

Advancing Pediatric Platform Trials: Streamlining Development, Maximizing Impact

29—30 October 2024

Biographies: Workshop Organizers and Speakers



Workshop Planning Committee Members

Name	Affiliation	Workshop Role
Susan Andrews, MPH	GSK	Planning Committee
Trevor Baker, MS	Multi-Regional Clinical Trials Center	Planning Committee
Danny Benjamin, MD, PhD, MPH	Duke University Medical Center	Planning Committee Keynote Speaker
Barbara Bierer, MD	Multi-Regional Clinical Trials Center	Planning Committee
Christina Bucci-Rechtweg, MD	Novartis Pharmaceuticals Corporation	Planning Committee
Laurie Conklin, MD	Johnson & Johnson	Planning Committee
Sneha Dave	Generation Patient	Planning Committee
Anthony "Tony" Garcia-Prats, MD, MSc, PhD	University of Wisconsin-Madison	Multi-Drug Resistant Tuberculosis Co-lead
Dionna Green, MD, FCP	U.S. Food and Drug Administration (FDA)	Planning Committee
Dominik Karres, MD	European Medicines Agency (EMA)	Planning Committee Oncology Co-lead
E. Anders Kolb, MD	The Leukemia & Lymphoma Society (LLS)	Planning Committee Oncology Co-lead
Lisa Koppelman, MSW, LICSW, MPH	Multi-Regional Clinical Trials Center	Planning Committee
Adam Levy, MD	Bristol Myers Squibb	Oncology Co-lead
Susan McCune, MD	PPD Clinical Research Business of Thermo Fisher Scientific	Planning Committee
Sumati Nambiar, MD, MPH	Johnson & Johnson	Planning Committee Multi-Drug Resistant Tuberculosis Co-lead
Robert (Skip) Nelson, MD, PhD	Johnson & Johnson	Planning Committee Major Depressive Disorder Co-lead
Manpreet Kaur Singh, MD	University of California-Davis	Planning Committee Major Depressive Disorder Co-lead
Lionel Tan, MBBS, FRCP, PhD	ViiV Healthcare	Planning Committee
Lynne Yao, MD	U.S. Food and Drug Administration (FDA)	Planning Committee



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Chloe de Campos, MRCT Center

Lindsey Henriques, MRCT Center

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Biographies

Breakout Session Co-leads and Core Group Members, Keynote Speakers, and Workshop Planning Committee

Susan Andrews, MPH (*Planning Committee*) holds a Bachelor of Science, Medical Biology, from the University of New England and a Master of Public Health from the George Washington University, Milken Institute School of Public Health. She has had roles as a clinical research coordinator, clinical research associate (monitoring), principal clinical research scientist, study delivery lead, and is currently a clinical science lead.

A special interest of hers is pediatric clinical trials; she has one (adult) child, Olivia, who has lifelong disabilities. Their hobbies include motorcycles (street), recreational and technical scuba diving (Dive Master/teaching), Land Rovers, music, horses, and textile crafts (remaking traditional Japanese garments, sashiko, yubinuki, and knitting).



Trevor Baker, MS (*Planning Committee*) is a Program Manager at the Multi-Regional Clinical Trials Center of Brigham and Women's Hospital and Harvard (MRCT Center). Trevor primarily focuses on MRCT Center projects involving Artificial Intelligence, Environmental Sustainability, and Platform Studies. Before joining the MRCT center, Trevor worked as a Research Project Manager at Boston Medical Center, implementing a multi-regional trial in the research area of overdose prevention. Trevor graduated from the University of Notre Dame with a Master of Science in Global Health. He earned a Bachelor of Science in Biology from Santa Clara University.



Danny Benjamin, MD, PhD, MPH (*Planning Committee & Keynote Speaker*) is the Principal Investigator and Chair of the National Institute of Child Health and Human Development's Pediatric Trials Network. The Network is responsible for designing and leading clinical trials of off-patent medicines in children of all ages across all therapeutic areas. The team has established, or is actively studying, the correct dosing and safety of more than 100 of the most commonly used medicines in children. Each of these trials is conducted under an Investigational New Drug (IND) application with guidance from the U.S. FDA for labeling.



Danny has led platform trials enrolling nearly 10,000 participants under an IND in the following patient populations: premature and term infants, breastfeeding mother-infant pairs, pharmacokinetics of off-patent therapeutics pediatrics (POPS, enrolling since 2010) of children birth to age 18 years, children with obesity, children on ECMO, analgesia and pain medicines, anti-seizure medicines in children, antipsychotics in children, and adults with COVID. The primary outcomes from trials include pharmacokinetics, pharmacodynamics, dosing, safety, and efficacy leading to labeling changes for over a dozen different molecules. Danny is recognized by the National Institutes of Health as a premiere mentor and educator. His research program serves as a platform to train students and early career investigators. Over 250 students and junior physicians from around the globe have accessed Pediatric Trials Network data and resources and subsequently earned co-authorship on peer-reviewed manuscripts retrievable on PubMed.



Barbara E. Bierer, MD, (*Planning Committee*) is the Faculty Director of the Multi-Regional Clinical Trials Center of Brigham and Women's Hospital and Harvard (MRCT Center), a Professor of Medicine, Harvard Medical School and Brigham and Women's Hospital, Boston, and a hematologist/oncologist. She is the Director of the Regulatory Foundations, Ethics and the Law Program of the Harvard clinical and translational sciences center. Previously, she served as senior vice president, research, at the Brigham and Women's Hospital for 11 years, and was the institutional official for human subjects and animal research, for biosafety, and for research integrity. She initiated the Brigham Research Institute and the Innovation Hub (iHub), a focus for entrepreneurship and innovation. In addition, she was the Founding Director of the Center for Faculty Development and Diversity at the BWH.

In addition to her academic responsibilities, Dr. Bierer serves on the Board of Directors of Vivli, Inc., a non-profit organization founded by the MRCT Center dedicated to global clinical trial sharing; North Star Research Board; Clinithink, Inc., Generation Patient, and the Edward P Evans Foundation. Previously she has served on the Board of Directors of Public Responsibility in Medicine and Research (PRIM&R) and the Association for Accreditation of Human Research Protection Programs (AAHRPP) and as chair of the Secretary's Advisory Committee on Human Research Protections, HHS, among others. She has authored



or co-authored over 300 publications. Dr. Bierer received a B.S. from Yale University and an M.D. from Harvard Medical School.

Christina Bucci-Rechtweg, MD (*Planning Committee*) graduated with a medical degree from the University of Rochester School of Medicine & Dentistry, was Residency trained in Pediatrics and Fellowship trained in Pediatric Critical Care Medicine at the State University of New York @ Buffalo. She has over 20 years of pharmaceutical industry experience with roles in translational research, clinical development & medical affairs, as well as regulatory and development policy. In her career she has been responsible for the oversight and registration of global medicines development programs. She is widely regarded for her negotiating skills in adult and pediatric drug development, as well as health policy and international regulatory consensus building.



Christina is actively involved in numerous external organizations advancing the regulatory and development environment for pediatric and maternal health globally, including the NIH/NICHD National Advisory Council for Child Health and Human Development, International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use (ICH) Pediatric Standing Advisory Committee, ICH E11A Pediatric Extrapolation Expert Working Group, Critical Path Institute's International Neonatal Consortium, European Forum for Good Clinical Practice Children's Medicines Working Party, the IQ Consortium's Pediatric Clinical Pharmacology Leadership Group, Health & Human Services Task Force on Research in Pregnant Women & Lactating Women, as well as numerous professional societies and the trades. Christina is the Global Head for Pediatric and Maternal Health Policy at Novartis Pharmaceuticals Corporation.



Laurie Conklin, MD (*Planning Committee*) has a combined 15+ years' experience in a number of roles including academic pediatric gastroenterology physician at Childrens National Hospital, rare disease researcher, medical officer at the Center for Drug Evaluation & Research (CDER) in Pediatrics and Maternal Health at FDA, as well as a clinical development physician-scientist for both small and large pharma.

She is board certified in both Pediatrics and Pediatric Gastroenterology and has authored and published numerous articles in peer-reviewed and other journals, book chapters and white papers. She has participated in international rare disease task forces and public workshops and has been an invited speaker and panelist both nationally and internationally on a variety of scientific topics.



Christoph Correll, MD (*Major Depressive Disorder Core Group Member*) is Professor of Psychiatry at Zucker School of Medicine at Hofstra/Northwell, New York, USA, and also Professor and Chair, Department of Child and Adolescent Psychiatry, Charité University, Berlin, Germany. He completed his medical studies at Free University of Berlin, Germany, and Dundee University, Scotland. He is board certified in both general and child and adolescent psychiatry. Dr. Correll focuses on youth and adults with severe mental illness, clinical trials, real-world evidence, psychopharmacology, meta-research, and physical health in mental health.



He has authored >900 articles that have been cited >82,000 times and received over 40 research awards. In 08/2024, his h-index was 145, with an h-index >100 in the last 5 years alone. Since 2017, Dr. Correll has consistently been the number one cited scientist in >10 areas, including “central nervous system agents”, “psychotropic drugs”, “schizophrenia”, “schizophrenia spectrum and other psychotic disorders”, “antipsychotics”, “delayed action preparations” and “weight gain”.



Sneha Dave (*Planning Committee*) is the Founder and Executive Director of Generation Patient. Generation Patient drives meaningful change for young adults with chronic conditions, providing nearly 600 peer support meetings, leadership programming, and scholarship and fellowship programs. On a systemic level, Generation Patient leads research and policy reform, focusing on the patent system, diversifying clinical trials, and increasing oversight of pharmaceutical advertisements on social media.

Sneha's leadership recognized her as the youngest principal investigator by the Patient-Centered Outcomes Research Institute and an American Association of People with Disabilities Emerging Leader. Sneha has spoken at the White House, Aspen Ideas Health, and Capitol Hill, been featured nationally on C-SPAN, and contributed to the U.S. News and World Report. She serves on various advisory councils and boards, including the Institute for Clinical and Economic Review, a panel for the British Medical Journal, a grantmaking committee with the Robert Wood Johnson Foundation, and the Yale Collaboration for Regulatory Rigor, Integrity, and Transparency Advisory Board. Sneha is building a sustainable movement to empower young adults with chronic conditions, ensuring their voices are positioned to drive change.



Melissa DelBello, MD, MS (*Major Depressive Disorder Core Group Member*) is a Professor of Psychiatry and Pediatrics and Dr. Stanley and Mickey Kaplan Chair of Psychiatry and Behavioral Neurosciences at the University of Cincinnati College of Medicine (UCCOM).

Dr. DelBello received her medical degree from the University of Rochester and completed her psychiatry residencies at the UCCOM and Payne Whitney Psychiatric Clinic of the New York Hospital-Cornell Medical Center. Dr. DelBello completed a fellowship in Child and Adolescent Psychiatry at Cincinnati Children's Hospital Medical Center and obtained a master's degree in Epidemiology and Biostatistics from UCCOM. Dr. DelBello has co-authored over 400 articles, and has served as principal or co-investigator of several NIH, PCORI, foundation, and pharmaceutical research grants investigating risk and resilience factors associated with the onset and course of mood disorders.



Martha Donoghue, MD (*Oncology Core Group Member*) is the Associate Director for Pediatric Oncology and Rare Cancers in the FDA Oncology Center of Excellence, Office of the Commissioner and the Acting Associate Director for Pediatric Oncology in the Office of Oncologic Diseases, Center of Drug Evaluation and Research (CDER). In these roles, she oversees implementation of pediatric regulations to facilitate timely investigation of drugs and biological products for pediatric patients with cancer, supports and promotes consistency of regulatory work relating to pediatric oncology and rare cancer drug development across CDER and the Center for Biologics Evaluation and Research (CBER), and works with stakeholders to address challenges and foster development of drugs to treat pediatric and other rare cancers.

Prior to joining FDA in 2009, Dr. Donoghue completed a fellowship in Pediatric Hematology and Oncology at the Children's National Medical Center after working for several years as a general pediatrician in private practice. She received her medical degree from Emory University and completed a residency in general pediatrics at the Georgetown University Medical Center.



Robert Findling, MD (*Major Depressive Disorder Core Group Member*) is currently the chair Psychiatry at Virginia Commonwealth University's School of Medicine. He is also a C. Kenneth and Dianne Wright Distinguished Chair in Clinical & Translational Research at VCU. Prior to coming to Richmond, Dr. Findling was the Director of Child and Adolescent Psychiatry and the Leonard and Helen R. Stulman Professor of Child & Adolescent Psychiatry at Johns Hopkins.

He also was the Vice President for Psychiatry Services and Research at the Kennedy Krieger Institute while in Baltimore. Dr. Findling completed training in Pediatrics, Psychiatry, and Child & Adolescent Psychiatry as part of the "Triple Board" joint training program at Mt. Sinai in New York. He subsequently earned his Master of Business Administration degree at a joint program run by the London School of Economics, NYU Stern, and École des Hautes Études Commerciales de Paris.



Anthony Garcia-Prats, MD, MSc, PhD (*Multi-Drug Resistant Tuberculosis Co-lead*) is an Associate Professor in the Divisions of Global Pediatrics and General Pediatrics and Adolescent Medicine at UW, as well as an Extraordinary Associate Professor at Stellenbosch University (Cape Town, South Africa). His primary interests are the management of tuberculosis and multidrug-resistant tuberculosis in children, and the pharmacokinetics, safety and acceptability of anti-tuberculosis drugs in children.

He is leading or supporting multiple pediatric trials of new and existing TB drugs, and is the Principal Investigator of the Unitaied-funded BENEFIT Kids project that aims to improve access for children to better MDR-TB treatment and prevention. He is Co-leading CHEETA (Chasing Expedited and Equitable Treatment Access for Children with TB) that aims to accelerate pediatric evaluations of new TB drugs. He works regularly with the World Health Organization on TB and MDR-TB treatment policy.



Meg Grabb, PhD (*Major Depressive Disorder Core Group Member*) is Program Officer for the Novel Pharmacologic and Device-based Intervention Program for Pediatrics, in the Division of Translational Research at NIMH. She leads efforts at NIMH in developing funding opportunities to support early-stage drug and brain stimulation trials in psychiatry, both in pediatric and adult populations, and she participates in several NIH-wide committees involving these intervention types.



She is a member of two different consortia focused on further validation of biomarkers for use in future trials: the Autism Biomarker Consortium for Clinical Trials (ABC-CT), and Accelerating Medicines Partnership - Schizophrenia (AMP-SCZ). She has received the NIH Director's Award for establishing the Fast Fail Autism Program to enable testing of investigational agents in autism spectrum disorder. Dr. Grabb oversees the Small Business Research Programs at NIMH, with an annual budget of > \$60M that supports a diverse technology portfolio. She also co-leads the BRAIN Initiative's Dissemination Team at NIH.



Dionna Green, MD, FCP (*Planning Committee*) is the Director of the Office of Pediatric Therapeutics (OPT) in the Office of the Clinical Policy and Programs in the Office of the Commissioner at the US Food and Drug Administration (FDA). OPT is a congressionally mandated office whose mission is to assure access for children to innovative, safe, and effective medical products. Dr. Green leads an organization that is responsible for spearheading, coordinating, and facilitating cross-cutting activities of the FDA in the areas of pediatric health and product development, ethics, neonatology, rare diseases, pharmacovigilance, international collaboration, regulatory science, and education and outreach.

Dr. Green received her medical degree from the Howard University College of Medicine and her clinical training in pediatric medicine from the Herman & Walter Samuelson Children's Hospital at Sinai. She completed a clinical pharmacology research fellowship with the Georgetown University Drug Discovery Program, and a regulatory science fellowship with the FDA Commissioner's Fellowship Program. Dr. Green is currently the President of the American College of Clinical Pharmacology (ACCP), as well as a Fellow of ACCP.



Stefan Gold, PhD, MSc (*Keynote Speaker*) is professor of Clinical Neuroscience and Immunology at Charité Universitätsmedizin Berlin, Germany. Previously, he trained and worked at University of Hamburg in Germany, as well as New York University (NYU), and University of California Los Angeles (UCLA) in the United States. The group's main interest is to develop novel treatment approaches for complex CNS disorders such as multiple sclerosis (MS) and major depressive disorder (MDD) and test their efficacy and safety in clinical trials. The research program is designed to combine research on (psycho)biological mechanisms with clinical trials flanked by biomarker studies to create a feed-forward cycle that steadily advances our understanding of disease mechanisms and ultimately improves clinical care. The overall goal is to advance treatments by understanding the biopsychological mechanisms, by developing novel interventions, by enhancing the evidence base through improvements in trial design, and by facilitating implementation (e.g. through digital delivery options).



Stefan has more than 15 years' experience in conducting investigator-initiated clinical trials in Europe and the US, evaluating both pharmacological and non-pharmacological treatments for neurological and psychiatric diseases. A particular focus area in recent years has been trial design innovations, including platform trials. Stefan is part of the EU-PEARL consortium (<https://eu-pearl.eu>), where he co-led the development of the master protocol for MDD, and serves as the scientific director of the integrated platform trial in treatment resistant depression (PEARLDIVER). Stefan also serves as the scientific advisor to the Good Clinical Trials Collaborative (<https://www.goodtrials.org>).

Anneke Hesseling, MBChB, MSc, PhD, Desmond Tutu TB Centre at Stellenbosch University (*Multi-Drug Resistant Tuberculosis Core Group Member*)



Patrick Jean-Philippe, MD (*Multi-Drug Resistant Tuberculosis Core Group Member*) is the Chief of the Maternal Adolescent Pediatric Research Branch, in the Prevention Sciences Program at the Division of AIDS of the National Institute of Allergy and Infectious Diseases, National Institute of Health. He oversees a portfolio of basic, translational, and clinical research focused on antiretroviral and anti TB drug development in children, including pediatric friendly formulations development, and is involved in several clinical trials evaluating novel drugs and regimen for RR-TB treatment in children. Dr. Jean-Philippe is U.S. Board-Certified in Pediatrics and Pediatric Infectious Diseases. He obtained his medical degree from Faculté de Médecine et Pharmacie, State University of Haiti, completed his pediatric residency training and chief residency at Harlem Hospital Center in affiliation with



Columbia University College of Physicians and Surgeons, then completed his pediatric infectious diseases training at New York University School of Medicine.

Dominik Karres, MD (*Oncology Co-lead*) has a medical degree, followed by a MD in paediatric drug development. He held a training post in paediatric haematology/ oncology in Germany. From there he developed an interest in paediatric oncology drug development, further strengthened with posts in the UK in an early clinical trials unit.

Since over 10 years now he works in drug regulation, including various scientific roles at the UK's regulatory agency, MHRA and now at EMA as Senior Scientific Officer in the Paediatric Medicines Office. He oversees scientific evaluations of anti cancer drug development plans and supports the agency's efforts fostering innovation in paediatric drug development in various roles and responsibilities globally and in line with EU policy initiatives. Expanding on this role and building on his experience, Dominik has recently also been appointed engagement specialist supporting technical regulatory/ HTA collaboration efforts.



Pam Kearns, PhD FRCPCH (*Oncology Core Group Member*) is Emeritus Professor of Clinical Paediatric Oncology at the University of Birmingham, where she was Director of the University of Birmingham's Cancer Research UK Clinical Trials Unit from 2011-2023 and Director of the Institute of Cancer and Genomic Sciences from 2021-2024. Her research is focused on drug development and innovation in the design and delivery of national and international clinical trials for childhood cancers.

She is President of 'Innovative Therapeutics in Childhood Cancer' (ITCC) and a Founding Board member of 'ACCELERATE'. She was President of the European Society of Paediatric Oncology (SIOPE) from 2019 -2021 and continues to serve on the SIOPE Board. She chairs the Research Assessment Panel for GOSH Charity and the Independent Scientific Advisory Panel for Bone Cancer Research Trust and is a member of the NIHR Invention for Innovation (i4i) funding Committee. She is Chair of the Board of Trustees for A Child of Mine, a charity dedicated to supporting bereaved parents.



George Kirk, PhD (*Oncology Core Group Member*) has been at AstraZeneca for 28 years and has spent the last 15 years supporting Oncology projects and is currently VP Global Product Leader (GPL) for a number of late stage projects, including Monalizumab (NK2GA inhibitor); Oleclumab (CD73 inhibitor); and Savolitinib (MET inhibitor) focusing on a range of tumour types, including NSCLC, pRCC, and Gastric Cancer. George also leads the Paediatric Oncology Development Team (PODT) which develops and delivers paediatric strategies for programs across AstraZeneca's oncology portfolio. The PODT is a new team in AstraZeneca Oncology formed to help prioritise activities to develop better medicines for paediatric patients with cancer with initial focus on paediatric brain tumours and sarcomas. George is also responsible for the leadership of the Oncology Sub-Cutaneous Strategy team.



Previously George was the GPL for Koselugo (selumetinib), a program he worked on since it entered late stage development in 2012. George was instrumental in driving the Koselugo program forward and played a pivotal role in achieving approval from US in 2020 and the EU in 2021 for the treatment of children with NF1-PN. In this role George successfully collaborated with the team at the NIH, NTAP, CTEP/NCI and the CTF to support the Koselugo development program. Prior to the above George spent his first 13 years in AstraZeneca as a Project Manager and Team Manager in Pharmaceutical Development. During his time in these roles he worked on a variety of Oncology, Infection and Respiratory and Inflammation projects in both Early and Late Phase Development. George's background/training is Chemistry, having a PhD in Organic Chemistry from the University of Strathclyde in Glasgow and he also undertook an oncology preceptorship program at St James' hospital in Leeds in 2014.



E. Anders Kolb, MD (*Oncology Co-lead*) is pediatric hematologist oncologist and researcher with more than 20 years experience in developing drugs for children with cancer. He is the president and chief executive officer of The Leukemia & Lymphoma Society (LLS), a global leader in the fight against cancer. Dr. Kolb has devoted his life's work to caring for children with pediatric blood cancer and conducting research to find cures. Before joining LLS, he spent 15 years at Nemours Children's Health, where he served as chief of the Division of Hematology and Oncology, Director of the Moseley Foundation Institute for Cancer and Blood Disorders, and Vice Chairman for Research in the Department of Pediatrics at the Sidney Kimmel Medical College at Thomas Jefferson University.

Dr. Kolb has been the principal or co-principal investigator of numerous studies and grants, including clinical trials with the Children's Oncology Group, the Nemours NCI Community Oncology Research Program and a NIGMS Center of Biomedical Research Excellence grant to establish the Delaware



Comprehensive Sickle Cell Research Center. From 2013 to 2023, he served as the Chair of the Children's Oncology Group Myeloid Disease Committee. From 2018 to 2023 he was co-chair of the Leukemia & Lymphoma Society Pediatric Acute Leukemia (PedAL) Master Clinical Trial. Dr. Kolb has authored or co-authored more than 200 articles in peer-reviewed journals and received numerous awards.

Lisa Koppelman, MSW, LICSW, MPH (*Planning Committee*) joined the MRCT Center in 2019 and has focused on issues related to the global harmonization of pediatric clinical trials, elevating the voices of young people in clinical research, and community engagement. Lisa assumed the position of Team & Program Director in June 2022, incorporating key elements of coaching and professional development into her work that supports the Center's staff. Lisa brings over fifteen years' experience as a public health professional to her work, with a particular focus on qualitative research methods.



Prior to her immersion in the public health realm, Lisa worked for 15+ years as a clinical social worker in a variety of settings. Lisa earned her Bachelor of Arts degree from Tufts University, her Master of Social Work degree from Columbia University School of Social Work, and her Master of Public Health degree in International Health from Boston University School of Public Health. She is a certified yoga teacher, a trained Executive Coach, and conducts professional development trainings focused on emotional intelligence in the workplace.



Oncology Group.

Adam Levy, MD (*Oncology Co-lead*) is Executive Director, Head of the BMS Pediatric Center of Excellence. In his position, Adam is responsible for BMS' strategic approach to Pediatric Development and leads a cross-functional matrix team to ensure BMS is positioned to deliver robust, informed, and timely pediatric plans in response to the evolving pediatric global regulatory and therapeutic environment.

Prior to working at BMS he spent 18 years at the Montefiore Health System where he most recently served as Vice Chair of Clinical Affairs and Strategy at the Children's Hospital at Montefiore and Professor of Pediatrics at the Albert Einstein College of Medicine. Over the course of his time at Montefiore/Einstein, Adam directed the pediatric oncology clinical trials program and was a Principal Investigator for the Children's



Susan McCune, MD (*Planning Committee*) is a board-certified pediatrician and neonatologist. She joined the FDA (2003) after an academic career at Children's National Medical Center and Johns Hopkins Medicine. Dr. McCune was the Director in the Office of Pediatric Therapeutics in the Office of the Commissioner, FDA (2017-2021) and joined PPD/Thermo Fisher Scientific in 2021. She obtained her MD from George Washington University Medical School and her BA in Biochemistry from Harvard University. Dr. McCune is focused on facilitating pediatric drug development with a particular emphasis on global trials for neonates and rare diseases, development of biomarkers for pediatric studies, and inclusion of the voice of the pediatric patient in trial designs.



Lindsay McKenna, MPH (*Multi-Drug Resistant Tuberculosis Core Group Member*) is co-director of the tuberculosis (TB) project. Lindsay works with community advocates, researchers, policy makers, developers, and donors to expedite the development of and access to novel TB treatment, diagnostic, and prevention technologies.

As coordinator of the Global TB Community Advisory Board—a group of research-literate activists from around the world that acts in an advisory capacity to institutions conducting TB clinical trials—she also works to ensure that research and development efforts respond to community needs and priorities

Sumati Nambiar, MD, MPH (*Multi-Drug Resistant Tuberculosis Co-lead*) is board certified in pediatrics and pediatric infectious diseases. She is a Fellow of the American Academy of Pediatrics and has over 20 years of experience in drug development and regulatory science. Currently, she is a Senior Director in the Child Health Innovation and Leadership Department at Johnson and Johnson.

Prior to joining Johnson & Johnson, Dr. Nambiar worked at the US Food and Drug Administration for 22 years in various capacities, including 8 years as Director, Division of Anti-Infectives providing regulatory oversight for the development of anti-infective products. Dr. Nambiar trained and worked as a pediatrician in India prior to moving to the US. She completed her residency in pediatrics, including a year as Chief Resident at Inova Fairfax Hospital for Children, VA and a fellowship in pediatric infectious diseases at Children's National Hospital, Washington DC. She has a master's degree in public health from the George Washington University School of Public Health.





Robert "Skip" Nelson, MD, PhD (*Planning Committee & Major Depressive Disorder Co-lead*) is Executive Director, Pediatric Drug Development in the Child Health Innovation Leadership Department at Johnson & Johnson. Previously (2006-2017), Dr. Nelson was the Deputy Director and Senior Pediatric Ethicist in the Office of Pediatric Therapeutics at the U.S. Food and Drug Administration. Prior to joining FDA, Dr. Nelson spent 20 years in the academic practice of pediatric critical care medicine, most recently as Professor of Anesthesiology, Critical Care and Pediatrics at The Children's Hospital of Philadelphia (CHOP) and University of Pennsylvania School of Medicine.

Dr. Nelson is a member of the International Council for Harmonisation (ICH) E11A Working Group developing a guideline on the use of extrapolation in pediatric drug development plans. He recently completed a four-year term as a member of the Secretary's Advisory Committee on Human Research Protections (SACHRP) and serves as the Industry Representative to the FDA Pediatric Advisory Committee. After receiving his M.D. degree from Yale University, Dr. Nelson trained in pediatrics (Massachusetts General Hospital), neonatology and pediatric critical care (University of California, San Francisco). He has a Master of Divinity degree from Yale Divinity School and a Ph.D. in The Study of Religion from Harvard University, specializing in ethics.

Martina Penazzato, MD (*Multi-Drug Resistant Tuberculosis Core Group Member*) is an infectious disease specialist and over the last 15 years, in addition to providing major contribution to several WHO Guidelines in the areas of HIV, TB and child health, she has provided technical assistance to several countries globally and contributed to shape the research agenda with tangible impact on improving treatment and care for children living with HIV. In her current role as co-founder and lead of the Global accelerator for Paediatric formulations within WHO's Science Division she is contributing to a number of global initiatives to improve access to better medicines for children and pregnant people.



Melissa Rones, JD, PhD Ropes & Gray LLP (*Keynote Speaker*)





Manpreet Kaur Singh, MD (*Planning Committee & Major Depressive Disorder Co-lead*) is Professor of Psychiatry and Behavioral Sciences at University of California Davis. She completed residency in Pediatrics, Psychiatry, and Child and Adolescent Psychiatry at Cincinnati Children's Hospital Medical Center. After postdoctoral training, she joined Stanford's professoriate, where she conducted family studies integrating neuroimaging, cognitive, genetic, behavioral, peripheral biomarker data characterizing risk for and resilience from developing bipolar disorder.

Dr. Singh's research now at UC Davis aims to address unmet clinical needs in mood disorders by bridging early-stage discoveries to clinical practice through: innovations in trial design, investigations of placebo response, and pursuit of novel therapeutic targets. Through reverse and forward translation, fast-fail clinical trials, and direct modulation of key brain regions using transcranial magnetic stimulation and real-time neurofeedback, Dr. Singh seeks to generate evidence for the most vexing questions in our field, like who gets which treatment and when? She also conducts mechanistic and comparative effectiveness trials to improve precision, reduce illness burden, and optimize functioning.

Lionel Tan, MBBS, FRCP, PhD (*Planning Committee*) is physician-scientist and currently the Medicine Development Lead for Paediatric HIV at ViiV Healthcare, with oversight of the paediatric R&D portfolio including studies supporting paediatric regulatory approvals. Lionel joined ViiV Healthcare from GSK where he had led adult and paediatric clinical programmes in Global Health and Infectious Diseases. He co-chairs the GSK Medicine for Children Advisory Network and the ViiV Paediatric Advisory Group. He also represents GSK on the network committee and project steering committee of Conect4children (c4c), an Innovative Medicine Initiative project aimed at establishing a paediatric clinical trial network in Europe. Prior to joining GSK, Lionel was a clinical academic and Consultant Infectious Diseases Physician at Imperial College London. He has a Diploma in Tropical Medicine and Hygiene and a PhD in Molecular Microbiology. He is a Fellow of the Royal College of Physicians.





Charles Wells, MD (*Multi-Drug Resistant Tuberculosis Core Group Member*) currently serves as Head of Therapeutics Development at the Bill & Melinda Gates Medical Research Institute in Cambridge, Massachusetts, where he joined in 2019. Previously he served as Executive VP for Global Health and Development for Infectious Diseases at Evotec carrying forward in this role with the transfer of the Infectious Diseases Therapeutics R&D group from Sanofi to Evotec in July 2018. His work in this role included overseeing development activities for new therapeutic agents for malaria, bacterial diseases and biologics development for HIV and other viral infections.

Prior to joining Sanofi in 2015, he served as Senior Medical Director for the development and initial registration of delamanid for treatment of multidrug resistant-tuberculosis at Otsuka Pharmaceuticals, during 2007-2015. Before Otsuka, he served as Chief of the International Research and Programs Branch of the Division of Tuberculosis Elimination at the U.S. Centers for Disease Control and Prevention (CDC) during 2000-2007. He is a native of North Carolina where he completed his medical studies at the University of North Carolina at Chapel Hill in 1992 and then his post-graduate medical training in internal medicine and infectious diseases at Emory University in 1998, as well as the Epidemic Intelligence Service at CDC.

Lynne Yao, MD (*Planning Committee*) is the Director, Division of Pediatric and Maternal Health (DPMH) in the Office of New Drugs, Center for Drug Evaluation and Research. Dr. Yao received a B.S. degree in Biology from Yale University, and an M.D. degree from the George Washington University School of Medicine. She is board certified in both Pediatrics and Pediatric Nephrology.



Prior to joining FDA, Dr. Yao was the Director of Dialysis and Associate Pediatric Residency Program Director at the Inova Fairfax Hospital for Children in Fairfax, VA. She has been with the FDA since 2008. Dr. Yao also served as the Rapporteur for the International Conference on Harmonization (ICH) E11A Pediatric Extrapolation Guideline Expert Working Group. As DPMH Director, Dr. Yao oversees quality initiatives which promote and necessitate the study of drug and biological products in the pediatric population; and improve collection of data to support the safe use of drugs and biological products in pregnant and lactating individuals. She collaborates with numerous stakeholders both inside and outside of FDA to advance development of safe and effective therapies for children, and pregnant and lactating women.

