Advancing the Development of Pediatric Therapeutics (ADEPT 7)

M-CERSI Workshop | September 1-2, 2021
Virtual via Adobe Connect

Speaker Biographies

John Alexander, MD is a pediatrician who joined the US Food and Drug Administration as part of a joint fellowship in pediatric infectious diseases with FDA and Children's National Medical Center in Washington, DC. After completion of his fellowship, he became a full-time medical officer, and subsequently a team leader, in the Division of Anti-Infective Products. He has been involved in drug regulation and pediatric drug development for more than 20 years. Dr. Alexander is currently Deputy Director in the Division of Pediatric and Maternal Health in the Center for Drug Evaluation and Research at FDA.

Nicky Best, PhD is a Professor and VP and Head of Statistics and Data Science Innovation at GSK. In 2015, she was awarded the RSS/PSI award for Statistical Excellence in the Pharmaceutical Industry for her role in implementing prior elicitation and statistical assurance to improve decision making in clinical drug development. In 2018 she received the Royal Statistical Society Bradford Hill Medal for her work on Bayesian methods in clinical trials, cost-effectiveness, epidemiology and drug development. She currently co-chairs the EFSPI/PSI Historical Data Special Interest Group.

Before joining the pharmaceutical industry, Nicky was an academic statistician at the Medical Research Council Biostatistics Unit in Cambridge UK and at Imperial College London, where she was Professor of Statistics and Epidemiology. Her academic research focused on development and application of Bayesian methods in health and social science; she has over 100 peer-reviewed publications and she is co-developer of the BUGS Bayesian software package.

Laurie Conklin, MD joined the Pediatric Development Team at Janssen in September 2020. Her primary responsibilities are leading regulatory strategy in pediatric inflammatory bowel disease, leading trials of Stelara in pediatric IBD, and developing strategies for rare disease development.

Prior to joining Janssen, Dr. Conklin worked with ReveraGen BioPharma on trials of vamorolone, a novel treatment for boys with Duchenne muscular dystrophy. She was the recipient of a Pediatric Pharmacology grant from NICHD to study biomarkers in pediatric inflammatory diseases and has experience at the FDA as a Medical Officer in Pediatrics at CDER. Dr. Conklin was a practicing pediatric gastroenterologist for over 11 years, and director of the IBD Program at Children's National Hospital from 2011-2018.

Dr. Conklin received her M.D. from New York Medical College. After completing a Pediatrics residency and Chief Resident year at New York Presbyterian-Weill Cornell Medical Center, she completed a fellowship in Pediatric Gastroenterology Hepatology, and Nutrition at Johns Hopkins Hospital. She earned a graduate certificate in Clinical and Translational Science from the George Washington University School of Medicine.

Thomas R. Fleming, PhD, is Professor and former Chair of the Department of Biostatistics at the University of Washington in Seattle, Member of the Fred Hutchinson Cancer Research Center, and the former Director of the Statistical Center for HIV/AIDS Prevention Trial Network, NIAID. Dr. Fleming received his BA in 1972 from the University of St. Thomas and his MA in 1974 and PhD in 1976 from the University of Maryland, College Park. He has chaired or served on Data Monitoring Committees for more than 200 clinical trials.

Dr Fleming has authored or co authored several books and more than 270 research articles in peer-reviewed journals, many regarding the development of state-of-the-art methods for the design, conduct and analysis of clinical trials, and many others reporting the results of landmark trials, including the 2011 publication in NEJM on prevention of transmission of HIV. This research, on which he was senior author, was recognized by Science Magazine to be the scientific "Breakthrough of the Year."

Margaret (Meg) Gamalo, PhD is Senior Director – Biostatistics, Global Product Development – Inflammation and Immunology at Pfizer Innovative Health. She combines expertise in biostatistics, regulatory and adult and pediatric

drug development. She recently was a Research Advisor, Global Statistical Sciences at Eli Lilly and Company and prior to that was a Mathematical Statistician at the Food and Drug Administration. Meg leads the Pediatric Innovation Task Force at the Biotechnology Innovation Organization. She also actively contributes to research topics within the European Forum for Good Clinical Practice – Children's Medicine Working Party.

Christine Garnett, Pharm. D., is a clinical reviewer in the Division of Cardiology and Nephrology. She leads CDER's interdisciplinary review team for cardiac safety studies. Since 2009, Dr. Garnett has represented the FDA in the International Council for Harmonisation for the E14 guideline, and currently serves as FDA's Topic Leader. For her work on the ICH E14 guideline and the scientific white paper on concentration-QTc modeling, she received ASCPT's Gary Neil prize for innovation in drug development in 2019. Dr. Garnett has published over 40 manuscripts in peer-reviewed journals in the area of cardiac safety, and received both Critical Path and Office of Women's Health grants. Prior to joining the Division of Cardiology and Nephrology, she was a Team Leader and Associate Director of Operations in the Division of Pharmacometrics in the Office of Clinical Pharmacology. She obtained her PharmD degree from the University of Maryland and completed her clinical pharmacology fellowship with a focus in pharmacometrics and regulatory science at Georgetown University.

Dieter A. Häring, PhD, Sr. Director Biostatistics Novartis. Dieter is the Biostatistics Group Head for Neuroinflammation in Neuroscience Novartis. Dieter joined Novartis in 2008 and has been working in roles of increasing responsibility in Neuroscience Novartis on various Multiple Sclerosis programs, including the fingolimod (Gilenya), siponimod (Mayzent) and ofatumumab (Kesimpta) programs.

He planned the re-design of the PARADIG*MS* study, which was the first successfully completed randomized controlled clinical trial in pediatric in MS and led to the approval of Gilenya in pediatric MS. Currently Dieter is working on the NEOS trial, a 2-year randomized double blind triple dummy trial comparing Kesimpta and Mayzent to the active control Gilenya in a Bayesian non-inferiority design (see joint presentation with Marius Thomas on Day 2).

Prior to joining Novartis, Dieter studied biology and statistics. Dieter holds an MSc in Biology from University of Basel, Switzerland; a PhD in Biology from ETH Zurich, Switzerland; an MSc in Statistics from the University of Neuchâtel, Switzerland. He

worked for his post-Doc at the School of Mathematical Sciences at the University College Dublin, Ireland.

Anne Hammer, Statistical Leader at GlaxoSmithKline, received her BSPH from the UNC School of Public Health. She has worked in the pharmaceutical industry for over 25 years. She has supported many different therapeutic areas, including gastrointestinal, neurosciences, and immunology. She has supported pediatric indications for an epilepsy compound and most recently a lupus compound.

Daphne Hsu, MD, a Professor in the Departments of Pediatrics and Medicine at the Albert Einstein College of Medicine. Her clinical interests are in pediatric heart failure, transplantation, cardiomyopathies and adults with congenital heart disease. Her main research efforts focus on improving outcomes in children and adults with congenital heart disease, cardiomyopathy and heart failure using medical and advanced therapies such as novel drug therapies, mechanical heart devices and heart transplantation. She has served in leadership positions in the American Heart Association, American College of Cardiology, International Society for Heart and Lung Transplantation, American Academy of Pediatrics, American Board of Pediatrics and is a member of the American Pediatric Society. She is certified by the American Board of Pediatrics in Pediatrics and Pediatric Cardiology and is certified by the American Board of Internal Medicine in Adult Congenital Heart Disease.

Paul R. Lee, PhD, is the Deputy Director of the Division of Neurology 2 in the Office of Neuroscience at the Food and Drug Administration (FDA). He is also currently the Team Leader for the Neuroimmune Team and was the primary clinical review for the supplemental NDA submission that provided the basis for adding the pediatric indication to Gilenya (fingolimod). Before joining the FDA, Dr. Lee was a staff clinician in the Undiagnosed Diseases Program within the National Human Genome Research Institute at the National Institutes of Health in Bethesda, Maryland. Dr. Lee did fellowship training in neuroimmunology at the National Institute of Neurological Disorders and Stroke after completing a residency

in adult and child neurology at the Johns Hopkins Hospital. He earned his medical degree from the University of Maryland School of Medicine in 2006 and prior to that earned a Ph.D. in Neuroscience from the University of Maryland, Baltimore in 2004. In addition to specialty board certifications in pediatrics and adult neurology with special qualifications in children, Dr. Lee is a certified through the Consortium of Multiple Sclerosis Centers as a specialist in multiple sclerosis and rare neuroimmunologic disorders.

Guenther Mueller-Velten, Dipl. Wi.-Math is Global Biostatistics Development Unit Head, Cardio-Renal-Metabolic at Novartis, Switzerland and is a Biostatistician with broad experience in international clinical drug development.

He has spent 18 years at Novartis Pharma in Switzerland in various expert and leadership positions in Biostatistics supporting the Cardiovascular, Transplantation and Infectious Disease areas.

In his current role as Global Biostatistics Head for the Cardio-Renal-Metabolic Development Unit, he leads a global group of Biostatisticians, providing strategic and scientific input to all Novartis clinical development programs and regulatory submissions in the cardio-renal-metabolic area and ensuring the consistent use and implementation of state-of-the-art and appropriate statistical methodologies in all trials (including pediatric trials and large outcome trials). In collaboration with cross-functional teams, the group strives for bringing new medicines in the right dose to patients with unmet need in a safe and efficient manner.

Prior to joining Novartis, he worked as Trial and Project Statistician at Behringwerke in Germany in multiple therapeutic areas and was Head of Biostatistics and Clinical Statistics Americas at Centeon / Aventis-Behring in the USA.

Statistical areas of interest and expertise include multiplicity, recurrent event data analysis, estimands and sample size re-estimation.

Lily Mulugeta is an Associate Director for Policy and Research in the Division of Pediatrics and Maternal Health at the FDA. She engages in scientific and regulatory review and research related to pediatric drug development. Dr. Mulugeta serves as a representative for the Division on the FDA Pediatric Review Committee (PeRC). Prior to joining the Division in 2017, Lily was the Scientific Lead for Pediatrics in the Division of Pharmacometrics at the FDA. Prior to joining the FDA, Dr. Mulugeta practiced as a Critical Care Specialist at Children's National Medical Center in Washington D.C. She also served as a faculty member in the Department of Pediatrics at the George

Washington School of Medicine and in the Department of Pharmacy at the University of Maryland College of Pharmacy School of Pharmacy. Dr. Mulugeta received her Pharm.D. degree from the University of Kentucky College of Pharmacy and completed a pediatric residency at Inova Fairfax Hospital in Falls Church, Virginia.

Ronald J. Portman, MD is a board certified pediatrician and pediatric nephrologist who is Head, Pediatric Clinical Development of the Pediatric Center of Excellence at Novartis Pharmaceuticals. He joined BMS (2007) and later Novartis (2014) after his academic career at the University of Texas Medical School at Houston where he was a Professor and Director of the Division of Pediatric Nephrology and Hypertension. His current focus has been on facilitating pediatric drug development in industry with particular emphasis on consideration of pediatrics early in drug development, extrapolation and innovative study design, adolescent/pediatric inclusion in adult trials, efficiencies in studies of drugs with multiple indications and development of global pediatric clinical trial networks. He has served on many national committees or study groups effecting the practice of pediatrics and medicine. He has numerous publications in reviewed journals. He served as a consultant to the FDA and a member of the FDA Cardiovascular and Renal Drugs Advisory Committee from 2003 – 2007 and is currently Industry Representative to the FDA Pediatric Advisory Committee.

Mark Rothmann, PhD is the Director of the Division of Biometrics II. He earned his Ph. D. in Statistics at the University of Iowa in 1990. He then spent the next nine years as a professor at various universities before coming to the FDA in 1999. At the FDA, when he was a reviewer and team leader he was involved in the review on Oncology, Hematology, and Metabolism and Endocrinology products. He has done research in several areas involving the design and analysis of clinical trials and co-authored the book Design and Analysis of Non-Inferiority Trials. He currently leads three Office of Biostatistics Working Groups and Committees in Bayesian Analysis, Drug Trials Snapshots and Pediatric Studies and has served on many working groups and committees.

Marius Thomas, PhD is currently working as a Senior Principal Biostatistician in the Neuroscience development unit of Novartis. Since joining Novartis, Marius has worked on various projects in the area of multiple sclerosis. He is the trial statistician for the NEOS trial, a 2-year randomized double blind triple dummy trial comparing Kesimpta and Mayzent to the active control Gilenya in a Bayesian non-inferiority design (see joint presentation with Dieter Häring on Day 2).

Before joining Novartis, he was an early-stage researcher in the IDEAS (Improving Design, Evaluation and Analysis of early drug development Studies) European Union training network, where he was working on the development of new approaches for subgroup identification in clinical trials in close collaboration with the Novartis Statistical Methodology Group. Marius holds a PhD in Statistics from TU Dortmund University.

Andrew Thomson, **PhD** is a statistician at the EMA Taskforce dedicated to Data, Analytics and Methodology, joining the Agency in 2014. He supports the methodological aspects of the assessments of Marketing Authorisation Applications, as well as Scientific Advice, and methodological aspects of Paediatric Investigational Plans. Additionally he is the EC lead for ICH E11A where he leads the statistical workstream. Prior to the EMA, he worked at the UK regulator, the Medicines and Healthcare product Regulatory Agency as a statistical assessor.

James Travis, PhD is a statistical reviewer supporting the Division of Pediatric and Maternal Health within the Center for Drug Evaluation and Research at the FDA. He is a member of the Complex Innovative Trial Design Program Steering Committee and the Pediatric Review Committee. He has been with the FDA for six years and received his Ph.D. in Statistics from the University of Maryland Baltimore County.

Simon Wandel, **MSc, PhD** holds a MSc in Statistics from the University of Bern, and a PhD in Medical Statistics/Epidemiology also from the University of Bern. After finishing his PhD, Simon joined Novartis' Oncology Early Clinical Biostatistics group in 2010, taking on roles of increasing responsibility in different

teams. Simon has been a member of the Biostatistics group in the Cardio-Renal-Metabolic Development Unit since 2018. He has a broad range of experience in clinical study design, analysis and interpretation of clinical data across the development chain (phase I to IV), including submissions and F2F meetings with various regulatory agencies.

Simon has a particular interest in the use of Bayesian methods for drug development and is co-lead of the cross-industry/academia EFSPI/PSI special interest group “Historical data”.