FDA CDER – Johns Hopkins CERSI Workshop

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

May 2, 2023, 9:00 am – 12:00 pm

Collection and Use of Fit-for-Purpose Data for Rare Disease Drug Development

9:00 – 9:05 am **Welcome: Kerry Jo Lee, MD**, Associate Director for Rare Diseases, Rare Diseases Team (RDT), Division of Rare Diseases and Medical Genetics (DRDMG), Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine (ORPURM), Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration (FDA)

9:05 – 10:25 am **Session 1**: **How to Collect Quality and Fit-for-Purpose Data**
The collection of high quality and fit-for-purpose data is challenging when working with small populations. This session will focus on an overview of FDA's approach to Real World Data/Real World Evidence (RWD/RWE), how to maximize the value of scarce and heterogeneous data on rare disease patients, and infrastructure needed to support data collection and analysis.

Moderator: Scott Winiecki, MD, Team Lead, RDT, DRDMG, ORPURM, OND, CDER, FDA

Panelists:

John Concato, MD, MS, MPH, Associate Director for Real-World Evidence Analytics, Office of Medical Policy, CDER, FDA (20 min)
Regulatory Perspectives on Real-World Data

Ramona Walls, PhD, Executive Director of Data Science, Critical Path Institute (C-PATH) (20 min)
How C-Path is Using the Latest Data Management and Data Science Techniques to Maximize the Value of Data

Vanessa Vogel-Farley, BA, BS, Senior Director, Research & Data Analytics, Global Genes and Principal Investigator, Rare-X Data Collection Platform (20 min)
Increasing the Speed and Productivity of Innovation in Rare Diseases by Increasing Collection and Access of Structured and Standardized Patient Data

Q&A: John Concato, Ramona Walls and Vanessa Vogel-Farley (20 min)

10:25 – 10:35 am **Break**

10:35 – 11:55 am **Session 2: Use of Data Sources to Inform Rare Disease Drug Development**
This session will focus on examples of how data collected from rare disease patients can be used to inform rare disease drug development. Examples include the use of RWD/RWE in trial planning, such as the selection of cohorts and endpoints, as well as validation of a kidney biomarker.

Moderator: Christine Nguyen, MD, Deputy Director, Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine, Office of New Drugs, CDER, FDA

Panelists:

Sorin Fedeles, PhD, MBA, MS, Executive Director, Polycystic Kidney Disease Outcomes, Consortium, C-PATH (30 min)
Advancement of Drug Development Tools for Polycystic Kidney Disease (PKD) as Told Through the PKD Outcomes Consortium Story

Caitlin Nichols, PhD, Research Director, AllStripes Research (30 min)
Leveraging Patient Engagement and Real-World Data to Inform Rare Disease Drug Development

Q&A: Sorin Fedeles, Caitlin Nichols and **Aliza Thompson, MD, MS**, Deputy Director of the Division of Cardiology and Nephrology, Office of Cardiology, Hematology, Endocrinology, and Nephrology, OND, CDER, FDA (20 min)

11:55 – 12:00 pm **Concluding Remarks: Kerry Jo Lee, MD**, Associate Director for Rare Diseases, RDT, DRDMG, ORPURM, OND, CDER, FDA

<u>May 3, 2023, 9:00 am – 12:00 pm</u>

Design and Analysis Methods for Clinical Trials for Rare Diseases

9:00 - 9:05 am **Welcome: Dionne Price, PhD,** Deputy Director, Office of Biostatistics, Office of Translational Sciences (OTS), CDER, FDA

9:05 – 10:25 am **Session 1: Adaptive Designs in Small Populations**
This session will focus on different types of adaptive designs for small sample size studies.

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

Panelists:

Kelley Kidwell, PhD, Associate Professor & Associate Chair for Academic Affairs in Biostatistics, School of Public Health, University of Michigan (20 min)
SMART Design and Bayesian Methods for Rare Disease Trials

Noah Simon, PhD, Associate Professor, Department of Biostatistics, University of Washington (20 min)
Adaptive Enrichment Designs in the Rare Disease Setting

Nigel Stallard, **MSc, PhD,** Professor of Medical Statistics and Deputy Director of the Clinical Trials Unit, Warwick Medical School, United Kingdom (20 min)
Clinical Trials in Rare Diseases: Should We Do Them Differently?

Q&A: Kelley Kidwell, Noah Simon, Nigel Stallard and **Gregory Levin, PhD**, Associate Director for Statistical Science and Policy, Office of Biostatistics, CDER, FDA (20 min)

10:25 – 10:35 am **10 min Break**

10:35 – 11:55 am **Session 2: Analysis Methods in Small Populations**

This session will discuss different analysis methods in small populations, including the target of inference (estimand), assumptions required, and performance.

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

Panelists:

Karen Price, PhD, Associate Vice President and Statistical Officer, Statistical Innovation Center, Eli Lily & Company (20 min)

Bayesian Methods and Master Protocols in Rare Disease Drug Development

J. Jack Lee, PhD, Professor, Department of Biostatistics, University of Texas MD Anderson Cancer Center (20 min)

Bayesian Information Borrowing for Efficient and Accurate Statistical Inference in Rare Diseases

Rima Izem, PhD, Director of Statistical Methodology Group in Analytics, Novartis Pharma AG (20 min)

Randomized and Non-Randomized Designs for Causal Inference with Longitudinal Data in Rare Disorders

Q&A: Karen Price, Jack Lee, Rima Izem, and **Frank Harrell, PhD,** Expert Biostatistics Advisor to CDER, FDA, and Professor of Biostatistics, Vanderbilt University (20 min)

11:55 – 12:00 pm **Concluding Remarks: Dionne Price, PhD,** Deputy Director, Office of Biostatistics, OTS, CDER, FDA