

ISCTM Orphan Diseases Working Group, 19 February 2019
Patient Centricity Subgroup Summary
15th Annual Meeting

The Patient Centricity Subgroup of the Orphan Diseases Working Group met and discussed plans for a future ISCTM session (half-day) focused on drug development in Orphan Diseases. The group proposed a half-day session, involving family members, to review and discuss best practices in protocol design and implementation. Tentative topics will range from involving caregivers in protocol design, lessons learned from past protocols, taking the drug trial to the patient, age-related issues in patient centricity, rating scales and measurement issues, and caregiver awareness of clinical trials. The goal is to have several papers, including a white paper, generated as a result of the series of prepared talks and discussion. The group will discuss potential duration and timing of the session with ISCTM leadership, in advance of the fall 2019 meeting. Participation in the fall meeting is highly encouraged! Multiple publications are anticipated, and attendees can participate in manuscript development in topic areas of interest.