



August 2, 2024

Dear NPC Community,

Today, the Genetic Metabolic Diseases Advisory Committee (GeMDAC) met to review Zevra Therapeutics' arimoclomol as an orally delivered treatment for Niemann-Pick disease type C (NPC). The committee, an independent panel appointed by the U.S. Food and Drug Administration (FDA), discussed the benefits and risks of arimoclomol and reviewed comments received from independent experts, NPC patients and families, and patient advocacy group representatives. View press release [here](#).

We are pleased to share that the 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). The GeMDAC's recommendation will serve as a key consideration for the FDA as it continues to review the NDA for arimoclomol. Though the GeMDAC's perspective is important, its decision is not binding, and the FDA will consider all data and information to inform their ultimate decision about arimoclomol's approval.

Zevra will continue to work closely with the FDA and respond to any outstanding questions they may have in the coming weeks. The assigned Prescription Drug User Fee Act (PDUFA) action date for arimoclomol is September 21, 2024.

We are optimistic about arimoclomol's continued path to approval and will continue to be transparent and keep the community informed of important developments and remain committed to the Arimoclomol Expanded Access Program. The support of countless members of the NPC community throughout our journey to bring a much-needed treatment option to patients with NPC is truly appreciated. We would like to express our sincere appreciation to those who took time to share their experience and stories in the docket and during the open public hearing. Thank you for your continued support of those living daily with NPC and their loved ones.

We value your continued partnership as we work to improve the lives of NPC patients. If you have any questions, please feel free to reach out to us at [patientadvocacy@zevra.com](mailto:patientadvocacy@zevra.com).

Kind regards,

The Zevra Team