FDA-MCERSI Workshop on Creating a Roadmap to Quantitative Systems Pharmacology-Informed Rare Disease Drug Development

Virtual Public Workshop
May 11, 2023
10:00 AM-5:05 PM Eastern Time

WORKSHOP AGENDA:

10:00 AM – 10:10 AM **Opening Remarks**
Robert Schuck, PharmD, PhD — Deputy Director, DTPM, OCP, CDER, FDA
Audra Stinchcomb, PhD — Professor, School of Pharmacy, University of Maryland

SESSION 1: RARE DISEASE DRUG DEVELOPMENT NEEDS THAT MAY BE ADDRESSED VIA QSP APPROACHES

Session Chair: Robert Schuck, PharmD, PhD — Deputy Director, DTPM, OCP, CDER, FDA

10:10 AM – 10:35 AM ***Considerations in Rare Disease Drug Development and How CDER is Accelerating Rare Disease Treatments***
Kerry Jo Lee, MD — Associate Director for Rare Diseases, DRDMG, OND, CDER, FDA

10:35 AM – 11:00 AM ***The Quantitative and the Mechanistic: Strategies to Advance Rare Disease Drug Development through Regulatory Science***
Issam Zineh, PharmD, MPH, FCP, FCCP — Director, OCP, OTS, CDER, FDA

11:00 AM – 11:25 AM ***Supporting Orphan Drug Development with Retrospective Quantitative Natural History Modeling – Conceptual Framework, Opportunities and Limitations***
Markus Ries, MD, PhD, MHSc, FCP — Professor, Pediatric Neurology and Metabolic Medicine, Center for Rare Diseases, Center for Pediatric and Adolescent Medicine, Heidelberg University Hospital, Heidelberg, Germany

11:25 AM – 12:00 PM
Moderator: **Valeriu Damian, PhD** — Senior Director, System Modeling & Translational Biology, Computational Sciences, Medicine Design, R&D, GlaxoSmithKline-Upper Providence

Panelists: **Issam Zineh, PharmD, MPH, FCP, FCCP** — Director, OCP, OTS, CDER, FDA
Kerry Jo Lee, MD — Associate Director for Rare Diseases, DRDMG, OND, CDER, FDA
Markus Ries, MD, PhD, MHSc, FCP — Professor, Pediatric Neurology and Metabolic Medicine, Center for Rare Diseases, Center for Pediatric and Adolescent Medicine, Heidelberg University Hospital, Heidelberg, Germany
Steven Chang, BS EE, MS EE — President & CEO, Immunetrics, Inc.
Hilary Vernon, MD, PhD — Associate Professor of Genetic Medicine, Department of Genetic Medicine, Johns Hopkins University School of Medicine
Robert Schuck, PharmD, PhD — Deputy Director, DTPM, OCP, CDER, FDA

12:00 PM – 1:00 PM ***Lunch Break***

SESSION 2: OVERVIEW OF QSP AND SUCCESSFUL USES OF QSP IN DRUG DEVELOPMENT

Session Chair: Jane Bai, PhD — Expert Regulatory Scientist (Systems Pharmacology), DARS, OCP, CDER, FDA

1:00 PM – 1:25 PM ***Challenges and Opportunities for QSP in Drug Development and Regulatory Evaluation***
Stephan Schmidt, BPharm, PhD, FCP — Endowed Professor, Department of Pharmaceutics, Director, Center for Pharmaceutics and Systems Pharmacology, University of Florida

1:25 PM – 1:50 PM ***Development and Application of a Quantitative Systems Pharmacology Model of Tumor Receptor Occupancy to Support New Dosing Regimens for Nivolumab Across Indications***
Brian J. Schmidt, PhD — Executive Director, Head QSP & PBPK Department, Bristol Myers Squibb

1:50 PM – 2:15 PM ***QSP-Model Based Assessment of Mechanistic Similarity of Disease and Response to Olipudase alfa Between Pediatric and Adult Acid Sphingomyelinase Deficiency (ASMD) Patients***
Susana Zaph, PhD — Senior Director, Head of Translational Disease Modeling-Rare & Neuro, Sanofi

2:15 PM – 2:50 PM
Moderator: **Justin Earp, PhD** — Lead Pharmacokineticist, DPM, OCP, OTS, CDER, FDA

Panelists:	**Stephan Schmidt, BPharm, PhD, FCP**	Endowed Professor, Department of Pharmaceutics Director, Center for Pharmaceutics and Systems Pharmacology, University of Florida
	Brian Schmidt, PhD	Executive Director, Head QSP & PBPK Department, Bristol Myers Squibb
	Susana Zaph, PhD	Senior Director, Head of Translational Disease Modeling-Rare & Neuro, Sanofi
	Rajanikanth (Raj) Madabushi, PhD	Associate Director, Guidance and Scientific Policy, OCP, OTS, CDER, FDA
	Christina Friedrich, PhD	Chief Engineer, Rosa & Co.
	Jane Bai, PhD	Expert Regulatory Scientist (Systems Pharmacology), DARS, OCP, CDER, FDA

2:50 PM – 3:00 PM ***Coffee Break***

SESSION 3: HOW CAN WE DEVELOP USEFUL QSP MODELS IN RARE DISEASES?

Session Chair: Audra Stinchcomb, PhD — Professor, School of Pharmacy, University of Maryland

3:00 PM – 3:35 PM ***Maximizing the Potential of Digital Twin Technology in Drug Development for Rare Diseases***

Tina Hernandez-Boussard, PhD, MPH, MS, FACMI — Associate Dean for Research; Professor of Medicine, of Biomedical Data Science, of Surgery, and by courtesy Epidemiology and Population Health; Director, Health Informatics, Stanford Center for Clinical and Translational Research, and Education, Stanford School of Medicine

3:35 PM – 4:00 PM ***QSP Modeling Evaluates CNS Enzyme Delivery for Different Treatment Modalities for Hunter Syndrome***

Kapil Gadkar, PhD — Staff Scientist, Senior Director, Denali Therapeutics

4:00 PM – 4:25 PM ***What it Will Take to Cross the Valley of Death: Dealing with Biological Heterogeneity and Epistemic Uncertainty with Agent-Based Modeling Using an Adaptation of the Principle of Maximal Entropy/Ignorance***

Gary An, MD, FACS — Professor of Surgery and Vice Chair of Surgical Research, Department of Surgery, University of Vermont Larner College of Medicine

4:25 PM – 5:00 PM

Moderator:	**Cynthia J Musante, PhD**	Vice President of Scientific Research & Global Head of QSP, Pfizer, Inc.
Panelists:	**Tina Hernandez-Boussard, PhD, MPH, MS, FACMI**	Associate Dean for Research; Professor of Medicine, of Biomedical Data Science, of Surgery, and by courtesy Epidemiology and Population Health; Director, Health Informatics, Stanford Center for Clinical and Translational Research, and Education, Stanford School of Medicine
	Kapil Gadkar, PhD	Staff Scientist, Senior Director, Denali Therapeutics
	Gary An, MD, FACS	Professor of Surgery and Vice Chair of Surgical Research, Department of Surgery, University of Vermont Larner College of Medicine
	Jie (Jack) Wang, PhD	Team Leader, Rare Diseases and Inborn Errors of Metabolism**,** DTPM, OCP, CDER, FDA
	Hao Zhu, PhD, Mstat	Division Director, Division of Pharmacometrics, OCP, OTS, CDER, FDA

5:00 PM – 5:05 PM **Closing Remarks**

Jane Bai, PhD — Expert Regulatory Scientist (Systems Pharmacology), DARS, OCP, CDER, FDA