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

Peripheral and Central Nervous System Drugs Advisory Committee (PCNS) Meeting
September 7, 2022

DRAFT QUESTIONS

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1. **DISCUSSION:** Discuss the strength of the currently available data regarding the effectiveness of sodium phenylbutyrate/taurursodiol (AMX0035), to include the new information submitted and the information presented at the March 30, 2022, PCNS meeting. The discussion may include considerations regarding the unmet need in amyotrophic lateral sclerosis (ALS), the status of the ongoing Phase 3 trial, and the seriousness of ALS.

2. **VOTE:** Considering the new information submitted and the information presented at the March 30, 2022, PCNS meeting, is the available evidence of effectiveness sufficient to support approval of sodium phenylbutyrate/taurursodiol (AMX0035) for the treatment of patients with ALS? In addition to the prior and new evidence presented, you may take into account in your vote the unmet need in ALS, the status of the ongoing Phase 3 trial, and the seriousness of ALS.