












Symposium - November 6th, 2019

-  **8:00** Registration and Breakfast
-  **9:00** Opening Remarks
-  **9:15** Drug Development in Rare Diseases - Need for Innovation in Statistical Thinking
Kannan Natarajan, Pfizer
-  **10:15** MIDD in Rare Disease: Experiences and Opportunities
Dr. Gianluca Nucci, Pfizer
-  **11:00** Historical Control Borrowing: Overview, Advancement and New Methodologies
Jianchang Lin, Takeda
-  **11:45** Lunch
-  **12:45** Cytel Innovations & Roadmap
Geoffrey Grove, Cytel Inc.
-  **1:15** Challenges in Rare Disease Clinical Trials: The PREVENT Trial for NMOSD
Amy Pace, Alexion
-  **2:15** Data Monitoring Committees – Behind Closed Doors
David Kerr, Axio
-  **3:00** Break
-  **3:15** The Use of Natural History Data in Clinical Trials for Rare Diseases
Clare Elkins, Alexion
-  **4:00** Using Historical Control for Regulatory Approvals in US – A Practical Review
Focusing on Diseases in Small Population
Ziliang Li and Chenkun Wang, Vertex
-  **4:45** Closing Remarks

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Challenges in Rare Disease Clinical Trials Symposium & Hands-on Training November 6th-7th, 2019

Hands-on Training - November 7th, 2019

-  **8:00** Breakfast and Introduction to East with an Overview East Modules
-  **9:00** Phase 1 Dose Escalation (ESCALATE) with Cohort Expansion (PROGRAM)
-  **10:45** Coffee Break
-  **11:00** Phase 2: MCPMod (Design and Analysis)
-  **12:00** Lunch and Networking
-  **1:00** Phase 2/3: Multiple-Arm, Multiple-Stage Designs (MAMS)
-  **2:00** Phase 3: Group Sequential Designs (SURVIVAL)
-  **3:00** Break
-  **3:15** Phase 3: Adaptive Designs; Sample Size Re-estimation (SURVADAPT)
-  **4:15** Phase 3: Adaptive Population Enrichment (ENRICH)
-  **5:00** Closing Remarks

Agenda subject to change on the day.

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