UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of Earliest Event Reported): January 8, 2024

Zevra Therapeutics, Inc.
(Exact Name of Registrant as Specified in Its Charter)

Delaware	**001-36913**	**20-5894398**
(State or Other Jurisdiction of Incorporation)	**(Commission File Number)**	**(I.R.S. Employer Identification No.)**
1180 Celebration Boulevard, Suite 103, Celebration, FL		**34747**
(Address of Principal Executive Offices)		**(Zip Code)**

Registrant's Telephone Number, Including Area Code: (321) 939-3416

(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock	ZVRA	The Nasdaq Stock Market LLC (Nasdaq Global Select Market)

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 7.01 Regulation FD Disclosure.

On January 8, 2024, Zevra Therapeutics, Inc., or the Company, issued a press release announcing that the U.S. Food and Drug Administration, or the FDA, has acknowledged receipt of the Company's resubmission of the New Drug Application, or NDA, for arimoclomol as an orally-delivered, first-in-class treatment for Niemann-Pick disease type C. In addition, the Company updated its corporate presentation.

A copy of the press release and presentation are furnished as Exhibit 99.1 and Exhibit 99.2, respectively, to this Current Report on Form 8-K. The information contained in this Item 7.01, the press release furnished as Exhibit 99.1 and the presentation furnished as Exhibit 99.2, shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and are not incorporated by reference into any of the Company's filings under the Securities Act of 1933, as amended, whether made before or after the date hereof, except as shall be expressly set forth by specific reference in any such filing

Item 8.01. Other Events.

On January 8, 2024, under the Prescription Drug User Fee Act ("PDUFA"), the FDA has deemed the arimoclomol NDA resubmission to be a Class I complete response which has a six-month review period from the date of resubmission. As a result, the FDA has assigned a PDUFA action date of June 21 2024, and currently intends to present the resubmission for discussion in an advisory committee.

Item 9.01. Financial Statements and Exhibits.

The following exhibits relating to Item 7.01 shall be deemed to be furnished, and not filed:

(d) Exhibits

Exhibit No.	Description
99.1	Press Release dated January 8, 2024.
99.2	Corporate Presentation dated January 2024.
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Zevra Therapeutics, Inc.

Date: January 8, 2024

By: /s/ R. LaDuane Clifton
R. LaDuane Clifton, CPA
Chief Financial Officer, Secretary and Treasurer

Zevra Therapeutics Receives FDA Acceptance of Resubmission of NDA for Arimoclomol as a Treatment for Niemann-Pick Disease Type C

Arimoclomol NDA has been assigned a PDUFA action date of June 21, 2024

CELEBRATION, Fla., **January 8, 2024** (GLOBE NEWSWIRE) -- **Zevra Therapeutics, Inc. (NasdaqGS: ZVRA)** (Zevra or the Company), a rare disease therapeutics company, today announced that the U.S. Food and Drug Administration (FDA) has acknowledged receipt of the resubmission of the New Drug Application (NDA) for arimoclomol as an orally-delivered, first-in-class treatment for Niemann-Pick disease type C. Under the Prescription Drug User Fee Act ("PDUFA"), the FDA has deemed the arimoclomol NDA resubmission to be a Class II complete response which has a six-month review period from the date of resubmission. As a result, the FDA has assigned a PDUFA action date of June 21, 2024, and currently intends to present the resubmission for discussion in an advisory committee.

"We are very pleased that the FDA has accepted the resubmission of the arimoclomol NDA following multiple collaborative and constructive meetings," said Neil F. McFarlane, President and Chief Executive Officer of Zevra. "This significant milestone brings us one step closer to the potential approval of arimoclomol for a community of patients with debilitating unmet medical needs. We would like to take the opportunity to acknowledge the NPC community for their continued support throughout the development of arimoclomol."

Zevra believes that its resubmission of the arimoclomol NDA addresses the concerns previously raised in the June 2021 complete response letter ("CRL") issued by the FDA in response to the prior arimoclomol NDA filing. The resubmission includes additiona evidence supporting trial metrics, FDA-preferred analyses, and data from multiple additional studies that provide supporting evidence of arimoclomol's efficacy in clinical and non-clinical settings.

About Niemann-Pick Disease Type C (NPC):

Niemann-Pick disease type C (NPC) is an ultra-rare, progressive, neurodegenerative lysosomal storage disorder characterized by an inability of the body to transport cholesterol and other lipids within the cell, leading to an accumulation of these substances in various tissue areas, including brain tissue. The disease is caused by mutations in the NPC1 or NPC2 genes, which are responsible for making lysosomal proteins. Both children and adults can be affected by NPC with varying clinical presentations. Those living with NPC lose independence due to physical and cognitive limitations, with key neurological impairments presenting in speech, cognition, swallowing, ambulation, and fine motor skills. Disease progression is irreversible and can be fatal within months or take years to be diagnosed and advance in severity.

About Arimoclomol:

Arimoclomol, Zevra's orally delivered, first-in-class investigational product candidate for the treatment of NPC, has been granted Orphan Drug designation, Fast Track designation, Breakthrough Therapy designation, and Rare Pediatric Disease designation by the FDA, and Orphan Medicinal Product designation for the treatment of NPC by the European Medicines Agency (EMA).

About Zevra Therapeutics:

Zevra Therapeutics is a rare disease company melding science, data, and patient needs to create transformational therapies for diseases with limited or no treatment options. With unique, data-driven clinical, regulatory, and commercialization strategies, the Company is overcoming complex drug development challenges to bring much-needed therapies to patients. With regulatory, clinical-stage and commercial assets, the Company is building its capabilities to make new therapies available to the rare disease community.

Expanded access programs are made available by Zevra Therapeutics and its affiliates and are subject to the Company's Expanded Access Program (EAP) policy as published on its website at www.zevra.com. Participation in these programs is subject to the laws and regulations of each jurisdiction under which each respective program is operated. Eligibility for participation in any such program is at the treating physician's discretion.

Cautionary Note Concerning Forward-Looking Statements:

This press release may contain forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements include all statements that do not relate solely to historical or current facts, and which can be identified by the use of words such as "may," "will," "expect," "project," "estimate," "anticipate," "plan," "believe," "potential," "should," "continue," "could," "intend," "target," "predict," or the negative versions of those words or other comparable words or expressions, although not all forward-looking statements contain these identifying words or expressions. Forward-looking statements are not guarantees of future actions or performance. These forward-looking statements include without limitation statements regarding Zevra's strategic and product development objectives, including with respect to becoming a leading rare disease company, the content, timing or results of any NDA submissions or resubmissions for arimoclomol or any other product candidates for any specific disease indication or at any dosage, the potential therapeutic benefits and effectiveness of arimoclomol and any other products and product candidates, and Zevra's plans, goals and expectations concerning market position, future operations and other financial and operating information. Forward-looking statements are based on information currently available to Zevra and its current plans or expectations, and are subject to several known and unknown uncertainties, risks, and other important factors that may cause actual results, performance, or achievements to be materially different from any future results, performance, or achievements expressed or implied by the forward-looking statements. These and other important factors are described in detail in the "Risk Factors" section of Zevra's Annual Report on Form 10-K for the year ended December 31, 2022, as updated in Zevra's Quarterly Report on Form 10-Q for the quarter ended September 30, 2023, and Zevra's other filings with the Securities and Exchange Commission. While Zevra may elect to update such forward-looking statements at some point in the future, it disclaims any obligation to do so, except as required by law, even if subsequent events cause their respective views to change. Although Zevra believes the expectations reflected in such forward-looking statements are reasonable, it cannot assure that such expectations will prove correct. These forward-looking statements should not be relied upon as representing Zevra's views as of any date after the date of this press release.

Zevra Contacts:

Nichol Ochsner
+1 (732) 754-2545
nochsner@zevra.com

Russo Partners Contacts:
David Schull
+1 (858) 717-2310
david.schull@russopartnersllc.com

Ignacio Guerrero-Ros
+1 (646) 942-5604
ignacio.guerrero-ros@russopartnersllc.com

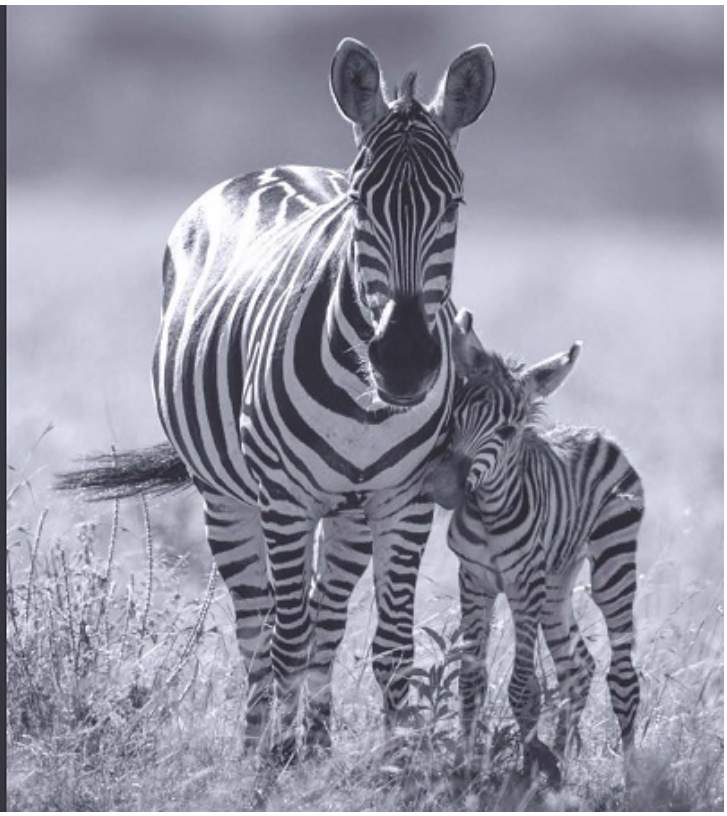
ZEVRA THERAPEUTICS

Corporate Presentation

January 2024

A Rare Approach to Therapeutics

NASDAQ: ZVRA



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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

Focused on Key Pillars for Strategic Growth

ZEVRA THERAPEUTICS

Our Mission:

Bringing life-changing therapeutics to people living with rare diseases

Experienced team with rare disease expertise

Commercial excellence to ensure patient access to therapeutics

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

Experienced Team with Rare Disease Expertise

ZEVRA THERAPEUTICS

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

Neil F. McFarlane
CEO and President

Adrian W. Quartel, MD FFPM
Chief Medical Officer

Joshua Schafer
Chief Commercial Officer & EVP of BD

Christal Mickle
Chief Development Officer & Co-Founder

R. LaDuane Clifton, CPA
CFO, Secretary & Treasurer

Sven Guenther, Ph.D.
Chief Scientific Officer

RARE DISEASE EXPERIENCE

ADAMAS · genzyme · BIOMARIN · Mallinckrodt Pharmaceuticals · astellas · Retrophin · Takeda

RARE PRODUCT EXPERIENCE

GOCOVRI · Thiola EC (tiopronin) · INOmax (nitric oxide) · EVENITY (romosozumab-aqqg) · Cholbam (cholic acid) capsules · Xtandi (enzalutamide) · OLPRUVA (sodium phenylbutyrate) for oral suspension · XOSPATA gilteritinib

Commercial Excellence to Ensure Patient Access to Rare Disease Therapies

ZEVRA THERAPEUTICS

Overlap in Prescribers and Centers of Excellence between UCD and NPC indications allow for efficient team approach

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

Patient Services and Resources support to assist patients navigate reimbursement and treatment journey

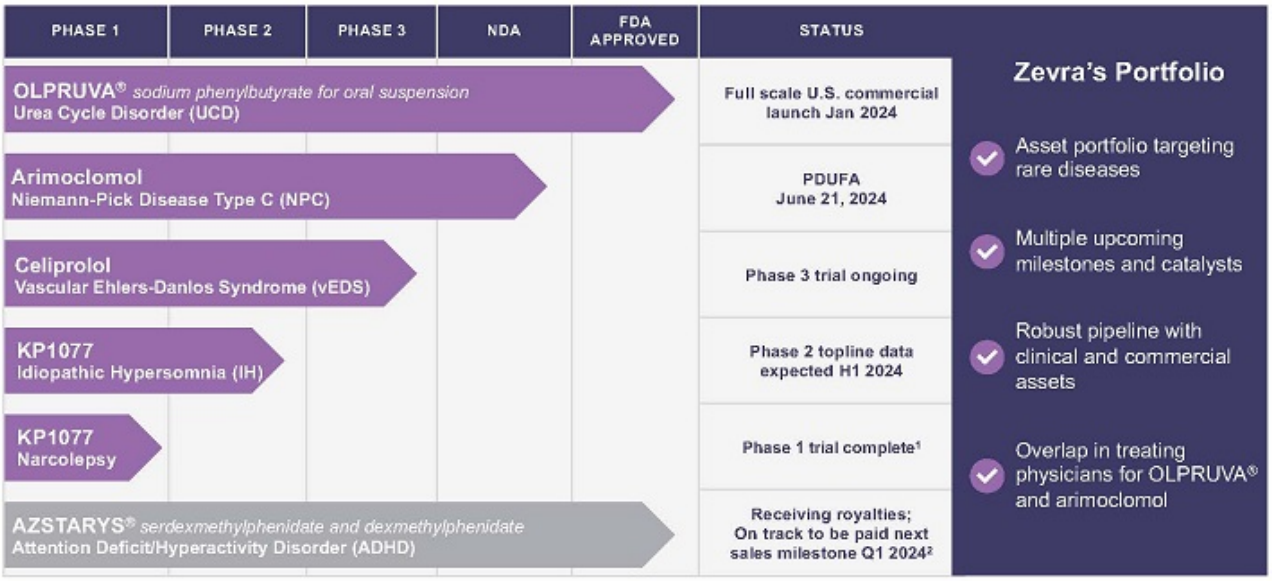
Marketing team to define appropriate patient identification and product positioning in treatment landscape

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

Account Management team to ensure market access and contracting with payors

Growing Pipeline with Potential to Bring New Products and Deliver Value for Patients

ZEVRA THERAPEUTICS

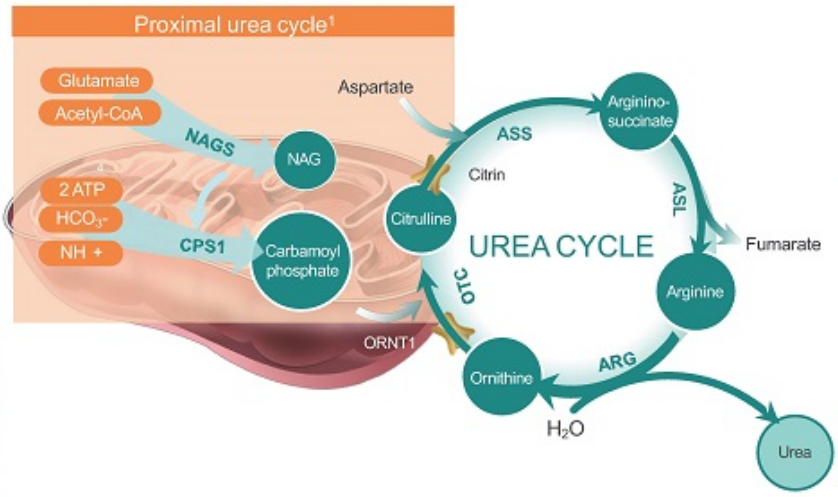


1. Data generated from this trial will be analyzed alongside the Phase 2 IH data to support clinical development of both narcolepsy and IH programs.
2. Zevra partnered asset

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

ZEVRA THERAPEUTICS

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 (HAC) crises
- Elevated ammonia levels can be neurotoxic, leading to neurocognitive damage, potentially coma 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.

Unmet Need in Urea Cycle Disorders

ZEVRA THERAPEUTICS

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

Orphan Designation

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

Unmet Need

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

United States (U.S.) Market
1. https://www.drugs.com/slideshow/top-10-most-expensive-drugs-1274
2. HealthVerity 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 UCDs

ZEVRA THERAPEUTICS

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

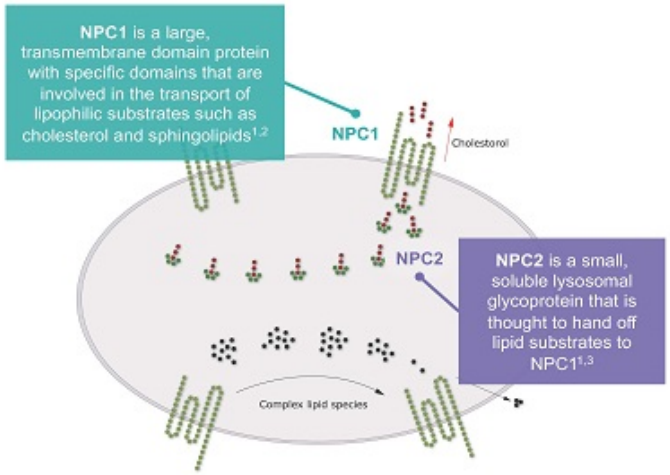
OLPRUVA helps the body get rid of excess nitrogen to help avoid dangerous buildup of ammonia

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

Niemann-Pick Disease Type C is a Progressive Lysosomal Storage Disorder

ZEVRA THERAPEUTICS

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



- 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.

No Approved NPC Treatments in the U.S.

ZEVRA THERAPEUTICS

Ultra-rare, relentlessly progressive and fatal neurodegenerative disease

Orphan Designation

- Incidence: 1 in 100,000-120,000 live births[1]
- Prevalence:
  - 1,800 patients diagnosed worldwide
  - 900 patients estimated in U.S.[2]
  - 300-350 patients diagnosed in U.S.

Significant Unmet Need

- Neurocognitive decline adversely impacts daily living
- Irreversible and potentially fatal disease
- Mean age of death is 13 years[2]
- No approved treatments exist in the U.S.

(1) https://rarediseases.org/
(2) Molecular Genetics and Metabolism Volume 134, Issues 1–2, September–October 2021, Pages 182-187 (Bianconi, 2019)

Arimoclomol is Positioned to Become First-Line Treatment for NPC Patients

ZEVRA THERAPEUTICS

Evidence indicates that arimoclomol acts on multiple fronts to help reduce lipid build-up in cells with improved lysosomal function

FIRST-IN-CLASS, ORAL TREATMENT

- Potential to be a foundational therapy in U.S. for NPC, if approved
- Oral capsules can be swallowed whole, mixed with foods/liquids or delivered through feeding tube

EXTENSIVE CLINICAL EXPERIENCE WITH DEMONSTRATED SAFETY

- No significant safety findings (600+ patients treated)
- NPC pivotal trial data demonstrate reduced disease progression[1]
- Long-term data suggest improved outcomes vs. historical controls[2]
- Ongoing global Expanded Access Program (EAP) with >150 patients treated in U.S. and E.U.

ADVANTAGEOUS REGULATORY DESIGNATION

- Orphan Drug Designation for NPC
- Fast-Track and Breakthrough Therapy Designations
- Eligible for Pediatric Rare Disease Voucher if approved
- Estimated value of ~$100M

Synergies and scale with an efficient customer-facing team supporting both launches

1. Mengel E et al. J Inherit Metab Dis. 2021 Nov;44(6):1463-1480.
2. 4-year open-label extension of Phase 2/3 trial

ZEVRA THERAPEUTICS

Growing Pipeline in Rare Diseases

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

ZEVRA THERAPEUTICS

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

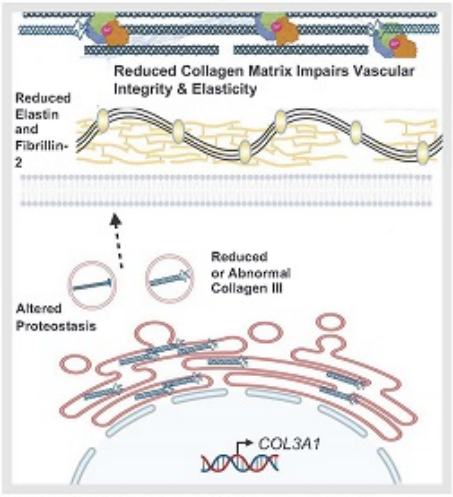


Figure adapted from Omar R, et al. Matrix Biol Plus. 2021 Nov 9;12:100090.

- vEDS (EDS type IV) is the severe subtype:
  - Characterized by aneurysms, dissections and/or ruptures
  - Vascular
  - Hollow Organs (e.g., gastrointestinal, uterine)
- Autosomal dominant (50%); spontaneous mutations (50%)
- Diagnosed by clinical symptoms and confirmed by presence of mutations in the COL3A1 gene
- Events occur in 25% of patients before the age of 20, and 90% by the age of 40
- 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

Orphan Designation

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

Significant Unmet Need

- No approved options 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 is a Selective Adrenergic Modulator for Potential Treatment of patients with COL3A1+ vEDS

ZEVRA THERAPEUTICS

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





CELIPROLOL FOR VEDS

- 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 Trial: 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 trial ongoing

REGULATORY & IP ADVANTAGES

- New Chemical Entity in the U.S.
- Orphan Drug designation and Breakthrough Therapy Designation
- Special Protocol Assessment in place
- Registration enabling Phase 3 trial
- Solid IP until 2038

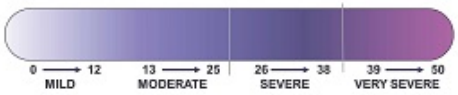
(1) https://www.sciencedirect.com/science/article/pii/S0735109719336939

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

ZEVRA THERAPEUTICS

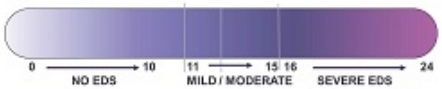
KP1077 may provide optimal exposure of methylphenidate to better address these unmet needs

Idiopathic Hypersomnia Severity Scale



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

Epworth Sleepiness Scale



- 8 questions on a scale of 0 to 3, totaling 24 points[4]
- Higher scores, indicate more severe daytime sleepiness
- 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
- Excessively long sleep times
- Sleep inertia or difficulty waking
- Long and unrefreshing nap[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

Orphan Designation

- 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 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 is a Novel Approach to Treating IH

ZEVRA THERAPEUTICS

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
  - 2x daily: once in the morning and once at bedtime

IMPROVED SAFETY & TOLERABILITY OVER EXISTING TREATMENTS

- Unique pharmacokinetic profile
- Greater tolerability and lower cardiovascular effects
- No DDI potential with hormonal contraceptives; antidepressants

REGULATORY & IP ADVANTAGES

- Orphan Drug designation in IH
- Solid IP through 2037 and potentially beyond
- Less abuse potential (SDX is designated Schedule IV controlled substance by DEA in the U.S.)[1]

1. 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).

Focused on Key Pillars for Strategic Growth

ZEVRA THERAPEUTICS

Our Vision: To give every promising rare disease therapy a fighting chance to improve the lives of the patients we serve

Rare Disease Team

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

Commercial Excellence

- Growing capabilities in-line with vision for a bespoke patient services approach
- Immediate focus on driving awareness and demand for OLPRUVA®
- Preparing for arimoclomol launch

Growing Pipeline

- Arimoclomol: PDUFA date on Jun 21, 2024
- Celiprolol: Phase 3 program
- KP1077: Topline results H1 2024

Financial strength to execute on our key priorities

Appendix

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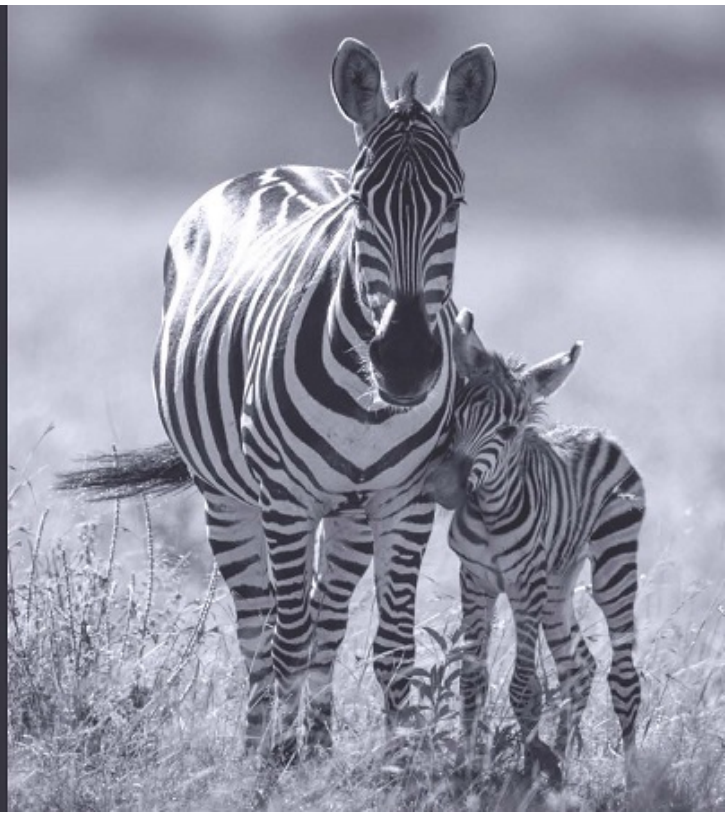

Arimoclomol NDA Resubmitted to FDA

Complete Response Letter (CRL) and FDA feedback gathered through multiple interactions/meetings has provided added clarity on resubmission package.

CRL	ZEVRA'S ONGOING RESPONSE
1 Sufficiency of validation and reliability of the Niemann-Pick type C Clinical Severity Scale (NPCCSS) instrument	Additional evidence being provided to support use of the NPCCSS as the primary instrument in measuring NPC disease progression
2 Appropriateness of how to handle data affected by certain patient events and method of primary endpoint analysis	Using FDA preferred primary analysis and supportive additional analyses
3 Robustness of confirmatory evidence to support single efficacy trial	Additional data from multiple new nonclinical studies being provided, data from the 4-year open label extension of the Phase 2/3 clinical trial

PDUFA: June 21, 2024

Two Dosing Regimens Being Explored to Achieve Sustained Symptom Management in IH

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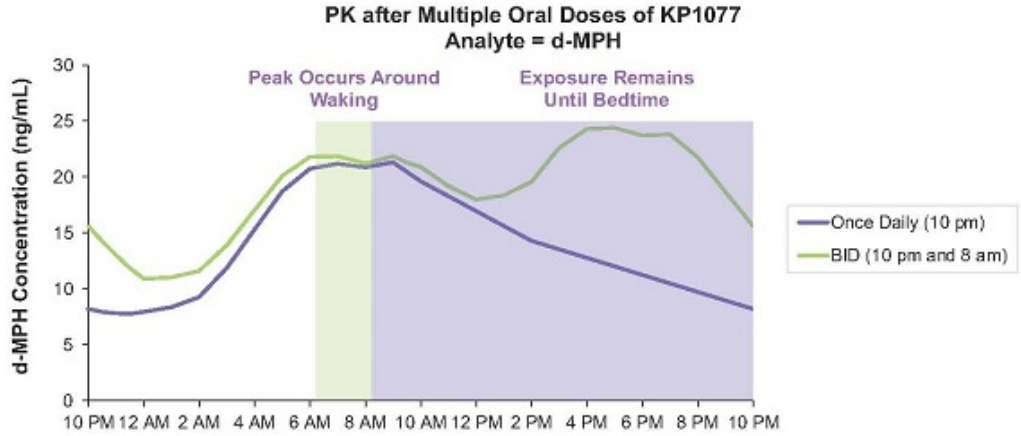

Phase 1 clinical trial results confirmed cardiovascular safety risk of KP1077 improved vs. immediate-release and long-acting formulations of Ritalin®, and KP1077 provided higher total exposure to d-MPH

Plasma concentrations were estimated based on data collected in study KP879.101

Predicted PK is shown for steady state of 240 mg KP1077 based on single oral dose

Phase 2 Clinical Trial of KP1077 in IH

ZEVRA THERAPEUTICS

Multi-center, dose-optimizing, double-blind, placebo-controlled, randomized-withdrawal study to evaluate safety of KP1077, as well as potential efficacy endpoints

PHASE 2 TRIAL (N=48)

Part 1:

- Five-week open-label titration phase
- Patients optimized to one of the four doses of KP1077 (80, 160, 240, or 320 mg/day)

Part 2:

- Two-week randomized, double-blind, withdrawal phase
- 2/3 receive KP1077; 1/3 placebo
- 50% receive single daily dose; 50% receive half daily dose upon awakening and at bedtime

INTERIM DATA:
To inform the design of the Phase 3 trial
Potential key differentiators:

1. Alignment of peak efficacy with patient need through dose optimized timing
2. Expanded exposure range through unique PK

PRIMARY ENDPOINT

- Safety and tolerability of KP1077

MAJOR SECONDARY ENDPOINT

- Change in Epworth Sleepiness Scale (ESS) total score

ADDITIONAL EXPLORATORY ENDPOINTS

- Patient Global Impression of Severity (PGI-S)
- Clinical Global Impression of Severity (CGI-S)
- Change in total score on the Idiopathic Hypersomnia Severity Scale (IHSS)
- Sleep Inertia at 1 hour after awakening
- New scale to assess the symptoms and severity of "Brain Fog"