DIA/FDA Statistics Forum 2013
PDUFA V Statistical Topics and Emerging New Issues

Tutorials: April 28, 2013
Conference: April 29 to May 1, 2013
Bethesda North Marriott Hotel & Conference Center
Bethesda, MD

STEERING COMMITTEE CHAIRPERSONS

Stephen E. Wilson, DrPH, CAPT, USPHS
Director, Division of Biometrics III
Office of Biostatistics, OTS, CDER, FDA

Walter W. Offen, PhD
Global Head of Statistical Innovation
Data and Statistical Sciences
AbbVie

STEERING COMMITTEE

Joan Buenconsejo, PhD
Statistics Team Lead, Division of Biometrics II,
Office of Biostatistics, Office of Translational Science
CDER, FDA

Christy Chuang-Stein, PhD
Vice President, Statistics
Pfizer Inc.

José C. Pinheiro, PhD
Senior Director, Quantitative Decision Strategies
Janssen Research and Development, LLC

Estelle Russek-Cohen, PhD
Division Director, Division of Biostatistics,
CBER, FDA

Barry Schwab, PhD
Vice President, Clinical Biostatistics
Janssen Research and Development, LLC

Jerald S. Schindler, DrPH
Vice President, Biostatistics and Research Decision Sciences
Merck Research Laboratories
North American Co-Chair of the DIA Statistics SIAC

Ram C. Tiwari, PhD
Associate Director, Office of Biostatistics
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KEYNOTE SPEAKER

Robert N. Rodriguez, PhD
Senior Director, Statistical Research and Development
Past President, American Statistical Association
SAS Institute

KEYNOTE SPEAKER

Janet Woodcock, MD
Director, Center for Drug Evaluation and Research
FDA

Now in its seventh year, this unique workshop continues the dialogue on issues including FDA guidance development and regulatory science initiatives including PDUFA V initiatives. The dialogue will focus on statistical opportunities and challenges associated with data standards and innovative approaches to the design, monitoring, analysis and reporting of clinical trials and assessments of safety and effectiveness in the pre- and post-market settings.

This conference will offer in-depth discussions on key topics relevant to the evaluation of therapeutic products and includes input from key thought leaders from regulatory agencies, industry, and academia.

FEATURED SESSION TOPICS

- PDUFA V Overview
- Statisticians as Leaders
- Dichotomizing Continuous Measures for a Primary Efficacy Endpoint – Weighing the Benefits and Risks
- Measures for Primary Efficacy Endpoint – Are we Substantially Compromising Statistical Power?
- Pre-Competitive Collaboration
- Meta Analysis for Safety Data
- Companion Diagnostics
- (Premarketing) Benefit-risk Assessment in Clinical Development and Regulatory Review
- Health Technology Assessment and Comparative Effectiveness Research: Their Impact on Access to Pharmaceutical Products and Their Role in Designing Product Development Strategies
- Missing Data: When is it Important to Collect Data After Study Drug Discontinuation?
- Missing Data: Case Study Presentation and Panel Discussion
- Overview and Commentary of FDA Guidances: Multiplicity and/or Enriched Populations

WHO SHOULD ATTEND

- Statisticians
- Clinicians
- Epidemiologists
- Pharmacometricians
- Drug safety professionals
- Regulatory and medical communication scientists

LEARNING OBJECTIVES

At the conclusion of this conference, participants should be able to:

- Recognize innovative statistical solutions to issues associated with the evidence and regulatory review of medical products
- Describe the application of statistical methodologies and thinking to the development of new medical products
- Assess the impact of regulations and guidance on statistical practice
- Discuss ideas for improving the communication between industry Statisticians and Reviewers

This year's program will offer additional avenues for interaction and scientific discourse via a **scientific poster session** and an interactive **round-table luncheon event**.

This program will be developed by FDA and the DIA Statistics Community.

In collaboration with:

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CONTINUING EDUCATION CREDITS

DIA is accredited by the Accreditation Council for Pharmacy Education as a provider of continuing pharmacy education. This program is designated for up to 21.5 contact hours or 2.125 continuing education units (CEU's).

Type of Activity: Knowledge

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Disclosure Policy

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CONTINUING EDUCATION CREDIT ALLOCATION

Tutorials:

Tutorial #1, Statistical Methods for Safety Surveillance: IACET: .3 CEUs
Tutorial #2, Missing Data in Clinical Trials: IACET: .3 IACET
Tutorial #3, Benefit-risk Evaluation: Pharmacy: 3.25 contact hours or .325 CEUs, 0286-0000-13-047-L04-P; IACET: .3 CEUs

Forum:

IACET: 1.8 CEUs

Pharmacy:

Day 1: 6.25 contact hours or .625 CEUs, 0286-0000-13-048-L04-P
Day 2: 6 contact hours or .6 CEUs, 0286-0000-13-049-L04-P
Day 3: 6 contact hours or .6 CEUs, 0286-0000-13-050-L04-P

DIA'S CERTIFICATE PROGRAM

This program is part of DIA's Certificate Program and is awarded the following:

- Clinical Research Certificate Program: 12 Elective Units
- Clinical Safety and Pharmacovigilance Certificate Program: 4 Elective Units

For more information go to
diahome.org/certificateprograms

TUTORIAL DAY | **SUNDAY, APRIL 28**

9:00 AM – 12:30 PM **TUTORIAL #1**

Tutorial #1 – Statistical Methods for Safety Surveillance

TUTORIAL CHAIRPERSONS:

Ram Tiwari, PhD
Associate Director, Office of Biostatistics
CDER, FDA

Jyoti Zalkikar, PhD
Mathematical Statistician - Team Leader
Office of Biostatistics
CDER, FDA

INSTRUCTORS:

Lan Huang, PhD
Mathematical Statistician
Office of Biostatistics
CDER, FDA

Ted Guo, PhD
Mathematical Statistician
Office of Biostatistics
CDER, FDA

The statistical methods used for data-mining or signal detection of drug-adverse event combinations from large drug safety databases such as FDA's Adverse Event Reporting System (AERS, now FAERS), consisting of spontaneous reports on adverse events for post-market drugs are called passive surveillance methods. On the other hand, the statistical signal detection methods for longitudinal data, as the data accrues in time, are called active surveillance methods. A review of the most commonly used passive surveillance statistical methods, along with a likelihood ratio test (LRT) based method, recently developed by the instructors, will be discussed in detail. A live demo of the LRT tool on AERS data will be presented. Extensions of LRT for active surveillance will also be presented in detail.

The tutorial will consist of four modules. In Module-I, a review of commonly used Bayesian and Frequentist methods for signal detection in passive surveillance will be given. In Module-II, the LRT method will be discussed and a simulation study will be presented to assess the performance characteristics of LRT such as type-I error, false discovery rate, power and sensitivity. In Module-III, a live demonstration of the LRT tool to certain drugs (or drug classes) or adverse events (AEs) (or combinations of AEs) from the AERS database will be given, and the signals detected from the LRT method will be compared with the ones from some other commonly used methods. Finally, in Module-IV, extensions of the LRT methodology to a longitudinal clinical database and a brief discussion on how to handle excessive zeros in the data will be presented.

LEARNING OBJECTIVES:

- Explain commonly used methods for disproportionality analysis in drug safety database;
- Demonstrate a new method for disproportionality method that controls false positive and have good sensitivity and power, and a live demo of the accompanying tool, and;
- Review extensions of newly developed method to longitudinal data from active surveillance.

TARGET AUDIENCE:

Participants should have a fundamental knowledge of Statistics and Pharmacovigilance.

Please note that lunch is not served on tutorial day

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1:30 – 5:00 PM CONCURRENT TUTORIALS – #2 AND #3

Tutorial #2 – Missing Data in Clinical Trials

Instructor:

Roderick Little, PhD
Biostatistics Department
Statistics Department
University of Michigan

This short course will discuss methods for the statistical analysis of data sets with missing values, emphasizing applications to clinical trials. Topics include: Definition of missing data; assumptions about mechanisms, including missing at random; pros and cons of simple methods such as complete-case analysis and imputation; weighting methods; maximum likelihood and Bayesian inference with missing data; multiple imputation; computational techniques, included EM algorithm and extensions, and Gibbs sampler; software for handling missing data; missing data in common statistical applications, including regression, repeated-measures analysis, clinical trials; selection and pattern-mixture models for nonrandom nonresponse; sensitivity analysis for deviations from missing at random.

Prerequisites: Course requires knowledge of standard statistical models such as the multivariate normal, multiple linear regression, contingency tables, as well as matrix algebra, calculus, and basic maximum likelihood for common distributions, at the level of Statistical Inference; 2nd Ed. by G. Cassella and R. L. Berger

Learning Objectives:

- Discuss methods for the statistical analysis of data sets that have missing values in clinical trials
- Discuss application of missing data methods in the context of missing data

Recommended texts:

1. Little, R.J. and Rubin, D.B. (2002), Statistical Analysis with Missing Data, 2nd edition, Wiley.
2. National Research Council (2010). The Prevention and Treatment of Missing Data in Clinical Trials. National Academy Press: Washington DC. A pdf copy can be obtained without charge from the list of publications at http://www7.nationalacademies.org/cnstat/

Target Audience:

Statisticians, clinicians, and others working in clinical trials with an interest in how to address issues of missing data.

Tutorial #3 – Benefit-risk Evaluation

Instructor:

Scott Evans, PhD
Senior Research Scientist
Harvard School of Public Health, Harvard University

John Powers, MD
National Institutes of Health

The monitoring and evaluation of Benefit-risk is a fundamental element of clinical trials and development programs. Despite Benefit-risk evaluation lying at the heart of this research, there is a need for improved evaluation through critical thought and more structured evaluation. We discuss the challenges to Benefit-risk assessment, review methods that have been proposed for Benefit-risk evaluation, and present ideas for improved design, monitoring, analyses, and reporting of benefits and risks. Methods for incorporating patient preferences and weighting outcomes, and novel ideas for tailored/personalized medicine using within-patient analyses of benefits and risks will be presented.

Learning Objectives:

At the conclusion of this tutorial, participants should be able to:

- Recognize the challenges of Benefit-risk evaluation
- Discuss approaches to Benefit-risk evaluation that can be used as a foundation for building new approaches for improved design, monitoring, analyses, and reporting

Target Audience:

Statisticians, clinicians, and others working in clinical trials and development programs with interest in Benefit-risk evaluation.

6:00 PM GENERAL ATTENDEE REGISTRATION

FORUM DAY 1 | **MONDAY, APRIL 29**

7:30 – 8:45 AM REGISTRATION AND CONTINENTAL BREAKFAST

8:45 – 9:00 AM WELCOME AND OPENING REMARKS

Stephen E. Wilson, DrPH, CAPT, USPHS
Director, Division of Biometrics III
Office of Biostatistics,
Office of Translational Science
CDER, FDA

Walter W. Offen, PhD
Global Head of Statistical Innovation
Data and Statistical Sciences
AbbVie

9:00 – 10:30 AM KEYNOTE ADDRESSES

International Year of Statistics

Robert N. Rodriguez, PhD
Senior Director, Statistical Research and Development
Past President, American Statistical Association
SAS Institute

Janet Woodcock, MD
Director, Center for Drug Evaluation and Research
FDA

10:30 – 11:00 AM MORNING BREAK

11:00 – 12:30 PM SESSION 1

PDUFA V Overview

Stephen J. Ruberg, PhD
Distinguished Research Fellow
Scientific Leader, Advanced Analytics
Global Statistical Sciences
Eli Lilly & Company

This session will discuss the key PDUFA V commitments, current plans for their implementation, areas of interest for statisticians in particular, and perspectives on the potential impact on stakeholders.

Speakers:

Theresa Mullin, PhD
Director, Office of Planning and Informatics
CDER, FDA

Jay P. Siegel, MD
Chief Biotechnology Officer
Head, Global Regulatory Affairs
Janssen Research & Development, LLC

Stephen E. Wilson, DrPH, CAPT, USPHS
Director, Division of Biometrics III
Office of Biostatistics
Office of Translational Sciences
CDER, FDA

Lisa M. LaVange, PhD
Director, Office of Biostatistics
Office of Translational Sciences
CDER, FDA

12:30 – 1:30 PM LUNCHEON

1:30 – 3:00 PM SESSION 2

Missing Data: When is it Important to Collect Data After Study Drug Discontinuation?

Session Chairpersons:

José C. Pinheiro, PhD
Senior Director, Quantitative Decision Strategies
Janssen Research and Development, LLC

John Scott, PhD
Acting Deputy Director, Division of Biostatistics
Office of Biostatistics and Epidemiology
CBER, FDA

This is an intertwined pair of sessions on missing data in clinical trials. In the first session, three expert speakers (from FDA, industry and academia) will address the questions, "When is it important to collect data after study drug discontinuation and how can it be done in practice?" The recent National Academy of Sciences panel report on The Prevention and Treatment of Missing Data in Clinical Trials recommended that sponsors continue to collect key outcome data on study participants after discontinuation of study drug, except when a cost-benefit analysis argues otherwise. However, the report also noted that the decision on continued data collection depends on the estimand and study design. The speakers in this session will discuss the whens, hows and whys of continued data collection after study drug termination.

In the second session, a case study in the collection of outcomes data after study drug termination will be presented, followed by a panel discussion involving the case study presenter, a discussant, and the three speakers from the previous session.

Speakers:

Thomas Permutt, PhD
Director, Division of Biostatistics II
Office of Biostatistics
Office of Translational Sciences
CDER, FDA

Craig H. Mallinckrodt, PhD
Research Fellow and Technical Lead
Department of Strategy and Decision Science
Eli Lilly

Roderick Little, PhD
Biostatistics Department
Statistics Department
University of Michigan

3:00 – 3:30 PM AFTERNOON BREAK

3:30 – 5:00 PM SESSION 3

Missing Data: Case Study Presentation & Panel Discussion

Session Chairpersons:

José C. Pinheiro, PhD
Senior Director, Quantitative Decision Strategies
Janssen Research and Development, LLC

John Scott, PhD
Acting Deputy Director, Division of Biostatistics
Office of Biostatistics and Epidemiology
CBER, FDA

Case Study Presenter:

Rajeshwari Sridhara, PhD
Director, Division of Biometrics V
Office of Biostatistics
CDER, FDA

Panelists:

Gary G. Koch, PhD
Director, Biometric Consulting Lab. Biostatistics
Professor, University of North Carolina at Chapel Hill

Craig H. Mallinckrodt, PhD
Research Fellow and Technical Lead
Department of Strategy and Decision Science
Eli Lilly

Thomas Permutt, PhD
Director, Division of Biostatistics (DB) II
Office of Biostatistics (OB)
Office of Translational Sciences (OTS)
CDER, FDA

Jay P. Siegel, MD
Chief Biotechnology Officer
Head, Global Regulatory Affairs
Janssen Research & Development, LLC

FORUM DAY 2 | **TUESDAY, APRIL 30**

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

8:30 – 10:00 AM SESSION 4

Statisticians as Leaders

Session Chairpersons:

Jerald S. Schindler, DrPH
Vice President, Biostatistics and Research Decision Sciences
Merck Research Laboratories
North American Co-Chair of the DIA Statistics Community

Joan Buenconsejo, PhD
Statistics Team Lead
Division of Biometrics II
Office of Biostatistics
Office of Translational Science
CDER, FDA

In the last few years, we have seen a rapid expansion in the amount of data collected and analyzed in drug development. In addition to clinical trial results we also collect pk, pharmacogenomic, comparative effectiveness, observational data and reimbursement data. This expansion of data has resulted in a wider variety of research objectives and analyses performed during drug development. As the scope has increased, the range of departments involved in quantitative analyses has also increased dramatically. Now in addition to a central Biostatistics department, Epidemiology, Modeling & Simulation, Outcomes Research, Marketing among others are engaged in these Quantitative Analyses. This has created new challenges for pharmaceutical companies. In the past most Quantitative Analyses were coordinated by a centrally located Statistics Department. In the currently evolving model, Quantitative Analyses are distributed among many departments in the pharmaceutical company. Often, these departments have a specific functional expertise but only limited Statistical expertise. This trend has begun to move the statistician in the direction of a support role. This session will explore this new trend and will also examine opportunities for statisticians to move back to a leadership role in drug development.

In this session, four leaders from regulatory agency, pharmaceutical industry and professional organization will engage in a conversation to share their thoughts and perspectives regarding these challenges. They will collectively discuss what are the stakes, the potential impact, and possible destination we can all strive for in our chosen profession. They will also engage the audience with an open dialogue about the ideal state of statistical leadership.

Panelists:

Lisa M. LaVange, PhD
Director, Office of Biostatistics
Office of Translational Sciences
CDER, FDA

Robert N. Rodriguez, PhD
Senior Director, Statistical Research and Development
Past President, American Statistical Association
SAS Institute

Jerald S. Schindler, DrPH
Vice President, Biostatistics and Research Decision Sciences
Merck Research Laboratories
North American Co-Chair of the DIA Statistics SIAC

Aarti S. Shah, PhD
Vice President, Biometrics & Advanced Analytics
Eli Lilly & Co

10:00 – 10:30 AM MORNING BREAK

10:30 – 12:00 PM SESSION 5

Dichotomizing Continuous Measures for a Primary Efficacy Endpoint – Weighing the Benefits and Risks

Session Chairpersons:

Steven M. Snapinn, PhD
Vice President, Global Biostatistical Science
Amgen Inc.

Dionne Price, PhD
Team Lead, Division of Biometrics II
Office of Biometrics
Office of Translational Sciences
CDER, FDA

In many disease areas, it is common for an endpoint measured on a continuous scale to be dichotomized prior to analysis. Typically, a subject is classified as a responder or a non-responder on the basis of a measurement taken at the end of the treatment period or based on a change from baseline; for this reason, this approach is often referred to as a responder analysis. For example, in a trial of an antihypertensive treatment, a subject might be classified as a responder if he or she achieves a diastolic blood pressure value of 80 mmHg or less. While this approach has some appeal, it also has disadvantages, most notably a substantial cost in statistical power. In this session, two speakers will outline the rationale for the approach and some of the associated statistical issues. A panel discussion will follow whereby panelists will discuss varying views of the benefits and risks of dichotomizing continuous measures.

Speakers:

Lisa A. Kammerman, PhD
Master Reviewer
Office of Biostatistics
Office of Translational Science
CDER, FDA

Qi Jiang, PhD
Executive Director
Global Biostatistical Science
Amgen, Inc.

Panelists:

Qi Jiang, PhD
Executive Director
Global Biostatistical Science
Amgen, Inc.

Lisa A. Kammerman, PhD
Master Reviewer
Office of Biostatistics
Office of Translational Science
CDER, FDA

Gary G. Koch, PhD
Director, Biometric Consulting Lab. Biostatistics
Professor, University of North Carolina at Chapel Hill

Stephen J. Ruberg, PhD
Distinguished Research Fellow
Scientific Leader, Advanced Analytics
Global Statistical Sciences
Eli Lilly & Company

Rajeshwari Sridhara, PhD
Director, Division of Biometrics V
Office of Biostatistics
CDER, FDA

12:00 – 1:30 PM LUNCHEON AND ROUNDTABLE DISCUSSIONS

Roundtable Chairpersons:

Tammy Massie, PhD
Mathematical Statistician
Division of Biostatistics
CBER, FDA

Brenda Crowe, PhD
Research Advisor
Global Statistical Sciences
Eli Lilly and Company

Carmen Mak, PhD
Manager, Statistics
Merck Research Laboratories

Topics:

1. Issues in dichotomizing a continuous variable
2. Innovative approaches for pre-licensure evaluation of safety
3. The design and analysis of meta-analysis for rare safety events
4. Practical missing data handling technique for clinical trial
5. Prevention and treatment of missing data: current practices
6. Multiplicity adjustment in confirmatory clinical trials
7. The use of freeware in FDA submissions and in further analyses
8. Effective use of table, figure, and listing in the study report of a clinical trial
9. Meta-analysis for safety data
10. Patient enrollment statistical prediction for clinical trial data monitoring
11. Using patient reported outcome (PRO) for product labels

1:30 – 3:00 PM SESSION 6

(Premarketing) Benefit-Risk Assessment in Clinical Development and Regulatory Review

Session Chairpersons:

Qi Jiang, PhD
Executive Director
Global Biostatistical Science
Amgen, Inc.

Jonathan D. Norton, PhD
DB V
Office of Biostatistics
CDER, FDA

The evaluation of new pharmaceutical products involves careful assessments of both efficacy and safety, and, based on these evaluations, a product is deemed approvable if the benefits are determined to exceed the harms (risks). In recent years there has been a growing consensus that more formal methods are needed to make this comparison. In particular, PDUFA V calls for FDA to develop a structured Benefit-risk framework. As this framework is developed and implemented, statisticians have an important role to play in ensuring that quantitative results and reasoning are appropriately incorporated. While a number of quantitative methods for Benefit-risk assessment have been proposed, none have been universally accepted by regulators. In this session we will discuss various Benefit-risk assessment approaches that can be potentially used to meet the needs of global regulatory agencies and prescribers, as well as challenges that may arise in implementing them.

Speakers:

Scott Evans, PhD
Senior Research Scientist
Harvard School of Public Health
Harvard University

Steven M. Snapinn, PhD
Vice President, Global Biostatistical Science
Amgen Inc

Panelists:

Frank Bretz, PhD
Global Head of Statistical Methodology
Novartis Pharma AG
Switzerland

Scott Evans, PhD
Senior Research Scientist
Harvard School of Public Health
Harvard University

Patrick Frey, MPP
Director, Office of Planning and Analysis
CDER, FDA

Frank W. Rockhold, PhD
Senior Vice President, Global Clinical Safety and Pharmacovigilance
GlaxoSmithKline

Steven M. Snapinn, PhD
Vice President, Global Biostatistical Science
Amgen Inc.

3:00 – 3:30 PM AFTERNOON BREAK

3:30 – 5:00 PM SESSION 7

Health Technology Assessment and Comparative Effectiveness Research: Their Impact on Access to Pharmaceutical Products and Their Role in Designing Product Development Strategies

Session Chairpersons:

Christy Chuang-Stein, PhD
Vice President, Statistics
Pfizer Inc.

Estelle Russek-Cohen, PhD
Division Director, Division of Biostatistics,
CBER, FDA

After a product received marketing authorizations, access to the product by the general public outside of the US is largely controlled by decisions from health technology assessment (HTA) agencies who determine if the product is cost-effectiveness for the indication authorized. While cost has not been a formal part of coverage decisions in health care systems supported by government in the US (e.g. Medicare, Medicaid), considerable attention has been paid to the subject of comparative effectiveness research (CER) since the Affordable Care Act was signed into law in 2010. In this session, we will look at issues associated with HTA and CER and how they need to be part of the overall considerations when designing a product development program.

Speakers:

Sally Morton, PhD
Professor and Chair, Biostatistics
Department of Biostatistics
University of Pittsburgh

Richard J. Willke, PhD
Head, Global Health Economics & Outcomes Research Acting
Head, Regional HE & OR
Global Market Access Primary Care Business Unit
Pfizer, Inc.

Chrissie Fletcher
Executive Director, Biostatistics
Amgen, Inc

5:00 – 7:00 PM NETWORKING RECEPTION AND POSTER SESSION

Poster Sessions Chairpersons:

Rima Izem, PhD
Mathematical Statistician
Division of Biometrics IV
Office of Biostatistics
CDER, FDA

Cristiana Gassmann-Mayer, PhD
Associate Director, Clinical Biostatistics
Janssen Research and Development, LLC

Zoran Antonijevic
Senior Director, Strategic Consulting and Adaptive Implementation
Cytel, Inc.

Program Committee Liaison:

Barry Schwab, PhD
Vice President, Clinical Biostatistics
Janssen Research and Development, LLC

FORUM DAY 3 | **WEDNESDAY, MAY 1**

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

8:30 – 10:00 AM SESSION 8

Companion Diagnostics

Session Chairpersons:

Keaven Anderson, PhD
Executive Director, Statistics: Late Stage Development
Merck Research Laboratories

Thomas E. Gwise, PhD
Deputy Division Director
Division of Biometrics V
Office of Biostatistics
CDER, FDA

Personalized medicine success stories such as trastuzumab and Vemurafenib require FDA approved diagnostics to describe the patients for whom the treatments are indicated. Through the presentation of several case studies and interactive audience discussion with presenters, this session will examine the drug-diagnostic co-development pathway as it has been travelled, pointing out typical bumps in the road, such as data and analysis challenges created by misaligned development schedules, and considering more complex diagnostics such as genomic or imaging systems. We hope the session will provide some possible routes around the potholes.

Speakers:

Estelle Russek-Cohen, PhD
Division Director, Division of Biostatistics,
CBER, FDA

Lisa McShane, PhD
Senior Statistician
Biometric Research Branch
Cancer Therapy Evaluation Program
National Cancer Institute

James Symanowski, PhD
Chair, Department of Cancer Biostatistics
Levine Cancer Institute
Carolinas HealthCare System

10:00 – 10:30 AM MORNING BREAK

10:30 – 12:00 PM SESSION 9

Meta Analysis for Safety Data

Session Chairpersons:

Aloka Chakavarty, PhD
Director, Division of Biometrics VII
Office of Biostatistics
CDER, FDA

Brenda Crowe, PhD
Research Advisor, Global Statistical Sciences
Eli Lilly and Company

This session will explore several aspects of meta-analysis of safety data. These include innovative ways of handling the display of risks/probabilities for multiple studies so as to avoid potentially paradoxical results, answers to frequently asked question, and information from the FDA perspective on meta-analysis.

Speakers:

Christy Chuang-Stein, PhD
Vice President, Statistics
Pfizer Inc.

Jesse A. Berlin, PhD
Vice President, Epidemiology
Janssen Research & Development LLC

Eugenio Andraca-Carrera, PhD
Mathematical Statistician
Office of Biostatistics
Office of Translational Science
CDER, FDA

12:00 – 1:00 PM NETWORKING AND LUNCHEON

1:00 – 2:30 PM SESSION 10

Pre-Competitive Collaboration

Session Chairpersons:

Frank W. Rockhold, PhD
Senior Vice President, Global Clinical Safety and Pharmacovigilance
GlaxoSmithKline

Mat Soukup, PhD
Team Leader, Division of Biometrics VII
Office of Biostatistics
Office of Translational Sciences
CDER, FDA

There is a new dawn in pharmaceutical and biotech drug development where data and knowledge is shared openly, sometimes with specific non-profit organizations, and sometimes fully publicly. This session will address the challenges and risks in this movement.

Speakers:

Frank W. Rockhold, PhD
Senior Vice President, Global Clinical Safety and Pharmacovigilance
GlaxoSmithKline

Lynn Hudson, PhD
Chief Science Officer
Critical Path Institute

Susan K. McCune, MD
Deputy Director
Office of Translational Sciences
CDER, FDA

2:30 – 3:00 PM AFTERNOON BREAK

3:00 – 4:30 PM SESSION 11

Overview and Commentary of FDA Guidance: Enriched Populations

Session Co-Chairpersons:

Walter W. Offen, PhD
Global Head of Statistical Innovation
Data and Statistical Sciences
AbbVie

Mohamed A. Alosh
Team Leader
Office of Biostatistics
CDER, FDA

This session will consist of key contributors from FDA to the recent FDA Guidances to present highlights of the guidances, along with areas for which they would particularly like feedback. The industry speaker will provide key suggestions and comments on the draft guidances. And finally, the academic speaker will serve as a discussant offering their viewpoint.

Moderator:

Walter W. Offen, PhD
Global Head of Statistical Innovation
Data and Statistical Sciences
AbbVie

Panelists:

Frank Bretz, PhD
Global Head of Statistical Methodology
Novartis Pharma AG
Switzerland

Kathleen S. Fritsch, PhD
Mathematical Statistician
Office of Translational Sciences
CDER, FDA

Gary G. Koch, PhD
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DIA/FDA Statistics Forum 2013

Event #13008 • Statistics Forum: April 29-May 1 • Tutorials: April 28

Bethesda North Marriott Hotel & Conference Center, Bethesda, MD

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CANCELLATION POLICY: Two weeks before the start of the event

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