Zevra Therapeutics Provides Update on Regulatory Submission for Arimoclomol for the Treatment of Niemann-Pick Disease Type C (NPC) in the European Union

July 24, 2026

Company plans to request re-examination following negative CHMP opinion for arimoclomol MAA

Zevra continues to support access to arimoclomol for eligible patients through its global Expanded Access Program

BOSTON, July 24, 2026 (GLOBE NEWSWIRE) -- Zevra Therapeutics, Inc. (NasdaqGS: ZVRA) (Zevra, or the Company), a commercial-stage company focused on bringing life-changing therapeutics to people living with rare diseases, today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a negative opinion regarding the Marketing Authorization Application (MAA) for arimoclomol for the treatment of Niemann-Pick disease type C (NPC), a rare, progressive and life-threatening neurodegenerative disorder.

Under European regulatory procedures, the Company plans to request a re-examination of the CHMP opinion and submit a detailed explanation outlining the basis for the request.

“While the review did not result in the outcome we had hoped for, we are committed to investigating appropriate pathways to bring arimoclomol to patients in Europe,” said Neil F. McFarlane, President and Chief Executive Officer of Zevra Therapeutics. “We appreciate the important role the CHMP plays in assessing therapies across Europe, and we remain confident in the totality of evidence supporting the potential of arimoclomol to make a meaningful difference in the lives of patients living with NPC.”

Zevra remains steadfast in its commitment to the NPC community and believes that patients facing this devastating disease deserve access to potential treatment options. Through its global Expanded Access Program (EAP), the Company continues to support eligible patients to advance access to arimoclomol and generate additional real-world evidence of its clinical impact. The recently updated international Consensus Clinical Management Guidelines for Niemann-Pick disease type C further underscore the role of arimoclomol as an important treatment option for individuals living with NPC.[1]

“In my experience, arimoclomol has demonstrated meaningful treatment effects in NPC, with stabilization and slowed progression in a condition with significant unmet need,” said Prof. Thorsten Marquardt, University of Münster. “Real-world clinical experience in NPC can provide additional insights into how patients are managed in routine care settings, contributing to the broader understanding of disease management in ultra-rare, progressive diseases.”

About Zevra Therapeutics, Inc.

Zevra Therapeutics, Inc. is a commercial-stage company with a late-stage pipeline committed to redefining what is possible in bringing life-changing therapies to people living with rare diseases. The Company is focused on broadening access through geographic expansion opportunities, progressing its pipeline toward key milestones, and delivering meaningful therapeutics. The commercialization of its lead product, marketed in the U.S. for Niemann-Pick disease type C (NPC), a rare, progressive neurodegenerative disease, provides a strong corporate foundation and validates its ability to advance therapies from development to market. Zevra's vision is realized through disciplined execution of its strategic plan and core values — patient centricity, integrity, accountability, innovation, and courage — which guide its efforts to deliver long-term value.

For more information, please visit www.zevra.com or follow us on X and LinkedIn.

Caution 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 intent to request a re-examination of the CHMP opinion and the timing related thereto, and the intent to continue to support access to arimoclomol through its global EAP. 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, 2025, filed on March 9, 2026, as well as 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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1. T. Hiwot, F. D. Porter, T. Bremova-Ertl, et al., "*2025 Consensus Clinical Management Guidelines for Niemann-Pick Disease Type C*," *Journal of Inherited Metabolic Disease* 49, no. 3 (2026): e70185, https://doi.org/10.1002/jimd.70185.