Best Practices for Development and Application of Disease Progression Models

November 19, 2021

FDA – Virtual Workshop

9:30 AM to 2:30 PM

AGENDA

9:30-9:40	**Welcome and Opening Remarks** **Maryanne Dingman, MPH** Regulatory Health Project Manager, Office of Clinical Pharmacology, Office of Translational Sciences, CDER, FDA **Patrizia Cavazzoni, MD** Director for Center for Drug Evaluation and Research
9:40-10:40	**Session 1: Multi-stakeholder Perspectives on the Public Health Utility of Disease Models** ***Moderator:*** **Rajanikanth Madabushi, PhD** Associate Director, Guidance and Policy Team, Office of Clinical Pharmacology, Office of Translational Sciences, CDER, FDA ***Speakers:*** **Hao Zhu, PhD** Acting Director, Division of Pharmacometrics, Office of Clinical Pharmacology, Office of Translational Sciences, CDER, FDA "Role of disease models in new drug development and approval"

10:40-11:30	**Steven Kern, PhD** **Deputy Director, Quantitative Sciences, Global Health – Integrated Development, Bill & Melinda Gates Foundation** "Impact of modeling in global health drug development" **Jin Jin, PhD** Executive Director and Senior Fellow, Global Head of Modeling and Simulation (M&S) and Ophthalmology/Neuroscience Clin Pharm, Clinical Pharmacology, Genentech, Inc. "Opportunities and Challenges of Disease Modeling in Drug Development – IQ Consortium Multi-industry Perspective" ***Panelists:*** **Hao Zhu, PhD.,** Deputy Director, Division of Pharmacometrics, Office of Clinical Pharmacology, Office of Translational Sciences, CDER, FDA **Steven Kern, PhD.,** Deputy Director, Quantitative Sciences Global Health – Integrated Development, Bill & Melinda Gates Foundation **Jin Jin, PhD.,** Executive Director and Senior Fellow, Global Head of Modeling and Simulation (M&S) and Ophthalmology/Neuroscience Clin Pharm, Clinical Pharmacology, Genentech, Inc. **Piet van der Graaf, PharmD, PhD.,** Senior Vice President, Quantitative Systems Pharmacology, Editor-in-Chief *Clinical Pharmacology & Therapeutics* **Theodore Rieger, PhD,** Associate Research Fellow, Early Clinical Development, Pfizer **Billy Dunn, MD,** Director, Office of Neuroscience, Office of New Drugs
11:30-12:30	***LUNCH***
12:30-1:30	**Session 2: Challenges, Progress, and Future Directions: Toward Best Practices in Disease Modeling for Development of Innovative New Treatments** ***Moderator:*** **Qi Liu, PhD, MStat, FCP** Associate Director for Innovation & Partnership Office of Clinical Pharmacology, Office of Translational Sciences, CDER, FDA

	Speakers: **Jeff Barrett, PhD, FCP** Senior Vice President, Critical Path Institute "Landscape analysis for a disease progression model of bronchopulmonary dysplasia (BPD) in neonates: leveraging clinical trial experience and real-world patient data" **Kristin Karlsson, PhD** Senior Pharmacometrician, Swedish Medical Products Agency Chair Modelling and Simulation Working Party, EMA "Opportunities and Challenges in Disease Progression Modeling - an EU Perspective" **Cynthia J. Musante, PhD** Head of Quantitative Systems Pharmacology, Worldwide Research, Development & Medical, Pfizer "The Science and Fiction of Disease Modeling: Can One "Best Practices" Fit All?"
1:30-2:20	***Panelists:*** **Jeff Barrett, PhD, FCP,** Senior Vice President, Critical Path Institute **Cynthia J. Musante, PhD.,** Head of Quantitative Systems Pharmacology, Worldwide Research, Development & Medical, Pfizer **Flora Musuamba Tshinanu, PhD.,** Vice-Chair of the Modeling and Simulation Working Party, European Medical Agency, assessor at the Belgian medicine's agency Federal Agency for Medicines and Health Products, and Professor at University of Namur **Kosalaram Goteti, PhD,** GradCert (Statistics), Senior Scientific Director & Portfolio Section Head, Quantitative Pharmacology, EMD Serono, Inc. **Karim Azer, PhD,** Vice President, Head of Systems Biology & Discovery Axcella Therapeutics **David Strauss, MD, PhD,** Director, Division of Applied Regulatory Science Office of Clinical Pharmacology, Office of Translational Sciences
2:20-2:30	**Meeting Summary and Closing Remarks** **Issam Zineh, PharmD, MPH, FCP, FCCP** Director, Office of Clinical Pharmacology, Office of Translational Sciences, CDER, FDA
	Meeting adjourns