

## **9/27 CTGTAC**

# **Questions for the Committee**

**Please note that the indication within the questions is different from the indication within the FDA Briefing Document. The applicant modified their proposed indication on September 22, 2023, from “treatment of amyotrophic lateral sclerosis (ALS)” to “treatment of mild to moderate amyotrophic lateral sclerosis (ALS).”**

1. Please discuss the data presented in support of effectiveness for treatment of mild to moderate amyotrophic lateral sclerosis (ALS), including consideration of the mechanisms of action proposed by the sponsor, biomarker data including neurofilament data, and the clinical data.
  
2. Voting question: Do the data presented demonstrate substantial evidence of effectiveness for treatment of mild to moderate ALS?
  - a) Yes
  - b) No
  - c) Abstain
  
3. If the answer to Question 2 is no, please discuss potential designs for a trial to demonstrate substantial evidence of effectiveness for MSC-NTF for the treatment of mild to moderate ALS.