Zevra Launches New Disease State Awareness Campaign, ‘Learn NPC, Read Between the Signs,’ to Drive Early Recognition and Treatment of Niemann-Pick Disease Type C

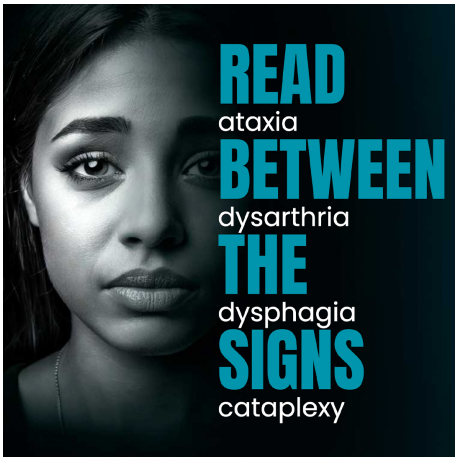
February 28, 2025

New website launched in honor of Rare Disease Day, Feb. 28, 2025

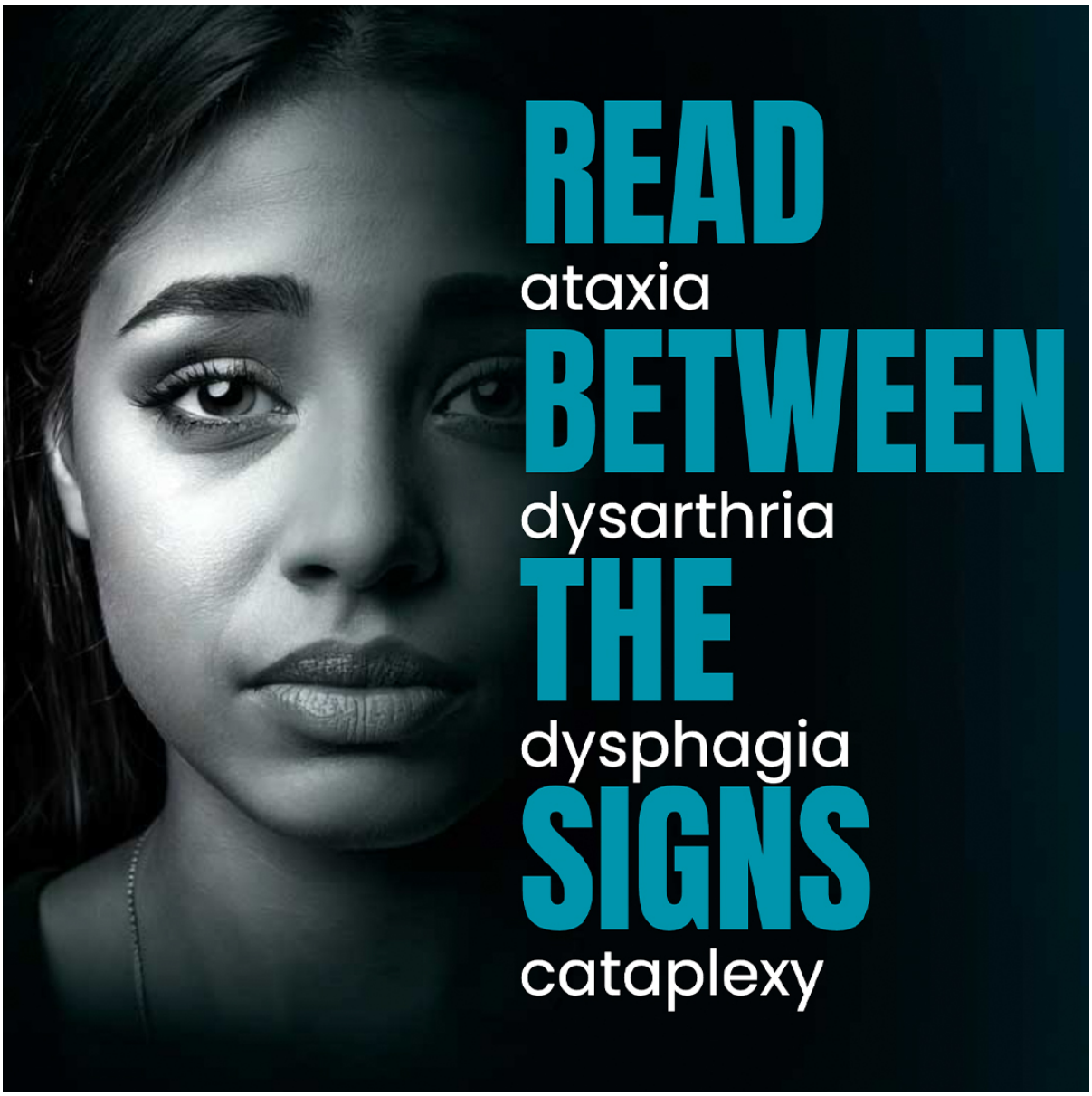
Awareness campaign includes educational resources and testing information for individuals with suspected NPC.

CELEBRATION, Fla., Feb. 28, 2025 (GLOBE NEWSWIRE) -- Zevra Therapeutics, Inc. (NasdaqGS: ZVRA) (Zevra, or the Company), a commercial-stage company focused on providing therapies to people living with rare disease, today announced, on Rare Disease Day, the launch of a new disease state awareness campaign, ’*Learn NPC, Read Between the Signs*,’ to highlight the need for early recognition and diagnosis due to the heterogeneity of symptoms related to Niemann-Pick disease type C (NPC). NPC is an ultra-rare, progressive, and neurodegenerative lysosomal storage disorder with varying age of onset and symptom presentation, often making the pathway to diagnosis difficult.

Learn NPC, Read Between the Signs



Learn NPC, Read Between the Signs



"We are proud to launch the *' Learn NPC, Read Between the Signs'* campaign to provide integral educational and testing resources to NPC treatment teams in the U.S.," said **Joshua Schafer, Zevra's Chief Commercial Officer and Executive Vice President of Business Development.** "Just over two years ago, the Company announced its mission to develop innovative therapies for people living with rare diseases that face the greatest unmet need. Today, we are honored to stand united with the NPC and broader rare disease community and reaffirm our commitment to raising awareness of remaining unmet need and championing the work ahead."

"During this year's Rare Disease Week, we are excited to see Zevra's educational campaign, *'Learn NPC, Read Between the Signs,'* focused on increasing the awareness of NPC among healthcare professionals and how to recognize its symptoms," **said Laurie Turner, Family Services Manager, National Niemann-Pick Disease Foundation (NNPDF).** "Efforts like these are essential to increase the suspicion of this difficult to diagnose rare disease and support patients seeking a diagnosis."

The awareness campaign aims to provide physicians and treatment teams with educational resources, including a summary of the disease and the challenges associated with diagnosis, an overview of symptoms often associated with NPC, and testing options for individuals with suspected NPC.

Learn more at www.LearnNPC.com

About MIPLYFFA™ (arimoclomol)

MIPLYFFA (arimoclomol) is Zevra's approved therapy for the treatment of Niemann-Pick disease type C (NPC). Approved by the U.S. Food and Drug Administration on Sep. 20, 2024, MIPLYFFA (arimoclomol) increases the activation of the transcription factors EB (TFEB) and E3 (TFE3) resulting in the upregulation of coordinated lysosomal expression and regulation (CLEAR) genes. MIPLYFFA has also been shown to reduce unesterified cholesterol in the lysosomes of human NPC fibroblasts. The clinical

significance of these findings is not fully understood. In the pivotal phase 3 trial, MIPLYFFA halted disease progression compared to placebo over the one-year duration of the trial when measured by the only validated disease progression measurement tool, the NPC Clinical Severity Scale. MIPLYFFA has also received Orphan Medicinal Product designation by the European Medicines Agency (EMA) for the treatment of NPC.

INDICATIONS AND USAGE

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.

IMPORTANT SAFETY INFORMATION

Hypersensitivity Reactions:

Hypersensitivity reactions such as urticaria and angioedema have been reported in patients treated with MIPLYFFA during Trial 1: two patients reported both urticaria and angioedema (6%) and one patient (3%) experienced urticaria alone within the first two months of treatment. Discontinue MIPLYFFA in patients who develop severe hypersensitivity reactions. If a mild or moderate hypersensitivity reaction occurs, stop MIPLYFFA and treat promptly. Monitor the patient until signs and symptoms resolve.

Embryofetal Toxicity:

MIPLYFFA may cause embryofetal harm when administered during pregnancy based on findings from animal reproduction studies. Advise pregnant females of the potential risk to the fetus and consider pregnancy planning and prevention for females of reproductive potential.

Increased Creatinine without Affecting Glomerular Function:
Across clinical trials of MIPLYFFA, mean increases in serum creatinine of 10% to 20% compared to baseline were reported. These increases occurred mostly in the first month of MIPLYFFA treatment and were not associated with changes in glomerular function.

During MIPLYFFA treatment, use alternative measures that are not based on creatinine to assess renal function. Increases in creatinine reversed upon MIPLYFFA discontinuation.

The most common adverse reactions in Trial 1 (≥15%) in MIPLYFFA-treated patients who also received miglustat were upper respiratory tract infection, diarrhea, and decreased weight.

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.

To report SUSPECTED ADVERSE REACTIONS, contact Zevra Therapeutics, Inc. at toll-free phone 1-844-600-2237 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

Drug Interaction(s):
Arimoclomol is an inhibitor of the organic cationic transporter 2 (OCT2) transporter and may increase the exposure of drugs that are OCT2 substrates. When MIPLYFFA is used concomitantly with OCT2 substrates, monitor for adverse reactions and reduce the dosage of the OCT2 substrate.

Use in Females and Males of Reproductive Potential:
Based on animal findings, MIPLYFFA may impair fertility and may increase post-implantation loss and reduce maternal, placental, and fetal weights.

Renal Impairment:
The recommended dosage of MIPLYFFA, in combination with miglustat, in patients with an eGFR ≥15 mL/minute to <50 mL/minute is lower than the recommended dosage (less frequent dosing) in patients with normal renal function.

MIPLYFFA capsules for oral use are available in the following strengths: 47 mg, 62 mg, 93 mg, and 124 mg.

About Niemann-Pick Disease Type C (NPC)

Niemann-Pick disease type C (NPC) is an ultra-rare, progressive, and 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 cell types, including neurons. The disease is caused by mutations in the *NPC1* or *NPC2* genes, which are responsible for making the *NPC1* and *NPC2* lysosomal proteins. Both children and adults can be affected by NPC with varying clinical presentations. Those living with NPC can lose independence due to physical and cognitive limitations, with key neurological impairments presenting in speech, cognition, swallowing, ambulation, and fine motor skills. Disease diagnosis can often take years, with disease progression being irreversible and often leading to early mortality.

About Zevra Therapeutics, Inc.

Zevra Therapeutics, Inc. is a commercial-stage company combining science, data, and patient needs to create transformational

therapies for rare diseases with limited or no treatment options. Our mission is to bring life-changing therapeutics to people living with rare diseases. With unique, data-driven development and commercialization strategies, the Company is overcoming complex drug development challenges to make new therapies available to the rare disease community.

Expanded access programs are made available by Zevra Therapeutics, Inc. and its affiliates and are subject to the Company's Expanded Access Program (EAP) policy, as published on its website. 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.

For more information, please visit www.zevra.com or follow us on X and LinkedIn.

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, including without limitation statements regarding the potential benefits of any of our products or product candidates for any specific disease or at any dosage; our strategic and product development objectives; prescription enrollments; our ability to support patients as they navigate the benefits verification process to obtain MIPLYFFA; and availability of and access to MIPLYFFA. Forward-looking statements are based on information currently available to Zevra and its current plans or expectations. They are subject to several known and unknown uncertainties, risks, and other important factors that may cause our 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, 2023, Zevra's quarterly report for the three and nine months ended September 30, 2024, and Zevra's other filings with the Securities and Exchange Commission. 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 cannot assure that such expectations will prove correct. These forward-looking statements should not be relied upon as representing our views as of any date after the date of this press release.

Zevra Contact

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

A photo accompanying this announcement is available at https://www.globenewswire.com/NewsRoom/AttachmentNg/93a58208-5f60-404b-99fd-9d5e27854cd1