March 4, 2024

Dear NNPDF and the NPC Community:

As a follow up to the previous communication on January 8th, we wanted to inform you that the U.S. Food and Drug Administration (FDA) extended by three months the review period for the New Drug Application (NDA) for arimoclomol for the treatment of Niemann-Pick disease type C. The FDA has set a new Prescription Drug User Fee Act (PDUFA) action date of September 21, 2024. The FDA still intends to present the resubmission for discussion in an advisory committee, consistent with the initial review process.

Zevra submitted a robust NDA package to the FDA in December 2023, meaningfully connecting the preclinical and clinical data on arimoclomol's safety and efficacy, as well as real-world Expanded Access Program outcomes with this investigational therapeutic product. As part of the ongoing review, Zevra submitted responses to information requests by the FDA. In this case, the FDA considered the additional information it received to be a "Major Amendment" to the application. This allows the agency more time to review the information. Though we are disheartened by the delay in providing this promising therapy to the NPC community, we are simultaneously encouraged that this development bodes well for the focused assessment of arimoclomol as a first-in-class treatment for this debilitating disease.

We at Zevra want to express our continued support of the arimoclomol Expanded Access Program, representing our enduring commitment to individuals affected by NPC and their families. We continue to prepare for arimoclomol's launch, pending approval in September, and continue our efforts to evaluate and identify the optimal pathway for arimoclomol regulatory approval in the E.U.

We express our gratitude to the National Niemann-Pick Disease Foundation and the entire NPC community for your enduring partnership. We're especially grateful for the compelling Community Response and Support Statement for the FDA review of arimoclomol. Your ongoing advocacy continues to inspire us and will play a crucial role as the review advances.

We will keep you informed as we continue to work toward making this groundbreaking therapy accessible to patients as soon as possible. Thank you for your unending support.

Sincerely,

The Zevra Team
patientadvocacy@zevra.com

*Arimoclomol is an investigational product for which safety and efficacy have not yet been established.