

ADVANTAGES AND CHALLENGES OF ADAPTIVE CNS TRIALS REAL WORLD LESSIONS

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Introduction

Problem

- Pharmaceutical companies have a never-ending search to improve the speed, quality, efficiency, and generation of decision-making data in clinical research trials

Solutions

- This search has led to the adoption of innovative research methodologies including adaptive design clinical trials
- Adaptive design trials provide flexibility in making prospectively defined trial adaptations on the basis of data generated during the conduct of the trial in a way that maintains the validity and integrity of the clinical study

Adaptive Design Working Group

- 2015: Adaptive Design session at the ISCTM annual meeting
- 2016: Two Webinars
 - Are Adaptive Trials an Appropriate Design Choice in Neuroscience?: *Judith Quinlan*
 - Case Studies In Adaptive Design: Re-Estimation of Sample Size and Sequential Parallel Comparison Design: *Roseann White and Michael Pencina*

Objectives of the Adaptive Design Session

- To build on the 2015 Adaptive Design session
- Provide a brief review of adaptive design methodology
- Review the obstacles, barriers, problems and logistical issues with adaptive design trials, describe when adaptive design trial methodology is useful or when not useful for CNS trials
- Present three new real world examples of adaptive design trials in neuroscience
- Provide a FDA perspective on the presentations along with the implications for neuroscience clinical studies

Agenda

- Introduction: *Ron Marcus*
- Adaptive population enrichment design: Drug development efficacy in rare epileptic syndromes: *Joanne Segieth*
- Adaptive design comparative effectiveness in status epilepticus: *Kristine Broglio*
- Identification of meaningful cognitive endpoints in studies of pharmacological therapies for cognitive impairment in schizophrenia: *Michael Sand*
- Layers of an onion: Considerations for a confirmatory, adaptive CNS trial: *Brian Millen*
- Comments from FDA statistician on Adaptive Design presentations: *H M Hung*
- Q & A and panel mediated discussion: *Judy Kando*