

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

Peripheral and Central Nervous System Drugs Advisory Committee (PCNS) Meeting

Tommy Douglas Conference Center
10000 New Hampshire Avenue, Silver Spring, Maryland
September 28, 2017

DRAFT QUESTIONS

1. **VOTE:** The best interpretation of the information presented today regarding the use of ataluren for the treatment of dystrophinopathies resulting from nonsense mutations in the dystrophin gene is that:
 - a. The data suggest that ataluren is not effective
 - b. Although it is possible that ataluren may be effective, the data are inconclusive, and more work would be needed to establish whether ataluren is effective
 - c. The data are sufficient to conclude that ataluren is effective