Zevra Therapeutics Announces Final CHMP Opinion on Arimoclomol for the Treatment of Niemann-Pick Disease Type C (NPC) in the European Union

November 13, 2026

European Commission to make final decision on arimoclomol MAA following negative CHMP opinion

BOSTON, Nov. 13, 2026 (GLOBE NEWSWIRE) -- Zevra Therapeutics, Inc. (NasdaqGS: ZVRA) (Zevra, or the Company), a commercial-stage company committed to redefining what's possible in 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 on 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 that affects approximately 1,000 people in Europe. The European Commission is expected to issue its final decision on the Marketing Authorization Application within 67 days.

Zevra will continue to support eligible patients currently receiving arimoclomol through its global Expanded Access Program (EAP), subject to applicable regulatory requirements and in coordination with national authorities. The Company remains focused on helping ensure continuity of treatment for patients currently benefiting from arimoclomol through the program.

“We are deeply disappointed by the CHMP's opinion, particularly given the significant unmet medical need faced by individuals and families living with NPC,” said Neil F. McFarlane, President and Chief Executive Officer of Zevra Therapeutics. “We remain grateful to the patients, caregivers, investigators, advocacy organizations and healthcare providers who have been part of the arimoclomol journey. While this outcome is not what the NPC community hoped for, our priority remains supporting patients and families.”

Zevra believes the robust data package, which included clinical and real-world evidence for arimoclomol, provides important support for its therapeutic benefit in NPC. The application was supported by data from the pivotal NPC 002 Phase 2/3 trial, which demonstrated that arimoclomol in combination with miglustat reduced disease progression compared with placebo over 12 months. Long-term open-label extension data showed that the treatment effect was sustained over multiple years. The Company will continue to advance scientific understanding of NPC and collaborate with stakeholders across the global NPC community.

“The NPC community is profoundly disappointed by this decision,” said Sandra Cowie, President of the International Niemann-Pick Disease Alliance (INPDA). “Families affected by NPC face a relentlessly progressive disease that impacts every aspect of daily life. People living with NPC and their caregivers across Europe have been waiting for access to additional treatment options, making this outcome particularly difficult for those who continue to face the daily realities of this devastating disease. INPDA remains committed to advocating for people living with NPC and ensuring that the voices of those living with NPC and their families are heard.”

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 ensuring continuity of treatment for patients currently receiving 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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