIENCE

GOCOVRI® (amantadine) extended release capsules 68.5 mg | 137 mg · Thiola EC (tiopronin) Delayed-Release Tablets 100mg | 300mg · EVENITY® (romosozumab-aqqg) injection 105 mg/1.17 mL · Cholbam® (cholic acid) capsules · INOmax® (nitric oxide) GAS FOR INHALATION · XOSPATA® gilteritinib 40mg tablets · Xtandi® (enzalutamide) 40 mg tablets | 80 mg tablets · NEW MIPLYFFA® arimoclomol capsules · OLPRUVA® (sodium phenylbutyrate) for oral suspension · Opsumit® macitentan tablets 10 mg · Uptravi® selexipag tablets 200-1600 mcg

Establishing Zevra as a Rare Disease Company Through Robust Advocacy Partnerships

ZEVRA THERAPEUTICS

National Urea Cycle Disorders Foundation

nnpdf NATIONAL NIEMANN PICK DISEASE FOUNDATION

Sleep consortium

Ara Parseghian Medical Research Fund University of Notre Dame

Connecting Families UCD Foundation

np uk

Firefly Fund Shining a Light on NPC1

Progress Together Inpda International Niemann-Pick Disease Alliance

EveryLife Foundation for Rare Diseases

Hope for Marian

hypersomnia foundation.org

NORD®

Wake Up Narcolepsy Inc. Celebrating 15 Years

Narcolepsy Network Supporting people with narcolepsy since 1986

projectsleep

Rare Disease Company Coalition

Diversified Portfolio to Bring New Products and Deliver Value for Patients

PHASE 1	PHASE 2	PHASE 3	NDA	FDA APPROVED	STATUS
MIPLYFFA™ *arimoclomol* **Niemann-Pick Disease Type C (NPC)**					**FDA approval: Sep 20, 2024** **Launch: Nov 21, 2024**
OLPRUVA® *sodium phenylbutyrate for oral suspension* **Urea Cycle Disorders (UCD)**					**FDA approval: Dec 22, 2022** **Launch: Jan 29, 2024**
Celiprolol **Vascular Ehlers-Danlos Syndrome (VEDS)**					**Ph. 3 trial ongoing**
KP1077 **Idiopathic Hypersomnia (IH)**					**FDA EOP2 meeting completed; Ph. 3 trial ready**
KP1077 **Narcolepsy**					**Ph. 3 trial potential[1]**
AZSTARYS® *serdexmethylphenidate and dexmethylphenidate* **Attention Deficit Hyperactivity Disorder (ADHD)***					**Receiving royalties and milestones on net sales[2]**

Zevra's Portfolio

- Asset portfolio targeting rare diseases
- Overlap in treating physicians for OLPRUVA® and MIPLYFFA™
- Multiple upcoming milestones and catalysts
- Pipeline with clinical and commercial assets

Certain products may be subject to royalty obligations, details and required disclosures are available in our SEC filings or on our website: www.zevra.com.

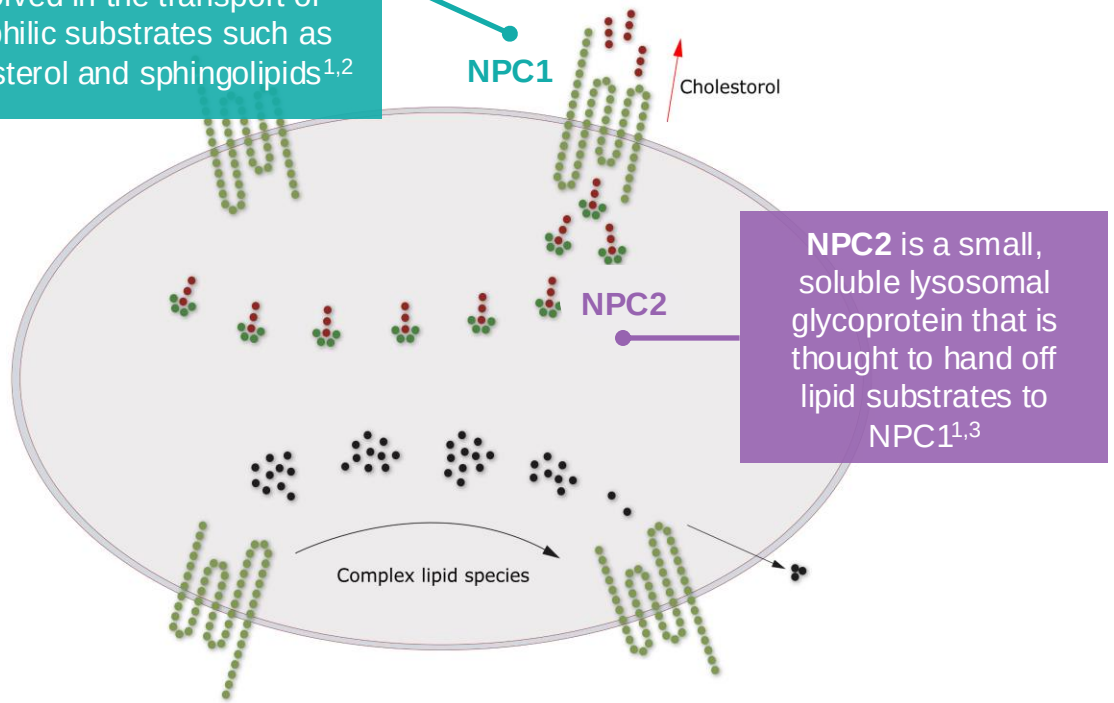
1. Data generated from IH Ph.2 trial will be analyzed to potentially support clinical development of both narcolepsy and IH programs; 2. Zevra partnered asset

MIPLYFFA™
Approved for the Treatment of NPC

Niemann-Pick Disease Type C (NPC) is a Neurodegenerative Lysosomal Storage Disorder

Cholesterol buildup leads to cell death; MIPLYFFA may enhance cholesterol metabolism through improved lysosomal function

NPC1 is a large, transmembrane domain protein with specific domains that are involved in the transport of lipophilic substrates such as cholesterol and sphingolipids[1,2]



NPC2 is a small, soluble lysosomal glycoprotein that is thought to hand off lipid substrates to NPC1[1,3]

- NPC gene mutations produce abnormal, absent or non-functional NPC proteins[4]
- Progressive lipid accumulation and cellular impairment leads to cell death and ultimately organ dysfunction in the spleen, liver and brain
- Disease results in impairment and loss of cognition, speech, swallow ability, fine motor skills and ambulation
- Heterogenous onset and rate of progression, always fatal

NPC, Niemann-Pick disease type C.

1. Carstea ED et al. *Science.* 1997;277:228-231. 2. Platt FM et al. *Annu Rev Genomics Hum Genet.* 2014;15:173-194. 3. Ingemann L, Kirkegaard T *J Lipid Res.* 2014;55:2198-2210. 4. Geberhiwot T, et al. Orphanet J Rare Dis. 2018 Apr 6;13(1):50.

MIPLYFFA: First Approved NPC Treatment in U.S.

Ultra-rare, relentlessly progressive and fatal neurodegenerative disease

Ultra-Rare Disease

- Incidence: ~1 in 120,000 live births[1]
- Prevalence:
  - 1,800 patients estimated in EU and U.S.
  - 900 patients estimated in U.S.[2]
  - ~300 U.S. patients currently diagnosed or treated[2]

Significant Unmet Need

- Neurocognitive decline adversely impacts daily living
- Irreversible and fatal disease
- Mean age of death is 13 years[2]
- Difficult to diagnose and can affect patients at any time
- First approved treatment for NPC in U.S.

1. https://link.springer.com/article/10.1186/1750-1172-5-16
2. Burton et.al., Molecular Genetics and Metabolism Volume 134, Issues 1–2, September–October 2021, Pages 182-187

MIPLYFFA Addresses an Unmet Need in NPC

MIPLYFFA in combination with miglustat is well-positioned to become the cornerstone of NPC treatment





FIRST-IN-CLASS, ORAL TREATMENT

- FDA approved on Sept 20, 2024
- Cornerstone therapy in U.S. for NPC
- Oral capsules can be swallowed whole, mixed with foods/liquids or delivered through feeding tube

EXTENSIVE CLINICAL AND SAFETY DATA GENERATED

- Halted disease progression through 12 months of treatment
- In clinical study, MIPLYFFA was well tolerated compared to placebo
- > 270 NPC patients have been treated, including pivotal, open label and Expanded Access Program
- Some patients have been on MIPLYFFA for more than 5 years

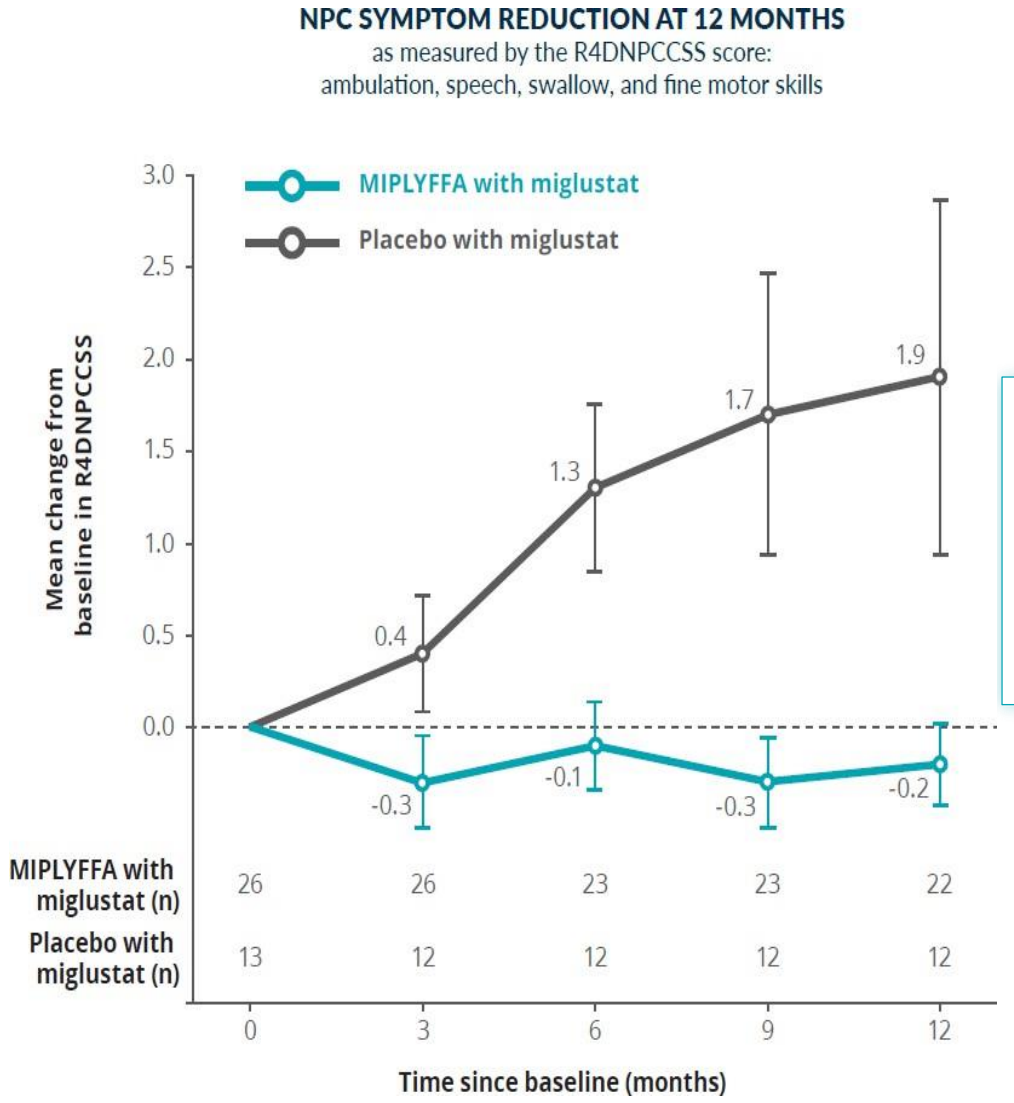
REGULATORY HIGHLIGHTS

- Advancing pathway to European registration
- Orphan Drug Designation for NPC
- Received Pediatric Rare Disease Priority Review Voucher
- Ongoing global Expanded Access Program (EAP) with >150 patients participating in U.S. and E.U.

Synergies and scale with OLPRUVA customer-facing team supporting both launches

Proven Effectiveness in Stopping Disease Progression in Patients who also Received Miglustat[1]

- MIPLYFFA, in combination with miglustat, **stopped disease progression through 12 months** of treatment, as demonstrated by a **decrease of 0.2 points** from baseline on the **R4DNPCCSS** compared to **1.9 points of progression** for patients with **miglustat alone**[1]
- There was insufficient data to determine the effectiveness of the use of MIPLYFFA without miglustat for the treatment of neurological manifestations in patients with NPC[1]



The estimated placebo adjusted mean change from baseline at month 12 was

-2.2 pts.

IMPORTANT SAFETY INFORMATION

Most common adverse reactions (≥15%) were upper respiratory tract infection, diarrhea, and decreased weight.

[1] MIPLYFFA Full Prescribing Information. Celebration, FL, US, Zevra Therapeutics Inc.; 09/2024.

MIPLYFFA Safety Profile

Adverse events were generally mild to moderate in severity and few led to a withdrawal of treatment.[1]

Adverse reaction[1]	MIPLYFFA with miglustat n=26 n (%)	Placebo with miglustat n=13 n (%)
Upper respiratory tract infection*	**8** (31)	**2** (15)
Diarrhea	**6** (23)	**3** (23)
Decreased weight	**4** (15)	**0**
Decreased appetite	**3** (12)	**0**
Tremor	**3** (12)	**0**
Urticaria**	**3** (12)	**0**
Headache	**3** (12)	**1** (8)
Lower respiratory tract infection	**3** (12)	**1** (8)
Seizure	**3** (12)	**1** (8)

*Upper respiratory tract infection: combined incidence of upper respiratory tract infection and rhinitis.
**Urticaria: Includes one patient in which urticaria occurred alone (3%) and two patients who had urticaria with angioedema (6%).

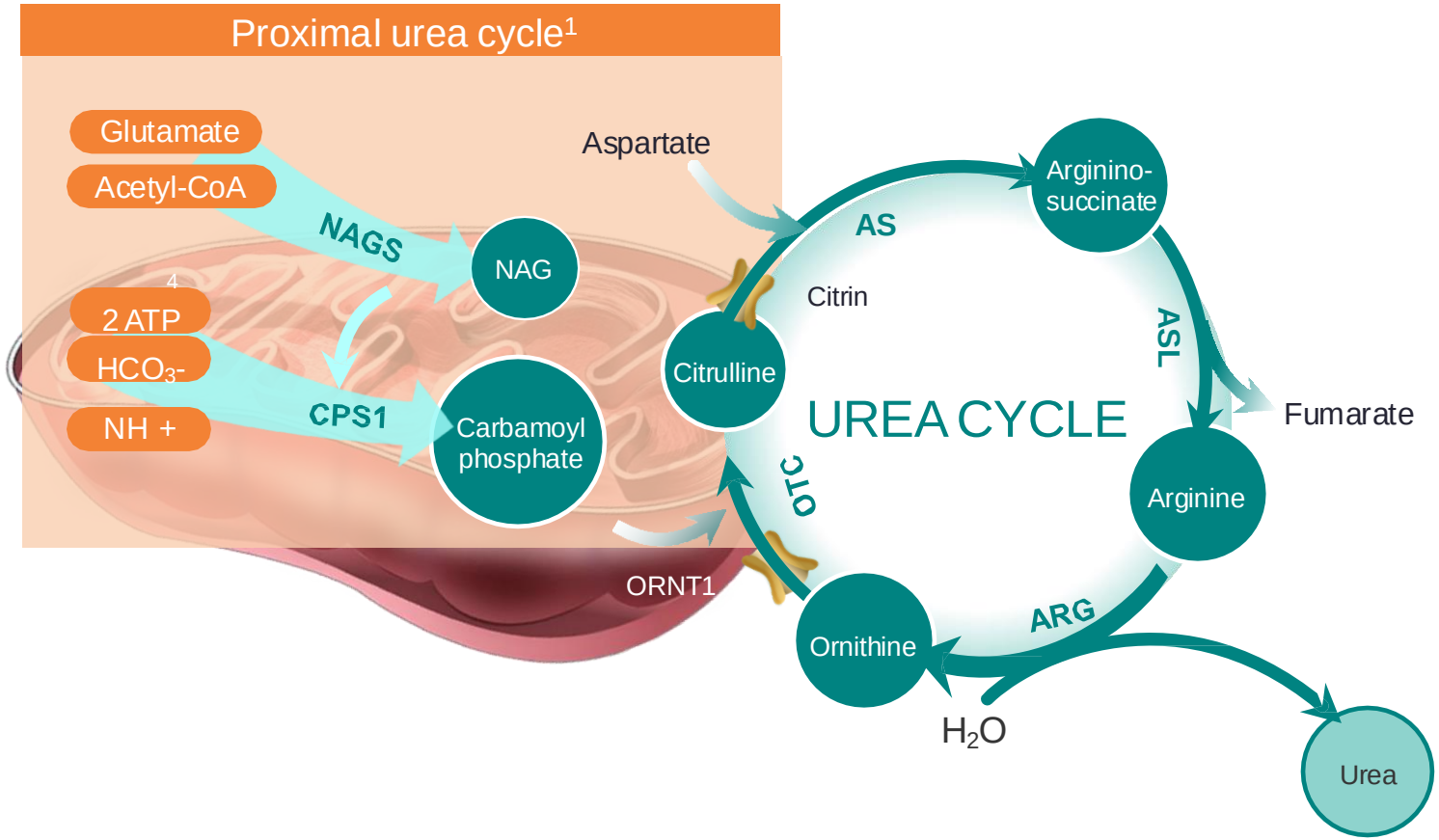
- Three (6%) of the MIPLYFFA-treated patients had the following adverse reactions that led to withdrawal from Trial 1: increased serum creatinine (one patient), and progressive urticaria and angioedema (two patients).
- Serious adverse reactions reported in MIPLYFFA-treated patients were hypersensitivity reactions including urticaria and angioedema.
- Thrombocytopenia was observed in three patients during the trial, all of whom were receiving miglustat for six months or longer at the time of enrollment. In two of these patients, the thrombocytopenia was present at baseline and persisted throughout the trial. In the other patient, the thrombocytopenia developed and resolved during the trial.
- Across the clinical trials, increases in serum creatinine (mean increase was 10%-20%) occurred mainly within the first month of dosing and were reversible upon treatment discontinuation.

[1] MIPLYFFA Full Prescribing Information. Celebration, FL, US, Zevra Therapeutics Inc.; 09/2024.

OLPRUVA®
For the Treatment of UCDs

Urea Cycle Disorders Cause Hyperammonemia, Leading to Brain Damage or Death

OLPRUVA is a nitrogen scavenger that removes excess ammonia



- Defect in one of the **6 enzymes** or **2 transporters** in the urea cycle leads to accumulation of ammonia
- A clinical hallmark of UCDs is hyperammonemic crises (HAC)
- Elevated ammonia levels can be neurotoxic, leading to neurocognitive damage, neurocognitive impairment and even death, if untreated
- Duration and severity of HAC correlates with brain damage, often requiring emergency visits and hospitalization

ARG, arginase; AS, argininosuccinate synthetase; ASL, argininosuccinate lyase; ATP, adenosine triphosphate; CoA, coenzyme A; CPS1, carbamoyl phosphate synthetase-1; NAG, N-acetylglutamate; NAGS, N-acetylglutamate synthetase; ORNT1, ornithine transporter; OTC, ornithine transcarbamylase.

1. Summar ML, Mew NA. *Pediatr Clin North Am*. 2018;65(2):231-246.

OLPRUVA: Addresses Unmet Needs in Urea Cycle Disorders (UCD)

Poor treatment adherence can lead to neurocognitive damage, coma and even death

Rare Disease

- US Incidence: 1 in 35,000 births[1]
- US Prevalence:
  - Approximately 1 in 100,000[1]
  - ~1,100 patients diagnosed[2]
  - >800 treated[2]
- About 80% of patients have mutations in either CPS, OTC or AS enzymes[3]

Unmet Need

- Phenylbutyrates are approved to treat UCDs
- More than 25% of HACs stem from poor treatment adherence[4]
- Palatability, odor, route of administration and packaging affect adherence

United States (U.S.) Market

1. https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4364413/
2. HealthVerify Payer Claims data analysis 2021
3. carbamylphosphate synthetase (CPS), ornithine transcarbamylase (OTC), or argininosuccinic acid synthetase (AS)
4. Enns GM, Porter MH, Francis-Sedlak M, Burdett A, Vockley J. 2019

OLPRUVA: Designed to Address Unmet Needs in Treatment of Certain UCDs

Unique formulation in single-dose envelopes for "ammonia control on the go"

UNIQUE FORMULATION DRIVES PALATABILITY AND ADHERENCE

- Novel formulation of phenylbutyrate
- Dual-coated formulation delays release in water for up to 5 minutes, rapidly dissolves in stomach
- Convenient, single-dose envelopes

FDA-APPROVED FOR LONG-TERM MANAGEMENT[1]

- Adjunctive therapy to standard of care
- Long-term management of adults and children
- UCDs involving deficiencies of CPS, OTC, AS[1]

COMPETITIVE ADVANTAGE

- Physicians attribute improved adherence to:
  - Better palatability
  - Less odor
  - Ease of administration
- Patent protection through 2036
- Current market estimated $350M[2]

OLPRUVA helps the body get rid of excess nitrogen to help avoid dangerous buildup of ammonia

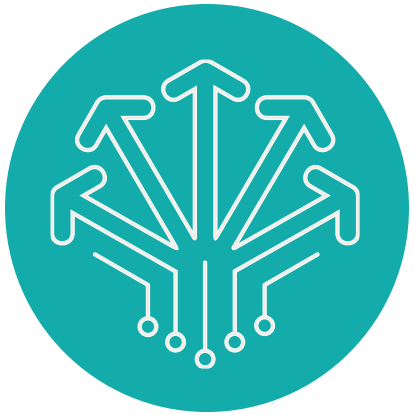
1. OLPRUVA is indicated as adjunctive therapy to standard of care, which includes dietary management, for the chronic management of adult and pediatric patients weighing 20 kg (44 pounds) or greater and with a body surface area (BSA) of 1.2 m2 or greater, with urea cycle disorders (UCDs) involving deficiencies of carbamylphosphate synthetase (CPS), ornithine transcarbamylase (OTC), or argininosuccinic acid synthetase (AS). Product Insert can be found at https://olpruva.com/wp-content/uploads/OLPRUVA-Prescribing-Information.pdf; Important safety information can be found at https://olpruva.com/#ImportantSafetyInformation
2. Company estimate

Commercial Excellence to Enable Patient Access

Resources in Place to Enable Patient Access to Our Therapies

Overlap in prescribers and centers of excellence for OLPRUVA and MIPLYFFA allow for efficient team approach

Rare Disease Sales Specialists calling on prescribing clinicians and Centers of Excellence



Patient Reimbursement Services assist patients in navigating reimbursement and treatment journey

Marketing identify appropriate patients and product positioning in treatment landscape

Account Management & Contracting enable market access and contracting with payors

Medical Affairs and Patient Advocacy work with Key Opinion Leaders and Advocacy Groups to advance scientific knowledge and patient care

Growing Pipeline in Rare Diseases

Vascular Ehlers-Danlos Syndrome Impairs Connective Tissue and Leads to Vascular Ruptures

Celiprolol is designed to reduce the mechanical stress on collagen fibers within the arterial wall

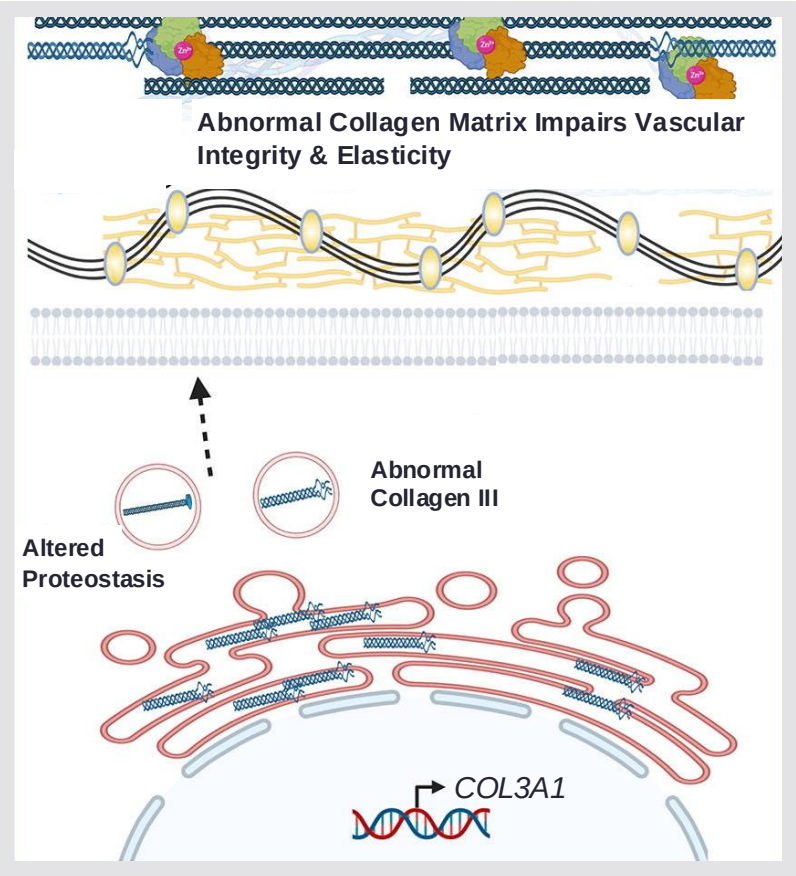


Figure adapted from Omar R, et al. Matrix Biol Plus. 2021 Nov 9;12:100090.

- VEDS (Vascular Ehlers-Danlos Syndrome) type IV is the most severe subtype[1]:
  - Characterized by aneurysms, dissections and/or ruptures
  - Large and medium sized arteries
  - Hollow organs (e.g., gastrointestinal, uterine)
- Autosomal dominant (50%); spontaneous mutations (50%)[1]
- Diagnosed by clinical symptoms and confirmed by presence of mutations in the *COL3A1* gene[1]
- Events occur in 25% of patients before the age of 20, and 90% by the age of 40[1]
- The median survival age is 51 years, with arterial rupture being the most common cause of sudden death[1]

1. Pepin M, et al. Genet Med. 2014 Dec;16(12):881-8.

Unmet Need in VEDS

Mutation in COL3A1 gene impairs connective tissue and leads to vascular ruptures

Rare Disease

- Incidence: 1 in 50,00 to 200,000 people[1]
- Prevalence 7,500 diagnosed patients in U.S. [2]

Significant Unmet Need

- No approved therapeutic options currently in the U.S.
- Current treatment is focused on surgical intervention
- Celiprolol has become the primary treatment for VEDS patients in several European countries[3]

1. https://www.orpha.net
2. Estimate based on an analysis of diagnosed vEDS patients from the Truven MarketScan® database and U.S. population data.
3. FightvEds.org

Celiprolol: Potential Treatment of Patients with *COL3A1*+ VEDS

Phase 3 primary endpoint: time to first occurrence of primary cardiac or arterial clinical event

CELIPROLOL FOR VEDS

- Selective adrenergic modulator
- Mechanism of action in VEDS patients is thought to be through vascular dilatation and smooth muscle relaxation
- May reduce the mechanical stress on collagen fibers within the arterial wall
- Unique pharmacological profile

CLINICAL EXPERIENCE

- BBEST Clinical study: 76% reduction in risk of arterial events observed in *COL3A1*+ subpopulation[1]
- Additional data from long-term observational study in France
- DiSCOVER Phase 3 decentralized (virtual) pivotal study ongoing

REGULATORY & IP ADVANTAGES

- New Chemical Entity in the U.S.
- Orphan Drug designation and Breakthrough Therapy Designation
- Special Protocol Assessment in place
- IP to 2038

1. https://www.sciencedirect.com/science/article/pii/S0735109719336939

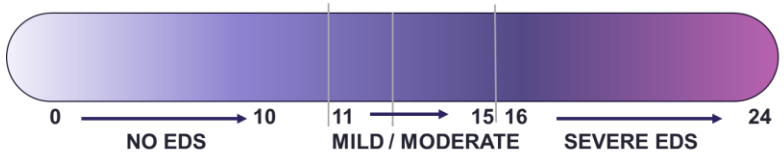
Idiopathic Hypersomnia (IH) Causes Excessive Daytime Sleepiness, Sleep Inertia and Brain Fog

KP1077 may provide optimal exposure of methylphenidate to better address these unmet needs

Idiopathic Hypersomnia Severity Scale

- 14 questions on a scale of 0 to 3 or 0 to 4, totaling 50 points[1]
- Higher scores, indicate more severe/frequent symptoms[1]
- Minimal Clinically Important Difference (MCID) of 4 points[2]

Epworth Sleepiness Scale



- 8 questions on a scale of 0 to 3, totaling 24 points[4]
- Higher scores, indicate more severe daytime sleepiness[4]
- 2- to 3-point change is considered MCID in sleep disorders[5]

- IH is a rare, debilitating, chronic neurologic disorder with an unknown pathophysiology
- Characterized by excessive daytime sleepiness (EDS)
- Excessively long sleep times
- Sleep inertia or difficulty waking
- Long and unrefreshing naps[3]
- Brain fog, memory problems, errors in habitual activities, mind blank and attention problems

*Idiopathic Hypersomnia Severity Scale is a self-report instrument designed to measure the severity of key symptoms of hypersomnolence

1. Dauvilliers Y, Evangelista E, Barateau L, et al. Measurement of symptoms in idiopathic hypersomnia: the Idiopathic Hypersomnia Severity Scale. *Neurology*. 2019;92(15):e1754-e1762.
2. Rassu AL et al. Idiopathic hypersomnia severity scale to better quantify symptoms severity and their consequences in idiopathic hypersomnia. *J Clin Sleep Med*. 2022;18(2):617-629.
3. ~25% of patients "long sleepers," >10hrs.
4. Johns MW. A new method for measuring daytime sleepiness: the Epworth Sleepiness Scale. *Sleep*. 1991;14(6):540–545
5. Patel S, et al.The Epworth Sleepiness Scale: Minimum Clinically Important Difference in Obstructive Sleep Apnea. Am J Respir Crit Care Med. 2018 Apr 1;197(7):961-963. doi: 10.1164/rccm.201704-0672LE.

Unmet Need in Idiopathic Hypersomnia

ZEVRA THERAPEUTICS

IH is a rare, debilitating, chronic neurologic disorder with an unknown pathophysiology

Rare Disease

- Incidence: 10.3 per 100,000 people in the US[1]
- Prevalence: ~37,000 patients diagnosed[2]
- Total population may be much larger

Current Treatments Don't Address Needs

- Patients rated current medication effectiveness as poor (5.4 on a 10-point scale)[3]
- Tolerable stimulant treatment doses currently available are inadequate to treat brain fog
- Comorbidities complicate treatment (cardiovascular and patient demographics)
- Potential drug-drug interactions (DDI) with contraceptives, antidepressants, antihistamines

1. https://doi.org/10.1093/sleep/zsy061.624
2. https://www.sleepcountshcp.com/what-is-idiopathic-hypersomnia
3. https://www.sleepcountshcp.com/idiopathic-hypersomnia-treatment-options

KP1077: Potentially Novel Approach for IH

Unique PK profile and dosing regimen designed to address EDS and sleep inertia

KP1077 FOR IH

- Proprietary prodrug of d-MPH
- Potential to address primary IH symptoms
- Two dosing regimens being explored
  - Once daily at bedtime
  - Twice daily: once in the morning and once at bedtime
- Full data package from completed Ph. 2 trial to inform Ph. 3 design[1]

IMPROVED SAFETY AND TOLERABILITY OVER EXISTING TREATMENTS

- Unique pharmacokinetic profile to overcome key symptoms
- Greater tolerability and lower cardiovascular effects
- No DDI potential with hormonal contraceptives; antidepressants
- Lower abuse potential

REGULATORY & IP ADVANTAGES

- Orphan Drug designation in IH
- IP through 2037
- Conducted End-of-Phase 2 meeting with the FDA at the end of Q3 2024
- SDX is designated Schedule IV controlled substance by DEA in the U.S.[2]

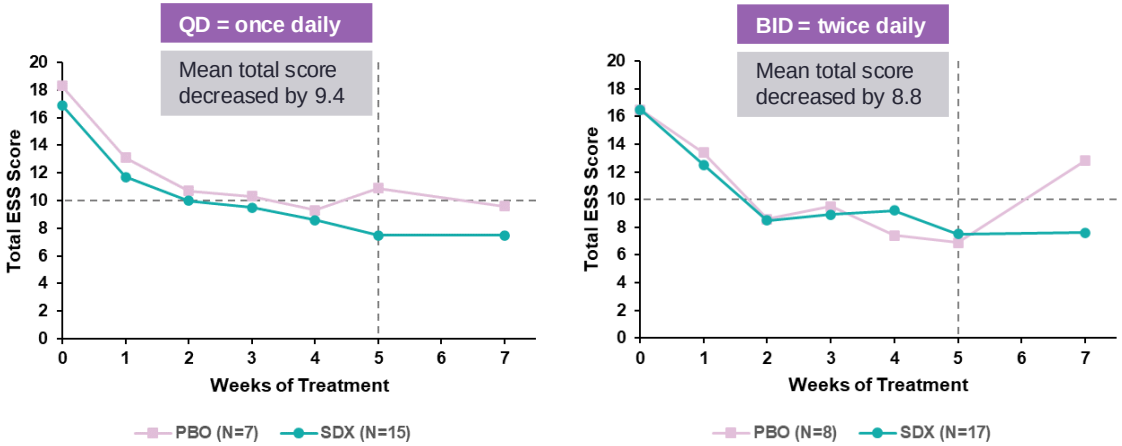
1. Data presented at SLEEP 2024, Houston, Texas, June 1-5, 2024
2. Zevra's proprietary prodrug of d-methylphenidate (d-MPH) has been classified as a Schedule IV controlled substance by the U.S. Drug Enforcement Administration (DEA).

KP1077 Ph. 2 Trial Produced Clinically Meaningful Improvements at All Endpoints

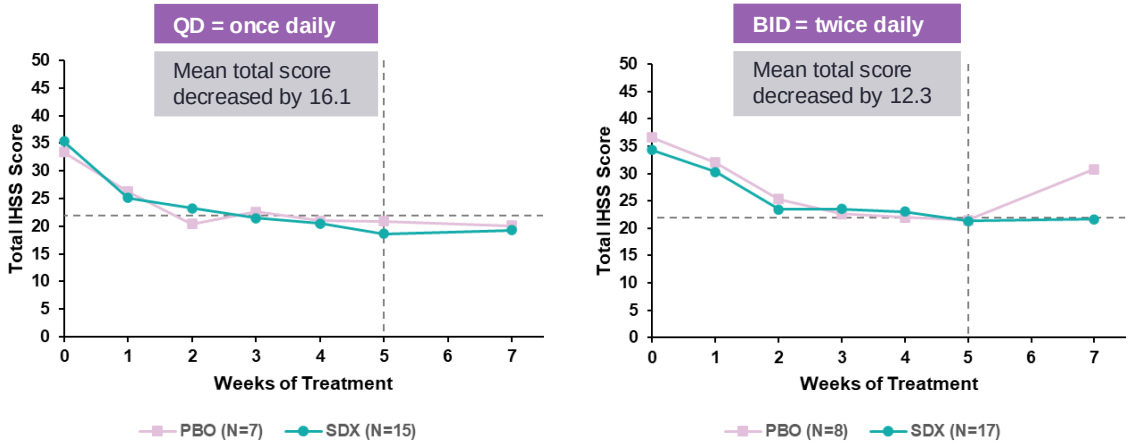
ZEVRA THERAPEUTICS

All patients received active drug in Weeks 1-5; (decrease = improvement)

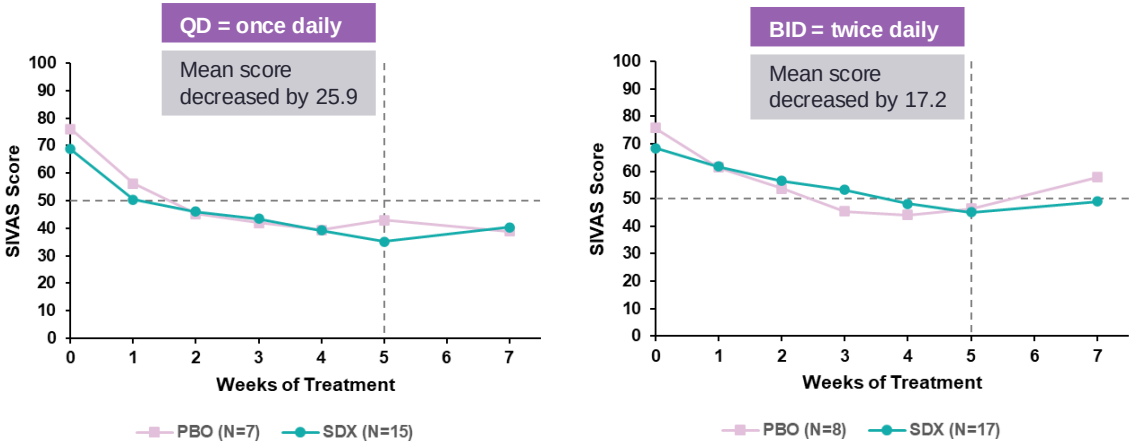
A. Epworth Sleepiness Scale (ESS)



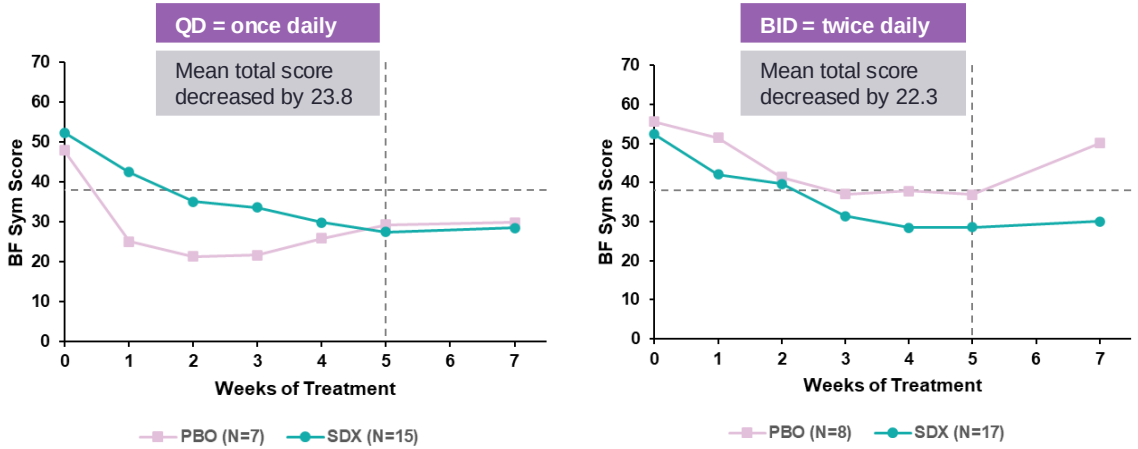
B. Idiopathic Hypersomnia Severity Score (IHSS)



C. Sleep Inertia Visual Analog Scale (SIVAS)



D. Brain Fog Symptom Scale (BFS)



Data presented at SLEEP 2024, Houston, Texas, June 1-5, 2024

Focused on Key Pillars for Strategic Growth

Our Mission:
Bringing life-changing therapeutics to people living with rare diseases




Talent and Culture

- Strong experience in rare disease commercial launches
- Track record of success in drug development and in overcoming complex regulatory challenges

Commercial Excellence

- Rare disease patient-centric capabilities
- MIPLYFFA launch progressing well
- OLPRUVA commercialization underway

Pipeline and Innovation

- Celiprolol: Ongoing Ph. 3 program
- KP1077: Ph. 2 trial complete; evaluating strategic alternatives to advance clinical development

Corporate Foundation: Financial runway into 2027

Thank You

ZEVRA THERAPEUTICS