Methodological Challenges Related to Rare and Orphan Disease Drug Development

Chairs: Joan Busner, Ravi Anand, Judith Dunn
Facilitator: Adam Butler
Disclosure

- Full time employee, Bracket
Session Genesis

• Much requested topic on ISCTM evaluations
• Many ISCTM members involved in or contemplating orphan disease development programs
• Many unique challenges to be explored...
Orphan Disease Working Group
Leadership Team

- Munaf Ali
- Ravi Anand
- Thomas Blaettler
- Adam Butler
- Judy Dunn
- Robert Farber

- Lew Fredane
- Bill Jacobson
- Randall Marshall
- Joanna Segieth
- Jeannie Visootsak
- Simon Day
Session Today: Excellent Speakers

- **Ron DeBellis**, Scientific Advisor of National Organization of Rare Disorders (NORD): *The Unmet Need – Partnership with Patient Advocacy Groups*
- **Dennis Revicki**, Senior Vice President Outcomes Research, Evidera – *Clinical Outcome Assessment Endpoints for Rare Disorders: Challenges and Methods for Clinical Trials*
- **Simon Day**, Expert Consultant in Orphan Disease Statistical Approaches, Formerly of MHRA and EMEA – *Statistical Challenges: Designs for Clinical Trials with Limited Patient Populations*
- **Ravi Anand**, CNS Consultant – *Clinical and Regulatory Challenges in Developing New Treatments for Rare Diseases*
- **Judith Ng-Cashin**, Chief Scientific Officer, Syneos Health – *Logistical Challenges. Rare Disease Clinical Research: Collaboration the Key To Success*
- **Frank Sasinowski**, Orphan Disease Legislation Consultant, Formerly of FDA – *Regulatory Perspective: Data Requirements and Approval Pathways for CNS Orphan Drugs*
Today’s Format

• 20 minutes for each speaker
• 10 minutes for questions
• 20 minutes Panel Discussion
Working Group Open to All

• We hope to see you at the next ISCTM meeting!