THE NEXT GENERATION OF TRIAL DESIGN INNOVATION

Strategies, Methods, and Logistical Considerations for Flexible Trial Designs

November 15th & 16th 2012
Loews Philadelphia Hotel • Philadelphia, PA

Top Reasons to Attend

✓ In-depth discussion on methodology, including statistical paradigms
✓ An adaptive design portfolio analysis on quantifying benefits
✓ Designs and adaptive analysis plans for pivotal clinical trials
✓ Designing and implementing a new breed of adaptive trials

Featured Sessions Include

Strategies, Methods and Logistical Considerations for Flexible Trial Designs
Roseann White, Director, Health Technology Assessment and Market Access, ABBOTT

Bayesian Statistics – Utilizing Bayesian Methods to Create the Optimal Trial Design
Jeff Palmer, Associate Director, Biostatistics, GENZYME CORPORATION

Adaptive Designs for Personalized Medicine and the Importance of Appropriate Design Selection
Sandeep Menon, MPH, PhD, Director of Biostatistics, PFIZER BIOThERAPEUTICS RESEARCH, and Adjunct Assistant Professor of Biostatistics, BOSTON UNIVERSITY

Workshop: Implementing Adaptive Trial Design in Any Company
Terry Katz, Director, Global Data Management and Statistics, MERCK ANIMAL HEALTH

Distinguished Speakers From:

ABBOTT
AMGEN
PFIZER
CASE WESTERN RESERVE UNIVERSITY
GENZYME CORPORATION
JOHNSON & JOHNSON
MERCK & CO.
VDDI PHARMACEUTICALS

Register Now! Call 866-207-6528 • Web: www.exlpharma.com/trialdesign • Email: register@exlpharma.com
DEAR COLLEAGUE,

The industry is determined to bring new drugs to market faster despite the increasing complexity of clinical trial execution therefore, there is a strong need to share current practices of trial design innovation.

Currently the industry is buzzing with fresh case studies, technological advances, simulations and other resources on Adaptive Trial Design. This interactive conference will showcase these innovations through case studies, panels, and workshops as a valuable resource to modify aspects of the study as it continues, without undermining the validity of the trial. In addition, multiple networking opportunities will also showcase new technologies available to harness this successful method of design.

The conference will focus on FDA Draft Guidance and its impact on industry efforts, as well as design strategies such as the Bayesian Method, the use of Biomarkers, and the role of personalized medicine.

We look forward to seeing you in November 15th - 16th in Philadelphia!

Sincerely,

Meredith Becker
Meredith Becker
Conference Director, The Next Generation of Trial Design Innovation
mbecker@exlpharma.com

MISSION STATEMENT:
The mission of this conference is to bring to light the use of Adaptive Trial Design in practice, share technology enhancements, protect trial integrity and examine the effect that FDA Draft Guidance has had on industry.

Who Should Attend
This conference is designed for executives from the pharmaceutical, biotechnology, and medical device industries within the following professional areas:

- Biostatistics and Data Management
- Clinical Development
- Project Management
- Translational Medicine
- Clinical Research/Clinical Affairs
- Discovery Biometrics
- Clinical Studies and Clinical Compliance
- Adaptive Design
- Statistical Research & Applications

This conference is also designed for companies providing the following services:

- Adaptive Design
- Data Management
- Statistical Analysis
- Clinical Research

HOTEL INFORMATION

Loews Philadelphia
1200 Market Street
Philadelphia, PA 19107
Phone: (215) 627-1200

Venue: Directly across from the Pennsylvania Convention Center, the Loews Philadelphia Hotel is 15 minutes from Philadelphia International Airport, five minutes from Amtrak’s 30th Street Station, and steps away from the historic district, shopping, restaurants, and sports arenas.

Room Reservations: To make reservations, please call The Loews Reservation Center at 1-888-575-6397 and request the negotiated rate for ExL’s November Meeting. The group rate is guaranteed until October 24, 2012.

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Main Conference Begins

1:15 Chairperson’s Day One Welcome and Opening Remarks
Laurent Spiess, VP Business Development, APTIV SOLUTIONS

1:30 FDA Draft Guidance Review and Impact on the Industry
Review the Details Behind the FDA Draft Guidance and the Thought Process That Led to the Decisions Made
• Identifying and discussing the key elements of the guidance to better understand the implications
• Recommended interaction between industry and FDA when deciding and executing new clinical methodologies
• Open discussion between presenter and audience
The Impact the Draft Guidance has Made and the Changes to Remain Compliant
Qing Liu, Ph.D., Senior Research Fellow of Statistical Science, JOHNSON & JOHNSON

2:30 Adaptive Designs for Personalized Medicine and the Importance of Appropriate Design Selection
• The background of personalized medicine and how it is a relatively young but rapidly evolving field of clinical research
• Identifying genetic, genomic, and clinical characteristics that have the potential to accurately predict patient’s susceptibility of developing a certain disease and its response to treatment
• Personalized medicine is the translation of this genetic knowledge to patient care. However, this “translation” can be very challenging in the phase of limited knowledge of the biomarker and/or appropriate diagnostics
• Appropriate selection of the study design is important to critically determine biomarker performance, reliability and eventually regulatory acceptance
• This session will discuss various designs including adaptive designs available at our disposal and its merits and limitations
Sandeep Menon, MPH, PhD, Director of Biostatistics, PFIZER BIOTHERAPEUTICS RESEARCH and Adjunct Assistant Professor of Biostatistics, BOSTON UNIVERSITY

3:45 Designing an Adaptive Strategy at the Portfolio Level
• Analyzing a pipeline of products to identify where an adaptive design strategy would add value
• Assessing adaptive design options at each phase of development
• Quantifying the benefits of adaptive design across a development portfolio
• Developing innovative ways to identify early winners and losers at the portfolio level
Vlad Dragalin, Senior Vice President, Innovation Centre, APTIV SOLUTIONS
Moira Thomson, Vice President, Translational Sciences, APTIV SOLUTIONS
Sarah-Arbe Barnes, SVP, Translational Sciences, APTIV SOLUTIONS

4:30 BREAKOUTS: Learn about various trial designs and available tools in a group discussion setting
Design Roundtable 1 – Multiple Ascending Dose (MAD)
Design Roundtable 2 – Adaptive Dose Escalation Design
Design Roundtable 3 – Drop Loser Design (DLD)
Design Roundtable 4 – Response Adaptive Randomization Design
Design Roundtable 5 – Group Sequential Design (GSD)
Design Roundtable 6 – Sample Size Re-Estimation Design (SSR)

5:30 Day One Concludes
8:00  Continental Breakfast

9:00  Chairperson’s Day One Recap and Day Two Welcome
Laurent Spiess, VP Business Development, APTIV SOLUTIONS

9:15  Case Study: Designing and Implementing a New Breed of Adaptive Trials
• The Sponsor’s perspective – pivotal study, risk tolerance, therapeutic indications and lessons from all research to date
• Constructing a “Promising Zone” design based adaptive sample size re-estimation
• Benefits: staged investment versus large up-front investment, any increase in sample size – patients – determined by interim analysis
• Avoidance of operational bias: interacting with regulators, investors and investigators
Zoran Antonijevic, Senior Director, Strategic Consulting and Adaptive Implementation, CYTEL INC.

10:00  The Challenges of Investigating Genetic Data in Adaptive Clinical Trials
• It will soon be feasible to examine 1 million or more genetic markers, or even sequence the whole genome of 3.2 billion nucleotide pairs, for a few hundred dollars
• The multiple testing issue and how it dominates any other problem there may be
• Result of Including the testing of only a few candidate genes, carefully chosen depending on what the trial is testing
• The appropriate approach for trials of drugs for which there is already sufficient pharmacodynamic knowledge, with the aim of determining subgroups who do and do not respond
Dr. Robert Elston, Chair, Distinguished University Professor, CASE WESTERN RESERVE UNIVERSITY, SCHOOL OF MEDICINE

10:30  Networking & Refreshment Break

11:00  Comprehending the Role of Biomarkers during Adaptive Trials to Advance the Growth of New Medical Therapies
• Adaptive Trials for personalized medicine – biomarkers for patient stratification
• Using genomics in clinical trial design
• Designs and adaptive analysis plans for pivotal clinical trials of therapeutics and companion diagnostics
R. Stephen Porter, Pharm.D., FCP, MRCP, Chairman, President and CEO, VDDI PHARMACEUTICALS

11:45  Strategies, Methods and Logistical Considerations for Flexible Trial Designs
Design Strategies:
• Timing
• Binary versus continuous endpoints
• Surrogate versus actual
Methods to Consider:
• Bayesian Versus Frequentist
• Adjusting for type I error
• Assessment using multiple comparators
• Making decisions on actual or conditional power
Logistics to Account For:
• Avoiding Bias
• What to do if your enrollment model is wrong
Roseann White, Director, Health Technology Assessment and Market Access, ABBOTT

12:30  Luncheon

1:30  Bayesian Statistics – Utilizing Bayesian Methods to Create the Optimal Trial Design
What is the Bayesian Method?
• Background and origins
• Current adoption rate
The Benefits of Utilizing the Bayesian Adaptive Approach
• Increase in efficiency of clinical trials and development
• Substantial cost savings
• Conducts seamless phase II and II trials
Jeff Palmer, Associate Director, Biostatistics, GENZYME CORPORATION

2:15  Panel Discussion: Conventional Design vs. Adaptive Trial Design
• Which design is going to lead the next generation of clinical trials?
• What have we learned thus far that can be applied to future Adaptive Trials?
• When is it appropriate to utilize Adaptive Design vs. Traditional Design?
• How have past trials shaped current Adaptive Design Methods?
Panelists Include:
Darcy Hille, MS, Sr. Biometrician, MERCK & CO
Sandeep Menon, MPH, PhD, Director of Biostatistics, PFIZER BIOOTHERAPEUTICS RESEARCH and Adjunct Assistant Professor of Biostatistics, BOSTON UNIVERSITY
Visit www.exlpharma.com/trialdesign for panel additions

3:00  Chair Closing Remarks

3:15  Conference Concludes

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Five Ways to Register:

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- **Four weeks or more:** A full refund (minus a $295 processing fee), or a voucher to another ExL event valid for 18 months from the voucher issue date.
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To receive a refund or voucher, please fax your request to 888-221-6750.

There will be an administrative charge of $300 to substitute, exchange and/or replace attendance badges with a colleague occurring within five business days of the conference.

ExL Pharma’s liability is limited to the conference registration fee in the event of a cancellation.

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Questions? Comments?
Do you have a question or comments that you would like to be addressed at this event? Would you like to get involved as a speaker or discussion leader?

Please email Program Director, Meredith Becker at mbecker@exlpharma.com
Register Now...

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Medical Product Claims Event
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