



Session 6

Clinical Trial Statistics

Accelerating Therapies for Rare Diseases

October 18-20, 2010

Rockville, MD

USP Building

Statistical & Review Perspectives

- **IND** and NDA/BLA
- Same Scientific Standards (Regulations and Guidance)
- Reproducible Research (Confirmatory Trials, ICH E9)
- Prospective/Pre-specified (Protocol / Statistical Analysis Plans)
- Flexible (Communication – We are learning. Please Include Statisticians...early and often)
- Innovation/Collaboration (e.g., Study Designs)
- Data/Information Quality (Missing Data, Standards)
- IND/NDA/BLA Submission (eCTD, Analysis Files) 2



Understanding the FDA

Regulation

Guidance

Outline

- ***Statistical Overview for Clinical Trials – Basics of Design and Analysis of Controlled Clinical Trials***

Behrang Vali, M.S., Biostatistician, Office of Biostatistics, CDER, FDA

- ***Adaptive Designs and Small Clinical Trials***

Christopher Coffey, Ph.D., Professor, Department of Biostatistics, University of Iowa

- ***Challenges in Rare Disease Studies***

Gary Cutter, Ph.D., Professor, Biostatistics, University of Alabama

- ***Level of Evidence to Support Marketing Applications***

Lisa Kammerman, Ph.D., Biostatistician, Office of Biostatistics, CDER, FDA



THANK YOU

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