CDER - JHU CERSI Workshop

Addressing Challenges in the Design and Analysis of Rare Disease Clinical Trials: Considerations and Tools

May 2-3, 2023

Participant Biographies

Day 2: Design and Analysis Methods for Clinical Trials for Rare Diseases
May 3, 2023

Welcome

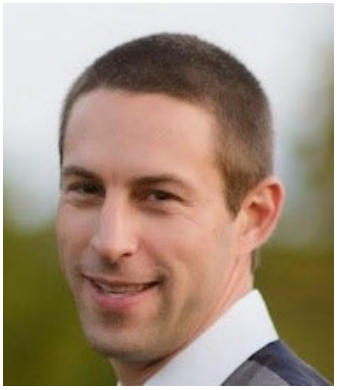
Dionne Price, PhD
Deputy Director, Office of Biostatistics
Office of Translational Sciences, Center for Drug Evaluation and Research, FDA

Dionne Price is the Deputy Director of the Office of Biostatistics in the Office of Translational Sciences, Center for Drug Evaluation and Research, FDA. In this role, Dr. Price provides leadership to statisticians involved in the development and application of methodology used in the regulation of drug products. She currently leads cross-cutting, collaborative efforts across FDA to advance and facilitate the use of innovative trial designs in pharmaceutical drug development. Dr. Price received her MS in Biostatistics from the University of North Carolina at Chapel Hill and a PhD in Biostatistics from Emory University. Dr. Price is an active member of the American Statistical Association (ASA) and the Eastern North American Region of the International Biometrics Society. She is a Fellow of the ASA and the 2023 President of the ASA.

Session 1 Participants

Kelley Kidwell, PhD
Associate Professor & Associate Chair for Academic Affairs in Biostatistics
School of Public Health, University of Michigan

Kelley M. Kidwell is an Associate Professor and the Associate Chair of Academic Affairs of Biostatistics at the University of Michigan (UM) with extensive experience in the design and analysis of clinical trials. Her particular expertise is in sequential, multiple assignment, randomized trials (SMARTs) and more novel trial designs. Over the past ten years at the UM, she has collaborated across a wide variety of fields, including oncology,mental health, and rare and chronic diseases. She has been the principal investigator of two Patient Centered Outcomes Research Initiative (PCORI)-funded contracts developing methods for SMARTs applied to rare diseases and incorporating patient preferences and a Food and Drug Administration (FDA) Broad Agency Announcement contract developing design and methods for small sample SMART designs and is on the Clinical Trial Advisory Board of PCORI. She has served as a co-investigator on many NIH- and foundation-funded projects, including 7 NIH-funded SMART designs, and has over 130 publications across statistical and medical journals and two book chapters about SMART designs.



Greg Levin, PhD
Associate Director for Statistical Science and Policy
Office of Biostatistics
Center for Drug Evaluation and Research, FDA

Gregory Levin is the Associate Director for Statistical Science and Policy in the Office of Biostatistics in the FDA's Center for Drug Evaluation and Research. He received a Ph.D. in biostatistics from the University of Washington in 2012. Greg has experience supporting drug review across a wide range of therapeutic areas and has represented CDER on several policy and guidance working groups, including efforts related to adaptive design, master protocols, benefit-risk, and the evaluation of effectiveness.

Michael Rosenblum, PhD
Professor of Biostatistics
Johns Hopkins Bloomberg School of Public Health

Michael's research is in causal inference, the design and analysis of clinical trials, evaluating accuracy of forensic methods, and enhancing capacity in low and middle income countries in statistical methods for clinical trial design and analysis. He is a Fellow of the American Statistical Association.

Noah Simon, PhD
Associate Professor, Department of Biostatistics
University of Washington

Dr. Simon is an Associate Professor in the department of biostatistics at the University of Washington. He is also an investigator for the Therapeutics Development Network at Seattle Children's Hospital; and an affiliate investigator at Kaiser Permanente Washington Health Research Institute. His research interests include biomarker development, clinical trial design, and machine learning (though rarely all together). He primarily engages with trials in oncology and cystic fibrosis.

Nigel Stallard, MSc, PhD
Professor of Medical Statistics
Warwick Medical School

Nigel Stallard is Professor of Medical Statistics and Deputy Director of the Clinical Trials Unit at Warwick Medical School in the UK, and is an Editor-in-Chief of the journal Statistics in Medicine. He has a first degree in Mathematics from the University of Cambridge and an MSc and PhD in Applied Statistics from the University of Reading, where he worked for ten years until moving to Warwick in 2005. His primary research interests are in the statistical design and analysis of clinical trials. In particular, he has worked on optimal design for clinical trials in rare diseases and small populations and on methodology for trials with interim analyses and adaptations such as treatment or subgroup selection, publishing 200 peer reviewed articles in methodological and medical journals.

Session 2 Participants

Frank Harrell, PhD
Expert Biostatistics Advisor, CDER
Professor of Biostatistics, Vanderbilt University

Dr. Harrell received his PhD in Biostatistics from UNC in 1979. Since 2003 he has been Professor of Biostatistics, Vanderbilt University School of Medicine, and was the department chairman from 2003-2017. He is Expert Biostatistics Advisor to FDA CDER and was Expert Biostatistics Advisor for the Office of Biostatistics for FDA CDER from 2016-2020. He is Associate Editor of Statistics in Medicine, and a member of the Scientific Advisory Board for Science Translational Medicine. He is a Fellow of the American Statistical Association and winner of the Association's WJ Dixon Award for Excellence in Statistical Consulting for 2014. His specialties are development of accurate prognostic and diagnostic models, model validation, clinical trials, observational clinical research, cardiovascular research, technology evaluation, pharmaceutical safety, Bayesian methods, quantifying predictive accuracy, missing data imputation, and statistical graphics and reporting.

Rima Izem, PhD
Director of Statistical Methodology Group in Analytics
Novartis Pharma AG

Dr. Rima Izem is a Director in the Statistical Methodology group in Novartis where she supports the use of best practice methodologies as well as development and implementation of novel statistical methods using real-world data or hybrid designs in all phases of clinical development and across therapeutic areas. Her research experience includes pioneering work in regulatory statistics using causal inference for comparative safety, signal detection, and survey research at the US FDA. Her methodological experience also includes comparative effectiveness in rare diseases at Children's National Research Institute, and dimension reduction methods at Harvard University. Her experience with real-world data includes work with US insurance claims databases and electronic healthcare data, international registries, and electronic clinical outcome assessments

J. Jack Lee, PhD
Professor, Department of Biostatistics
University of Texas MD Anderson Cancer Center

J. Jack Lee, Ph.D. is Professor of Biostatistics and Kenedy Foundation Chair in Cancer Research. His areas of statistical research include design and analysis of clinical trials, Bayesian adaptive designs, statistical computation/graphics, drug combination studies, and biomarkers identification and validation. He is an elected Fellow of American Statistical Association, Society for Clinical Trials, and American Association for the Advancement of Science. He has more than 500 publications in statistical and medical journals. He co-authored two books entitled: "Bayesian Adaptive Methods for Clinical Trials" and "Model-Assisted Bayesian Designs for Dose Finding and Optimization: Methods and Applications."

Karen L. Price, PhD
Associate Vice President and Statistical Officer, Statistical Innovation Center
Eli Lilly & Company

Karen Price received her Ph.D. in Statistics from Baylor University in 2001, and joined Lilly at that time. She is Associate Vice President and Statistical Officer for the Statistical Innovation Center, a team focused on innovative design and analysis of clinical trials. Her research interests include Bayesian methods, innovative clinical trials, and quantitative decision making. She previously led the DIA Bayesian Scientific Working Group, and currently serves as past-chair. Karen is a Fellow of the American Statistical Association