

FOOD AND DRUG ADMINISTRATION (FDA)
Center for Drug Evaluation and Research (CDER)

Genetic Metabolic Diseases Advisory Committee (GeMDAC) Meeting
August 2, 2024

QUESTIONS

1. **DISCUSSION:** Discuss your assessment of the efficacy results of trial CT-ORZY-NPC-002 (NPC-002). In your discussion, please comment on:
 - a. The 5-domain Niemann-Pick disease type C Clinical Severity Scale (5DNPCCSS) and the rescored 4-domain Niemann-Pick disease type C Clinical Severity Scale (R4DNPCCSS).
 - b. Your assessment of whether the trial results demonstrate a treatment effect of arimoclomol on the treatment of Niemann-Pick disease type C (NPC).

2. **DISCUSSION:** Discuss your assessment of other data (specifically the additional clinical and nonclinical data) with respect to support for the effectiveness of arimoclomol.

3. **VOTE:** Do the results of trial NPC-002 in concert with the other data (clinical and nonclinical in particular) support a conclusion that arimoclomol is effective in the treatment of patients with NPC? Provide a rationale for your vote.
 - a. If you voted no, provide recommendations for additional data that may support a conclusion that arimoclomol is effective.