Zevra Therapeutics to Present Data Demonstrating Relevance of Swallow Domain in Niemann-Pick Disease Type C Clinical Severity Scale at 53rd Child Neurology Society Annual Meeting

November 11, 2024

Data show that the NPCCSS swallow score reflects the patient's level of dysfunction

Study indicates that a change in score reflects actual improvement or worsening in a patient's swallowing function

CELEBRATION, Fla., Nov. 11, 2024 (GLOBE NEWSWIRE) -- Zevra Therapeutics, Inc. (NasdaqGS: ZVRA) (Zevra, or the Company), a commercial-stage company focused on addressing unmet needs for the treatment of rare diseases, today announced that Elizabeth Berry-Kravis, M.D., Ph.D., Professor of Pediatrics, Neurological Sciences, and Biochemistry at Rush University Medical Center in Chicago, is presenting data demonstrating the relevance of the swallow domain as part of the Niemann-Pick disease Type C Clinical Severity Scale (NPCCSS) at the 53rd Child Neurology Society Annual Meeting, which takes place Nov. 11-14 in San Diego.

The poster presentation includes a reassessment of individual swallow domain scoring categories, rater scoring instructions, and evaluation of the score linearity based on a qualitative study in which an independent research organization interviewed 12 NPC and swallow experts. Findings showed that the descriptions of the NPCCSS swallow response categories can be clearly and consistently interpreted and that a change in score reflects actual improvement or worsening in a patient's swallow function.

This study and outside expert feedback were the basis for Zevra's revised scoring algorithm of the swallow domain. This rescored swallow domain imparts improved linearity by re-ranking the scoring categories for dysphagia by frequency regardless of food texture. In addition, supplemental tube-feeding was placed in its own scoring category. The composite score of this revised swallow domain and the three NPCCSS domains for ambulation, fine motor skills, and speech, formed the primary 4-domain R4DNPCCSS endpoint used to evaluate the effectiveness of arimoclomol (MIPLYFFA™) in the Phase 2/3 study (NCT02612129). The treatment difference based on the R4DNPCCSS score change from baseline to 12 months or last visit while on treatment was -1.51 (*P*=0.0413) in favor of arimoclomol when compared to placebo.

Presentation Details

Poster Number:	279
Title:	Qualitative Assessment of the Validity and Standardization of the Swallow Domain in the 5-Domain Niemann-Pick Disease Type C (NPC) Clinical Severity Scale (5DNPCCSS)
Date/Time:	Tuesday, Nov. 12, 2024, 12:30 p.m. – 1:45 p.m. PT, and 5:30 p.m. – 7:00 p.m. PT
Presenter:	Elizabeth Berry-Kravis, MD, Ph.D., Professor of Pediatrics, Neurological Sciences, and Biochemistry at Rush University Medical Center in Chicago

Members of Zevra's team will be available at the meeting in San Diego; visit Zevra at its commercial booth (#515) and medical booth (#614).

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 rare disease company combining science, data, and patient needs to create

transformational therapies for 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.

About MIPLYFFA™ (arimoclomol)

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. MIPLYFFA was granted Breakthrough Therapy designation, Rare Pediatric Disease designation, Orphan Drug designation, and Fast Track designation by the FDA for the treatment of NPC. MIPLYFFA was further granted Orphan Medicinal Product designation by the European Medicines Agency (EMA) for the treatment of NPC.

For Full Prescribing and Safety Information for MIPLYFFA, please visit www.MIPLYFFA.com.

Cautionary Note Concerning Forward-Looking Statements

This news 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 promise and potential impact of our preclinical or clinical trial data; 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, including with respect to becoming a leading, commercially focused rare disease company; the presentation of data at conferences; and the timing of any of the foregoing. 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 on Form 10-Q for the three months ended June 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

Russo Partners Contact

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