



Automated speech measures are optimal for clinical trial efficiency.

©2026 Mayo Foundation for Medical Education and Research

Optimizing Outcome Measure Selection for Clinical Trials in PAOS

Rene L. Utianski¹, Joseph R. Duffy¹, Hugo Botha¹, Heather M. Clark¹,
Jennifer L. Whitwell², Keith A. Josephs¹, Gabriela Meade¹

Departments of Neurology¹ and Radiology², Mayo Clinic, MN, USA

BACKGROUND

- Progressive apraxia of speech (PAOS) is a likely target for tau-targeted therapies; however, clinical trials in rare neurodegenerative diseases are often limited by the large sample sizes required to detect treatment effects using traditional perceptual scales.
- Whitwell, et al., (2016) established neuroimaging as a sensitive marker for PAOS trials, with rates of atrophy of the precentral gyrus and supplementary motor area each requiring less than 50 subjects per arm to detect a 25% effect.
- Here, we investigated whether and which perceptual-acoustic measures could provide similar or superior efficiency as primary endpoints, anchored to clinically meaningful change (minimally clinical important difference, or MCID).

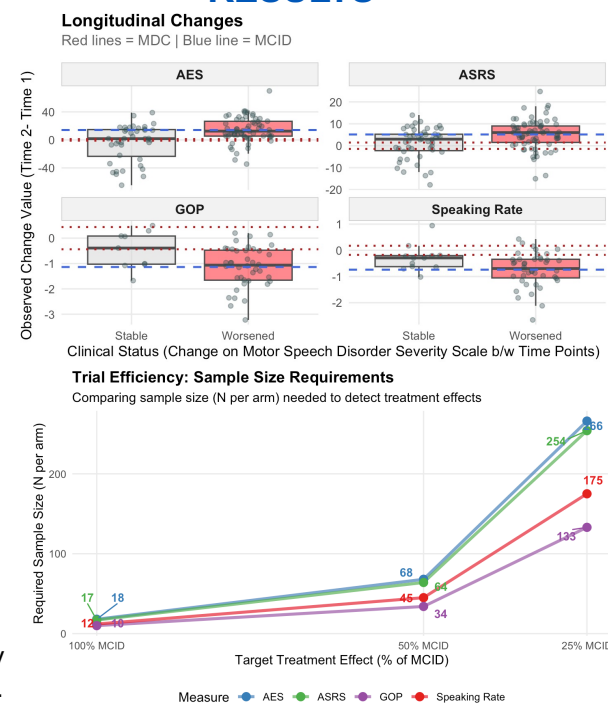
METHOD

- MCID was calculated based on the change for each measure for those who with worsening on the motor speech disorder severity rating scale.
- Power simulations modeled the required sample size per arm to detect 100%, 50%, and 25% of the MCID at 80% power ($\alpha = 0.05$).

CONCLUSION

Objective measures like Goodness of Pronunciation (GOP) and speaking rate (syllables/ second) required fewer participants per arm to detect clinically meaningful change relative to perceptual measures like the articulatory error score (AES) and ASRS. These measures are more suitable for automation for large-scale research.

RESULTS



CONTACT INFORMATION:

Utianski.Rene@mayo.edu