December 27, 2023

Dear Joslyn, NNPDF and the NPC community,

Today marks a significant milestone in our collective efforts to bring a possible new treatment option to the Niemann-Pick disease type C (NPC) community. Zevra Therapeutics, Inc., has resubmitted the New Drug Application (NDA) for arimoclomol to the U.S. Food and Drug Administration (FDA). The resubmission moves this potential therapy one step closer to addressing the unmet needs of individuals affected by NPC, their loved ones, and caregivers.

Thank you to the National Niemann-Pick disease Foundation and the entire NPC community for your partnership over the years, particularly since the complete response letter (CRL) in June 2021. Your consistent support and collective insights have informed our approach to addressing the questions the FDA raised in the CRL and assembling the most robust case we can make in favor of the benefit-risk profile of arimoclomol in NPC. This includes addressing the FDA's specific questions on the validity and reliability of the 5-Domain NPC Clinical Severity Scale and providing additional supportive evidence for the benefits of arimoclomol in NPC. This resubmission is a significant milestone in the development process of arimoclomol, and represents our commitment to regulatory rigor and transparency in all we do.

Now that the NDA has been resubmitted, the FDA will reengage in a thorough review process. Your efforts in capturing the patient voice through development of the international petition will play a fundamental role in ensuring that the agency understands the unmet need and patient experience going into this review process. Thank you for your leadership and for continuing to share your voice.

We will hear in 30 days if the NDA is accepted and anticipate a six-month review period thereafter. We are prepared to assist the FDA with any further information they may need for their analysis. While this submission signifies important progress in our shared journey, it is not the final destination. Along with our continued support of the Arimoclomol Expanded Access Program, it represents our unwavering commitment to the NPC community.

We will update you as we move through this review period toward our ultimate goal of delivering new treatment options to patients in need. This important milestone would not have been possible without your support, partnership, and advocacy. Thank you.

Sincerely,

The Zevra Team
patientadvocacy@zevra.com

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