INC
CRITICAL PATH INSTITUTE

2021 International Neonatal Consortium Scientific Meeting

Thank You
to our 2021 Speakers and Panelists

Gerri Baer, MD, Medical Officer and Team Leader, Pharmacovigilance and Neonatology in the Office of Pediatric Therapeutics, FDA

Dr. Baer completed pediatric residency and chief residency at Mount Sinai Medical Center and worked as a NICU hospitalist before starting her neonatology fellowship training at the Children's Hospital of Philadelphia. Prior to joining the FDA in September of 2015, she worked for eight years as an attending neonatologist in a level III NICU in Silver Spring, Maryland. She was a partner in the practice and site lead for the Vermont-Oxford Very Low Birth Weight Neonatal Database.

At FDA, she established the Neonatal-Perinatal Medicine consultation service and is involved with neonatal product development across the Centers. She now leads the OPT Pharmacovigilance team, which works with the Centers to improve pediatric product safety. Dr. Baer serves on the coordinating committee of the International Neonatal Consortium and represents the FDA on several INC working groups. She participated in creating the "Draft Guidance for Industry: General Clinical Pharmacology Considerations for Neonatal Studies for Drugs and Biological Products" with CDER OND and OTS and is working on additional guidance and regulatory science research.

Jeff Barrett, PhD, FCP, Senior Vice President and Rare Disease Cures Accelerator - Data Analytics Platform Lead, C-Path

Dr. Barrett serves as a critical liaison between C-Path and the pharmaceutical industry, foundations and other key stakeholders, helping grow C-Path's portfolio in drug development solutions, with a focus, but not limited to model-informed drug development (MIDD) and real-world data (RWD) technologies. Jeff was previously Head of Quantitative Sciences at the Bill & Melinda Gates Medical Research Institute. In this role he was responsible for implementing model-based drug development, employing PK/PD modeling, statistics, and clinical trial simulations to advance the discovery and development of new medicines and vaccines. Prior to MRI, he was Vice President, of Translational Informatics at Sanofi Pharmaceuticals. He led various aspects of model-based decision-making spanning and provided leadership for Sanofi's cloud-based, high-performance computing and "big data" initiatives. Jeff spent 10+ years at the University of Pennsylvania where he was Professor, Pediatrics and Director, Laboratory for Applied PK/PD at the Children's Hospital of Philadelphia. Jeff received his B.S. in Chemical Engineering from Drexel University and Ph.D. in Pharmacokinetics from University of Michigan. He has co-authored over 175 manuscripts, is fellow of ACCP and AAPS and the recipient of numerous honors including ACCP awards for Young Investigator (2002) and Mentorship in Clinical Pharmacology (2007) and the AAPS Award in Clinical Pharmacology and Translational Research (2011). Dr. Barrett was awarded for Exceptional Innovation and Advancing the Discipline of Pharmacometrics at the International Society for Pharmacometrics (2013) and elected ISOP Fellow (2017). He is co-Specialty Chief Editor of Frontiers in Obstetric and Pediatric Pharmacology Journal and an active member of the Child Health and Human Development Pediatrics Subcommittee as a study section reviewer. He was a past acting chair of the FDA Advisory Committee for Pharmaceutical Science and Clinical Pharmacology; a voting member of the committee for eight years.

Alison Bateman-House, PhD, MPH, MA, Assistant Professor, Division of Medical Ethics at NYU Grossman School of Medicine

Dr. Bateman-House is co-chair, with Arthur Caplan Ph.D., of the Working Group on Compassionate Use and Preapproval Access (CUPA), an academic group that studies ethical issues concerning access to investigational medical products and which is composed of patient advocates, clinicians, members of industry, former FDA staffers, lawyers, and academics. Dr Bateman-House also co-chairs, with Lesha Shah MD, the Pediatric Gene Therapy and Medical Ethics (PGTME) working group, which includes academics, patient advocates, industry representatives, and a wide array of clinical and research professionals. She advises a wide array of biopharmaceutical companies, patient advocacy organizations, governmental and non-governmental entities about clinical trial design and non-trial access programs, and she serves as ethicist for three data safety monitoring boards overseeing clinical trials. Dr. Bateman-House also serves as the non-voting, non-paid chair of the NYU/Janssen Pharmaceutical Compassionate Use Advisory Committees (CompACs) for Infectious Diseases and Neurology/Psychology. CompAC won the Reagan-Udall Foundation for the FDA's 2019 Innovation Award. She has published and spoken extensively on non-trial access to investigational drugs, on clinical trial accessibility, on the history and ethics of using humans as research subjects, and on public health ethics.

Ralph Bax, MD, Head of the Paediatric Medicines Office, EMA

After studying medicine and health care management in Germany, Ralph Bax completed his training as a paediatrician at the Children's University Hospital in Munich/Germany. His research focused on the area of EEG and brain haemodynamics in preterm neonates, he specialized in neonatology and neonatal neurology and lead the department of developmental neurology. In 2007, he joined the paediatric team at the European Medicines Agency (EMA) supporting the Paediatric Committee (PDCO) assessing paediatric investigation plans mainly in neonatology, neurology and child psychiatry and working on public health issues such as unmet paediatric needs for medicines, the Paediatric Regulator Network (WHO) and the International Neonatal Consortium (INC). Dr Bax is the EMA contact point for neonatology and since beginning of 2017 Head of the Paediatric Medicines Office at EMA.

Andrew A. Bremer, MD, PhD, Chief of the Pediatric Growth and Nutrition Branch within the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD)

Dr. Andrew A. Bremer joined the National Institutes of Health (NIH) as a Medical Officer in November 2013 within the National Institute of Diabetes and Digestive and Kidney Diseases' Division of Diabetes, Endocrinology, and Metabolic Diseases, and was appointed in May 2018 to be the Chief of the Pediatric Growth and Nutrition Branch within the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD). He is a board-certified internist, pediatrician, and pediatric endocrinologist, and has a Ph.D. in pharmacology. Prior to joining the NIH, he was an Associate Professor of Medicine and Pediatrics at the Vanderbilt University School of Medicine. His areas of interest and expertise include pediatric and adult endocrine disorders, the impact of maternal diet and gestational obesity/diabetes on fetal programming, the developmental origins of health and disease, the impact of the intrauterine environment on long-term maternal and child health outcomes, childhood obesity, and the role of nutrition in development throughout the life cycle. He is also the Acting Chief of the Pregnancy and Perinatology Branch at NICHD and the Program Official for NICHD's Neonatal Research Network, Maternal-Fetal Medicine Units Network, and Global Network for Women's and Children's Health Research. Furthermore, he is a Co-Chair on the National Collaborative on Childhood Obesity Research Steering Committee, the NIH Liaison to the American Academy of Pediatrics' Committee on Nutrition and Section on Obesity, and a Senior Liaison to the NIH Office of Nutrition Research.

Megan Cala, PhD, Postdoctoral fellow of Pharmacometrics, Quantitative Medicine Program, C-Path

Megan Cala, Ph.D., is a postdoctoral fellow of pharmacometrics within the Quantitative Medicine Program at the C-Path. She is a chemical engineer by training from the University of Pittsburgh where her research was focused on developing a multiphase and multiscale computational model of blood coagulation. At C-Path, she is developing quantitative solutions such as disease progression models, placebo response models, and model-based clinical trial simulators using pharmacometrics methods to advance drug development. Her efforts are primarily focused on developing models and simulations to describe and quantify the biological variability of disease progression in Friedreich's Ataxia and bronchopulmonary dysplasia. She also provides quantitative analytical expertise to support the development of the Rare Disease Cures Accelerator Data and Analytics Platform (RDCA-DAP).

Chip Chambers, MD, Founder and President of the Board of Directors, DADA2 Foundation

As a physician and educator with years of experience in the business sector of healthcare, he is unusually well prepared to lead the DADA2 Foundation that forges productive and innovative partnerships between clinicians, researchers, and patients. He is a compelling advocate for rare disease patients, a tireless networker, and the co-author of multiple scientific papers on Deficiency of Adenosine Deaminase 2 (DADA2). Dr. Chambers two children were diagnosed with the rare disease DADA2 in March 2014, just one month after the first articles describing the disease were published in the New England Journal of Medicine. Dr. Chambers is the former Chief of Endocrine Surgery at Vanderbilt University Medical Center in Nashville, Tennessee, where he continues to hold his clinical faculty appointment. He received his M.D. from the University of Mississippi. He was recently named to the newly created Tennessee Rare Disease Advisory Council by Governor Bill Lee.

John Concato, MD, MS, MPH, Associate Director for Real-Word Evidence Analytics, Office of Medical Policy (OMP), Center for Drug Evaluation and Research (CDER), FDA

John Concato is the Associate Director for Real-Word Evidence Analytics in the Office of Medical Policy (OMP) at the Center for Drug Evaluation and Research (CDER), US Food and Drug Administration. In seeking to enhance policies related to drug development and regulatory review, his responsibilities involve coordinating CDER's real-world evidence (RWE) Program, serving as Chair of the RWE Subcommittee, supporting RWE guidance development and demonstration projects, interacting with external stakeholders regarding RWE, and developing internal Agency processes related to RWE. He also supports other activities and initiatives in the Office of Medical Policy Initiatives (OMPI). Prior to joining FDA in 2019, his career focused on generating research as an independent investigator and research center director at Yale University School of Medicine and the U.S. Department of Veterans Affairs (VA), including serving as one of two founding principal investigators of the VA Million Veteran Program. He received M.D. and M.S. degrees from New York University and an M.P.H. degree from Yale University.

Jonathan Davis, MD, Vice-Chair of Pediatrics and Chief of Newborn Medicine, Floating Hospital for Children at Tufts Medical Center; Professor of Pediatrics at Tufts University School of Medicine

Dr. Davis' research has focused on neonatal drug development for the prevention and treatment of a variety of neonatal conditions. He has authored over 200 manuscripts and book chapters and received numerous grant awards from the NIH, the FDA, the March of Dimes, and many others. He is currently funded by NIH to develop better outcome measures for clinical trials and new and existing therapeutics to improve outcomes in children. Dr. Davis is Chair of the Neonatal Advisory Committee in the Office of the Commissioner at FDA, the Director of the International Neonatal Consortium (FDA, EMA), and a member of Pediatric Policy Council representing the American Pediatric Society. These positions permit him to work closely with governments, academic leaders, industry and families worldwide to promote the development of important therapeutics for infants and children.

Deb Discenza

Mrs. Discenza is the mom to Becky, a 30-week twice exceptional preemie now 17 years old. Combining fields in technology and publishing, she has created PreemieWorld.com as the go-to space for education, support and resources for the preemie community including the acclaimed book, The Preemie Parent's Survival Guide to the NICU. Along with this she is vested in premature infant outcomes with a global patient-reported data portal, Preemie Crystal Ball that is forthcoming. And in speaking about racial and healthy equity for years, the latest round of disparities and inequality led Deb to co-found the non-profit the Alliance for Black NICU Families. She sits as a founding member and steering committee member of the National Premature Infant Health Coalition and a founding member and former Leadership team member of the NICU Parent Network, and was recently honored with a role on INC's Leadership Team. Deb is also a columnist with Neonatal Network's Neonatal Network Journal and for Neonatal Intensive Care magazine.

Most importantly she gives a heartfelt thank you to all who are attending today who have had a part in developing the care, the devices, the protocols and the pharmaceuticals that saved her daughter's life.

Maria Escolar, PhD, Professor of Pediatrics and Neurodevelopmental Disabilities Pediatrician, University of Pittsburgh

Dr. Escolar began her research on rare neurodegenerative disorders in 2000, when she established the Program for the Study of Neurodevelopment in Rare Disorders (NDRD) at The University of North Carolina (UNC) at Chapel Hill, comprehensive and multidisciplinary since inception. It aims to improve the lives of children and families living with rare neurodegenerative disorders. From 2010 to 2011, Dr. Escolar served as Associate Director for Translational Research at UNC Gene Therapy Center under the direction of Dr. Jude Samulski one of the pioneers of AAV gene therapy.

Dr. Escolar is best known for her work focused on rare, genetic, neurodegenerative diseases: such as Krabbe, MLD, & ALD leukodystrophies, Mucopolysaccharidosis disorders (MPS I, II, and III), Gaucher, GM1, GM2, Nieman Pick C, Mucolipidosis, Neuronal ceroid lipofuscinosis, Alpha-mannosidosis, neurodegeneration with brain iron accumulation (PKAN), vanishing white matter disease and many others.

Dr. Escolar is also passionate about training the new generation of health care providers that wish to develop careers in translational research of rare neurodevelopmental disorders.

Since the creation of the NDRD, Dr. Escolar has published over 80 original research papers in peer-reviewed journals, including two influential publications in the New England Journal of Medicine. She explores identifiers of disease progression that can predict long-term outcomes. She is interested in biomarker discovery and the use of standardized neurobehavioral, neuroradiological, and neurodevelopmental markers to determine the extent of disease progression in patients suffering from rare diseases. Dr. Escolar has strong interests in developing novel quantitative neuroimaging techniques that allow for longitudinally tracking changes in the brain throughout the disease process. She has been funded by the NIH to develop neuroimaging tools for newborn screening. In parallel, her focus on generating atlases of normal brain development allows for comparison between normal and aberrant neurological development and further contributes to the scientific community's understanding of abnormal brain morphology.

Lastly, Dr. Escolar is a leader in clinical trial design with a specific expertise in gene therapy trials. She has served as site PI for multiple gene therapy trials, including trials for MLD, Hunter's Syndrome, Sanfilippo Syndrome, Gaucher and GM2. Dr. Escolar has also led the development of a novel therapy for Krabbe disease, a combination treatment of hematopoietic stem cell transplantation and adeno-associated virus gene therapy. Forge Biologics Inc. has licensed the therapy and advanced it to a Phase I/II clinical trial. Additionally, Dr. Escolar is now serving as their Chief Medical Officer.

Laura Fabbri, PhD, Head of Clinical Neonatology, Global Clinical Development, R&D Chiesi Farmaceutici

Dr. Fabbri has been working in Chiesi Pharmaceutical for 19 years, at Global Clinical Development department; for the first four years as Neurology and Psychiatry Clinical Area Manager and then up to now, she is covering the role of Head of Neonatology Clinical Unit. Previously Laura worked at GlaxoSmithKline for 14 years, engaged in clinical product developments at local level (Verona Italy) and at corporate level for one year (Greenford-UK). She has large experience in the coordination and management of different clinical team groups to deliver clinical projects on phase I-IV drugs, nationally and worldwide, in writing clinical development plans and single clinical protocols, study reports and manuscripts for national and international journals; furthermore, always collaborates in writing the clinical sections of Pediatric Investigational Plans and other documents for EMA or FDA or other Agencies.

Laura has consolidated relationships with worldwide neonatologists, because of reciprocal faith and friendship due to the long- lasting collaboration; she is member of the International Neonatal Consortium, committed to organizational plans processes, improvement and global standardization in neonatology research.

Deborah R. Gill, PhD, Professor of Gene Medicine, University of Oxford

Dr. Gill completed her Ph.D. in molecular microbiology at the University of Warwick, UK, studying cell division proteins in E. coli, during which she discovered the defining bacterial member of the ABC (ATP-Binding Cassette) superfamily of proteins. In 1990, she moved to the University of Oxford, to undertake post-doctoral research under the mentorship of Professor Chris Higgins at the Weatherall Institute for Molecular Medicine, investigating human ABC proteins including the Multi-Drug Resistance p-glycoprotein and CFTR, the protein responsible for Cystic Fibrosis (CF). Her research developed to investigate novel gene therapies, in particular for treating Cystic Fibrosis lung disease. Two clinical trials in CF patients were undertaken in collaboration with Sir Martin Evans, and Professors Bill College and Alan Cuthbert at Cambridge University, demonstrating proof of principle for non-viral (plasmid-based) CF gene therapy in the upper airways.

As a director of the Gene Medicine Group at Oxford University, she was also a founding member of the UK CF Gene Therapy Consortium in 2001, joining with the Centre for Molecular Medicine and the Roslin Institute, at Edinburgh University in Scotland and the Department of Gene Therapy, Imperial College London. This Consortium completed the world's largest gene therapy trial - a phase ll b study involving 136 participants with CF - delivering an aerosol of non-viral gene therapy at monthly intervals for 12 months. The study showed a clinically relevant improvement in lung function in those receiving gene therapy, compared with those receiving a placebo treatment; this provided proof-of-concept for gene therapy for CF lung disease. This Consortium of scientists and clinicians continues to work towards making gene therapy a reality for patients with CF, now focusing on a lentiviral vector that is highly efficient for gene delivery to the lung and provides long-term gene expression. The team is now evaluating the potential of this lentiviral vector to deliver genes for other lung diseases, such as the rare lung surfactant deficiencies caused by mutations in the SFTPB and ABCA3 genes.

Elizabeth Hart, MD, Branch Chief of General Medicine Branch 1, Office of Tissue and Advanced Therapies (OTAT), Center for Biologics Evaluation and Research (CBER), FDA.

Dr. Hart completed her undergraduate medical training at the University of Pennsylvania, a residency in pediatrics at Rainbow Babies and Children's Hospital, and a fellowship in pediatric endocrinology at Boston Children's Hospital. She provided clinical care, taught and conducted clinical and translational research prior to joining the FDA in 2014. Upon joining the FDA, she initially served as a medical officer in the Division of Gastroenterology and Inborn Errors Products where she developed expertise in innovative clinical trial design and analysis for rare pediatric diseases. She joined OTAT in 2019 and currently supervises a team of medical officers who are responsible for regulating cellular and gene therapies for a variety of medical conditions, including pediatric rare diseases. Dr. Hart has served on multiple committees within CDER, CBER, and as part of FDA-NIH collaborations, and she has authored and edited multiple FDA guidances, book chapters, and presented at national and international meetings.

Kanwaljit Singh, MD, MPH, Executive Director, International Neonatal Consortium, C-Path

In addition to INC, Dr. Singh also manages the Pediatric Medical Device pre-consortium at C-Path. Dr. Singh comes to C-Path from University of Massachusetts Medical School (UMass), Worcester, MA, where he worked for more than seven years as Instructor of Pediatric Neurology. At UMass, Dr. Singh's research focused on evaluating novel treatment options for Autism Spectrum Disorders (ASD) and evaluating the safety and efficacy of a small molecule (Sulforaphane) present in broccoli sprouts in the treatment of ASD. Dr. Singh has also done extensive research and has numerous publications in pediatric epilepsy. Before UMass, he worked in ASD research at Lurie Center for Autism at Massachusetts General Hospital/Harvard Medical School in Boston. In addition to his research work, Dr. Singh also served on the IRB committees at Harvard Medical School and interacted with regulatory authorities, including the FDA. His experience also includes the testing of medical devices in hypertension and diabetes mellitus.

Stephen F. Kingsmore, MD, DSc, President and CEO Rady Children's Institute for Genomic Medicine, San Diego, CA

Stephen Kingsmore, MD, DSc is the founding President and CEO of Rady Children's Institute for Genomic Medicine (RCIGM) where he leads a multi-disciplinary team of scientists, physicians and researchers who are pioneering the use of rapid and ultra-rapid Whole Genome Sequencing (WGS) to diagnose rare genetic disorders and guide personalize care for critically-ill newborns and children. He is an expert in genomic and systems medicine research. The Institute is a non-profit, research organization embedded within Rady Children's Hospital- San Diego. RCIGM has developed a clinical Rapid Precision Medicine™ delivery system optimized for speed and accuracy. In medically urgent cases, RCIGM returns preliminary clinical results in less than three days.Dr. Kingsmore came to Rady Children's from Children's Mercy Kansas City, where he was the Founding Director of the Center for Pediatric Genomic Medicine. He also held the Dee Lyons/Missouri Endowed Chair in Genomic Medicine at the University of Missouri-Kansas City School of Medicine. In his distinguished career, Dr. Kingsmore has also served as President and CEO of the National Center for Genome Resources; COO of Molecular Staging Inc.; Vice President of Research at CuraGen Corporation; founder of GatorGen; and Assistant Professor at the University of Florida's School of Medicine. Dr. Kingsmore received MB ChB BAO and DSc degrees from the Queen's University of Belfast. He trained in clinical immunology in Northern Ireland and did residency in internal medicine and fellowship at Duke University Medical Center. He is a fellow of the Royal College of Pathologists.

In 2019, the National Human Genomic Research Institute cited the publication by RCIGM on the use of artificial intelligence to accelerate WGS to diagnose genetic diseases in seriously ill children among the year's 10 most significant advances in genomic medicine. Recently, Dr. Kingsmore and the team broke their own world speed record for the fastest molecular diagnosis using whole genome sequencing by reducing the time to 13.5 hours.

Nicole LaMarca, DNP, MSN, CPNP, PMHS, Global Medical Director & Data Exploration and Generation lead, Novartis Gene Therapies

Nicole LaMarca, DNP, MSN, CPNP, PMHS is a Global Medical Director & Data Exploration and Generation lead at Novartis Gene Therapies. In addition, Dr. LaMarca is currently an assistant clinical professor at Columbia University School of Nursing in New York City. Prior to joining Novartis Gene Therapies, Nicole led the interdisciplinary person and family-centered care team at Columbia University's Pediatric Neuromuscular Center and was a co-investigator at the Spinal Muscular Atrophy Research Center for over nine years. Dr. LaMarca co-led the Neuromuscular Newborn Screening Program at Columbia and collaborated to develop the newborn screening pilot for SMA. Dr. LaMarca has extensive experience working with families who have received a positive newborn screen, connecting the family to appropriate care teams quickly, and providing treatment and/or possibly enroll in clinical trials.

At Novartis Gene Therapies, Dr. LaMarca serves as a Global Medical Director leading the Medical Affairs Data Generation team. She maintains in-depth understanding of the disease state, product, competitors, marketplace, related medical areas, and regulatory guidelines. Nicole is involved in the strategic development and tactical execution of SMA therapeutic area gene therapy strategy.

John Lantos, PhD, Glasnapp Family Foundation Chair in Bioethics, Children's Mercy Hospital

At Children's Mercy Hospital, Dr. Lantos created and directs the Children's Mercy Bioethics Center, a research center focused on ethical issues in pediatric clinical care, research, and health policy. Prior to his current work at Children's Mercy, he was a Professor at University of Chicago where he was Division Chief of General Pediatrics and Associate Director of the MacLean Center for Clinical Medical Ethics. He is a Past President of the American Society of Bioethics and Humanities. He Co-Chaired the Clinical Trials Advisory Panel of the Patient-Centered Outcomes Research Institute, served on President Clinton's Health Reform Task Force, and received the Bartholome Award from the American Academy of Pediatrics. He has authored over 400 peer-reviewed papers and ten books.

Dr. Collin Hovinga, PharmD, Senior Vice President of Clinical and Scientific Affairs, I-ACT

Dr. Hovinga earned his Bachelor of Science and Doctor of Pharmacy degrees from Creighton University in Omaha, Nebraska. He completed a Residency and Fellowship in Pediatric Pharmacotherapy with emphasis in Pediatric Neuroscience at the University of Tennessee, Memphis, LeBonheur Children's Medical Center. He completed a second a Fellowship at the Food and Drug Administration Office of Clinical Pharmacology and a Masters of Epidemiology from the University of Tennessee Health Science Center. He has additional training in outcomes research from MD Anderson Cancer Center. Dr. Hovinga is recognized as an expert in pediatric pharmacology and has held appointments with the FDA's Peripheral and Central Nervous System Advisory Committee. Dr. Hovinga has had key roles in pediatric interventional clinical trials and neuroprotective drug pharmacology NIH projects. He was most recently Chair of ACCP's Practice Based Research Advisory Committee. He has been active as a leader in practice-based guidelines and evidence-based medicine projects for the American Academy of Neurology and American Epilepsy Society. He is a recognized consultant in the conduct of pediatric clinical trials, with expertise in ethical and methodological issues, as well as patient reported outcomes. Dr. Hovinga's understanding of developmental pharmacology and pediatric medical conditions has made him unique in the pharmacology arena and has led to him being a journal reviewer and an invited speaker at many national organizational meetings. He is active as a board member and advocate in many pediatric rare medical conditions and is currently a Clinical Associate Professor at the University of Texas at Austin.

Satoshi Kusuda, MD, Neonatologist, Kyorin University

Satoshi Kusuda is a neonatologist working at Kyorin University. He graduated from medical school in Osaka, Osaka City University. After completion of a residency in pediatrics, he started specialty training at Children's Hospital at Osaka City and completed his training at Osaka City General Hospital. He is on the Board of Japanese Society for Perinatal and Neonatal Medicine. He is also a President-elect of the Federation of Asia and Oceanian Perinatal Societies. His primary research interests include neonatal intensive care and quality improvement in NICU. To improve the outcomes in preterm infants through quality improvement activities, he has participated in the joint activity among neonatologists and epidemiologists to establish the Neonatal Research Network of Japan in 2003. Since then he has been working as an active member of the network and he became the president of the network in 2013. The network is running the database and also funding several neonatal research projects. The number of very preterm infants registered on the network database has exceeded 7,000 and more than 70 peer reviewed manuscripts analyzing the network database were already published. Because of this background, he is keen to contribute to the INC activities. Especially, he has been involved in working groups of databases and terminology. Furthermore, he has a great interest to develop a system for collecting RWD in Japan. Please visit to see his activity at http://plaza.umin.ac.jp/nrndata/indexe.htm.

Jill Maron, MD, Chief of Pediatrics at Women & Infants Hospital; Oh-Zopfi Endowed Chair of Pediatrics at Brown University

For nearly two decades, Dr. Maron's research has focused on the integration of novel testing platforms into newborn care. Specifically, she works to identify biomarkers in noninvasively obtained biofluids, such as saliva, to monitor development and pathology in the premature neonate with subsequent development of diagnostic assays for use in this population. She also serves as an MPI on a NIH-funded multi-center award integrating rapid genomic testing platforms into the care of critically ill newborns (the GEMINI Study) about which she will speak today. Her research has been funded by NIH since 2009.

Thomas F. Miller, PhD, Vice President & Global Head, Pediatric Development for Bayer's Pharmaceutical Division

In total, Dr. Miller's career in the life science industry spans more than 25 years, with a primary focus on the development of therapeutics, medical devices and combination products for infirmed children. He is a respiratory physiologist with a primary interest in translational and clinical research for pediatric critical care patients. Prior to joining Bayer, Dr. Miller served in the capacity of Chief Executive Officer of Therabron Therapeutics, Inc. with oversight of all operational functions for the company (R&D, manufacturing, quality, regulatory affairs, etc.). While at Therabron, he oversaw their clinical program through Phase 2 completion for their lead molecule (orphan pediatric disease) and secured both the Rare Pediatric Disease and Fast Track designations for this program. Prior to Therabron, Dr. Miller served in the capacity of Chief Operating Officer of Discovery Laboratories, Inc. During his tenure, the company successfully secured marketing authorization for their first approved therapeutic (rare pediatric disease), successfully registered their first medical device (pediatric drug delivery enabling technology) and advanced multiple pediatric-centric pipeline programs into the clinic. Earlier, Dr. Miller served in operational roles of increasing responsibility at Pfizer, Novartis, BASF Pharma, and Johnson & Johnson. He has authored several peer-reviewed publications, given numerous presentations at scientific symposia and is an inventor with an issued patent in the field of pediatric respiratory drug delivery.

Neena Modi, PhD, Professor of Neonatal Medicine, Imperial College London

Dr. Modi is Professor of Neonatal Medicine at Imperial College London, one of the world's top ten universities, and Consultant at Chelsea and Westminster NHS Foundation Trust.

She is president of the British Medical Association, immediate past-president of the UK Medical Women's Federation, past-president of the UK Royal College of Paediatrics and Child Health, and president-elect of the European Association of Perinatal Medicine. She has had several previous academic leadership roles that include president of the UK Neonatal Society, president of the Academic Paediatrics Association of Great Britain and Ireland, and Chair of the British Medical Journal Ethics Committee.

Dr. Modi is a trustee of the charities Theirworld and Action Cerebral Palsy, a patron of Keep Our NHS Public and Health Prom, and an elected fellow and member of council of the UK Academy of Medical Sciences.

She leads a multidisciplinary research group focused on improving the care and life-long health of preterm and sick newborn babies. She is a strong advocate for a publicly funded, publicly delivered National Health Service that is committed to the values of equity, effectiveness, efficiency, quality, and compassion.

Michael Padula, MD, MBI, Medical Director of Informatics, Division of Neonatology, Children's Hospital of Philadelphia

Dr. Padula is an Associate Professor of Clinical Pediatrics at the Perelman School of Medicine and a Senior Fellow of the Institute for Biomedical Medical Informatics at the University of Pennsylvania. He serves as the Co-chair of the Terminology Work Group of the International Neonatal Consortium, Vice Chair of the Children's Hospitals Neonatal Consortium (CHNC), and Co-chair of the Patient Care Work Group of Health Level Seven. He has led several efforts promoting the development of health information standards and terminology harmonization for neonatal and pediatric research.

Jagdeep Podichetty, PhD, Director of Predictive Analytics, C-Path

Dr. Podichetty has a PhD in chemical and biomedical engineering and postdoctoral training in computational biology and artificial intelligence. He has experience working in academia and pharmaceutical industry in both research and manufacturing of pharmaceutical products. He has received several awards for his research and leadership. Recently, he received the ACoP quality award for his work with Type 1 Diabetes. He has numerous publications in peer reviewed journal and conference proceedings. He is also an active member of the International Society of Pharmacometrics and American Society for Clinical Pharmacology and Therapeutics.

Sheuli Porkess, MRCP, FFPM, GFMD, Chair of the Policy and Communications Group and Vice-President Elect, Faculty of Pharmaceutical Medicine; Director, Actaros Consultancy

Dr. Porkess is an experienced pharmaceutical physician with industry expertise in medicines development, medical affairs and research policy within the UK and internationally, across multiple therapy areas. Porkess holds a number of roles including member of the scientific advisory board for C2-Ai, an AI in healthcare company, and membership of expert groups for COVID therapeutics, the NIHR Multiple Long-term conditions (AIM) Programme and the UK Pandemic Ethics Accelerator. Sheuli's career began in clinical medicine in the NHS in the UK. Heis a Fellow of the Faculty of Pharmaceutical Medicine in the UK and a Global Fellow in Medicine Development with the International Federation of Associations of Pharmaceutical Physicians and Pharmaceutical Medicine.

Cynthia (Cindy) Prows, MSN, APRN, Genetics Clinical Nurse Specialist, Cincinnati Children's Hospital Medical Center (CCHMC) and a Fellow in the American Academy of Nursing

Prows' career has focused on translation of genetic/genomic information and technology into clinical practice. Translation efforts began with NIH education research funding used to create, implement and evaluate a national Genetics Education Program for Nursing Faculty that later transitioned to an online Genetics Education Program for Nurses. More recently, translation efforts have been focused on research in pharmacogenetics implementation and the return of children's genomic research results. Currently, she is a site principal investigator in the Phase IV Electronic Medical Record and Genomics (eMERGE) Network.

Heike Rabe, MD, Professor of Perinatal Medicine/Honorary Consultant Neonatologist, Department of Neonatology, Brighton & Sussex Medical School, University of Sussex

Prof. Rabe's research interests are neonatal brain, haematology, oxygen and microcirculation -- she is actively performing studies within these areas. Rabe is a member of several national and international scientific societies. She is a fellow of the Royal College of Paediatrics and Child Health (UK). As a member of the European Society for Paediatric Research/European Society for Neonatology (ESPR/ESN), she served as a section secretary from 1998 to 2004 and a member of the Council from 2001 to 2004. The Council of the ESPR/ESN seconded her in 2004 to act as the representative on behalf of the Society on the EU directive on Paediatric Drug Regulation. From 2007-2013 she was Vice President of the ESPR. From 2014-2015 she was president of the International Paediatric Research Foundation. She currently chairs the ESPR European Mentoring Scheme committees and is co-chairing the INC Hemodynamic Adaptation working group.

William Roddy, Data Engineering Team Lead, C-Path

William Roddy, currently leads the Critical Path Institute's data engineering team and develops tooling to support the moving and processing of data from a diversity of data sources with a focus on extracting evidence to improve patient care. Mr. Roddy has a background in neuroscience and experience from a variety of biomedical informatics projects where he led key components of each phase of the research data lifecycle. His previous work has included the technical and scientific design and implementation databases for natural history, interventional, and retrospective studies in the areas of pain management, traumatic brain injury, stress disorders, and limb loss. Currently Mr. Roddy focuses on developing the data pipeline and platform for the RDCA-DAP project and leveraging data in the areas of rare diseases and complications of premature birth.

Klaus Romero, MD, MS, FCP, Chief Science Officer, C-Path

Dr. Romero, a clinical pharmacologist and epidemiologist with more than 17 years combined experience in clinical research, translational research and development, pharmacometrics, modeling and simulation and pharmacoepidemiology, has been with C-Path since December 2007. During his tenure with C-Path, he has led clinical pharmacology, pharmacoepidemiology and modeling and simulation projects in Alzheimer's disease (AD), polycystic kidney disease (PKD), tuberculosis, type 1 diabetes, Parkinson's disease, Duchenne muscular dystrophy, kidney transplantation, Huntington's disease and cardiovascular drug safety. His work has helped to achieve major milestones, including the first regulatory endorsement by the U.S. Food and Drug Administration and European Medicines Agency of a clinical trial simulation tool for mild and moderate Alzheimer's disease and the regulatory qualification of the first imaging biomarker for PKD. Dr. Romero's scientific production with C-Path has resulted in more than 60 peer-reviewed publications related to his work with the Institute. Dr. Romero is a fellow of the American College of Clinical Pharmacology, a founding member of the International Society of Pharmacometrics, as well as a member of the American Society for Clinical Pharmacology and Therapeutics, and the International Society for Pharmacoepidemiology. In addition to his duties at C-Path, he serves as Chairman of the Board of Directors for CredibleMeds. Dr. Romero is also a Research Associate Professor at the University of Arizona College of Medicine, Adjunct Professor at the College of Health Solutions at Arizona State University, and Adjunct Professor at the University of Southern California's School of Pharmacy.

Thomas Salaets, MD, PhD, Pediatric Cardiology Fellow, UZ Leuven

Thomas Salaets is a pediatrician working in the University Hospitals of Leuven in Belgium. He recently successfully defended his PhD-dissertation entitled 'Towards New Therapies for Bronchopulmonary Dysplasia'. His research includes preclinical work on the pathophysiology and experimental treatment of BPD and pulmonary vascular disease, but also several projects on how to improve the conduct of neonatal clinical trials under supervision of Prof. Karel Allegaert. Thomas led the development of the INC Neonatal Adverse Event Severity Scale, a tool for standardization of adverse event severity reporting in neonatal clinical trials.

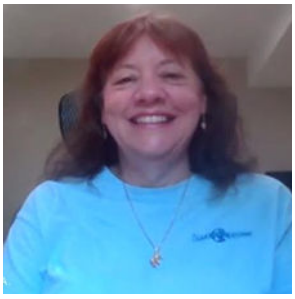
Richard Scott, MD, Chief Medical Officer, Genomics England

Dr. Scott is also a Consultant and Honorary Associate Professor in Clinical Genetics at Great Ormond Street Hospital for Children and the UCL Institute of Child Health where his practice focuses on diagnosing children with rare multisystem disorders. Richard trained in medicine at Cambridge University and University College London. He specialised in Paediatrics and subsequently Clinical Genetics in London and completed his PhD on childhood cancer syndromes at the Institute of Cancer Research. Through his clinical practice and in his role at Genomics England he is passionate about harnessing the power of new genomic technologies for the benefit of all patients in mainstream healthcare.

Laurent Servais, PhD, Professor of Pediatric Neuromuscular Diseases at the MDUK Oxford Neuromuscular Centre and Invited Professor of Child Neurology at Liège University

After graduating from Louvain Medical School, Brussels, Belgium in 1999, Dr. Servais completed a Ph.D. in Neuroscience (cerebellar electrophysiology in alert living mice) from Free University of Brussels, Belgium, followed by residencies in child neurology at the Free University of Brussels and Robert Debré Hospital, Paris. In 2008, he took a position in neuromuscular disease and clinical research at the Institute of Myology in Paris, where his interest and expertise in neuromuscular diseases flourished. He was subsequently appointed Head of Clinical Trials and Database Services. Most recently, he served as Head of the Institute of Myology's I-Motion (Institute of Muscle-Oriented Translational Innovation), and Head of the Neuromuscular Centre in Liège, Belgium. Dr. Servais has joined MD UK Oxford Neuromuscular Center and the University of Oxford in September 2019.

He has been involved as principal investigator in numerous clinical trials to test treatments for Duchenne muscular dystrophy and spinal muscular atrophy (SMA). He is the leader of the newborn screening program for SMA in southern Belgium where we are conducting a medico-economic analysis of newborn screening. His main research expertise covers the development of innovative outcome measures, including connected devices for real-life patients' evaluation.



Mary Short, (retired) Sr. Research Advisor for Pediatric Capabilities and former co-chair and charter member of the Pediatric Steering Committee, Eli Lilly and Company

Mary Short is a retired Sr Research Advisor for Pediatric Capabilities and former co-chair and charter member of the Pediatric Steering Committee at Eli Lilly and Company. Mary's involvement in pediatrics at Lilly included establishment of the Pediatric Steering Committee, Lilly Pediatric Symposium, Pediatric Excellence Awards, Lilly Parent Volunteer Group, Pediatric Capabilities Function and Lilly's membership in the International Neonatal Consortium, the International Children's Advisory Network and the Institute of Advanced Clinical Trials for Children. As a research advisor, Mary supported efforts to integrate pediatric requirements and considerations into the Lilly quality, training and development systems for support across therapeutic areas. She provided pediatric expertise and innovation for multiple pediatric clinical trials across the Biomedicines development area.

Mary is a Registered Nurse, a Perinatal Clinical Nurse Specialist, and has a Master of Science in Nursing from Indiana University. Prior to employment at Lilly, Mary was as a staff nurse and Clinical Nurse Specialist at Methodist Hospital of Indiana NICU where she was involved in research on neuromuscular development in low-birth-weight infants. Mary has publications on her research in neonatal neuromuscular development, neonatal sepsis, pediatric informed consent, and medication errors in children. Mary provides consultation services for pediatric drug development and has interest in promoting multistakeholder engagement to improve the successful completion of pediatric research. She co-chairs the INC Communication Workgroup. Mary serves as a Court Appointed Special Advocate for Children in Need of Services in Marion County, Indiana.

Jeffrey Siegel, PhD, Director of the Office of Drug Evaluation Sciences (ODES) in the Office of New Drug (OND), CDER, FDA

ODES oversees Clinical Outcome Assessments, Biomarker Qualification, Research and Bioinformatics in OND. Dr. Siegel has over 20 years of experience in research, regulatory, and clinical drug development. Jeff received his B.A. from Columbia University and M.D. from Yale University. He trained in internal medicine at University Hospitals of Cleveland. Then he did a fellowship in Immunology and Signal Transduction at NIH. He served at FDA from 1996-2010 as a medical officer and then Medical Team Leader. In 2010, he left FDA for industry and worked at Genentech/Roche as global lead for Rheumatology and Rare Diseases and subsequently at Gilead Sciences as Translational Medicine lead in Clinical Research/Inflammation before rejoining FDA in February, 2021.

Rebeccah Slater, MSc, Professor of Paediatric Neuroscience and Senior Wellcome Fellow, University of Oxford. Professorial Fellow at St John's College

Rebeccah studied Physics (BSc) at Imperial College and Neuroscience (MSc) at UCL, and in 2007 was awarded her PhD (UCL). Since 2013 she has led the Paediatric Neuroimaging Research Group, which focuses on understanding the mechanisms that underlie the development of pain perception. She uses a range of non-invasive brain imaging tools, including EEG and fMRI, to explore the development of pain perception in the human nervous system and has developed new approaches to measure the efficacy of analgesics in neonates.

She has published many articles about infant pain and has been enthusiastically involved in science communication and public engagement with science. Rebeccah holds an honorary research position in the Neonatal Care Services at the John Radcliffe Children's Hospital, NHS Foundation Trust and is a Principal Investigator at the Wellcome Centre for Integrative Neuroimaging.

Ryan J. Taft, PhD, Senior Director, Scientific Research, Illumina Inc.

Ryan J. Taft, is Vice President of Scientific Research at Illumina, where he leads a portfolio of programs focused on the development and deployment genomic technologies for patients worldwide, especially the use of whole genome sequencing as platform for genetic testing. At Illumina his activities include oversight of the Illumina Clinical Services Laboratory's clinical whole genome sequencing test, pediatric and adult clinical trials, genomic interpretation software, bioinformatic innovation and novel application development. At Illumina Dr Taft also leads iHope, a philanthropic program that provides patients in ten countries and 25 clinical sites pro bono access to clinical whole genome sequencing. Dr. Taft has helped describe more than a dozen novel genetic disorders, and is credited with being one of the first to use short-read whole genome sequencing of a family to resolve an undiagnosed genetic disease. He holds various positions in industry organizations including as scientific advisor to a biotechnology company and five rare disease non-profits. His work has been featured in WIRED magazine, Forbes, Scientific American, the television series Chasing The Cure and documentary series Australian Story.

Prior to joining Illumina Dr. Taft was a Group Leader at the Institute of Molecular Bioscience at the University of Queensland, where he focused on the discovery and function of novel regulatory RNAs and the role of non-coding DNA in animal evolution. He obtained his Bachelor of Science in Biochemistry and Molecular Biology from the University of California, Davis on a Regent's Scholarship, and his PhD in Genomics and Computational Biology, which received a Deans' Commendation for Academic Excellence, from the University of Queensland on a US National Science Foundation Graduate Research Fellowship. Dr Taft has received several awards, including a Biocom Life Science Catalyst Award for innovation in the life sciences and the 2021 University of Queensland International Alumnus of the Year for his work in rare genetic disease.

Toshiki Takenouchi, PhD, Assistant Professor of Pediatrics, Keio University School of Medicine, Tokyo, Japan

Dr. Takenouchi graduated from Keio University School of Medicine in 2002. He trained in general pediatrics, child neurology and neonatal neurology both in Japan and United States and board certified in pediatrics and child neurology in both countries. He has been working in Keio University since 2010. Dr. Takenouchi obtained Ph.D. in 2015. His research focuses on rare genetic disorders and clinical and molecular aspects of newborn neurology. He established a new human disease that was eponymised as Takenouchi-Kosaki syndrome (OMIM #616737). Currently, he is a primary investigator of a national project, 'Research and Development on Precise and Rapid Genetic Diagnosis in Neonatal Intensive Care Unit' under Baby and Infant in Research of Health and Development to Adolescent and Young adult (BIRTHDAY) funded by the Japan Agency for Medical Research and Development.

Bernard Thébaud, MD, Senior Scientist, Ottawa Hospital Research Institute; Neonatologist, Children's Hospital of Eastern Ontario

Dr. Thébaud is a clinician-scientist with a focus on the clinical translation of stem cell-based and gene therapies for lung diseases. He is a senior scientist with the Ottawa Hospital Research Institute and a neonatologist with the Children's Hospital of Eastern Ontario, providing care to critically ill newborns. He is also a Professor of Pediatrics at the University of Ottawa. Dr. Thébaud obtained his MD at the University Louis Pasteur in France and trained in Pediatrics and Neonatology at the University Paris V, where he obtained his MSc and PhD, before completing a 2-y ear postdoctoral fellowship at the University of Alberta.Dr. Thébaud studies the mechanisms of lung development, injury and repair to design new treatments for incurable lung diseases. His focus is on answering clinically relevant questions for translation into real-life applications. Over the next five years, his goal is to bring safe and effective cell and gene therapies for lung diseases into the clinic to improve patient outcomes. Dr. Thébaud has participated on numerous peer review committees and scientific advisory boards at the international, national and provincial level, including CIHR and NIH. Dr. Thébaud holds the University of Ottawa Partnership Research Chair in Regenerative Medicine. His research is funded by the Canadian Institutes of Health Research, the Heart and Stroke Foundation of Canada, and the Stem Cell Network.

Mark Turner, PhD, Professor of Neonatology and Research Delivery, University of Liverpool, UK

Dr. Turner graduated from Manchester University with a medical degree in 1991 and a PhD in 1999 (placental physiology). He trained in neonatal medicine in the North West of England and has worked as a Consultant Neonatologist in Liverpool since 2005.

His research aims to improve the access of newborn babies and children to high quality medicines. This has included studies of dosing, safety and efficacy of 15 medicines; excipient kinetics; manipulations of medicines; the assessment of adverse drug reactions; and the value of age-appropriate formulations.

He believes that the coherent integration of the design and conduct of clinical trials is key to improving the quality of medicines. He works to develop efficient medicines research infrastructure in Europe and globally as Chair of the European Network for Paediatric Research at the European Medicines Agency (EnprEMA) (2013 – 2019), Convenor of the European Paediatric Clinical Trials Research Infrastructure, co-Director of the International Neonatal Consortium and as co-Coordinator of the Collaborative Network for European Clinical Trials For Children (conect4children, c4c).

Jerry Vockley, MD, PhD, Chief of the Division of Medical Genetics, director of the Center for Rare Disease Therapy, Cleveland Family Endowed Professor of pediatric research, University of Pittsburgh School of Medicine

Dr. Vockley received his undergraduate degree at Carnegie-Mellon University in Pittsburgh, Pennsylvania, and received his degree in Medicine and Genetics from the University of Pennsylvania School of Medicine in Philadelphia, Pennsylvania. He completed his pediatric residency at the University of Colorado Health Science Center, and his postdoctoral fellowship in Human Genetic and Pediatrics at Yale University School of Medicine in New Haven, Connecticut. Before assuming his current position in Pittsburgh, Dr. Vockley was Chair of Medical Genetics in the Mayo Clinic School of Medicine. Dr. Vockley is internationally recognized as a leader in the field of inborn errors of metabolism. His lab has been responsible for identifying multiple new genetic disorders, many of them defects in mitochondrial energy metabolism, and he has published nearly 300 scientific articles in peer review journals. His current research focuses on the molecular architecture of mitochondrial energy metabolism, in which he is breaking new ground in describing the role of dysfunction of mitochondrial energy metabolism in such common conditions as diabetes, obesity, and Alzheimer disease, and branced chain amino acid metabolism. He also is a leader in the development and testing of novel therapeutic agents for treating inborn errors of metabolism. He is the principle investigator on four NIH grants and a co-investigator on 7 others. He holds multiple US and international patents and has had two drugs for treatment of metabolic disorders approved by the FDAHe is the founder of the Plain Communities Translational Medicine program at the Children's Hospital of Pittsburgh. He received a UPMC Wolff Center Award for Excellence in Patient Experience in 2017. Dr. Vockley has served on numerous national and international scientific boards including the Advisory Committee (to the Secretary of Health and Human Services) on Heritable Disorders in Newborns and Children where he was chair of the technology committee. He is co-chair of the International Network on Fatty Acid Oxidation Research and Therapy (INFORM). He is past chair of the Pennsylvania State Newborn Screening Advisory Committee and the American College of Medical Genetics and Genomics Therapeutics Committee. He recently joined the Board of Directors for the American College of Medical Genetics and Genomics. He is a past president of the International Organizing Committee for the International Congress on Inborn Errors of Metabolism and the Society for the Inherited Metabolic Disorders (SIMD). He is also a volunteer medical advisor for several parent and family support groups including the Fatty Acid Oxidation Family Support Group, Save Babies through Screening, United Mitochondrial Disease Foundation, and the Organic Acidemia Support Group. He speaks at multiple family support functions throughout the year for CanPKU and the NPKU Alliance. He was recognized as a Champion for Babies by the March of Dimes in 2015.

Dr. Vockley is the co-founder and director of the North American Metabolic Academy established by the SIMD to help educate the next generation of metabolic physicians in the United States, and serves as associate editor for the journal Molecular Genetics and Metabolism. He is founder of the International Network on Fatty Acid Oxidation Research and Management (INFORM). He is a Founding Fellow of the American College of Medical Genetics and Genomics, and currently serves on its board of directors. Dr. Vockley was recognized in 2002 as the Research Educator of the Year while at the Mayo Clinic, and received University Faculty Honors for his educational efforts in 2014. At the University of Pittsburgh, Dr. Vockley teaches in the both the Medical School and Graduate School of Public Health. Dr. Vockley has mentored numerous Ph.D. candidates, post-doctoral fellows, and undergraduate in their research.

Cate Walsh Vockley, MS, LCGC, Genetic Counselor and Coordinator of Plain Community Outreach at UPMC Children's Hospital of Pittsburgh, Division of Medical Genetics

Dr. Vockley earned her B.S. in molecular and cellular biology from Carnegie Mellon University and her Master of Science degree in Biophysics and Biochemistry with a focus on Human Genetics and Genetic Counseling from the University of Colorado Health Sciences Center. She is certified by the American Board of Medical Genetics and American Board of Genetic Counseling, licensed to practice in Pennsylvania, and is a member of several professional societies, including the American Society of Human Genetics, the National Society of Genetic Counselors, the Society for Inherited Metabolic Disorders, and the Society for the Study of Inborn Errors of Metabolism. Prior to joining UPMC Children's Hospital of Pittsburgh, she was a genetic counselor at the Mayo Clinic Department of Medical Genetics. and at Yale University Pediatric Genetics/Metabolic Clinic, DNA Diagnostic Laboratory, and the Prenatal Genetics Unit. Ms. Walsh Vockley's current clinical interests include genetic counseling for inborn errors

of metabolism, especially newborn screening disorders, and work with the Plain Communities of western Pennsylvania. She has been involved in studies of coping mechanisms and risk perception among high-risk cancer families, long term follow-up of patients identified by newborn screening, exome analysis for undiagnosed disorders in the Plain communities and whole genome sequencing in the NICU. Her interests also include genetics education and the ethical implications of clinical genetics and genetic testing. She serves as the National Society of Genetic Counselors Organizational representative to the HHS Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC), on the Education Subcommittee of the ACHDNC and on the State of Pennsylvania Newborn Screening Technical Advisory Board and Ethics Subcommittee. Medical ethics experience includes past positions as Chair of the Ethics Subcommittee of the National Society of Genetic Counselors and on the Mayo Clinic Institutional Review Board DNA Results Subcommittee.

Erin Ward, MEd, Co-Founder & President, MTM-CNM Family Connection

Erin is Co-Founder and President of MTM-CNM Family Connection, a non-profit dedicated to connecting individuals and families from the Myotubular and Centronuclear Myopathy community to research, resources, and life-enhancing relationships. Erin serves as Director of biennial, national, patient-professional collaborative conferences for the MTM-CNM community. Erin has also helped to lead the MTM-CNM community's recent patient engagement efforts with the FDA, including both a Patient Listening Session and Externally-Led Patient-Focused Drug Development Meeting. Holding a master's degree in Education and a Certificate of Advanced Study in Counseling, Erin combines personal experiences as a mother to a young adult son living with Myotubular Myopathy with her professional skills to work towards improving patient-professional partnerships across both clinical and therapeutic drug development systems. Erin has served as Associate Faculty for the Institute for Professionalism and Ethical Practice at Boston Children's Hospital and Harvard Medical School for 15 years. Erin helped co-design and facilitated the Program to Enhance Relational and Communication Skills: Difficult Conversations in the NICU course, aimed at improving shared decision-making among NICU multidisciplinary teams and families of children complex medical conditions. Erin also participated in the development of the Ethics and Professionalism Curriculum in Neonatology on OPENPediatrics. With the evolving landscape of gene and cell therapy development, Erin is interested in the field's impact on family experiences and decision-making in neonatal care settings and committed to thinking about how best to support families and medical care teams navigating these new developments.
