FDA-NIH Science of Small Clinical Trials Course

The Great Room • White Oak Campus
10903 New Hampshire Avenue • Silver Spring, Maryland 20993 November 27–28, 2012

Agenda

Day 1 — General Principles

Time	Session	Speakers	Session Chair
8:00-8:15	Welcome Remarks	Gayatri Rao M.D., J.D.(FDA/OOPD) Stephen Groft (NIH/ORDR)	n/a
8:15-8:35	FDA Introduction	Lisa Lavange, M.D. (FDA/CDER)	Gayatri Rao
8:35-8:55	Industry Introduction	Simon Day Ph.D. (Roche)	
8:55-9:15	Academic/NIH Introduction: Practical considerations for small trials	Jeffrey Krischer, Ph.D. (University of South Florida)	
9:15-9:30	Break		
9:30-9:50	Case 1: Vemurafenib for melanoma with BRAF mutation (*Enrichment*; *Companion Diagnostic)*	Geoffrey Kim, M.D. (FDA/CDER)	John Hyde
9:50-10:10	Case 2: Infliximab for Pediatric Ulcerative Colitis (*Extrapolation from adults)*	Jessica Lee, M.D., M.M.Sc. (FDA/CDER)	
10:10-10:30	Case 3**:** A Tale of Two Studies: Carbaglu & Kuvan	Lynne Yao, M.D. (FDA/CDER)	
10:30-10:50	Case 4: Naglazyme for MPS VI (*One-way crossover)*	Tamara Johnson, M.D. (FDA/CDER)	
10:50-11:10	Case 5: Rilonacept for CAPS (*Three-stage study)*	Keith Hull, M.D. (FDA/CDER)	
11:10-11:30	Case 6: Berlin Heart Pediatric EXCOR Ventricular Assist Device *(HUD)*	John Laschinger, M.D. (FDA/CDRH)	
11:30-11:50	Panel Discussion		
11:50-12:50	LUNCH		
12:50-1:30	Design Principles for small trials	Christopher Coffey, Ph.D. (University of Iowa)	Larry Bauer
1:30-2:10	Enrichment	Robert Temple, M.D. (FDA/CDER)	

2:10-2:50	Bayesian	John Scott, Ph.D. (FDA/CBER)	Larry Bauer
2:50-3:05	Break		
3:05-3:50	Benefit/Risk *(incl. Decision analysis)*	Telba Irony, Ph.D. (FDA/CDRH)	
3:50-4:30	Adaptive design - iSpy2 Trial	Donald Berry, Ph.D. (MD Anderson CA Center)	
4:30-5:00	Panel Discussion		

Day 2 — Special Considerations

Time	Session	Speakers	Session Chair
8:00-9:30	Rare Diseases: Challenges and opportunities	John Hyde, M.D. (FDA/CBER) Kathryn O'Connell, M.D., Ph.D. (FDA/CDER) Stephen Groft, Pharm.D. (NIH/ORDR)	David Eckstein
9:30-9:45	Panel discussion		
9:45-10:00	Break		
10:00-10:40	Historical controls	Marc Walton, M.D. (FDA/CDER)	Francesca Joseph
10:40-11:15	Device Topics: e.g. Propensity scores, Performance goals, etc.	Gregory Campbell, Ph.D. (FDA/CDRH)	
11:15-11:30	Panel discussion		
11:30-12:30	LUNCH		
12:30-12:45 12:45-1:00	The role of patient organizations	Ronald Bartek (Friedrich's Ataxia Research Alliance)	Kathryn O'Connell
1:00-1:45	Logistics/Practical Issues/Use of Foreign data	Eugene Sullivan, M.D. (EJS Consulting) Robert Schmouder, A.B., M.P.H., M.D. (Novartis Institutes for Biomedical Research)	
1:45--2:00	Panel discussion		
2:00-3:00	Course Wrap-Up		Francesca Joseph