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!