Adaptive Design for Clinical Trials: FDA Draft Guidance Symposium

March 26, 2010

National Labor College, Silver Spring, MD, USA

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Executive Director
Late Development Statistics
Merck Research Laboratories

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Senior Director
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Build a Solid Foundation in the Regulatory Aspects of Adaptive Designs.

There has been increasing interest in applying adaptive clinical trials throughout the various stages of clinical development. This conference will provide an overview of the FDA draft guidance on the topic of adaptive designs, and an opportunity for discussion of some topics involved with the implementation of adaptive clinical trial design in drug development and approval. The FDA will summarize the guidance content and highlight areas where they feel comments to the official Docket would be most helpful. There will be opportunities to seek clarifications of issues raised in the guidance. Study sponsors will discuss practical examples of designing and conducting clinical trials using adaptive designs for regulatory approval and focus on how to address potential concerns about adaptive designs.

Due to their complexity, adaptive design development programs require more (and earlier) planning and documentation. The conference will present how this planning should be summarized and discussed with the FDA prior to implementing an adaptive trial.

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Who Should Attend

Professionals involved in:

- Biostatistics
- Clinical research
- Compliance
- Data analysis
- Quality assurance/Quality control
- Regulatory affairs

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Conference Objectives

- Transmit a general understanding of the content of the draft guidance
- Ensure attendees understand how to formally submit comments on the draft guidance to the FDA Docket
- Provide solid understanding of adaptive design issues and the contexts for their use that are of greater or lesser concern to FDA. Potential issues include operational bias, design induced bias, necessary multiplicity adjustments, difficulties in result interpretation, and the potential effects of eliminating the time to thoughtfully evaluate data during a drug development program. The FDA will outline types of adaptive methods and stages of drug development where adaptive clinical trials may be advantageous in the drug development process
- Present practical issues about conduct of adaptive trials by those who had experiences in performing adaptive trials, with particular emphasis on maintaining the integrity of adaptive trials

Target Audience

Potential stakeholders interested in this guidance include the pharmaceutical industry, academic clinical investigators, contract research organizations, and other regulatory agencies. The conference will present perspectives from each of these groups.

FRIDAY, MARCH 26

7:30-8:30 AM CONTINENTAL BREAKFAST AND REGISTRATION

8:30-8:40 AM WELCOME AND OVERVIEW

Keaven M. Anderson, PhD
Executive Director, Late Development Statistics
Merck Research Laboratories

8:40-11:45 AM SESSION 1

FDA Draft Guidance on Adaptive Design Clinical Trials

Robert O'Neill, PhD
Director
Office of Biostatistics
Office of Translational Sciences, CDER, FDA

Robert Temple, MD
Deputy Director for Clinical Science
CDER, FDA

Marc Walton, MD, PhD
Associate Director
Office of Translational Sciences, CDER, FDA

Sue-Jane Wang, PhD
Associate Director for Adaptive Design and Pharmacogenomics, Office of Biostatistics
Office of Translational Sciences, CDER, FDA

8:40-9:45 AM PART I – DRAFT GUIDANCE

8:40-8:55 AM Overview
Sue-Jane Wang, PhD

8:55-9:20 AM Statistical Focus
Robert O'Neill, PhD

9:20-9:45 AM Clinical Regulatory Aspect
Robert Temple, MD

9:45-10:15 AM BREAK TO COLLECT/COLLATE QUESTIONS

10:15-11:15 AM PART II – DOCKET PROCESS AND RESPONSES TO QUESTIONS ON THE DRAFT GUIDANCE
MODERATOR:
Sue-Jane Wang, PhD

Robert O'Neill, PhD
Robert Temple, MD
Marc Walton, MD, PhD

11:15-11:45 AM PhRMA PERSPECTIVE ON ADAPTIVE DESIGNS FROM EXPLORATORY TO CONFIRMATORY TRIALS
Michael Krams, MD, PhD
VP, Neurology and Franchise Head for CNS
Johnson & Johnson

11:45 AM-12:45 PM LUNCH

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LEARNING OBJECTIVES At the conclusion of this meeting, participants should be able to:

- Transmit a general understanding of the content of the draft guidance
- Explain how to formally submit comments on the draft guidance to the FDA Docket.
- Discuss adaptive design issues and the contexts for their use that are of greater or lesser concern to FDA

12:45-1:45 PM SESSION 2 — PART I

Documenting Properties of Adaptive Trials and Communicating with the FDA

MODERATOR

Robert Hemmings, MSc, CStat
Statistics Unit Manager
Medicines and Healthcare Products Regulatory Agency, (MHRA), Committee for Medicinal Products for Human Use (CHMP), UK

12:45-1:15 PM **EXPLORATORY DOSE-FINDING TRIAL IN PHASE 2**
Frank (Xiaoyin) Fan, PhD
Associate Director, Scientific Staff, Late Development
Merck Research Laboratories

1:15-1:45 PM **COMMON PRACTICES ON TRIAL SIMULATION AND REPORTING FROM THE ADAPTIVE DESIGN WORKING GROUP**
Brenda Gaydos, PhD
Senior Research Advisor
Eli Lilly and Company

1:45-3:15 PM SESSION 2 — PART II

MODERATOR

Henry Hsu, PhD, MPH
Director, Division of Biostatistics
Office of Biostatistics and Epidemiology
CBER, FDA

1:45-2:15 PM **EXPERIENCE IN MULTIPLE ADAPTIVE TRIALS (EMPHASIS ON DECISION-MAKING, CONFIDENTIALITY/INTEGRITY, TYPES OF DESIGNS CONSIDERED)**
Paul Gallo, PhD
Biometrical Fellow - Biostatistics
Novartis Pharmaceuticals

2:15-2:45 PM **THE ROLE OF A DATA MONITORING COMMITTEE IN COMMUNICATION AND DECISION MAKING AT INTERIM ANALYSES**
Raymond Bain, PhD
Vice President
Biostatistics and Research Decision Sciences
Merck Research Laboratories

2:45-3:15 PM **ADAPTIVE TRIAL OPERATIONS WHEN WORKING WITH A DEVELOPMENT PARTNER**
Zoran Antonijevic, MSc
Senior Director
Center for Statistics in Drug Development Innovation
Quintiles

3:15-3:30 PM REFRESHMENT BREAK

3:30-4:00 PM SESSION 3

FDA's Current Thinking on Adaptive Trial Integrity

MODERATOR

Sue-Jane Wang, PhD
Associate Director for Adaptive Design and Pharmacogenomics, Office of Biostatistics
Office of Translational Sciences, CDER, FDA

CONFIRMING THE INTEGRITY OF ADAPTIVE CLINICAL TRIALS
Leslie Ball, MD
Director, Division of Scientific Investigations
CDER, FDA

4:00-4:45 PM SESSION 4

Panel Discussion

CO-MODERATORS

Sue-Jane Wang, PhD
Associate Director for Adaptive Design and Pharmacogenomics, Office of Biostatistics
Office of Translational Sciences, CDER, FDA

Christy Chuang-Stein, PhD
Executive Director, Statistical Research and Consulting Center
Pfizer Inc

PANELISTS:

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Medicines and Healthcare Products Regulatory Agency, (MHRA), Committee for Medicinal Products for Human Use (CHMP), UK

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Willi Maurer, PhD
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Senior Statistical Consultant, Statistical Methodology Group
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Office of Translational Sciences, CDER, FDA

Rick Sax, MD
Clinical Vice President, Design Strategies
AstraZeneca R&D

Marc Walton, MD, PhD
Associate Director, Office of Translational Sciences
CDER, FDA

4:45-5:00 PM SESSION 5

Summary and Path Forward

Keaven M. Anderson, PhD
Executive Director, Late Development Statistics
Merck Research Laboratories

John Jenkins, MD
Director, Office of New Drugs
CDER, FDA

5:00 PM CONFERENCE ADJOURNED

REGISTRATION FORM
Register online or fax this page to +1.215.442.6199

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Event #10020 • March 26, 2010

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Venue: Contact Lauren Samet, +1.301.628.5606

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The most convenient airport is Reagan National Airport and attendees should make airline reservations as early as possible to ensure availability.

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