DIA/FDA Statistics Forum

Short Courses: April 24 | Forum: April 24-26
Bethesda North Marriott Hotel and Conference Center

PROGRAM CO-CHAIRS

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Director, Division of Biometrics IV, Office of Biostatistics, Office of Translational Sciences
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Nevine Zariffa
Vice President and Head, Biometrics and Information Sciences
AstraZeneca Pharmaceuticals

"As industry statisticians, we can be close to FDA and discuss the current issues."
– 2016 Attendee

Overview

How do we respond to the ever-changing landscape in drug development? This question and many others related to real-world application of advanced approaches and solutions for statistical challenges surrounding the design and analysis of clinical trials will be collaboratively explored at the DIA/FDA Statistics Forum. Sessions will focus on regulatory science initiatives and the statistical methodologies and quantitative approaches necessary for providing evidence of the efficacy and safety of new therapies.

Highlights

- Keynote Addresses on Monday, April 24

Janet Woodcock, MD
Director, CDER, FDA

Kenneth I Kaitin, PhD
Professor of Medicine, Director, Tufts Center for the Study of Drug Development, Tufts University School of Medicine

- Town Hall: An expert panel of regulatory and industry thought leaders will engage with the audience in a fully open forum to discuss live questions as well as those collected throughout the meeting
- Each session Co-Chaired by an Industry/FDA team
- Luncheon Round Table Discussions with Key Thought Leaders
- Poster Presentations

Who Should Attend

Professionals from industry, academia, and government involved in all phases of medical product development who are interested in learning the latest state of the art techniques for pharmaceutical development:

- Biostatisticians
- Physicians
- Clinical Pharmacologists
- Health Economists
- Epidemiologists
- Regulatory Scientists

Message from Program Committee

Dear Colleagues,

It is a pleasure to welcome you to the DIA/FDA Statistics Forum!

This forum is unique in setting the stage for an open, collaborative discussion of important topics related to statistics in drug development among industry, academia, nonprofit, and regulatory agencies.

Lead by key thought leaders, the 2017 agenda encompasses accepted new concepts and approaches and the exploration of emerging ideas. Opportunities for cross-functional communication with related biopharmaceutical disciplines, such as pharmacology, clinical research, pharmacovigilance, or health outcomes research, that interface with statistics in practice have been meticulously placed to inspire important dialogue necessary for effectively providing evidence of the efficacy and safety of new therapies.

It is with great enthusiasm that we announce our Keynote Speakers: Janet Woodcock, MD, Director, Center for Drug Evaluation and Research, FDA, and Kenneth I Kaitin, PhD, Professor of Medicine; Director, Tufts Center for the Study of Drug Development, Tufts University School of Medicine.

We hope you take advantage of the many opportunities to actively engage in discussions and with each other. Be sure to join us Tuesday during the luncheon for our popular Round Table Discussions and in the evening for the Networking Reception.

Best Regards,
The DIA/FDA Statistics Forum Program Committee

Schedule At-A-Glance

DAY ONE | MONDAY, APRIL 24		
7:30–11:30AM	Short Course Registration	Glen Foyer, Lower Level
8:30AM–12:00PM	**Short Course 1:** New Graphical Methods for Clinical Trials	Forest Glen, Lower Level
8:30AM–12:00PM	**Short Course 2:** Assessing Biosimilarity and Interchangeability: Issues and Recent Developments	Glen Echo, Lower Level
11:00AM–6:00PM	Main Forum Registration	Grand Ballroom Salon D Foyer, Main Level
1:00–1:30PM	**Welcome and Opening Remarks**	Grand Ballroom Salon D, Main Level
1:30–2:30PM	**Keynote Addresses**	Grand Ballroom Salon D, Main Level
2:30–3:45PM	**Session 1:** The 21st Century Cures Act and PDUFA VI: Highlights and Impact	Grand Ballroom Salon D, Main Level
3:45–4:05PM	Refreshment and Networking Break	Grand Ballroom Salon D, Main Level
4:05–5:20PM	**Session 2:** Prescription Opioid Abuse Epidemic: Regulatory Actions and Quantitative Evaluation	Grand Ballroom Salon D, Main Level
5:30–6:30PM	DIA Statistics Community – Open Meeting	Forest Glen, Lower Level
DAY TWO | TUESDAY, APRIL 25		
7:30AM–7:00PM	Registration	Grand Ballroom Salon D Foyer, Main Level
7:30–8:30AM	Continental Breakfast	Grand Ballroom Salon D Foyer, Main Level
8:30–8:35AM	**Welcome to Day 2**	Grand Ballroom Salon D, Main Level
8:35–10:05AM	**Session 3:** Alternative Data Sources: Analytical Challenges and Opportunities for Statisticians to	Grand Ballroom Salon D, Main Level
10:05–10:30AM	Refreshment Break and Networking	Grand Ballroom Salon D, Main Level
10:30AM–12:00PM	**Session 4:** Synthesis of Real-World Evidence and Randomized Controlled Trials	Grand Ballroom Salon D, Main Level
12:00–1:30PM	Luncheon and Round Table Discussions	White Oak, Lower Level
1:30–3:00PM	**Session 5:** Statistical Innovation in Antimicrobial Drug Development	Grand Ballroom Salon D, Main Level
3:00–3:30PM	Refreshment Break and Networking	Grand Ballroom Salon D, Main Level
3:30–5:00PM	**Session 6:** Bridging Statistics and Pharmacometrics: Informing Drug Development Decisions	Grand Ballroom Salon D, Main Level
5:00–6:00PM	Networking Reception and Poster Session	Grand Ballroom Salon D Foyer, Main Level
DAY THREE | WEDNESDAY, APRIL 26		
7:30AM–3:00PM	Registration	Grand Ballroom Salon D Foyer, Main Level
7:30–8:30AM	Continental Breakfast	Grand Ballroom Salon D Foyer, Main Level
8:30–8:35AM	**Welcome to Day Three**	Grand Ballroom Salon D, Main Level
8:35–10:05AM	**Session 7:** Statistical Challenges and Opportunities in Drug Development with Immunotherapy	Grand Ballroom Salon D, Main Level
10:05–10:30AM	Refreshment Break and Networking	Grand Ballroom Salon D, Main Level
10:30AM–12:00PM	**Session 8:** Novel Statistical Methods in Oncology Studies	Grand Ballroom Salon D, Main Level
12:00–1:30PM	Luncheon	White Oak, Lower Level
1:30–2:45PM	**Session 9:** 21st Century Communication for Medical Product Development: Getting Better and Better	Grand Ballroom Salon D, Main Level
2:45–4:00PM	**Session 10:** Town Hall	Grand Ballroom Salon D, Main Level

Learning objectives

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

- Assess the impact of 21st Century Cures and PDUFA VI on statistical practice
- Introduce statistical solutions to assess the efficacy, safety, and abuse liability of opioids
- Discuss recent statistical and clinical trial developments in oncology
- Describe opportunities for statistical innovation in antimicrobial drug development
- Outline trends in modeling and decision support in drug development
- Identify non-traditional data sources and discuss their role in drug development
- Discuss the synthesis of real-world evidence and randomized controlled clinical trials
- Propose recommendations for improving inter-stakeholder communication about complex, multi-dimensional issues in developing new therapeutics
- Summarize the latest trends and updates from regulatory agencies

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Short Course 1 ..... 0.3
Short Course 2 ..... 0.3
Forum 1.6

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DAY ONE | MONDAY, APRIL 24

7:30AM-6:00PM — **Registration**

8:30AM-12:00PM

Short Course 1
New Graphical Methods for Clinical Trials

Instructor
Frank E. Harrell Jr., PhD
Professor and Chairman
School of Medicine, Vanderbilt University

In the never-ending quest to replace tables with graphics, new graphics solutions to common data display problems in clinical trials are becoming more readily available. This short course will focus on high-information graphics that faithfully convey characteristics of data and summary statistics using such tools as extended box plots, dot charts, and spike histograms. With the rapid evolution of HTML5 and html notebooks, new possibilities now exist, and graphics can be less cluttered with more information made available by merely hovering with the mouse or clicking the legend to activate the display of additional data layers.

Learning Objectives
At the conclusion of this short course, participants should be able to:

- Discuss principles of graph construction
- Determine which features of summary statistics should be emphasized in a graph
- Recognize examples of increasing information using modern graphics, whether static or interactive
- Understand features of html notebooks for statistical reports
- Demonstrate how to use RStudio to make html notebooks
- Exercise new functions in the R Hmisc package which use the R plotly package to produce somewhat interactive graphics
- Generate ideas for constructing your own interactive graphics for statistical reports by seeing examples of placing supplemental information in initially hidden layers of graphics

8:30AM-12:00PM

Short Course 2
Assessing Biosimilarity and Interchangeability: Issues and Recent Developments

Instructor
Shein-Chung Chow, PhD
Professor, Department of Biostatistics and Bioinformatics
Duke University School of Medicine

Biological drugs are much more complicated than chemically synthesized, small molecule drugs. Consequently, the assessment of biosimilarity and interchangeability calls for greater circumspection than the evaluation of bioequivalence. The FDA recommends the use of stepwise approach for obtaining totality-of-the-evidence for demonstration of biosimilarity and interchangeability. The stepwise approach involves analytical similarity assessment, animal studies for toxicity, pharmacokinetic and pharmacodynamics (PK/PD) studies for pharmacological activities, and clinical studies including immunogenicity for safety, tolerability, and efficacy. The present communication discusses some current issues and recent development related to the assessment of biosimilarity and interchangeability of biosimilar products.

Current issues to be discussed include:

1. Biosimilar versus biobetter
2. How many biosimilar studies are required
3. Multiple reference products
4. Criteria for highly variable drug products
5. Development of biosimilarity index

Learning Objectives
At the conclusion of this short course, participants should be able to:

- Develop a clear understanding regarding the concepts of biosimilarity and interchangeability for biosimilar drug development
- Focus statistical methods including sample size requirement and data analysis for demonstrating biosimilarity and interchangeability
- Discuss current issues and FDA's current thinking and recommendations regarding the assessment of biosimilarity and interchangeability
- Explain recent developments including regulatory guidance and statistical methodology development

DAY ONE | MONDAY, APRIL 24

1:00-1:30PM

Welcome and Opening Remarks

Dionne Price, PhD
Director, Division of Biometrics IV, Office of Biostatistics, Office of Translational Sciences
CDER, FDA

Sudip Parikh, PhD
Senior Vice President and Managing Director, DIA Americas
DIA

Jerald S. Schindler, DrPH
Vice President, Data Science and Statistics, Alnylam Pharmaceuticals and Adjunct Professor, Harvard Chan School of Public Health

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

1:30-2:30PM

Keynote Addresses

Session Co-Chairs

Dionne Price, PhD
Director, Division of Biometrics IV, Office of Biostatistics, Office of Translational Sciences
CDER, FDA

Jerald S. Schindler, DrPH
Vice President, Data Science and Statistics, Alnylam Pharmaceuticals and
Adjunct Professor, Harvard Chan School of Public Health

The 21st Century Vision for New Drug Review at CDER and the Role of Statisticians

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

The Times They Are A-Changin': How Economic Demands and Drug Development Challenges are Altering the Face of Pharmaceutical R&D

Kenneth I Kaitin, PhD
Professor of Medicine, Director, Tufts Center for the Study of Drug Development
Tufts University School of Medicine

2:30-3:45PM

Session 1

The 21st Century Cures Act and PDUFA VI: Highlights and Impact

Session Co-Chairs

Dionne Price, PhD
Director, Division of Biometrics IV, Office of Biostatistics, Office of Translational Sciences
CDER, FDA

Jerald S. Schindler, DrPH
Vice President, Data Science and Statistics, Alnylam Pharmaceuticals and Adjunct Professor, Harvard Chan School of Public Health

The 21st Century Cures Act was signed into law December 13, 2016 and the PDUFA VI proposed commitment letter rests with Congressional authorizing committees. The spirit of both legislative documents seeks to enhance the development and approval of drugs and biologics for the benefit of public health. In this session, overviews of the 21st Century Cures Act and PDUFA VI will be presented. The overviews will be germane in understanding the role of statisticians in the implementation of the legislation.

Learning Objectives

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

- Describe key components of the 21st Century Cures Act and PDUFA VI
- Assess the impact of the 21st Century Cures Act and PDUFA VI on statistical practice

21st Century Cures: Highlights and Expectations

Gregory Daniel, PhD
Deputy Director, Duke Robert J. Margolis, MD, Center for Health Policy; Clinical Professor, Fuqua School of Business

PDUFA VI: Highlights and Expectations

Theresa M Mullin, PhD
Director, Office of Strategic Programs
CDER, FDA

3:45-4:05PM

Refreshments and Networking Break

4:05-5:20PM

Session 2
Prescription Opioid Abuse Epidemic: Regulatory Actions and Quantitative Evaluation

Session Co-Chairs
William Wang, PhD
Executive Director, Clinical Safety Statistics, Biostatistics and Research Decision Sciences (BARDS)
Merck Research Laboratories

Mark S. Levenson, PhD
Director, Division of Biometrics VII, Office of Biostatistics
CDER, FDA

The prescription opioid abuse epidemic has grown in scope and has become a major public health concern. The FDA has developed an action plan to address this epidemic. The plan includes new professional labeling, postmarketing studies to measure and understand abuse, promotion of abuse deterrent formulations, and Risk Evaluation and Mitigation Strategies. This session provides an overview of the prescription opioid epidemic and future FDA actions. It then considers the unique challenges to evaluate the actions including the use of novel data sources and quantitative methods.

Learning Objectives
At the conclusion of this session, participants should be able to:
- Understand the unique challenges to measuring and understanding the prescription opioid epidemic
- Explain the use of novel data sources and quantitative methods

Overview of the Prescription Opioid Epidemic and the FDA Activities to Address It
Judy Staffa, PhD, RPh
Associate Director for Public Health Initiatives, Office of Surveillance and Epidemiology
CDER, FDA

Epidemiologist Perspective on the Prescription Opioid Abuse Epidemic
Scott Novak, PhD
Director of Prescription Drug Research
Battelle Memorial Institute

Estimating Prescription Opioid Abuse and Assessing Causality: Statistical Challenges
Kunthel By, PhD
Senior Statistical Reviewer, Office of Biostatistics
CDER, FDA

5:30-6:30PM

DIA Statistics Community – Open Meeting

DAY TWO | TUESDAY, APRIL 25

7:30AM-6:00PM	**Registration**
7:30-8:30AM	**Continental Breakfast and Networking**
8:30-8:35AM	**Welcome to Day Two** **Dionne Price, PhD** Director, Division of Biometrics IV, Office of Biostatistics, Office of Translational Sciences CDER, FDA
8:35-10:05AM	**Session 3** Alternative Data Sources: Analytical Challenges and Opportunities for Statisticians to Take the Lead **Session Co-Chairs** **Pandurang M. Kulkarni, PhD** Vice President, Global Biometrics and Advanced Analytics Eli Lilly and Company **Laura Lee Johnson, PhD** Deputy Director, Office of Biostatistics, DB III, Office of Translational Sciences CDER, FDA Based on published research, 90 percent of data in the world has been created in just the last two years. This growth is expected to continue at a rate of 40 percent each year. Health care data is a major contributor to this growth, accelerating at a phenomenal rate of 48 percent every year. New technologies also accelerate this speed, such as electronic patient reported outcomes (ePROs), wearable devices, social media, and more. Key challenges are two fold: one is collecting, integrating, and making the data available quickly to be able to draw insights from the data. The other is how to address the variability, and how to make sense of mass data being generated by doctors, researchers, patients, and hospitals, so we can improve patients' care. These challenges provide opportunities for improvements of current data storage/transfer infrastructure, analyzing methods, and a way to communicate the timely results. In this session, our distinguished speakers will provide their visionary insights and their recent works around these areas. **Learning Obectives** At the conclusion of this session, participants should be able to: • Understand data, technology, and analytics methods to address your health care questions with data from alternative sources **Predicting Health Metrics with Connected Glucose Monitoring** **Jesse Bridgewater, PhD** Vice President, Data Science Livongo **From Logbooks to Closed Loop Systems – How Data is Changing Diabetes Care** **Marie Schiller** Vice President, Drug Delivery/Device R&D Innovation Lilly and Company **Regulatory Perspectives on Alternative Data Sources** **David M. Petullo, MS** Mathematical Statistician CDER, FDA
10:05-10:30AM	**Refreshments and Networking Break**

10:30AM-12:00PM

Session 4

Synthesis of Real World Evidence and Randomized Controlled Trials

Session Co-Chairs

Amy Xia, PhD
Executive Director, Biostatistics
Amgen, Inc

Rima Izem, PhD
Lead Mathematical Statistician, Office of Translational Science
CDER, FDA

While randomized controlled trials (RCTs) continue to have an established place in the realm of evidence generation, real world evidence (RWE) has drawn increasing attention in the development of medical products. This session will review and discuss supplementing or integrating evidence from RCTs with RWE in the regulatory assessment of risk and benefit of drugs. Additionally, it will delve into the design and analysis of pragmatic clinical trials (PCT), as well as the strengths, limitations, and key issues relative to PCT. Speakers and panelists will discuss opportunities and challenges with access to RWE, design of PCT, and the future of clinical trials.

Learning Objectives

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

- Describe benefits and methods for integrating RWE and clinical trials such as using drug use information to improve clinical trial design, design and analysis of pragmatic trials, or re-assessing benefit-risk in user population postmarketing
- Discuss challenges and limitations in access and use of RWE

Where the Sidewalk Ends: Integrating RCTs, Meta-Analysis, and Observational Studies – And Not Just for Safety

Jesse Berlin, ScD
Senior Vice President, Epidemiology
Johnson & Johnson

Pragmatic Trials: A Compromise Between Traditional Clinical Trials and Observational Studies

Elizabeth DeLong, PhD
Chair, Biostatistics and Bioinformatics
Duke University

Panel Discussion

Joining session speakers

Brian Bradbury, PhD
Executive Director, Center for Observational Research
Amgen, Inc.

David Barrett Martin, MD, MPH
Liaison to the Reagan-Udall Foundation IMEDS Program, OMP
CDER, FDA

12:00-1:30PM

Luncheon and Round Table Discussions

Session Co-Chairs

Cristiana Mayer, PhD
Scientific Director, Statistical Modeling and Methodology, Statistics and Decision Sciences
Janssen Research and Development LLC

Annie Lin, PhD
Mathematical Statisitician
CBER, FDA

Mouna Akacha
Statistical Methodologist
Novartis Pharma AG, Switzerland

Shyla Jagannatha, PhD
Scientific Director
Janssen Research and Development LLC

1:30-3:00PM

Session 5
Statistical Innovation in Antimicrobial Drug Development

Session Co-Chairs

Amy Xia, PhD
Executive Director, Biostatistics
Amgen, Inc

Dionne Price, PhD
Director, Division of Biometrics IV,
Office of Biostatistics, Office of Translational Sciences
CDER, FDA

Antimicrobial drug development encompasses treatments for viruses and bacterial infections. Viruses, such as Ebola and Zika, often pose an immediate public health threat and unique clinical trial challenges. An equally concerning risk to public health is the simulataneous increase in the number of bacteria that are highly resistant to available antibiotics and lack of new antibiotics in drug development. The 21st Century Cures Act includes legislation pertaining to novel clinical trial designs as well as antimicrobial innovation and stewardship. In this session, the landscape of antimicrobial drug development will be reviewed and two innovative Bayesian adaptive designs will be presented.

Learning Objectives

At the conclusion of this session, participants should be able to:
- Describe unique challenges associated with antimicrobial drug development
- Identify opportunities for statistical innovation in antimicrobial drug development
- Discuss Bayesian adaptive design case studies

Landscape of Antimicrobial Drug Development

John Farley, MD
Deputy Director, Office of Antimicrobial Products
FDA

An Innovative Trial Design and Analysis Undertaken During the Ebola Crisis

Michael Proschan, PhD
Mathematical Statistician
National Institutes of Health

The ADAPT Platform Trial Design for Innovative Antibiotic Development

Kert Viele, PhD
Director and Senior Statistical Scientist
Berry Consultants

Panel Discussion

Joining session speakers

Telba Irony, PhD
Deputy Director, Office of Biostatistics
and Epidemiology
CBER, FDA

3:00-3:30PM

Refreshments and Networking Break

3:30-5:00PM

Session 6

Bridging Statistics and Pharmacometrics: Informing Drug Development Decisions Through Model-Based Approaches

Session Co-Chairs

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

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

Clinical drug development remains a mostly empirical, costly enterprise, in which decision-making is often based on qualitative assessment of risk-benefit, without systematically leveraging all the relevant data collected throughout the development program. Quantification of risk and combination of information from different sources across time using model-based approaches may modernize drug research towards a better informed drug development process. Such a shift in paradigm requires a close collaboration between biostatisticians and pharmacometricians. These disciplines share many common quantitative elements and are much more specialized and offer many capabilities that each individual discipline does not have. Speakers will share their experiences on opportunities and challenges when fostering the new model-based drug development paradigm in their organizations.

Moderator
Joan Buenconsejo, PhD
Statistics Team Leader, Inflammation, Neuroscience and Respiratory TA, B&I Science
AstraZeneca Pharmaceuticals LP

Introduction to Model Informed Drug Discovery and Development (MID3) Good Practice Guidelines: Current Status and Challenges

Peter A. Milligan, PhD
Head of Pharmacometrics
Pfizer Global Research & Development, United Kingdom

Structural Approach to Pediatric Extrapolation Through Bayesian Modeling

Margaret Gamalo, PhD
Statistical Scientist
Eli Lilly and Company

Application of Semi-Mechanistic Pharmacokinetic-Pharmacodynamic Model in Dose Selection for Medical Countermeasures Initiative Product Developed Under Animal Rule: Case Study of Pegfilgrastim

Lian Ma, PhD
Pharmacometrics Reviewer
CDER, FDA

An Extrapolation Approach for Pediatric Development in Transplantation

Eric Gibson, PhD
Vice President, Global Head Biostatistical Sciences and Pharmacometrics
Novartis Pharmaceuticals Corporation

5:00-6:00PM

Networking Reception and Poster Session

DAY THREE | WEDNESDAY, APRIL 26

7:30AM-4:00PM	**Registration**
7:30-8:30AM	**Continental Breakfast and Networking**
8:30-8:35AM	**Welcome to Day Three** **Dionne Price, PhD** Director, Division of Biometrics IV, Office of Biostatistics, Office of Translational Sciences CDER, FDA
8:35-10:05AM	**Session 7** Statistical Challenges and Opportunities in Drug Development with Immunotherapy Oncology Studies **Session Co-Chairs** **Jeff Maca, PhD** Senior Director, Biostatistics; Advisory Services Analytics Quintiles **Rajeshwari Sridhara , PhD** Director, Division of Biometric V, Office of Biostatistics, Office of Translational Sciences CDER, FDA Novel immunotherapies have recently been developed which have provided alternative and often more beneficial treatments for many types of cancers. Key features of these products include initial delay in treatment effect and relative change in time to disease progression measured radiologically not associated with overall survival between treatment arms. However, it is not clear how these delays and relationships will change when these immunotherapies are combined with other targeted or chemotherapy. Careful planning and design considerations of clinical trials are essential to accommodate the changing landscape including treatment for rare cancers. In this session we will discuss some of the statistical challenges in the development of immunotherapies encountered so far and expected in the future. **Learning Objectives** At the conclusion of this session, participants should be able to: • Understand the statistical considerations for combinations studies • Describe the issues with a delayed treatment effect often seen in immunotherapy studies • Design optimal interim analysis plans for immunosuppressive studies **Insights from a CrossPharma Working Group on Non-Proportional Hazards in Oncology Trials** **Renee Iacona, PhD, MPH** TA Biometrics Head, Oncology and Immuno-Oncology AstraZeneca Pharmaceuticals LP **Designing Immunotherapy Trials with Delayed Treatment Effect** **Zhenzhen Xu, PhD** Mathematical Statistician CBER, FDA **Statistical Considerations for Immunotherapy Trials** **Sumithra J. Mandrekar, PhD** Biostatistician Mayo Clinic
10:05-10:30AM	**Refreshments and Networking Break**

10:30AM-12:00PM

Session 8
Novel Statistical Methods in Oncology Studies

Session Co-Chairs

Jeff Maca, PhD
Senior Director, Biostatistics; Advisory Services Analytics
Quintiles

Rajeshwari Sridhara, PhD
Director, Division of Biometric V, Office of Biostatistics, Office of Translational Sciences
CDER, FDA

With the development novel targeted agents and immunotherapy, the clinical trial designs and the statistical considerations that can achieve the objectives to demonstrate the treatment effects are critical. The advances in science provide an opportunity to target the population that benefits most with least risk. This has led to development of adaptive enrichment design strategies with more complex trial designs and a comprehensive trial network that can answer multiple objectives with multiple outcomes. Such protocols need careful advance planning with often little or minimal prior information and process has to be adaptive to fast changing environment with the development of novel therapies and drug approvals. In this session we will discuss features of such novel designs.

Learning Objectives

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

- Explain statistical issues arising in oncology studies
- Understand the impact of biomarkers and their use in oncology studies
- Discuss advantages and disadvantages of Umbrella or basket trials

Issues in Clinical Trial Design for Late-Stage Immuno-Oncology Trials

Keaven Anderson, PhD
Distinguished Scientist, Late Development
Merck Research Laboratories

Trial Design Considerations in the Lung-MAP Trial: A Biomarker-Driven Master Protocol in Lung Cancer

Mary Redman, PhD
Lead Biostatistician, SWOG Lung Committee/LungMap,
Associate Member, Clinical Research Division
Fred Hutchinson Cancer Research Center

Regulatory Experience in Innovative Trial Designs

Kun He, PhD
Associate Director, Division of Biometrics V
CDER, FDA

12:00-1:30PM

Luncheon and Networking

DAY THREE | WEDNESDAY, APRIL 26

1:30-2:45PM

Session 9
21st Century Communication for Medical Product Development: Getting Better and Better

Session Co-Chairs
Cristiana Mayer, PhD
Scientific Director, Statistical Modeling and Methodology, Statistics and Decision Sciences
Janssen Research and Development LLC

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

The 21st Century Cures Discussion Document states that "FDA's current rules and policies governing what drug and device developers may say about their own products were designed decades ago. Since then, the way that medicine is practiced and delivered and the way that information is communicated have fundamentally changed."

We need to talk about science, guidance, clinical trial design, regulatory experience, submissions, data standards, and analysis. We need to make the right decisions. We need to know where we are and we need to get better. This is our opportunity!

Learning Objectives
At the conclusion of this session, participants should be able to:
- Become familiar with multiple aspects of scientific and regulatory communication with different audiences involved in drug development

Panelists
Frank Rockhold, PhD, ScM
Professor of Biostatistics
Duke Clinical Research Institute, Duke University Medical Center

Vladimir Dragalin, PhD
Vice President, Head of QS Consulting, Quantitative Science
Janssen R&D, at Johnson & Johnson

Frank E. Harrell Jr., PhD
Professor and Chairman
School of Medicine, Vanderbilt University

John Scott, PhD
Acting Director, Division of Biostatistics, OBE
CBER, FDA

2:45-4:00PM

Session 10
Town Hall

Session Co-Chairs
Jerald S. Schindler, DrPH
Vice President, Data Science and Statistics, Alnylam Pharmaceuticals and Adjunct Professor
Harvard Chan School of Public Health

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

Welcome to the Town Hall! Dedicated to sharing the latest information on new guidances, this session will allow open discussion between you and an esteemed panel of regulatory and industry experts. Questions asked may focus on the sessions held during this forum, but can also branch out into other areas of regulatory statistics. Panelists will address questions live from the Town Hall as well as those submitted throughout the forum.

Panelists
John Scott, PhD
Acting Director, Division of Biostatistics, OBE
CBER, FDA

Rajeshwari Sridhara, PhD
Director, Division of Biometric V, Office of Biostatistics, Office of Translational Sciences
CDER, FDA

Pandurang M. Kulkarni, PhD
Vice President, Global Biometrics and Advanced Analytics
Eli Lilly and Company

Aloka Chakravarty, PhD
Director, Division of Biometrics VII, Office of Biostatistics, Office of Translational Sciences
CDER, FDA

4:00PM

Forum Adjourned