Zevra Therapeutics Submits Marketing Authorization Application to European Medicines Agency to Review Arimoclomol for the Treatment of Niemann-Pick Disease Type C

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CELEBRATION, Fla., July 28, 2025 (GLOBE NEWSWIRE) -- Zevra Therapeutics, Inc. (NasdaqGS: ZVRA) (Zevra, or the Company), a commercial-stage company focused on providing therapies for people living with rare diseases, announced the company submitted a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) for the evaluation of arimoclomol for the treatment of Niemann-Pick Disease Type C (NPC). NPC is an ultra-rare, neurological disease caused by genetic mutations that result in lipid accumulation in cells, leading to visceral, neurological, and psychiatric symptoms. Arimoclomol is the only treatment shown to directly target the underlying pathology of NPC by increasing gene expression for improved lipid clearance. Arimoclomol for the treatment of NPC has been designated as an Orphan Medicinal Product by the EMA. Arimoclomol is marketed in the U.S. under the brand name MIPLYFFA®.

“This EMA submission marks a significant milestone for the Company as we continue to expand access to MIPLYFFA® in NPC patients across the globe,” said Neil F. McFarlane, Zevra’s President and Chief Executive Officer. “Concurrently, we continue to advance our global Expanded Access Program, with 89 patients enrolled in Europe at the end of Q2, reinforcing MIPLYFFA’s potential to serve as a foundational treatment option across European markets. We extend our deepest gratitude to everyone involved – especially those patients and clinicians who participated in our clinical development programs and look forward to potential European approval of arimoclomol to expand on our approval in the U.S. last year.”

The EMA will review the application under the centralized marketing authorization procedure. If a marketing authorization is granted by the European Commission, the authorization is valid in all EU Member States as well as in the European Economic Area (EEA) countries Iceland, Liechtenstein and Norway.

Adrian Quartel, M.D., Zevra’s Chief Medical Officer added, “The extensive data generated for arimoclomol has shown long-term, meaningful clinical outcomes with 5 to 7 years of patient experience across more than 270 NPC patients through a Phase 2/3 clinical trial, Open-Label Extension (OLE) study, Expanded Access Programs (EAP), and a pediatric sub-study, which is the most expansive clinical development program in NPC to date. We are confident in the strength of our MAA data package and look forward to our interactions with the EMA.”

Zevra currently offers an EAP for NPC patients in certain European countries, and more information can be found at: https://zevra.com/patients-and-providers/expanded-access-policy.

About Arimoclomol

Arimoclomol is Zevra’s therapy for the treatment of Niemann-Pick disease type C (NPC), which was approved by the U.S. Food and Drug Administration on Sep. 20, 2024. 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. Arimoclomol 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. In the pivotal Phase 3 trial, arimoclomol halted disease progression compared to placebo over the twelve month duration of the trial when measured by the only validated disease progression measurement tool, the NPC Clinical Severity Scale. Arimoclomol has also received Orphan Medicinal Product designation by the European Medicines Agency (EMA) for the treatment of NPC.

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.

For more information, please visit www.zevra.com or follow us on X 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; or the potential benefits of any of our products for any specific disease. 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, 2024, filed on March 12, 2025, and Zevra’s Quarterly Report on Form 10-Q for the quarter ended March 31, 2025, filed on May 13, 2025, and Zevra’s other filings with the SEC. 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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