



International Society for CNS Clinical Trials and Methodology

Orphan Diseases Working Group
ISCTM Annual Meeting, Mayflower Hotel
Washington, DC
Wednesday, February 18, 2026

Joan Busner, Ph.D

Gahan Pandina, Ph.D.

Co-Chairs

Disclosures

- Dr. Busner is a full-time employee of Signant Health, in which she may hold stock/equity.
- Dr. Pandina is a full-time employee of Janssen Research and Development, LLC, and holds stock in Johnson & Johnson

Welcome and Introductions to New Members

Updates

- Autumn, 2025 Meeting in Amsterdam
 - Interest expressed by many of our EU colleagues to join in manuscript development
- 5 excellent papers already published
- Three manuscripts currently underway
 - CGI in Rare Disease Trials – draft in progress
 - Measuring Cognition in Rare Disease – draft in progress
 - (Ethical Issues in Rare Disease Trials) – is this still of interest?
- Last presented Orphan Diseases WG-sponsored session: February 2025 session: “Using Novel Biomarkers and Advanced Analytics to Optimize Measurement, Endpoint Selection, and Signal Detection: Lessons for the Broader Neuroscience Community from Orphan Disease Trials”
- Potential future session ideas needed
 - Some discussions held post-Amsterdam with scientific committee about session on gene therapy

Brainstorming

- Other manuscript ideas?
- New areas to direct focus?
- Half-day sessions we might consider?