

Orphan Diseases Working Group Summary -- Autumn Conference 2018
Co-Chairs, Joan Busner and Ravi Anand

This was the first in-person meeting of the working group after multiple teleconferences. We had an excellent turnout. The group had decided ahead of time to form two subgroups so as to most effectively address important issues. Per group vote, the initial subgroups were Patient Centricity and Instructive Case Examples. Among the topics considered by the Patient Centricity group were plans for developing a patient centricity half-day session for the full ISCTM membership, and generation of white papers for industry on incorporating patient and caregiver perspectives, disseminating study results to participants, and special considerations for children and adolescents. The Instructive Case Examples subgroup has already generated a series of excellent examples, and discussed means of soliciting additional examples so as to form a database accessible by the ISCTM membership. Best means of soliciting cases on an ongoing basis, ways of sponsor-blinding, means of categorizing cases, and the need for a reviewing committee were discussed. The Orphan Diseases Working Group is collaborative and multidisciplinary – we welcome new members and new ideas!