ZEVRA THERAPEUTICS

A Rare Approach to Therapeutics

January 2025

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 celiprolol 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 April 1, 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.

Adult living with Niemann-Pick disease type C

Zevra Has a Unique Opportunity to Impact People Living with Rare Diseases

Two commercial-stage rare disease products

Advanced clinical development pipeline

Existing infrastructure supportive of future growth

Strong financial position with cash runway into 2027

ZEVRA THERAPEUTICS

Pursuing our Mission to Bring Life-Changing Therapeutics to the Rare Disease Community

Execute	Focus	Innovate

Resources prioritized across four pillars to achieve our vision

Commercial Excellence	Pipeline and Innovation	Talent and Culture	Corporate Foundation
Synergistic commercial products supporting patient access to therapeutics	Potential for growth in-line with commercial capabilities to deliver maximum value to patients	Experienced team with rare disease expertise	Demonstrating Financial Discipline

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

Alison Peters
Chief People Officer

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

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

RARE DISEASE EXPERIENCE

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

RARE DISEASE PRODUCT 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

ZEVRA THERAPEUTICS

Diversified Portfolio to Deliver Value for Patients

	PHASE 1	PHASE 2	PHASE 3	NDA/MAA[iv]	FDA APPROVED	STATUS AND IP
U.S. Commercial	**MIPLYFFA™** *arimoclomol* **Niemann-Pick Disease Type C (NPC)**					**FDA Approval: Sep 20, 2024** **Launch: Nov 21, 2024** **IP through 2031 via ODE[i]**
U.S. Commercial	**OLPRUVA®** *sodium phenylbutyrate for oral suspension* **Urea Cycle Disorders (UCD)**					**FDA Approval: Dec 22, 2022** **Launch: Jan 29, 2024** **IP through 2036**
Partnered	**AZSTARYS®** *serdexmethylphenidate and dexmethylphenidate* **Attention Deficit Hyperactivity Disorder (ADHD)**					**Receiving royalties and milestones on net sales[ii]** **IP through 2037**
Pipeline	**Arimoclomol** **Niemann-Pick Disease Type C (NPC)**					**Exploring regulatory path in Europe and other countries**
Pipeline	**Celiprolol** **Vascular Ehlers-Danlos Syndrome (VEDS)**					**Ph. 3 trial ongoing** **IP through 2038**
Pipeline	**KP1077** **Idiopathic Hypersomnia (IH)**					**Seeking strategic alternative; Ph. 3 trial ready[iii]** **IP through 2037**
Pipeline	**KP1077** **Narcolepsy**					**Ph. 3 trial potential[iii]**

Zevra's Portfolio

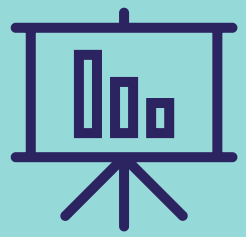
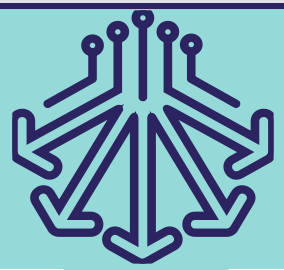
- Asset portfolio targeting rare diseases
- Leverage areas of synergy for commercial portfolio
- Lean infrastructure that can be leveraged with additional products
- Multiple upcoming milestones and catalysts
- Portfolio with commercial and clinical assets

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.
i. Orphan Drug Exclusivity ii. Zevra partnered asset; iii. Seeking strategic alternative iv. New Drug Application\Marketing Authorization Application

ZEVRA THERAPEUTICS

Efficient Team Approach to Providing Patient Access

Rare Disease Specialists	Marketing	Patient Reimbursement Services	Account Management & Contracting	Medical Affairs & Patient Advocacy

Zevra's comprehensive patient support program

AmplifyAssist™

Your ally in what's ahead.

To support the individual needs of eligible patients and those who care for them

Personalized insurance coverage education and support

Disease state information and tools for therapy management

Copay & alternate funding identification assistance for eligible patients' product needs

Interactions to address barriers while facilitating timely prescription refills

ZEVRA THERAPEUTICS

U.S. Prescribing Information for MIPLYFFA™ (arimoclomol capsules)

Indication
MIPLYFFA is indicated for use in combination with miglustat for the treatment of neurological manifestations of Niemann-Pick disease type C (NPC) in adult and pediatric patients 2 years of age and older.

Recommended Dose
Recommended MIPLYFFA oral dosage, in combination with miglustat, for patients with actual body weight of:

- 8 kg to 15 kg, is 47 mg three times a day
- > 15 kg to 30 kg, is 62 mg three times a day
- > 30 kg to 55 kg, is 93 mg three times a day
- > 55 kg, is 124 mg three times a day

MIPLYFFA can be administered with or without food

For full prescribing information, visit MIPLYFFA.com

ZEVRA THERAPEUTICS

NPC is a Neurodegenerative Lysosomal Storage Disorder

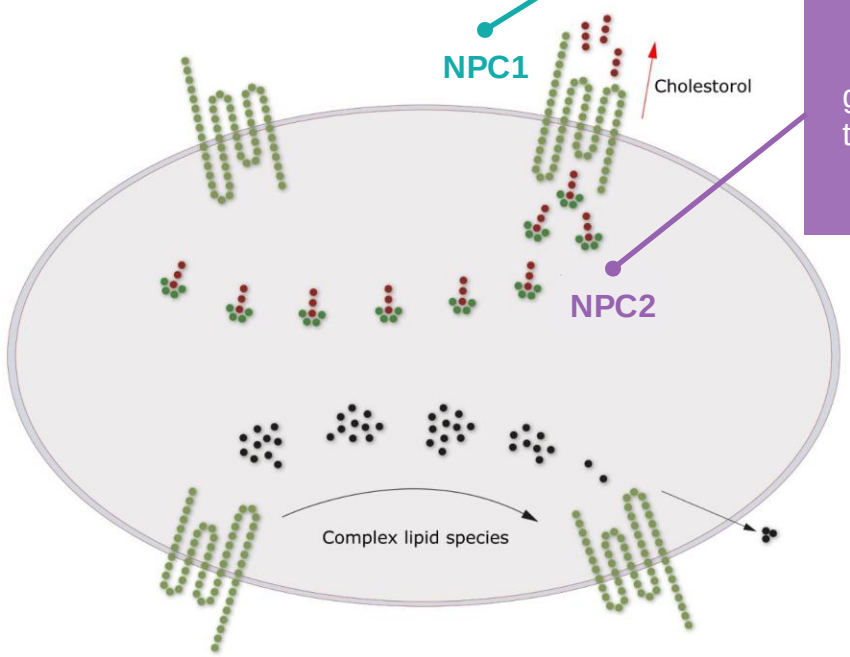
NPC causes cholesterol buildup that leads to cell death

NPC gene mutations produce abnormal, absent, or non-functional NPC proteins[1]

Progressive lipid build up 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

NPC1 is a large, transmembrane domain protein with specific domains that are involved in the transport of lipophilic substrates such as cholesterol and sphingolipids[2,3]



NPC2 is a small, soluble lysosomal glycoprotein that is thought to hand off lipid substrates to NPC1[2,4]

Cholesterol buildup leads to cell death; MIPLYFFA may enhance cholesterol metabolism through improved lysosomal function and upregulation of genes belonging to the CLEAR network[i,5]

i. Coordinated Lysosomal Expression and Regulation (CLEAR)

ZEVRA THERAPEUTICS

NPC is an Ultra-Rare, Heterogenous and Fatal Disease

Child living with Niemann-Pick disease type C

~1,800

Individuals in the U.S. and Europe diagnosed and live with NPC

~900 patients the U.S., of which ~300 are diagnosed or treated[6]

Onset at Any Age

Makes NPC particularly difficult to diagnose

Heterogeneous

Rate of progression, always fatal

~13 Years

Mean age of death[6]

~80%

Of patients who participated in the Phase 2/3 clinical trial took miglustat[7]

Miglustat

The primary treatment for NPC patients in the EU and U.S.[i]

i. Miglustat is not approved in the U.S. for the treatment of NPC

MIPLYFFA is Cornerstone Treatment for NPC

Efficacy	**Proven effectiveness using the rescored 4-domain NPC clinical severity scale (R4DNPCCSS)** MIPLYFFA used in combination with miglustat shown to halt disease progression through 12-months of treatment[7] ➢ Only U.S. approved product for NPC with 5+ years of evidence-based data outcomes
Safety	**Generally well-tolerated therapy** Adverse events were generally mild to moderate in severity and few led to a withdrawal of treatment[7] ➢ >270 NPC patients have been treated, including pivotal studies, open label extension, and Expanded Access Program (EAP)
Dosing	**Oral, weight-based dosing** 3x per day, either by swallowing the whole capsule with or without food, or by adding the contents to a small amount of water, apple juice, or soft foods, or via a feeding tube
Advantage	➢ **MIPLYFFA is the first product approved for treatment of NPC and the only product approved in combination with miglustat shown to halt disease progression through 12-months**

Please see full U.S. Prescribing Information and Important Safety Information including Instructions for Use at: https://zevra.com/documents/MIPLYFFA-Prescribing-Information.pdf

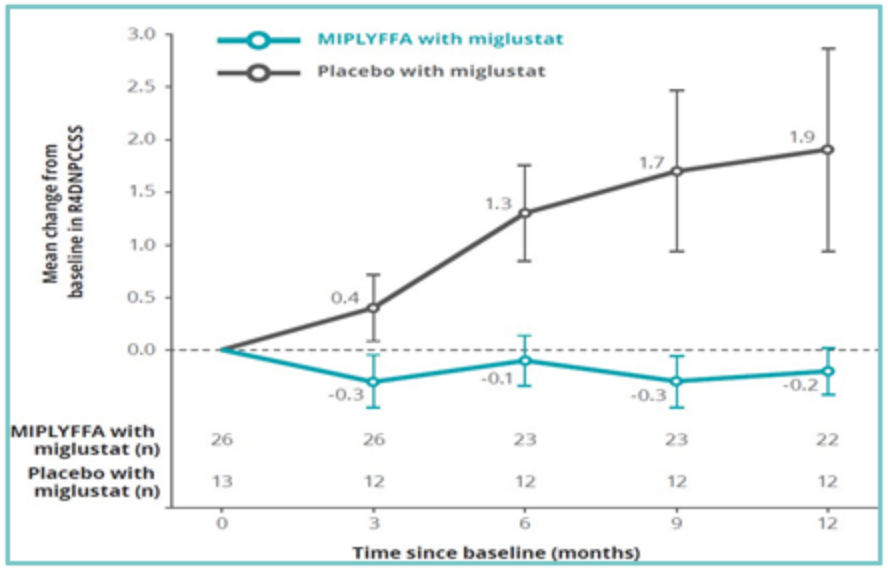
ZEVRA THERAPEUTICS

MIPLYFFA has Proven Effectiveness in Stopping Disease Progression in Patients who also Received Miglustat

Proven Effectiveness: Shown to halt disease progression in patients who also received miglustat[7]

The estimated placebo adjusted mean change from baseline at month 12 was -2.2 pts.

NPC SYMPTOM REDUCTION AT 12 MONTHS
as measured by the R4DNPCCSS score:
ambulation, speech, swallow, and fine motor skills

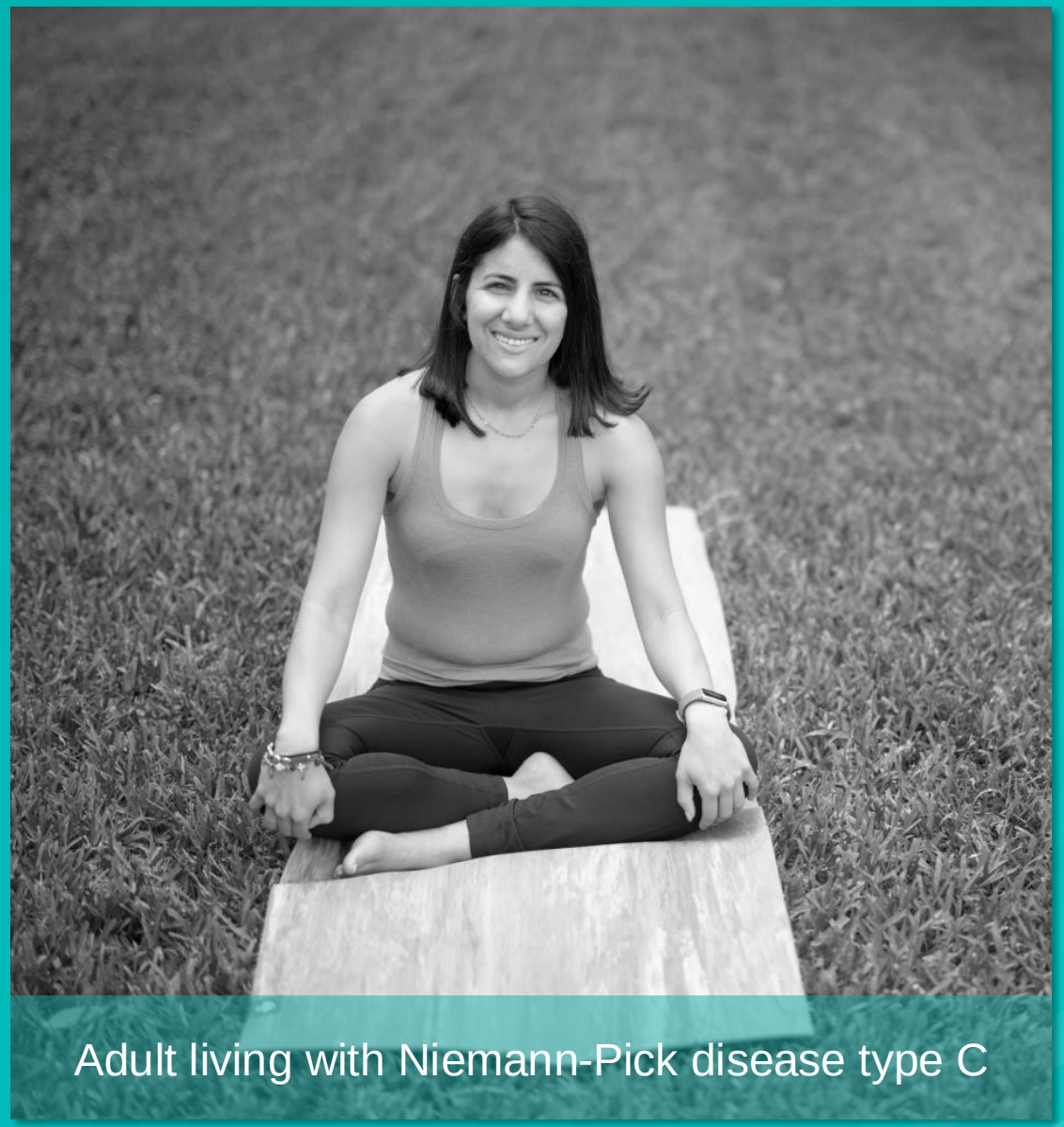


Well-Tolerated Safety Profile: Adverse events were generally mild to moderate in severity and few led to a withdrawal of treatment[7]

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%).

ZEVRA THERAPEUTICS



Adult living with Niemann-Pick disease type C

Early MIPLYFFA Launch Metrics

U.S. Launch Metrics
as of October 31, 2024[i]

- **90** prescription enrollment forms received
  - **69** of which were from U.S. EAP participants
  - **21** patients new to MIPLYFFA and outside of the EAP
- **30% of all prescription enrollment forms approved for reimbursement**

Exploring Regulatory Path in EU

- EU-based EAP continues with ~70 to 80 patients
- ~$2.1M per quarter in net reimbursements from French EAP
- Exploring regulatory path in Europe and other countries

i. Metrics announced during Zevra's 3Q24 earnings call on November 12, 2024

U.S. Prescribing Information for OLPRUVA® (sodium phenylbutyrate) for Oral Suspension

Indication
OLPRUVA is a nitrogen-binding agent indicated as adjunctive therapy to standard of care, which includes dietary management, for the chronic management of adult and pediatric patients weighing 20 kg 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).

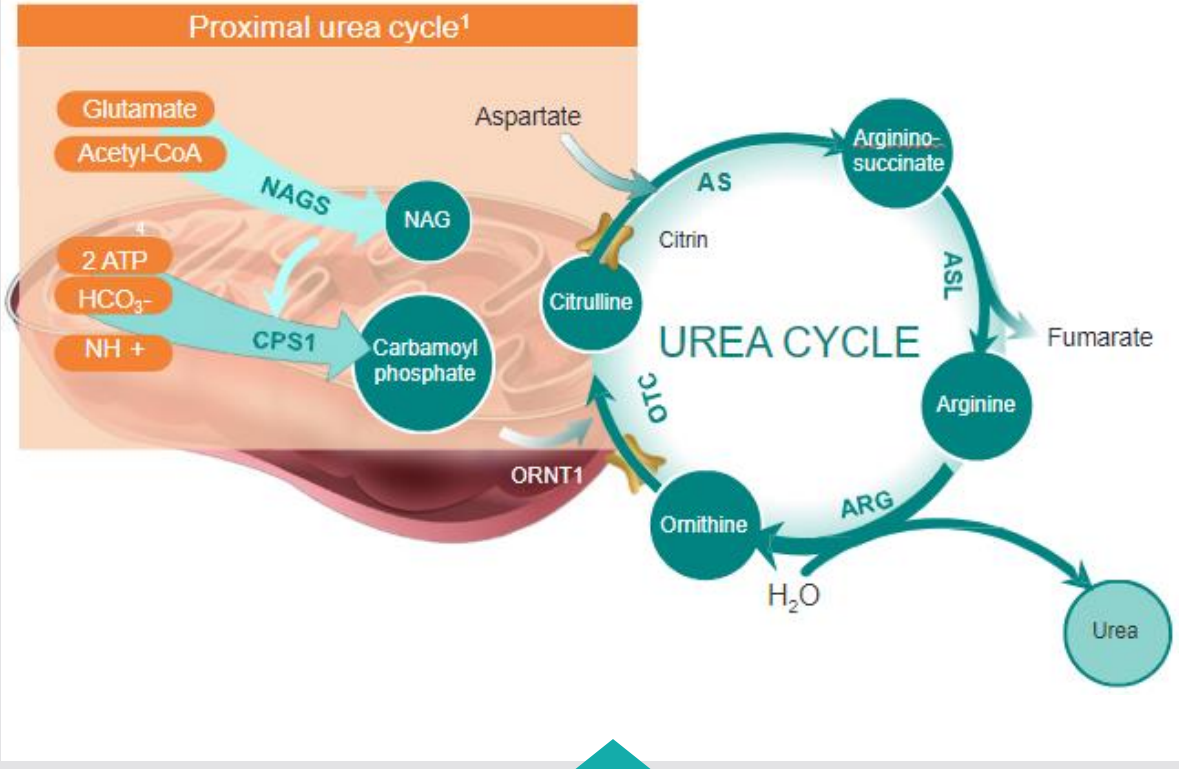
Recommended Dose
The recommended dosage is 9.9 -13 g/m2/day.

For full prescribing information, visit OLPRUVA.com

ZEVRA THERAPEUTICS

UCDs are Rare Inherited Metabolic Disorders Characterized by Hyperammonemia

- Defect in one of the **6 enzymes** or **2 transporters** in urea cycle leads to accumulation of ammonia[8,14]
- Elevated ammonia can be neurotoxic, leading to neurocognitive damage and even death[9-12]
- Signs and symptoms of hyperammonemia can present acutely or chronically, and can first manifest across the age spectrum[9,13-15]
- Treatment goals are to prevent hyperammonemia, achieve normal development & neurocognitive function, & minimize the risk of chronic complications[9,16]

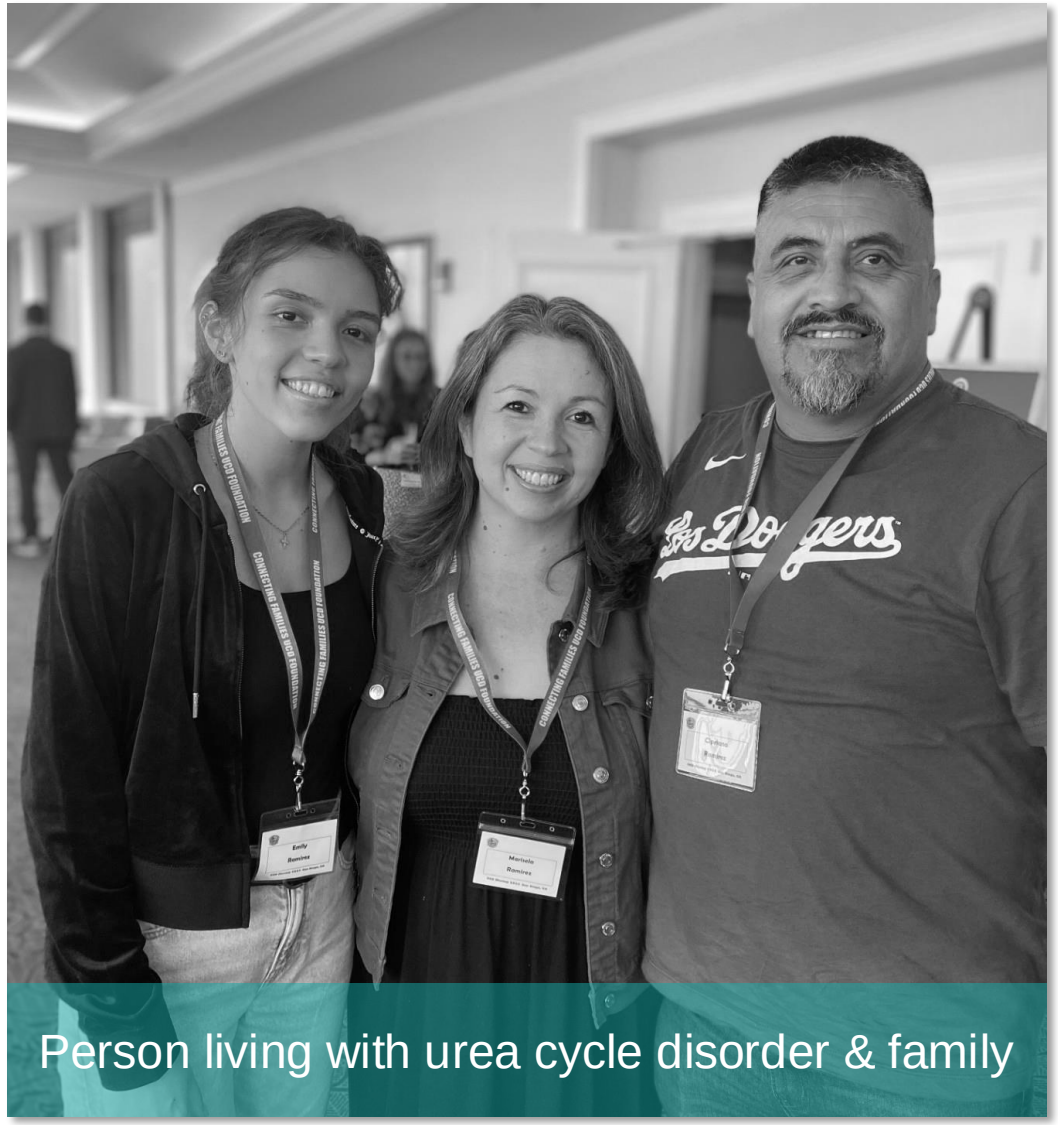


OLRPUVA is a nitrogen scavenger that removes excess ammonia

Figure adapted from Summar ML, Mew NA. Pediatr Clin North Am. 2018;65(2):231-246.[i]

i. 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.

Poor Treatment Adherence Remains an Unmet Need in UCDs



Person living with urea cycle disorder & family

~1,100
Individuals in the U.S. diagnosed in the U.S.[17]

~80%
Of patients have deficiencies in the CPS, OTC, or AS enzymes[i]

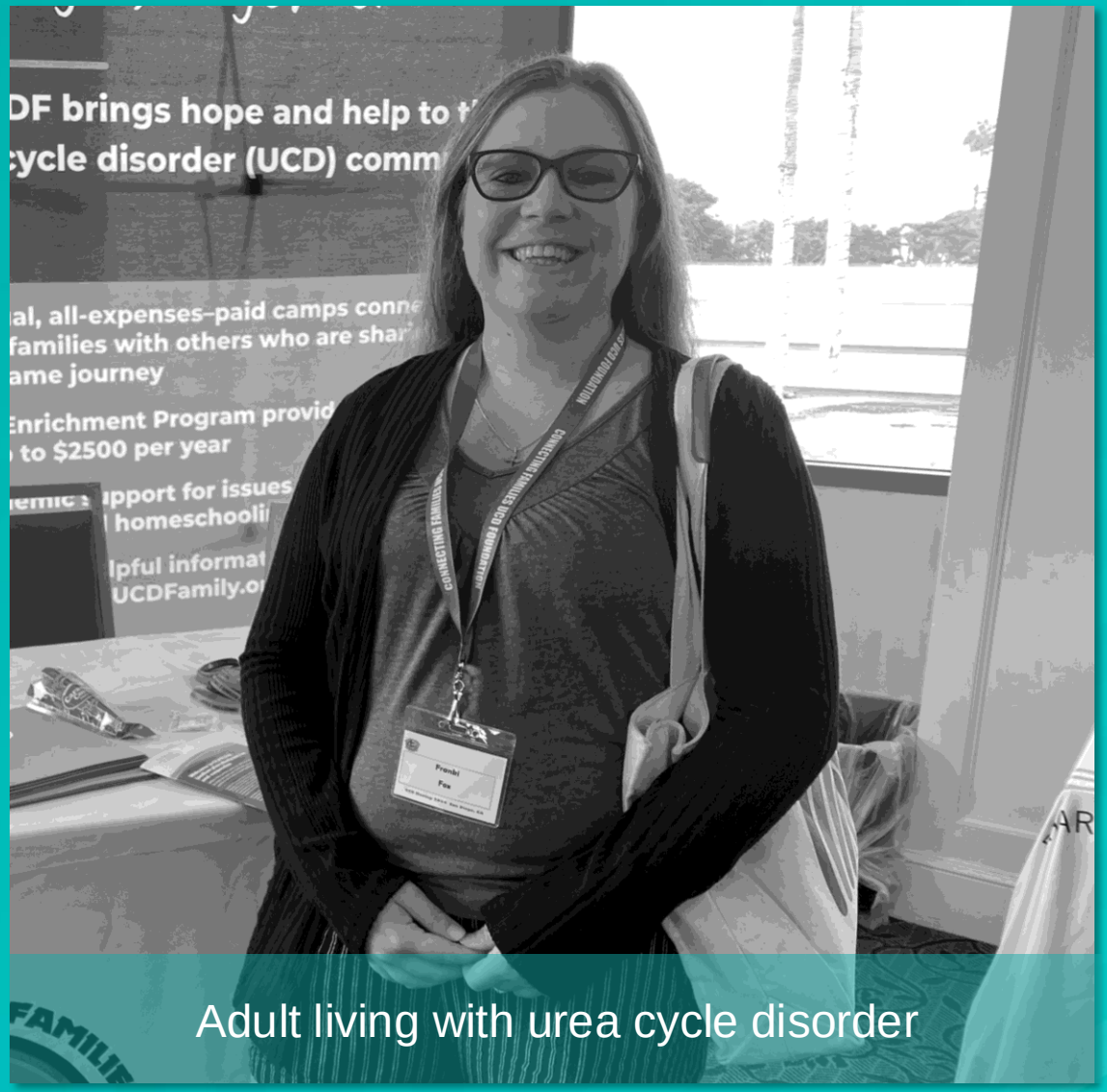
>800
Patients are currently receiving treatment in the U.S.[17]

>25%
Of hyperammonemic crises stem from poor treatment adherence[18]

i. carbamylphosphate synthetase (CPS), ornithine transcarbamylase (OTC), or argininosuccinic acid synthetase (AS)

Potential to Improve Compliance with OLPRUVA through Formulation Designed for Convenience and Palatability

Efficacy	**Established efficacy profile through 505(b)(2) filing in-line with market leader** ➢ OLPRUVA is indicated as adjunctive therapy to standard of care for long-term management of adults and children in UCDs involving deficiencies of CPS, OTC and AS
Safety	**Safety profile in-line with other nitrogen scavengers** ➢ Demonstrated by use of sodium phenylbutyrate for the treatment of UCDs since 1996
Dosing	**Oral, weight-based dosing** ➢ Convenient pre-measured, single-dose envelopes for ease of use and “ammonia control on the go” ➢ Unique formulation designed for palatability and adherence
Advantage	**Novel dual-coated formulation of sodium phenylbutyrate delays release in water for up to 5 minutes**



Adult living with urea cycle disorder

Unmet Medical Needs in UCDs

Important reasons to reassess therapy for patients currently on a nitrogen scavenger therapy

1

Poor Adherence

2

Recurrent Hospital Visits
(hyperammonemic crises)

3

Not on Optimal Treatment & Mild Undiagnosed UCD

ZEVRA THERAPEUTICS

Driving Adoption of OLPRUVA through Patient Experience

Adult Patients Diagnosed
with busy lifestyles

- **Need to take their medication at work**
- Have jobs that allow limited time for treatment
- **Missing their midday dose**
- Patients who travel
- **May want to be discreet or more private with their treatment**
- Experiencing symptoms such as vomiting, confusion, mental fogginess, lethargy

Diagnosed
and naïve to treatments

- Taking medication was inconvenient / not worth the trouble
- Couldn’t tolerate previous medications
- Didn’t like the taste or texture of previous medications
- **May want to be discreet or more private with their treatment**
- **Elevated ammonia and/or glutamine**
- Experiencing symptoms such as vomiting, confusion, mental fogginess, lethargy

Celiprolol

Potential Treatment for Vascular Ehlers-Danlos Syndrome (VEDS)

Celiprolol is investigational, safety and efficacy have not been established

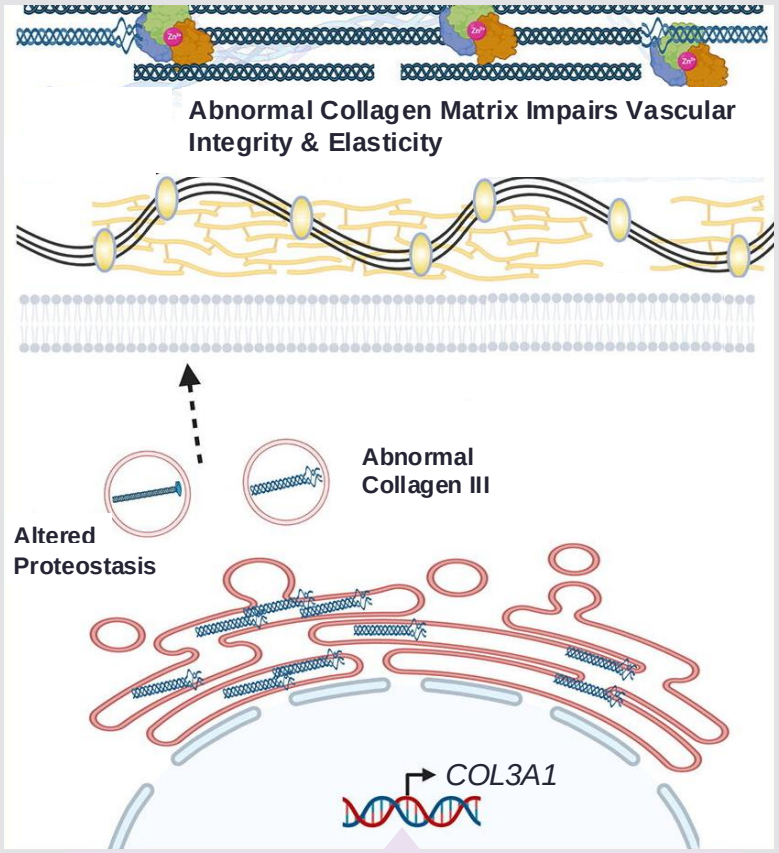
VEDS Impairs Connective Tissue

Most severe Ehlers-Danlos syndrome subtype[19]

Inherited **connective tissue disorder** caused by ***COL3A1*** gene mutations

Leads to defect in type III procollagen in vessel walls and hollow organs

Characterized by arterial aneurysms and hollow organ ruptures[19]



Diagnosis confirmed by presence of mutations in the *COL3A1* gene

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

Significant Unmet Need in VEDS with No Approved Treatments

Adult living with VEDS

~7,500

Individuals in the U.S. diagnosed and live with VEDS[21]

Majority (95%) have genetically confirmed (*COL3A1*) diagnosis[19, 20]

~25%

Of patients experience an event before the age of 20[19]

~90%

Of patients experience an event by the age of 40[19]

51 Years

Median survival age with arterial rupture being the most common cause of sudden death[19]

Surgical intervention

Is the current treatment paradigm

Celiprolol

Is the primary treatment for VEDS patients in several European countries[22]

ZEVRA THERAPEUTICS

Celiprolol is a Potential Treatment of Patients with *COL3A1*+ VEDS

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

New Chemical Entity in the U.S., Orphan Drug Designation and Breakthrough Therapy Designation, & IP to 2038

- Selective adrenergic modulator (SAM)
- 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

Previously Completed Studies & Ongoing DiSCOVER Trial

BBEST Trial, 2010[23]

- Multicenter, randomized, controlled, open-label, event blinded Ph3 study
- Results: 5% annual risk of major vascular event in celiprolol treated vs. 12% in not-treated

Long-Term Observational: French Cohort, 2019[24]

- Retrospective observational study
- Patients not treated with celiprolol had a worse survival outcome than treated patients (p=0.0002)

Long-Term Observational: Swedish Cohort, 2021[25]

- Observational study
- 66% reached target dose of 400mg and tolerated celiprolol well
- Follow up of 106 patient years, five patients suffered major vascular events, 4.7% annual risk

DiSCOVER Trial, Ongoing

- DiSCOVER Phase 3 decentralized (virtual) pivotal study ongoing
- Special Protocol Assignment (SPA) in place

ZEVRA THERAPEUTICS

KP1077 (Serdexmethylphenidate)

Potential Treatment for Idiopathic Hypersomnia (IH)

KP1077 is investigational, safety and efficacy have not been established

IH Causes Brain Fog, Excessive Daytime Sleepiness, & Sleep Inertia

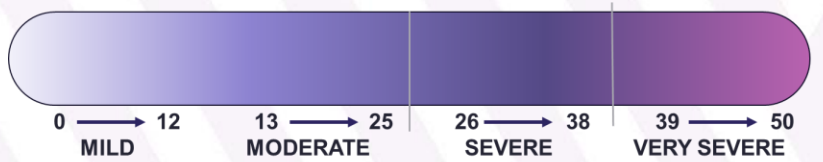
Rare, debilitating, chronic neurologic disorder

Unknown pathophysiology

Also characterized by excessively long sleep times, difficulty waking, & long and unrefreshing naps[i]

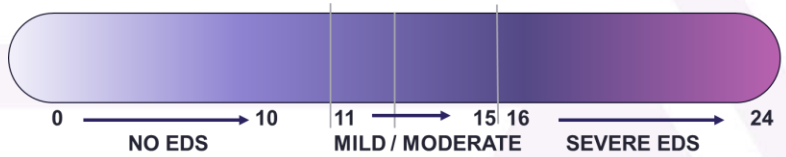
Symptoms measured by Idiopathic Hypersomnia Severity Scale & Epworth Sleepiness Scale

Idiopathic Hypersomnia Severity Scale



- 14 questions on a scale of 0 to 3 or 0 to 4, totaling 50 points[26]
- Higher scores, indicate more severe/frequent symptoms[26]
- Minimal Clinically Important Difference (MCID) of 4 points[27]

Epworth Sleepiness Scale



- 8 questions on a scale of 0 to 3, totaling 24 points[28]
- Higher scores, indicate more severe daytime sleepiness[28]
- 2- to 3-point change is considered MCID in sleep disorders[29]

i. ~25% of patients “long sleepers,” >10hrs.

ZEVRA THERAPEUTICS

Remaining Unmet Need in IH

Adult living with IH

~37,000

Individuals in the U.S. diagnosed and live with IH, though the total population may be much larger[30]

~66%

Report IH symptoms as High Impact including not feeling refreshed, fatigue, excessive daytime sleepiness, and brain fog[31]

~85%

Require more than a first-line therapy due to unresolved symptoms[32]

Current Treatments Don't Address Needs

Patients rated **current medication effectiveness as poor** (5.4 on a 10-point scale)[33]

KP1077 has a Differentiated Profile Addresses Unmet Needs in IH

KP1077 for IH

- SDX[i] is a proprietary prodrug of d-MPH
- Two dosing regimens being explored:
  - Once daily at bedtime
  - Twice daily once in the morning and once at bedtime

Improved Safety and Tolerability Over Existing Treatments

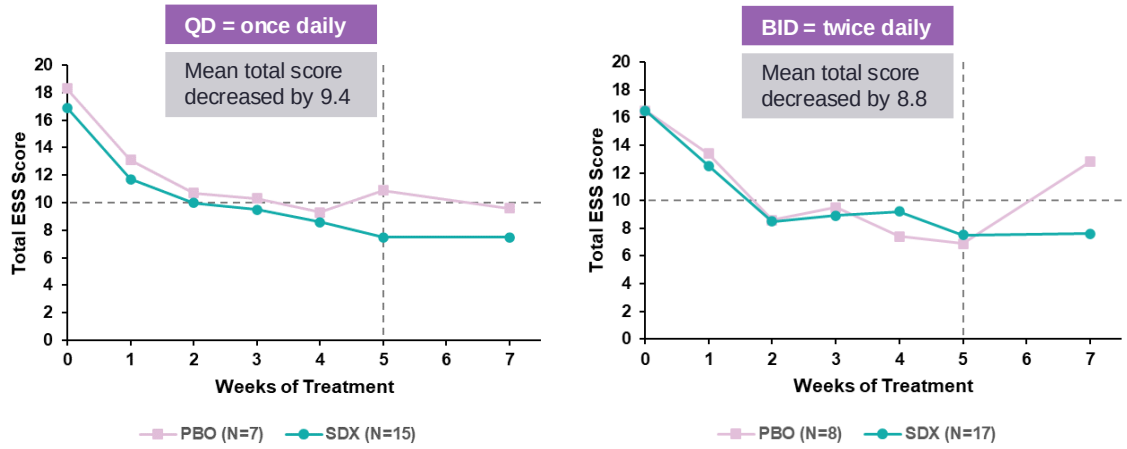
- Unique pharmacokinetic profile to overcome key symptoms
- Greater tolerability, lower cardiovascular effects, lower abuse potential
- No DDI potential with hormonal contraceptives; antidepressants

Regulatory & IP Advantages

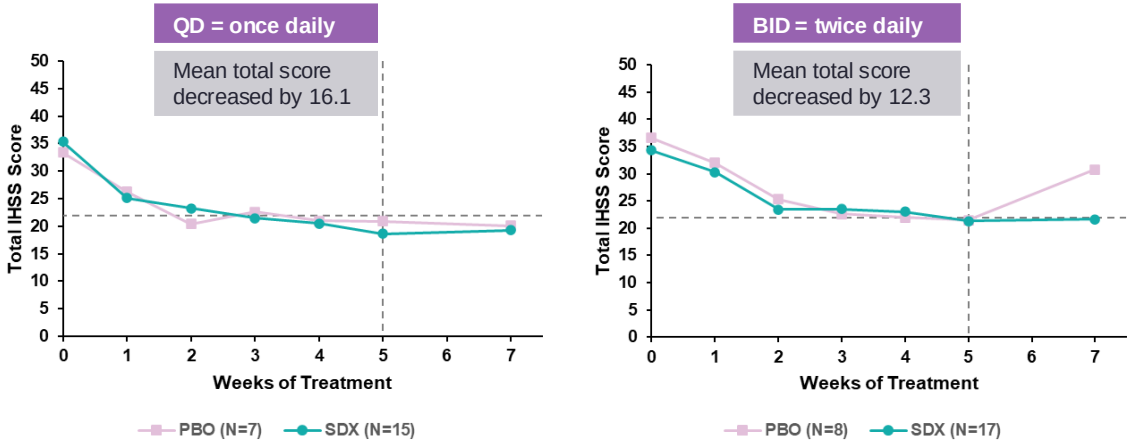
- Orphan Drug Designation in IH
- Conducted End-of-Phase 2 meeting with the FDA at the end of Q3 2024
- SDX is designated Schedule IV controlled substance by DEA in the U.S.[ii]

Results from Ph. 2 Clinical Trial[iii]; All patients received active drug in Weeks 1-5; (decrease = improvement)

A. Epworth Sleepiness Scale (ESS)



B. Idiopathic Hypersomnia Severity Score (IHSS)



i. Serdexmethylphenidate
ii. 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)
iii. Data presented at SLEEP 2024, Houston, Texas, June 1-5, 2024

ZEVRA THERAPEUTICS

Strong Corporate Foundation Demonstrating Financial Discipline

Financial Position is a Source of Strength

Q3 2024 Income Statement Details:

- Net revenue of $3.7M, comprised of $2.6M from the arimoclomol French EAP program, and $1.1M under the AZSTARYS® license agreement
- Q3 2024 net loss attributable to common stockholders of ($33.2M), or ($0.69) per basic and diluted share, driven primarily by R&D expense of $10.9M, and general and administrative expense of $16.2M

Q3 2024 Balance Sheet Details:

- Cash, cash equivalents and investments were $95.5M
- Available cash, cash equivalents and investments expected to extend cash runway into 2027
  - cash runway does not include potential proceeds from sale of PRV[i]
- Debt of $60M, additional $20M tranche available

Common and Fully Diluted Shares Outstanding:

- As of Sep 30, 2024, common and fully diluted shares outstanding were:

	(millions)
Common shares outstanding	**53.2**
Outstanding awards under equity incentive plans	9.0
Outstanding common stock warrants	5.5
Fully diluted shares outstanding	**67.7**

i. Rare pediatric disease Priority Review Voucher (PRV)

Zevra has a Unique Opportunity to Impact People Living with Rare Diseases

Two commercial-stage rare disease products

- Launch of MIPLYFFA, cornerstone therapy for people living with NPC
- OLPRUVA launched for the treatment of certain UCDs

Advanced clinical development pipeline

- Prioritizing arimoclomol's regulatory submission in Europe
- Ongoing Phase 3 program for treatment of VEDS
- Seeking strategic alternatives to develop and commercialize KP1077, Phase 3 ready, for rare sleep disorders

Existing infrastructure supportive of future growth

- Aspire to become a "partner of choice" for rare disease products
- Leverage current commercial infrastructure

Strong financial position with cash runway into 2027

- Disciplined capital allocation
- Cash runway into 2027 without potential proceeds from sale of PRV
- Potential to monetize PRV
- Royalty generating asset (AZSTARYS) and French EAP (arimoclomol) contribution

ZEVRA THERAPEUTICS

ZEVRA THERAPEUTICS

A Rare Approach to Therapeutics

January 2025

NasdaqGS: ZVRA

Children living with Niemann-Pick disease type C

SOURCES

1. Geberhiwot T, et al. Orphanet J Rare Dis. 2018 Apr 6;13(1):50.
2. Carstea ED et al. Science. 1997;277:228-231.
3. Platt FM et al. Annu Rev Genomics Hum Genet. 2014;15:173-194.
4. Ingemann L, Kirkegaard T J Lipid Res. 2014;55:2198-2210.
5. MRC-2400139-ASHG-2024-Arimoclomol-In-Vitro-Poster-11-11.pdf
6. Burton et.al., Molecular Genetics and Metabolism Volume 134, Issues 1–2, September–October 2021, Pages 182-187
7. MIPLYFFA Full Prescribing Information. Celebration, FL, US, Zevra Therapeutics Inc.; 09/2024.
8. Summar ML, Mew NA. Pediatr Clin North Am. 2018;65(2):231-246.
9. Häberle J, et al. *J Inherit Metab Dis.* 2019;42(6):1192-1230.
10. Helman G, et al. *Semin Neurol*. 2014;34(3):341-349.
11. Matsumoto S, et al. *J Human Genet*. 2019;64(9):833-847.
12. Auron A, Brophy PD. *Pediatr Nephrol*. 2012;27:207-222.
13. Berry GT, Steiner RD. *J Pediatr*. 2001;138:S56-S61.
14. Bernstein LE, Rohr F, van Calcar S. *Nutrition Management of Inherited Metabolic Diseases* (2nd ed.). Cham, Switzerland: Springer; 2022.
15. Strong A, et al. *Clin Liver Dis*. 2021;18(4):198-203.
16. Waisbren SE, et al. *J Inherit Metab Disord*. 2019;42:1176-1191.
17. HealthVerify Payer Claims data analysis 2021
18. Enns GM, Porter MH, Francis-Sedlak M, Burdett A, Vockley J. 2019
19. Pepin M, et al. Genet Med. 2014 Dec;16(12):881-8
20. Leistritz, D., Pepin, M., Schwarze, U. et al. COL3A1 haploinsufficiency results in a variety of Ehlers-Danlos syndrome type IV with delayed onset of complications and longer life expectancy. Genet Med 13, 717–722 (2011). https://doi.org/10.1097/GIM.0b013e3182180c89
21. Estimate based on an analysis of diagnosed vEDS patients from the Truven MarketScan® database and U.S. population data.
22. FightvEds.org
23. Ong et al., Lancet 2010; 376: 1476-1484
24. Frank et al., J Am Coll Cardiol. 2019 Apr 23;73(15):1948-1957
25. Baderkhan et al., Eur J Vasc Endovasc Surg (2021) 61, 326-331
26. 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.
27. 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.
28. Johns MW. A new method for measuring daytime sleepiness: the Epworth Sleepiness Scale. Sleep. 1991;14(6):540–545
29. 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.
30. https://www.sleepcountshcp.com/what-is-idiopathic-hypersomnia
31. https://sleepconsortium.org/illuminate-hypersomnia/
32. Zevra Research Report (2023)
33. https://www.sleepcountshcp.com/idiopathic-hypersomnia-treatment-options

ZEVRA THERAPEUTICS