



Zevra Therapeutics to Present Multiple Analyses on MIPLYFFA® at the 22nd Annual WORLDSymposium™

January 26, 2026

Four abstracts highlighting clinical data and real-world experience with MIPLYFFA accepted for presentation, including an oral presentation by Caroline Hastings, MD

Company to host satellite symposium on diagnostic and therapeutic advances in Niemann-Pick disease type C (NPC)

CELEBRATION, Fla., Jan. 26, 2026 (GLOBE NEWSWIRE) -- Zevra Therapeutics, Inc. (NasdaqGS: ZVRA) (Zevra, or the Company), a commercial-stage company focused on providing therapies for people living with rare disease, today announced that four abstracts discussing data and clinical experience associated with MIPLYFFA® (arimoclomol) have been accepted for presentation at the 22nd Annual WORLDSymposium™. Dr. Caroline Hastings will present four-year real-world safety and efficacy data of MIPLYFFA in patients with Niemann-Pick Disease Type C (NPC) during a podium presentation.

The poster presentations include outcomes from the U.S. Early Access Program (EAP), multi-year subgroup analyses, and efficacy results from a randomized, placebo-controlled clinical trial, further expanding the body of evidence characterizing MIPLYFFA in pediatric and adult NPC patients. In addition, Zevra will host a non-continuing education (Non-CE) sponsored satellite symposium focused on diagnostic and therapeutic challenges in NPC.

Podium Presentation Details

Poster Number:	41
Title:	<i>Real-world Safety and Effectiveness of Arimoclomol in Patients with NPC: Outcomes from the U.S. Early Access Program (EAP) Over a 4-Year Period</i>
Date/Time:	Thursday, February 5, 2026, at 2:30 p.m. PT
Presenter:	Caroline Hastings, MD, UCSF Benioff Children's Hospital

Satellite Symposium Details

Title:	<i>Charting a Path in Niemann-Pick Disease Type C: Diagnostic Challenges, Therapeutic Innovations, and Real-World Patient Cases</i>
Date/Time:	Thursday, February 5, 2026, at 12:15 p.m. PT
Presenter:	Caroline Hastings, MD, UCSF Benioff Children's Hospital

Poster Presentation Details

Poster Number:	193
Title:	<i>Multi-year Subgroup Analyses of Niemann-Pick Disease Type C Participants Treated with Arimoclomol in the U.S. Early Access Program</i>
Date/Time:	Thursday, February 5, 2026, at 3:30 p.m. PT
Presenter:	Kristina Julich, MD, Dell Children's Medical Center

Poster Number:	250
Title:	<i>Efficacy of Arimoclomol Combined with Miglustat at Months 3, 6, 9, and 12 of the Double-blind, Randomized, Placebo-controlled NPC002 Trial</i>
Date/Time:	Thursday, February 5, 2026, at 3:30 p.m. PT
Presenter:	Eugen Mengel, MD, SphinCS GmbH, Institute of Clinical Science for LSD, Hochheim, Germany

Poster Number:	273
Title:	<i>Long-term Safety and Effectiveness of Arimoclomol in Adult and Pediatric Niemann-Pick disease type C Patients in the US Early Access Program (EAP)</i>
Date/Time:	Thursday, February 5, 2026, at 3:30 p.m. PT
Presenter:	Damara Ortiz, MD, UPMC Children's Hospital of Pittsburgh

E-Posters will be available to all registered attendees via the *WORLDSymposium* mobile app beginning at 5:00 p.m. PT on Tuesday, February 3, 2026, and will remain accessible throughout the live meeting. Registered attendees may continue to access e-Posters on demand from February 11 through March 13, 2026.

Members of the Zevra team will be available during the meeting, taking place February 2-6, 2026, in San Diego, CA. Attendees are invited to visit Zevra at booths #213 and #507 for more information.

About Zevra Therapeutics, Inc.

Zevra Therapeutics, Inc. is a purpose-driven, commercial-stage company focused on bringing life-changing therapeutics to people living with rare diseases. The company's commercialization of its lead product, marketed in the U.S. for Niemann-Pick disease type C (NPC), a rare, progressive neurodegenerative disorder, provides a strong corporate foundation and validates its ability to advance therapies. In addition, the company is broadening access through geographic expansion opportunities and has a pipeline of rare disease programs. Zevra is a patient-centric organization guided by our values of accountability, integrity, innovation and courage, with the goal of creating long-term value for patients, partners, and shareholders.

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 Company's expanded access program. 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, Quarterly Report on Form 10-Q for the three and nine months ended September 30, 2025, filed on November 5, 2025, 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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