January 8, 2024

Dear Joslyn, Becky, and the NPC Community:

As a follow up to the previous communication on December 27th, we are excited to share another milestone in our collective efforts to bring a possible new treatment option to the NPC community. Zevra Therapeutics, Inc., announced today the U.S. Food and Drug Administration's (FDA) acceptance of the resubmission of the New Drug Application (NDA) for arimoclomol for treatment of Niemann-Pick disease type C. The FDA has assigned a response date (formally known as a PDUFA date), of June 21, 2024, and currently intends to present the resubmission for discussion in an advisory committee. For more information on the purpose of an advisory committee click here.

Thank you again to the National Niemann-Pick Disease Foundation and the entire NPC community for your partnership over the years. Your continued advocacy will become increasingly important as the review progresses. We will be transparent in our communications and continue to look to the NPC community for guidance and for strong advocacy to ensure maximum regulatory flexibility is applied.

Additionally, Zevra remains steadfast in our support of the Arimoclomol Expanded Access Program (EAP) both in the US and Europe. The EAP represents our unwavering commitment to those affected by NPC and their families.

In the spirit of transparency, commitment, and collaboration, we anticipate working with the NNPDF on future communications and connect points with the NPC Community. More to come!

Kind Regards,

Tara Greene
Sr. Director, US Medical Affairs and Patient Advocacy
Zevra Therapeutics