



November 20, 2023

Dear INPDA community and Sandy,

With today's announcement of the completion of Zevra's acquisition of Acer Therapeutics, we want to take this opportunity to reassure you of our continued commitment to the Niemann-Pick disease type C (NPC) community and the INPDA. At Zevra, our mission is to give every promising rare disease therapy candidate a fighting chance to reach patients and improve their quality of life, and we value your partnership in helping to make this vision a reality.

We remain committed to the Arimoclomol Expanded Access Program and the continued development and potential approval of arimoclomol* as a much-needed therapy for the treatment of NPC. As you know, our goal is to resubmit the arimoclomol new drug application (NDA) to the U.S. Food and Drug Administration (FDA) by the end of this year, followed by identifying a regulatory pathway to approval for arimoclomol with the European Medicines Agency (EMA). In addition, we look forward to attending both the International Niemann-Pick Disease Alliance (INPDA) and Niemann-Pick UK (NPUK) meetings this month.

As a newly combined company, we have expanded our already strong foundation and added capabilities that will help advance our programs for rare diseases, including NPC, as well as rare sleep disorders, urea cycle disorders (UCDs) and Vascular Ehlers-Danlos syndrome (VEDS). A very important aspect of this acquisition is the addition of OLPRUVA® (sodium phenylbutyrate) for oral suspension to Zevra's portfolio. OLPRUVA was approved by the FDA in December 2022 for patients with UCDs. While very different diseases, UCDs and NPC are both often diagnosed by clinical geneticists and metabolic specialists, many of whom work within a concentrated number of centers of excellence. This will help us efficiently reach and support the physicians who are diagnosing and treating patients with both rare genetic conditions.

We are grateful for the work we've done together and are proud to be your partner as we work toward our shared goal of bringing a much-needed potential therapy to people living with NPC. You can learn more about our advocacy programs here on the [Zevra](#) and [Acer](#) websites.

Sincerely,
The Zevra Team

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