



July 09, 2024

Dear Joslyn and the NNPDF Community,

Today, the U.S. Food and Drug Administration (FDA) announced that the recently formed Genetic Metabolic Diseases Advisory Committee (GeMDAC) will meet on August 2, 2024, to review Zevra Therapeutics' New Drug Application (NDA) for arimoclomol as an orally delivered, first-in-class treatment for Niemann-Pick disease type C (NPC). The press release can be found [here](#). The meeting is open to the public via livestream on YouTube. This meeting is an important and welcome step in our journey to bring this much-needed treatment option to those affected by NPC.

During the meeting, the GeMDAC, which is composed of experts in genetics, epidemiology, biostatistics, medical care, consumer issues, etc., will discuss the data relating to the benefits and risks of arimoclomol and provide recommendations to the FDA. The agency will make an approvability decision later, likely by arimoclomol's Prescription Drug User Fee Act (PDUFA) target action date of September 21, 2024. During the meeting, the GeMDAC will gather perspectives from Zevra, FDA review staff, panel members, and other subject matter experts, including NPC treating clinicians, patients, and families, and discuss the benefits and risks of arimoclomol. The FDA will take this advice into consideration when making its decision about arimoclomol's approval.

There are several voluntary (non-mandatory) avenues for the NPC community to participate in the GeMDAC meeting. The committee accepts letters describing experiences with arimoclomol from groups and individuals. The letters can be submitted to an electronic docket by July 25th. The link to the docket will be available in approximately 2 days. The meeting also includes an open public hearing session during which registered individuals can share their perspectives.

We recognize and appreciate that you share our commitment to addressing the unmet treatment need for individuals living with NPC. Thank you for your engagement, partnership, and most importantly, sharing your voice to influence positive change. If you have any questions, please feel free to reach out to us at patientadvocacy@zevra.com, to NNPDF at nnpdf@nnpdf.org or to the NPC Family Foundations.

Kind regards,

The Zevra Team