



Zevra Announces FDA Advisory Committee Meeting to Review Arimoclomol for the Treatment of Niemann-Pick Disease Type C

July 9, 2024

Meeting with the recently formed GeMDAC set for August 2, 2024

CELEBRATION, Fla., July 09, 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 indicated that it will convene a meeting with the recently formed Genetic Metabolic Diseases Advisory Committee (GeMDAC) on August 2, 2024, to review the New Drug Application (NDA) for arimoclomol as an orally delivered, first-in-class treatment for Niemann-Pick disease type C (NPC).

According to the notice provided, the FDA intends to make the background materials available to the public no later than two business days before the meeting. The GeMDAC consists of experts in the fields of medical genetics, inborn errors of metabolism, epidemiology, and other related specialties. The NDA for arimoclomol has been assigned a Prescription Drug User Fee Act (PDUFA) action date of September 21, 2024.

"We look forward to presenting data from the arimoclomol NDA to the recently formed GeMDAC, which will also provide the opportunity for patients and other members of the NPC community to share their treatment journeys and highlight the desperate need for an approved therapy," said Neil F. McFarlane, President and Chief Executive Officer of Zevra.

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 tissues and organs, including the brain. 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 drug 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). The FDA has accepted the resubmission of the NDA for arimoclomol and has set a PDUFA date of September 21, 2024.

About Zevra Therapeutics

Zevra Therapeutics is a 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 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.

For more information, please visit www.zevra.com or follow us on [X](#) (formerly Twitter) 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 impact of meetings or communications with GeMDAC or the FDA, the content, information used for, timing or results of any NDA submissions or resubmissions for arimoclomol or any other drug product candidates for any specific disease indication or at any dosage, the potential launch or commercialization of any of drug product candidates or products, and our strategic and product development objectives, including with respect to becoming a leading, commercially focused rare disease company. 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 March 31, 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.

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