

7 September 2019 Orphan Disease Working Group Summary

ISCTM Autumn Conference Includes Joint Day with ECNP

Copenhagen, Denmark

- The Orphan Disease Working Group met to refine concepts for a potential future ISCTM program (day) on pediatric drug development, with a half-day parallel session on patient centricity in Orphan Diseases.
- Topics were refined to include critical topics that impact the approach taken to drug development.
 - Highlighted topics for a potential day-long session were pediatric first vs. adult first indications, outcome measures and cognition, IRB/Ethical Concerns, role of consortia/pre-competitive networks, and rater training.
 - Patient centricity parallel session will include orientation to patient centricity, importance of and best practices to obtain caregiver/family input protocol design and implementation, case studies of successes and challenges,
- Potential speakers for key topics were proposed, and areas of need identified – the ODWG will be reaching out to the ISCTM membership, leadership, and beyond to put together a stellar program of speakers.
- The next face-to-face meeting will include another ODWG breakout session, with additional themes and key outputs from the session to be considered – please reserve the time in February and join us!