

ROUNDTABLE 1: Statistical Challenges in Clinical Study Design in Rare Diseases

- * **Moderator:** *Robert Temple, CDER, FDA*
- * **Panelists:** *Edmund Capparelli, University of California San Diego; Avital Cnaan, CNMC; Susan Ellenberg, University of Pennsylvania; Ed Kaye, Sarepta Therapeutics; Michael Pacanowski, FDA; Martin Posch, University of Vienna*