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 1: Collection and Use of Fit-for-Purpose Data for Rare Disease Drug Development
May 2, 2023

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), FDA

Dr. Kerry Jo Lee is the Associate Director for Rare Diseases in the Division of Rare Diseases and Medical Genetics, Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER). In this role she leads the Rare Diseases Team, a multidisciplinary rare disease programming and policy team that works to promote their mission to facilitate, support, and accelerate the development of drugs and therapeutic biologics for rare diseases and serves as the program management office for CDER's Accelerating Rare diseases Cures (ARC) Program.

Dr. Lee joined the FDA as a medical officer in 2014 with the former Division of Gastroenterology and Inborn Errors Products, OND, CDER. Dr. Lee then moved to a position as a clinical advisor for the Office of New Drug Policy, CDER, where she served as a lead in the areas of benefit-risk assessment, modernization efforts (including the integrated review for marketing applications), and real- world data/evidence programming before serving in her current position.

Dr. Lee is a pediatric gastroenterologist/hepatologist and a graduate of Princeton University and the New York University School of Medicine with an honors degree conferred in microbiology. She completed her residency in pediatrics at the Children's Hospital of Los Angeles followed by a post-doctoral clinical fellowship in Pediatric Gastroenterology, Hepatology, and Nutrition at Columbia University College of Physicians and Surgeons in New York. Dr. Lee maintains a steadfast interest in international policy and bioethics and worked for several years at the former

National Bioethics Advisory Commission on reports advising the executive branch on ethical and policy issues in both international and domestic clinical trials.

Session 1 Participants

John Concato, MD, MS, MPH
Associate Director for Real-World Evidence Analytics, Office of Medical Policy, Center for Drug Evaluation and Research
Food and Drug Administration

Dr. John Concato is Associate Director for Real-World Evidence Analytics in the Office of Medical Policy, Center for Drug Evaluation and Research (CDER), Food and Drug Administration. His responsibilities focus on FDA's Real-World Evidence (RWE) Program and include internal Agency processes, external stakeholder interaction, demonstration projects, and guidance development; he also serves as Chair of CDER's RWE Subcommittee. Dr. Concato joined FDA after a 27-year career at Yale School of Medicine and the U.S. Department of Veterans Affairs (VA), where he was a Professor of Medicine, Director of the VA Clinical Epidemiology Research Center, and one of two founding principal investigators of the VA Million Veteran Program genomic mega-biobank. He received a B.E. degree from The Cooper Union, M.D. & M.S. degrees from New York University, and an M.P.H. degree from Yale University.

Vanessa Vogel-Farley, BA, BS
Senior Director, Research & Data Analytics Global Genes
Principal Investigator- RARE-X Data Collection Platform

Vanessa Vogel-Farley is the Senior Director of Research & Data Analytics for Global Genes and serve as the Principal Investigator for RARE-X. She is the co-founder of the Commission on Novel Technologies for Complex Copy Number Variants and serve on the Coordinating Committee for the Rare Epilepsy network and the Alliance for Genetic Etiologies in Neurodevelopmental Disorders and/or Autism. She serves on the Dup15q Alliance Board and head Science and Research Strategy. She possesses 20 years of experience in data collection methods as well as expertise in non-profit and research operations, patient advocacy and support, and non-profit management. Her general research focus has been child development neuroscience/psychology research in rare disease clinical populations.

Ramona Walls, PhD
Executive Director of Data Science
Critical Path Institute (C-Path)

Ramona L. Walls, Ph.D. is Executive Director of Data Science at the Critical Path Institute (C-Path). She oversees multiple efforts including the development of C-Path's Data and Analytics Platform, expansion and modernization of C-Path's data integration pipeline to encompass new data types, and development of a rare disease knowledge graph. Walls joined C-Path in December 2020 as a Data Scientist in Ontologies, Standards, and Metadata. In 2021 she was promoted to Associate Director of Data Science and became executive director in 2022. Walls retains an appointment in the Bio5 Institute of the University of Arizona, where she has been Principal Investigator on multiple grants from the National Science Foundation and other funders. She has published over 50 peer-reviewed papers in fields as diverse as rare diseases, environmental health, evolution, biodiversity, sustainability, and space situational awareness. Walls received a bachelor's degree in Environmental Resource Management and Horticulture at Penn State and a Ph.D. in Ecology and Evolution from Stony Brook University and did a post-doc in data science at the New York Botanical Garden.

Scott Winiecki MD
Team Lead
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), FDA

Dr Scott Winiecki joined the Rare Diseases Team in Center of Drug Evaluation and Research (CDER) as a team lead in December 2022. He received his MD degree from the University of Maryland and completed his pediatric training at the Children's Hospital of Philadelphia. After 12 years in private pediatric practice, he joined Center for Biologics Evaluation and Research in 2011. In 2016, Dr. Winiecki joined CDER's Professional Affairs and Stakeholders Engagement (PASE) staff. During his six years on the PASE staff, Dr. Winiecki led the Safe Use Initiative, an initiative to reduce preventable harm from medications by funding extramural research.

Session 2 Participants

Sorin Fedeles, PhD, MBA, MS
Executive Director, Polycystic Kidney Disease Outcomes Consortium
Critical Path Institute

Sorin Fedeles is Executive Director of the Polycystic Kidney Disease Outcomes Consortium (PKDOC) at the Critical Path Institute. Here, he is overseeing the strategic vision, management, and activities of collaborative research endeavors with various stakeholders (academia, industry, FDA) to accomplish objectives of the consortium. Prior to C-Path, Sorin was a faculty member and entrepreneur at Yale University School of Medicine where he led a program focused on identifying new targets and pharmacological avenues for the treatment of Autosomal Dominant Polycystic Kidney Disease, the most common genetic cause of end-stage renal disease (ESRD). His previous work has led to publications as first/senior author in Nature Genetics, Journal of Clinical Investigation, JASN, etc., multiple grants from DoD, NIH, and the PKD Foundation and several patents. Sorin obtained his MS, PhD, and MBA from Yale University. He remains affiliated with Yale University as an Assistant Professor (Adjunct)

Christine Nguyen, MD
Deputy Director
Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine
Office of New Drugs, Center for Drug Evaluation and Research, FDA

Dr. Christine Nguyen is the Deputy Director of the Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine (ORPURM), in the Office of New Drugs, that oversees the development, review, and regulation of applications for drug and biologic products in the Division of Rare Diseases and Medical Genetics. In her role, she provides leadership in important scientific, clinical, regulatory and policy considerations related to the development of treatment of inborn errors of metabolism, including lysosomal storage disorders, organic acid disorders, and amino acid metabolism disorders. Prior to her Deputy Office Director position, Dr. Nguyen was the Deputy Director for Safety and then Division Director in the Division of Urology, Obstetrics and Gynecology and served as the acting Deputy Office Director in the Office of Drug Evaluation II that oversaw complex therapeutic areas related to endocrinology, metabolism, pulmonary, rheumatology, analgesia, pain, and addiction. Since joining the FDA in 2005, Dr. Nguyen has actively engaged in and contributed extensively to regulatory and scientific initiatives, within and outside of FDA, and has been the co-investigator on several FDA research grants. She has co-authored numerous publications on regulatory science and complex FDA decisions.

Dr. Nguyen received her Medical Doctor (MD) degree from the University of California at San Francisco (UCSF) School of Medicine, and then completed a residency in obstetrics and gynecology at the Johns Hopkins School of Medicine**.**

Caitlin Nichols, PhD
Research Director
AllStripes Research

Dr. Caitlin Nichols is the Director of Research at AllStripes Research, a medical data science company with the mission of accelerating new treatments for people impacted by rare disease. In this role, Dr. Nichols oversees scientific communications and the design and execution of real-world data research partnerships with industry, academic, government, and patient advocacy group stakeholders. She received a PhD in Biological and Biomedical Sciences from Harvard University, where she studied novel cancer therapeutic approaches leveraging copy number changes in cell-essential genes. Prior to her current role, Dr. Nichols was a scientific curator on the Product Science team at 23andMe, where she assisted in the development and improvement of carrier status and genetic health risk reports.

Aliza Thompson, MD, MS
Deputy Director of the Division of Cardiology and Nephrology
Office of Cardiology, Hematology, Endocrinology and Nephrology (OCHEN), OND, CDER, FDA

Aliza Thompson is Deputy Director of the Division of Cardiology and Nephrology, Center for Drug Evaluation and Research at the U.S. Food and Drug Administration (FDA). The Division of Cardiology and Nephrology regulates and reviews Investigational New Drug applications and marketing applications for drug and biologic products for the treatment of cardiovascular and kidney diseases. Dr. Thompson joined the FDA in 2007. Prior to her current position, Dr. Thompson served as a clinical team leader for products being developed to treat kidney diseases. Dr. Thompson received her medical degree from Johns Hopkins Medical School and completed her Internal Medicine and Nephrology training at Columbia University/New York-Presbyterian Hospital. She holds a Master of Science in Biostatistics/Patient Oriented Research Track from Columbia University Mailman School of Public Health.