FDA Advisory Committee Votes Favorably that the Data Support Arimoclomol as Effective Treatment for Patients with Niemann-Pick Disease Type C

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PDUFA Action Date for the Arimoclomol NDA is September 21, 2024

CELEBRATION, Fla., Aug. 02, 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) Genetic Metabolic Diseases Advisory Committee (GeMDAC) voted favorably (11 yes, 5 no) that the data support that arimoclomol is effective in the treatment of patients with Niemann-Pick disease type C (NPC).

"We are extremely pleased with the committee's recognition of the benefits of arimoclomol for people living with NPC," said **Neil F. McFarlane, President and Chief Executive Officer of Zevra**. "Based on the totality of the clinical data, including data from the pivotal trial, the long-term data from the arimoclomol open label extension study, and data from our expanded access programs (EAP: NCT04316637), we remain confident in the clinical benefit offered by arimoclomol as a treatment for NPC, and are optimistic about its continued path to approval."

The GeMDAC, which consists of experts in the fields of medical genetics, inborn errors of metabolism, epidemiology and other related specialties, discussed the benefits and risks of arimoclomol, including the data recently presented at the 45th Annual Meeting of the Society for Inherited Metabolic Disorders (SIMD), and reviewed comments received from independent experts, NPC patients, and patient advocacy group representatives. The committee's recommendation will be considered by the FDA as it completes its independent review of the arimoclomol NDA; however, the feedback from the GeMDAC is not binding upon the Agency. The arimoclomol NDA has been assigned a Prescription Drug User Fee Act (PDUFA) action date of September 21, 2024.

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, 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 user fee goal action 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.

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 potential benefits of any of our products or product candidates for any specific disease or at any dosage; the impact of meetings or communications with the FDA or any advisory committee; decisions by the FDA or any other entity for arimoclomol or any other product candidates; our strategic and product development objectives, including with respect to becoming a leading, commercially focused rare disease company; 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 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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