

Orphan Disease Working Group F2F Meeting Minutes – February 21, 2026

Attendees (TBC from staff).

- Meeting run by co-leads: Joan & Gahan.

Agenda Topics and Discussion

1. Introduction and welcome
2. Description of recent activities
 - a. Papers for CGI and cognition are in development, ethics paper in early stages as well. More detailed discussion
 - i. Status of current papers in development (CGI, Cognition, Ethics).

Papers in production	Status	Next Steps	Authors / Interests
CGI	<ul style="list-style-type: none"> • Outline of paper produced 	<ul style="list-style-type: none"> • Outline to be circulated for group review in the next 4-6 weeks • Meeting to be held with authors, to be coordinated by ISCTM staff – poll to be expected soon • Goal – produce solid draft by June 2026, with submission goal for September 	<ul style="list-style-type: none"> • Already working on it: Identified in draft documents • Expressed Interest at meeting: Sarah Barnum (sbarnum@cognitionmetrics.com), Scott Hunter, Lisa Kammerman (lisa@kammerman.com), Audrey Thurm,
Cognition	<ul style="list-style-type: none"> • Mature draft of paper available 	<ul style="list-style-type: none"> • Pre-final draft to be produced for group review in the next 4-6 weeks • Goal – submit for review by June 2026 	<ul style="list-style-type: none"> • Already working on it: identified in draft documents • Expressed Interest at meeting: Sarah Barnum, Meg Grabb, Scott Hunter, Audrey Thurm, Cristan Farmer, Brian Murphy (brian@cumulusneuro.com)
Ethical Issues in OD Drug Dev.	<ul style="list-style-type: none"> • Concept approved to move ahead, outline in development 	<ul style="list-style-type: none"> • Outline to be produced and discussed with co-author group in next 2-3 months • Draft paper to be developed by September 2026, for discussion at Philadelphia meeting and finalization by December 2026 • Concepts to be added: genetic variability/stratification by genotype, access to medications, data sharing from companies 	<ul style="list-style-type: none"> • Already working on it: Identified in draft documents • Expressed Interest at meeting: Joan Fallon, Holly Pearl (holly.pearl@faigredrinker.com)

3. Brainstorm on future topics of interest – for consideration as a short session or paper by ODWG:
 - a. Neurophysiological biomarkers, novel biomarkers (specificity) and relation to clinical outcome/efficacy and outcomes of interest to patients and families (interested: Dana Kim Murphey)
 - b. Clinical design in rare disease (interested: Mandeep Kaur).
 - i. Include topics on pros and cons of alternative trial designs including N of 1 designs, misuse of crossover designs
 - c. Regulatory hurdles and challenges in rare disease (interested: Mandeep Kaur)
 - d. Design paper in relation to roadmap for COAs (interested: Audrey Thurm)
 - e. Best practices for natural history studies, collaboration across rare disease
 - f. Special issues in gene therapy