Advancing the Development of Pediatric Therapeutics (ADEPT 6)

Pediatric Clinical Trial Endpoints for Rare Diseases

with a Focus on Patient Perspectives

November 12, 2019

Speakers' Biographies

**Alphabetically ordered by last name*

HEATHER ADAMS, MD, University of Rochester School of Medicine and Dentistry

Heather Adams is an Associate Professor in the Departments of Neurology and Pediatrics at the University of Rochester School of Medicine and Dentistry in Rochester, NY. She completed her PhD in Clinical Child Psychology, at Southern Illinois University at Carbondale, in 2002, an Internship in Clinical Psychology at the University of Rochester Medical Center (URMC) in 1999, and a post-doctoral fellowship in Child Neuropsychology (URMC) in 2003. She is a member of the University of Rochester Batten Center (URBC) and has been engaged in Batten disease research and clinical activities since 2003. Dr. Adams' research interests span three main areas - neurobehavioral phenotyping and clinical trial outcomes for rare pediatric genetic and metabolic diseases, cognitive function in pediatric essential hypertension, and studies in Tourette Syndrome. Dr. Adams also serves on the Medical Advisory Board for the Batten Disease Support and Research Association (BDSRA) and is the Co-Director of the University of Rochester's Tourette Association of America Center of Excellence. Her clinical activities include pediatric neuropsychological assessment for a range of conditions, and evidence-based psychosocial treatments for Tourette Syndrome, OCD, anxiety, and ADHD. Dr. Adams also serves as a Chair for one of five boards that comprise the University of Rochester's Research Subjects Review Board (IRB).

COURTNEY BLACKWELL, PhD, Northwestern University, Feinberg School of Medicine

Dr. Blackwell has expertise in the development and validation of Patient Reported Outcomes (PROs) and performance assessments, and recently led efforts to develop a series of Patient Reported Outcome Measurement Information System (PROMIS®) parent report measures to evaluate early childhood (1-5 years) physical, mental, and social health outcomes. Her research focuses on the developmental trajectories of positive health and well-being, with particular interest in social environmental risk and promotive factors. Dr. Blackwell is a co-Investigator for the NIH-funded Environmental influences on Child Health Outcomes (ECHO) PRO Core and a faculty affiliate of the Northwestern Institute for Innovations in Developmental Sciences (DevSci), where she leads the inaugural DevSci national parent survey to evaluates attitudes,

experiences, and perspectives on high-impact, policy-relevant early childhood health and development issues.

LORI CROSBY, PsyD, Cincinnati Children's Hospital Medical Center

Lori E. Crosby is a Professor in the Division of Behavioral Medicine and Clinical Psychology at Cincinnati Children's Hospital Medical Center (CCHMC) and Department of Pediatrics at the University of Cincinnati College of Medicine. Dr. Crosby is also Co-Director of the Cincinnati Center for Clinical and Translational Science (CTSA), Community Engagement Core, INNOVATIONS in Community Research and Program Evaluation, and directs a research program in pediatric sickle cell disease. Dr. Crosby has expertise in self-management, treatment adherence, healthcare transition, recruitment and retention of minorities in research, integrating design thinking into research, implementation science, and community engagement. She is the Co-Director of the Community Engagement Core at the CCHMC and UC where she directs the Community Leaders Institute, which trains community organizations and residents in research methods and quality improvement. Over 100 leaders have received training that has resulted in more than $5 million in grant funding for the Greater Cincinnati community. Dr. Crosby has been a Principal Investigator, Co-Investigator or Consultant on more than 18 federally-funded projects including serving as Principal Investigator for K07 and R21 grants funded by the National Institutes of Health and a contract award from the Patient Centered Outcomes Research Institute (PCORI) to disseminate a hydroxyurea shared decision-making intervention. She is also a Co-Investigator on the HRSA-funded Sickle Cell Treatment Outcomes and in the Midwest (STORM) regional network that has demonstrated improvements in clinical outcomes for patients affected by sickle cell disease in the Midwest. Dr. Crosby has served in a leadership role for a number of quality improvement projects at Cincinnati Children's including implementation of home pain management plan and transcranial Doppler protocols for pediatric patients with sickle cell disease. Parent and stakeholder partners have worked with Dr. Crosby's research team on a number of projects including the development and evaluation of a mobile self-management app for adolescents and young adults with sickle cell disease, iManage, and her current study evaluating dissemination and implementation strategies for a shared decision making intervention.

CARLA EPPS, MD, Office of the Commissioner, FDA

Carla Epps is a board-certified pediatrician. She joined the FDA in 2009 and currently serves as a medical officer in the Office of Pediatric Therapeutics. She has also served as a medical officer in the Division of Gastrointestinal and Inborn Errors Products (DGIEP) and in the Office of Orphan Products Development. She has been involved in a number of FDA efforts related to drug development for rare diseases, including development of guidances for industry, organization of educational programs on rare diseases for FDA staff, and participation in collaborative activities between FDA and external stakeholders to facilitate development of products for rare diseases.

LILI GARRARD, PhD, Center for Drug Evaluation and Research, FDA

Lili Garrard is a statistical reviewer on the Clinical Outcome Assessment (COA) Statistical Support Team at the Division of Biometrics III, Office of Biostatistics (OB), Office of Translational Sciences (OTS), Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration (FDA). Dr. Garrard primarily supports the Division of Gastroenterology and Inborn Errors Products (DGIEP) by providing statistical consultations and reviews related to the use of COAs. Dr. Garrard joined the FDA in 2016 from the University of Kansas Medical Center where she received a PhD in Biostatistics.

PAMELA GAVIN, National Organization for Rare Disorders

Pamela Gavin sets the strategic direction for National Organization for Rare Disorders (NORD) and implements programs and services that provide innovative solutions to address the needs of the rare disease community. She is responsible for bringing together all stakeholders within the rare disease space and works closely with NORD's board of directors, donors, corporate council and member organizations, other partners, and staff. Pam has led key multi-million dollar initiatives in both non-profit and for-profit environments to meet regulatory requirements, achieve operational effectiveness and improve health care services, patient outcomes, and patient safety. Pam has extensive experience implementing healthcare-related data systems and data standards and has been a longtime advocate for patient safety and rare diseases. Pam holds an MBA degree from Northeastern University and a BA in Biology from Smith College.

CRISTOPH HORNIK, MD, PhD, MPH, Duke Clinical Research Institute, Duke University

Christoph Hornik is devoting his professional life to improving drug and device labeling for critically ill infants and children. To accomplish this goal, Dr. Hornik is leveraging his advanced training in pediatric cardiopulmonary physiology, biostatistics, pharmaceutical sciences, and innovative clinical trial designs to develop novel and efficient methods to study drug dosing, efficacy, and safety. Dr. Hornik has implemented this vision through leadership of traditional prospective multi-center drug trials (5), real world databases (3), and novel study designs including registries. These projects, funded by both industry and government and executed under investigational new drug (or device) applications, several of which he personally holds, have provided regulatory-grade data for FDA label changes, and implemented several novel methods and approaches. Dr. Hornik's scholarly accomplishments include over 150 peer reviewed publications within my first 6 years on faculty. He has served as Protocol Development Principal Investigator of the NICHD funded Pediatric Trials Network (PTN; Network PI Benjamin, Duke), overseeing the development of innovative protocols within a network of >100 clinical sites that collectively enrolled >8000 children. As Chief of the Division of Quantitative Sciences, he led a team providing statistical analysis and other quantitative support services to investigators across the Department of Pediatrics at Duke University. As Associate Director of the Duke Clinical Research Institute Pharmacometrics

Center of Excellence, he oversees the day to day activities of a team of 3 PK/PD analysts, 4 PhD level pharmacometricians, 3 project leaders, 3 clinical pharmacology fellows, and 10 faculty that have collectively performed PK/PD analyses for >75 clinical trials.

JIN-SHEI LAI, MD, Northwestern University Feinberg School of Medicine

Jin-Shei Lai is a Professor of Medical Social Sciences and Pediatrics, Northwestern University Feinberg School of Medicine. Dr. Lai is a registered occupational therapist and a trained psychometrician. She is a full member of the Lurie Comprehensive Cancer Center at Northwestern University Feinberg School of Medicine, Proton Collaborative Group and the Pediatric Brain Tumor Consortium (PBTC). Dr. Lai has significant research experiences in quality of life, measurement, symptom monitoring and late effect due to childhood cancer and/or its treatment in chronic illness for both pediatric and adult populations. Dr. Lai is recognized as an expert in both outcomes measurement and Item Response Theory (IRT) and is the lead developer of the pediatric Functional Assessment of Chronic Illness scales, including Childhood Brain Tumor Survivor, Fatigue, Anorexia and Cachexia, and Cognition. She has been integral in the development of item banks using IRT to measure perceived cognitive function and fatigue for both children and adults with chronic conditions for NIH PROMIS and NINDS Neuro-QoL measurement systems.

BRADLEY S. MARINO, MD, MPP, MSCE, FAAP, FACC, FAHA, Northwestern University Feinberg School of Medicine

Bradley Marino is a Professor of Pediatrics and Medical Social Sciences at the Feinberg School of Medicine at Northwestern University. Dr. Marino is the creator and Director of the Center for Cardiovascular Innovation in the Stanley Manne Children's Research Institute at Ann & Robert H. Lurie Children's Hospital (Lurie Children's). He is the Heart Center Co-Director of Research and Academic Affairs, Cardiology Inpatient Director and Medical Director of the Regenstein Cardiac Care Unit, and the Co-Director of the NICU-Cardiac Neurodevelopmental Program at Lurie Children's. He also leads the Cardiovascular Bridge Programs between Lurie Children's and Northwestern Medicine. At Northwestern University he serves as the Co-Director of the Neurodevelopmental Core within the Institute for Innovations in Developmental Sciences. Dr. Marino earned his medical degree from Harvard Medical School and a Master's degree in Public Policy from the John F. Kennedy School of Government at Harvard University. He completed his pediatric residency at Johns Hopkins Hospital and a combined fellowship in Pediatric Cardiology and Pediatric Critical Care Medicine at The Children's Hospital of Philadelphia. While at the University of Pennsylvania Dr. Marino completed a Master's of Science degree in Clinical Epidemiology at the Center for Clinical Epidemiology and Biostatistics. He is presently completing a Master's degree in Business Administration in Healthcare Management at the University of Texas-Dallas Naveen Jindal School of Management. Dr. Marino is an internationally recognized pediatric cardiovascular disease outcomes researcher. Dr. Marino's research interests focus on the impact of surgical and intensive care unit factors on mortality and morbidity, as well as, the impact of neurodevelopmental, psychosocial, and physical morbidities

on quality of life, functional status, and behavioral and emotional outcomes in the high-risk complex congenital heart disease population. Dr. Marino is a national leader and advocate for children with congenital and acquired heart disease.

JANET MAYNARD, MD, MHS, Office of the Commissioner, FDA

Janet Maynard, as the Director of the Office of Orphan Products Development (OOPD), oversees legislatively mandated designation and grant programs intended to promote the development of products for rare diseases including, orphan drug, rare pediatric disease, and humanitarian use device designation programs, as well as clinical trial, natural history study, and pediatric device consortia grant programs. In her role, she serves as FDA's lead in coordinating cross-cutting rare disease issues and engages extensively with patients, sponsors, and other stakeholders. Prior to OOPD, Dr. Maynard worked in the Center for Drug Evaluation and Research (CDER), where she was a clinical team leader in the Division of Anesthesia, Analgesia, and Addiction Products (DAAAP). Dr. Maynard has been with FDA since 2011, when she joined FDA's Division of Pulmonary, Allergy, and Rheumatology Products (DPARP) as a Medical Officer, before becoming a clinical team leader in DPARP. Dr. Maynard received her medical degree from Vanderbilt University and completed a residency in internal medicine at Duke Hospital. Subsequently, she completed a fellowship in rheumatology at Johns Hopkins Hospital. During her fellowship, she completed a Master of Health Science at the Johns Hopkins Bloomberg School of Public Health in the Graduate Training Program in Clinical Investigation.

SUSAN MCCUNE, MD, MAEd, FAAP, Office of the Commissioner, FDA

Susan McCune is the Director in the Office of Pediatric Therapeutics (OPT) in the Office of the Commissioner at the Food and Drug Administration (FDA). She joined the Agency in 2003 in the Division of Pediatric Drug Development, Office of Counter-Terrorism and Pediatric Drug Development, in the Center for Drug Development and Research (CDER). She was the Deputy Director in the Office of Translational Sciences in CDER from February, 2010, until January, 2017, when she joined OPT. Dr. McCune received her medical degree from George Washington University following her undergraduate degree at Harvard University. She completed her internship, residency, chief residency, and neonatal fellowship at Children's National Medical Center in Washington, D.C. She is Board Certified in Pediatrics and Neonatal/Perinatal Medicine. For 15 years, while practicing academic pediatric and neonatal medicine at Johns Hopkins and Children's National Medical Center, Dr. McCune continued her molecular biology research on adrenergic receptor ontogeny and expression in models of newborn brain injury in the Lab of Developmental Neurobiology, NICHD, NIH. In addition, she has a Masters in Education Technology Leadership from George Washington University, and certificates in Public Health from Georgetown and Regulatory Science from USC.

ALI MOHAMADI, MD, BioMarin Pharmaceutical

Ali Mohamadi, M.D. is the Executive Director of Patient Advocacy at Biomarin Pharmaceutical. Ali is a pediatric endocrinologist who joined BioMarin in September, 2015, originally as Senior Director of Regulatory Patient Engagement and Policy and now in his current role. At BioMarin, Ali and his team work to incorporate the patient's voice into the company's clinical development programs, connecting with patients and advocates to advance therapies for rare diseases. Prior to BioMarin, Ali spent 6 years at the FDA, where he worked initially as a clinical reviewer and team lead in the Division of Metabolism and Endocrinology Products, then as a lead medical officer in CDER's Professional Affairs and Stakeholder Engagement staff. Ali completed his undergraduate studies at Yale University, followed by medical school at George Washington University, pediatric residency and chief residency at Mount Sinai School of Medicine, and pediatric endocrinology fellowship at Johns Hopkins School of Medicine. He is based in Washington, D.C, where lives with his wife and four children.

TONYA PALERMO, PhD, Seattle Children's Research Institute

Tonya Palermo is a pediatric psychologist and Professor of Anesthesiology and Pain Medicine at University of Washington with adjunct appointments in Pediatrics and Psychiatry. She also serves as Associate Director of the Center for Child Health, Behavior and Development at Seattle Children's Research Institute. he directs the Pediatric Pain & Sleep Innovations Lab that aims to improve the lives of children with pain and their families. Her NIH funded research investigates behavioral, psychosocial and family factors that affect pain experiences, and development and evaluation of innovative psychological treatments that can be delivered at low cost. Dr. Palermo has published over 200 peer-reviewed articles and two books on cognitive-behavioral therapy for chronic pain in children and adolescents. Dr. Palermo directs a T32 anesthesiology postdoctoral training program and is active in training clinician-scientists at the postdoctoral and junior faculty level. Dr. Palermo is Editor-in-Chief for the *Journal of Pediatric Psychology* and Associate Editor for *PAIN* and has been elected Fellow of the American Psychological Association. She previously served on the Federal Pain Research Strategy Chronic Pain work group, and presently is a member of the CSR Advisory Council.

PATROULA SMPOKOU, MD, FACMG, Center for Drug Evaluation and Research, FDA

Patroula Smpokou is a clinical team leader (lead medical officer) in the Division of Gastroenterology and Inborn Errors Products (DGIEP) at FDA's Center for Drug Evaluation and Research where she is involved in the review and regulation of products intended for the treatment of rare, biochemical genetic diseases known as inborn errors of metabolism (IEM). She received her medical degree from the University of South Florida and completed her residency in pediatrics at Yale-New Haven Children's Hospital and fellowship in clinical genetics & genomics at Harvard Medical School. She is board certified in general pediatrics and in clinical genetics and genomics. Prior to joining FDA, she practiced pediatric clinical genetics at Children's National Medical Center in Washington, DC and held an academic appointment as Assistant Professor of Pediatrics at The George Washington University School of Medicine &

Health Sciences. Her professional interests and expertise focus on the complex scientific and regulatory aspects of drug development in rare genetic diseases including the inborn errors of metabolism.

CHARLES THOMPSON, MD, FAAP, Pfizer Pediatric Center of Excellence

Charlie is a board-certified pediatrician who earned his M.D. at the University of Connecticut School of Medicine and completed his pediatric residency at Connecticut Children's Medical Center in Hartford. He is a Clinical Instructor of Pediatrics at the University of Connecticut School of Medicine and is a member of the medical staff at Connecticut Children's Medical Center. Currently, Charlie is the Global Lead for the Pfizer Pediatric Center of Excellence. Throughout his 22+ year career at Pfizer, Charlie has taken on diverse roles in clinical development, clinical safety/risk management, and field medical affairs. Charlie is also the Immediate Past Chair of the Executive Committee for the American Academy of Pediatrics Section on Advances in Therapeutics and Technology, a member of the Board of Directors for the Hezekiah Beardsley Connecticut Chapter of the American Academy of Pediatrics, and the Founder and Chair of iCAN (International Children's Advisory Network). He is serving his second term as a governor-appointed member of the Connecticut Pharmaceutical and Therapeutics committee and has served on the State of Connecticut Immunization Task Force following an appointment by the Speaker of the House. In his community, Charlie is an active youth leader, mentor, and sports coach. Charlie lives in Florida, USA, with his wife, Heather, and four children.

ROBBERT ZUSTERZEEL, MD, PhD, MPH, NEST Coordinating Center

Robbert Zusterzeel, MD, PhD, MPH is the Data Network Director for the NEST Coordinating Center. He joins MDIC/NESTcc from the U.S. Food and Drug Administration (FDA). Dr. Zusterzeel earned his M.D. and Ph.D. from Maastricht University, The Netherlands as well as a Master of Public Health (MPH) in epidemiology from Harvard University. He has published numerous peer-reviewed journal articles related to the safety and effectiveness of medical devices and drugs and predicting individualized response to therapies using and creating study data across the full product lifecycle. At MDIC/NESTcc he is responsible for overseeing the Data Network, Test-Cases, Data Quality and Methods as well as Active Surveillance activities.