



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

January 8, 2024

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

CELEBRATION, Fla., Jan. 08, 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 additional 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.

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