



August 31, 2023

Dear NPC Community,

Today, we announced that Zevra Therapeutics intends to acquire Acer Therapeutics. Both of our companies are focused on the acquisition, development and commercialization of therapies for rare diseases with significant unmet medical needs. Together, we are taking an important step forward in achieving our shared missions and strengthening our commitment to the communities we serve to deliver therapies to people who have limited or no treatment options.

Importantly, Acer recently received FDA approval for OLPRUVA™ (sodium phenylbutyrate) for the treatment of urea cycle disorders (UCDs). The commercialization of OLPRUVA is expected to be highly complementary to our plans for arimoclomol. Both conditions are often diagnosed at an early age and treated by metabolic specialists, geneticists, as well as other specialists.

In addition to OLPRUVA, this acquisition would augment our strategic capabilities and further enable our ability to advance our existing programs for rare diseases, including our development of investigational drug candidate arimoclomol for Nieman-Pick disease Type C (NPC) and investigational drug candidate KP1077 for rare sleep disorders. Both of these development programs will continue to have top priority as we move forward.

We remain steadfast in our key priorities to the NPC community: (i) continuing and full support of the compassionate use of arimoclomol for the treatment of NPC via our Early Access Programs in the US and several EU countries, (ii) resubmission of the arimoclomol NDA to the FDA in the fourth quarter this year, and (iii) evaluation and identification of a regulatory pathway to approval for arimoclomol with the European Medicines Agency (EMA).

We are encouraged by the strength of Acer's portfolio of ultra-rare programs and our shared goal of bringing life-changing therapeutics to the communities we serve. We look forward to our newfound collaboration and we commit to providing you with appropriate updates as we have more information available.

For inquiries, please contact patientadvocacy@zevra.com

Warm regards,

The Zevra Team

