Zevra Therapeutics Provides FDA Update on the PDUFA Action Date for Arimoclomol as a Treatment for Niemann-Pick Disease Type C

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The new Prescription Drug User Fee Act (PDUFA) action date set by the FDA is September 21, 2024

CELEBRATION, Fla., March 04, 2024 (GLOBE NEWSWIRE) -- **Zevra Therapeutics, Inc. (NasdaqGS: ZVRA)** (Zevra or the Company), a rare disease therapeutics company, today announced the U.S. Food and Drug Administration (FDA) has extended the review period for the New Drug Application (NDA) for arimoclomol, an investigational orally delivered, first-in-class treatment for Niemann-Pick disease type C (NPC). In addition, the FDA has set a new Prescription Drug User Fee Act ("PDUFA") action date of September 21, 2024, and has re-affirmed its intent to present the resubmission for discussion at an advisory committee meeting to be scheduled.

As previously reported, Zevra received acceptance of the NDA resubmission for arimoclomol in early January 2024, with an original PDUFA action date of June 21, 2024. The arimoclomol NDA resubmission included evidence supporting trial metrics, FDA-preferred analyses, and data from a multitude of additional studies that provide supportive evidence of arimoclomol's impact in clinical and nonclinical settings.

As part of the ongoing review, Zevra received notification from the FDA that it required more time to review the additional analyses provided by the Company in responses to recent information requests generated from the FDA's review. The FDA has determined that the additional information constitutes a Major Amendment to the NDA, thereby resulting in an extension of the PDUFA action date.

"While the PDUFA action date extension represents a delay, we remain confident in the potential for arimoclomol to help people living with NPC, and we will continue to work closely with the FDA as they complete their review," said Neil F. McFarlane, President and Chief Executive Officer of Zevra. "We applaud the NPC Patient Advocacy Community who recently submitted an informal petition to the FDA in support of arimoclomol and reinforcing the urgent need for an FDA-approved treatment to improve the course of this relentless and fatal disease. As the review continues, Zevra will maintain the early access program for arimoclomol and work tirelessly to bring this potential therapy to patients as soon as possible."

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 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). The FDA has accepted the resubmission of the NDA for arimoclomol and has set a user fee goal date (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 promise and potential impact of our preclinical or clinical trial data, the initiation, timing and results of any clinical trials or readouts, the content, information used for, 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 launch or commercialization of any of 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, 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 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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