

Individualized Therapies on the RISE

November 20, 2025

9:00 am – 5:00 pm ET

Hybrid Public Meeting | National Press Club

Speaker Biographies



Rebecca Ahrens-Nicklas is an Assistant Professor of Pediatrics in the Division of Human Genetics and Metabolism at The Children’s Hospital of Philadelphia (CHOP) and the University of Pennsylvania. She also directs the Gene Therapy for Inherited Metabolic Diseases Frontier Program at CHOP. After completing MD/PhD training in Physiology and Biophysics, she pursued clinical training in Pediatrics, Clinical Genetics, and Metabolism. She cares for children with rare diseases, with a special interest in neurometabolic disorders. Her research laboratory focuses both on gene discovery and elucidating the pathologic mechanisms underlying rare diseases to guide therapy development. She also partners with advocacy groups to conduct natural history and biomarker studies to promote clinical trial readiness for these rare conditions.



Terry Jo Vettters Bichell was a public health nurse-midwife until her youngest child, Lou, was diagnosed with Angelman syndrome in 2000. She pivoted to help move bench research into the first clinical trials for Angelman syndrome and design natural history studies. Dr. Bichell earned a PhD in neuroscience from Vanderbilt University in 2016, where she studied gene-environment interactions in Huntington’s disease rodent models. She was founding director of the A-BOM Alliance from 2016-2018. In 2019, Dr. Bichell launched COMBINEDBrain, a pre-competitive consortium of patient advocacy organizations working to identify outcome measures and biomarkers for rare genetic neurodevelopmental disorders. She serves as Vice Chair of the Tennessee Rare Disease Advisory

Council and teaches a course in Translational Neuroscience at Vanderbilt University. As a parent, she has accompanied her son Lou through multiple clinical trials in the search for a treatment for Angelman syndrome.



Lauren E. Black is Distinguished Scientist at Charles River Laboratories. She advises industry, research collaborators, and CRL staff on scientific strategy, translational research, and regulatory risk/benefit assessment. She has >35 years’ experience working with novel technologies for life-threatening, genetic or rare diseases, and inflammatory conditions. Dr. Black worked 11 years at FDA as a Reviewing Pharmacologist in the Centers for Drug Evaluation (Antivirals during the AIDS crisis) and CBER (Biologics during the advent of gene-modified stem cell therapies). She guided sponsors during pre-IND, IND, and NDA research; wrote FDA guidelines (immunotoxicology, starting doses, rheumatoid arthritis, biologics, xenotransplantation, and oligonucleotides); and represented FDA on topics related to relevant species and metrics used to set patient safety margins (PADs)

and NOAELs). She was a founding co-author of the Human Starting Dose guidance. Lauren assessed ~1000 preINDs and INDs for high-risk, Phase 2a trials conducted in patients. Starting in 2017, Dr. Black helped academics and patient families design single species toxicology plans for oligonucleotides and gene therapies intended for N=1 patient trials, and supported 4 academic centers in their approach to expanded access INDs. Lauren received numerous FDA awards for innovative review science. Appearing on The Today Show, an article in Chem. Eng. News, and soon, CNBC, Dr. Black also received the Society of Toxicology Career Achievement in Biotechnology Award. This year, Endpoint News selected her as a Top 20 Women Leader in Biopharma. She lost a son treated at CHOP for ultrarare mitral valve stenosis; and has two grown healthy kids and 2 rescue hounds that keep, blessedly, draining her accounts.



Teresa Buracchio is Director of the Office of Neuroscience in the Office of New Drugs, Center for Drug Evaluation and Research, Food and Drug Administration (FDA). She oversees the review of new drug programs for neurologic and psychiatric diseases, including Alzheimer's disease, Parkinson's disease, amyotrophic lateral sclerosis, neuromuscular diseases, neurogenetic disorders, major depressive disorder, and schizophrenia. Dr. Buracchio received her medical degree from Rush Medical College and completed a residency in neurology at Rush University Medical Center, Chicago, IL. Dr. Buracchio completed fellowship training in geriatric neurology at Oregon Health & Science University and Portland VA Medical Center in Portland, OR. Prior to joining FDA in 2013, Dr. Buracchio

worked at AbbVie as an Associate Medical Director for Neuroscience Clinical Development.



Scott Demarest is an associate professor in the Department of Pediatrics, Division of Neurology. He is board certified in Child Neurology and Epilepsy. His clinical practice and research focus on the evaluation and treatment of neurogenetic conditions. This includes clinical trials for novel therapeutics, natural history studies, and the development of improved outcome measures for neurogenetic conditions. He is the Chief Precision Medicine Officer at Children's Hospital Colorado, co-director of the neurology complex drug program and medical director of the Batten and Neurogenetic Multi-disciplinary Clinics. He is also the director for the International CDKL5 Clinical Research Network.



Shelby Elenburg currently serves as a clinical team lead and acting branch chief in the Office of Therapeutic Products (OTP) in FDA's Center for Biologics Evaluation and Research (CBER), where her work is primarily focused on leading her team in the clinical review of cell and gene therapy products for rare diseases. She has also been involved in numerous working groups and committees across the FDA, and has presented on regulatory topics at internal and public conferences and workshops. Prior to joining FDA in 2021, Dr. Elenburg was in Allergy/Immunology clinical practice in Maryland and DC for 6 years. Dr. Elenburg received a BS in Psychology from The

Ohio State University in 2006 and an MD from University of Cincinnati College of Medicine in 2010. She completed her pediatric residency at Phoenix Children's Hospital in 2013 and her fellowship in Allergy/Immunology at The University of Tennessee Health Science Center in 2015. She is Board Certified in Allergy/Immunology.



Richard Finkel has nearly 30 years of experience in translational research into the biology and treatment of neurologic disorders, including Duchenne muscular dystrophy, spinal muscular atrophy, inherited neuropathies, and other neurogenetic diseases of children. He is an international leader in organizing key clinical trials for neuromuscular diseases and is an international expert in spinal muscular atrophy, playing a vital role in developing the first successful therapy for this disease. Since 2020, Dr. Finkel has served as a member of the St. Jude faculty, where he serves as the founding Director for the Center for Experimental Neurotherapeutics within the Pediatric Translational Neuroscience Initiative. He leads the clinical arm of the program that includes a novel effort to develop personalized gene-targeted treatments for children with ultrarare neurogenetic diseases. Prior to St. Jude, Dr. Finkel directed the neuromuscular program at the Children's Hospital of Philadelphia and then served as Chief of Neurology at Nemours Children's Hospital in Orlando and Professor of Neurology at the University of Central Florida College of Medicine. Dr. Finkel earned a B.A. in chemistry at Washington and Jefferson College and a M.D. at Washington University, St. Louis. He completed pediatric and neurology residency and neuromuscular training at Boston Children's Hospital, Harvard Medical School. He has published over 300 peer-reviewed manuscripts. Dr. Finkel is a member of numerous professional and scientific societies, including being elected a Fellow of the American Academy of Neurology, the American Neurological Association and the Association of American Physicians.



Emily Freilich is a board-certified pediatric neurologist and the Director of the Division of Neurology 1 within the Office of New Drugs in FDA's Center for Drug Evaluation and Research. Dr. Freilich graduated from Duke University with a Bachelor of Science degree in biology and received her medical degree from Rutgers-New Jersey Medical School. She completed her pediatric residency and child neurology training at Children's National Health System in Washington, D.C. Prior to joining FDA, Dr. Freilich worked at Children's National and the Pediatric Specialists of Virginia, where she was a general child neurologist with special interest in rare pediatric epilepsies, served as co-director of the Tuberous Sclerosis Clinic, and was an Assistant Professor of Pediatrics and Neurology at George Washington University School of Medicine. She joined the Division of Neurology Products at FDA in 2016 as a clinical reviewer working in the areas of epilepsy and migraine and subsequently transitioned to a team leader in the Division of Neurology 1 for review of treatments for neuromuscular and rare neurologic disorders. She subsequently served as Acting Associate Director for Therapeutic Review and Acting Deputy Director in the Division of Neurology 1 prior to her current role.

Lois Freed is the director of the Division of Pharmacology/Toxicology in the Office of Neuroscience in the Center for Drug Evaluation and Research at the U.S. Food and Drug Administration. She has been at the FDA since 1992, as a nonclinical reviewer in the Division of Pharmacological Drug Products and later as a supervisory pharmacologist in the Division of Neurology Products. She earned her undergraduate and Master's degrees from the University of Kansas and her Ph.D. from the University of Maryland. Prior to joining the FDA, she conducted research at the National Institute on Aging/NIH in the Laboratory of Neurosciences.



Sarah Glass is the chief operating officer of n-Lorem Foundation. Sarah received her Ph.D. in Molecular Genetics at Ohio State University where she trained in rare inherited cancer syndromes. She has over 20 years of experience in clinical development and research across academia, pharmaceutical companies, and CROs. Sarah brings significant strengths and experience as an accomplished research geneticist, rare disease drug developer, and clinical trialist. She is acclaimed for forging key strategic partnerships across rare disease sectors and has driven efficiencies to decrease patient/caregiver burden in clinical research. Most notably, Sarah combines her professional expertise and training with the perspective of a

parent of a child with a nano-rare disease. This allows Sarah to not only personally understand the challenges faced by n-Lorem patients and their families, but also to translate this understanding into n-Lorem's paradigm shifting platform solution for the nano-rare community. Outside of n-Lorem, Sarah volunteers as chair of the DYRK1A Medical and Scientific advisory board and is engaged in many rare disease organizations to ensure collaboration in reaching common goals.



Michelle Hastings is the Pfizer Upjohn Research Professor of Pharmacology at the University of Michigan Medical School and co-Director of the Center for RNA Biomedicine, directing the Nucleic Acids Therapeutics Core. She earned her Ph.D. from Marquette University and completed postdoctoral training at Cold Spring Harbor Laboratory. Her work focuses on RNA-based therapeutics, particularly antisense oligonucleotides (ASOs) that modulate splicing and gene expression to treat disease. Her lab has pioneered candidate therapies for Usher syndrome, Batten disease, cystic fibrosis, Alzheimer's, and Parkinson's disease and developed a personalized ASO medicine, Zebronkysen, that is being used to treat two children

with CLN3 Batten disease caused by an ultra-rare variant. Dr. Hastings holds many patents for her discoveries and serves on multiple editorial and advisory boards. She contributes extensive experience in RNA therapeutics, translational research, and clinical development to inform strategic decision-making.



Sadik Kassim is a biotechnology executive and scientist specializing in cell and gene therapy, bioprocessing, and translational research. He currently serves as Chief Technology Officer of Genomic Medicines for Danaher's Life Sciences companies. Previously, he was CTO at Vor Bio, where he built the technical operations team and led development for a CRISPR-edited HSPC product, as well as the company's preclinical CAR-T efforts. Before that, he was Executive Director at Kite Pharma, leading manufacturing process development for autologous CAR-T and TCR-based therapies. As CSO at Mustang Bio, he oversaw the company's preclinical and manufacturing foundation. He also led early analytical development at Novartis' Cell and Gene Therapies Unit. Sadik has contributed to the development of three commercial CAR-T therapies—Kymriah, Yescarta, and Tecartus—and, most recently, to K-abe, the world's first patientspecific, custom gene-edited drug product, developed and administered in a recordsetting six months. He completed postdoctoral training at the National Cancer Institute (with Dr. Steven Rosenberg), the University of Pennsylvania Gene Therapy Program (with Dr. Jim Wilson), and Johnson & Johnson's Immunology Discovery group. He holds a B.S. in Cell and Molecular Biology from Tulane University and a Ph.D. in Microbiology and Immunology from Louisiana State University.

and Gene Therapies Unit. Sadik has contributed to the development of three commercial CAR-T therapies—Kymriah, Yescarta, and Tecartus—and, most recently, to K-abe, the world's first patientspecific, custom gene-edited drug product, developed and administered in a recordsetting six months. He completed postdoctoral training at the National Cancer Institute (with Dr. Steven Rosenberg), the University of Pennsylvania Gene Therapy Program (with Dr. Jim Wilson), and Johnson & Johnson's Immunology Discovery group. He holds a B.S. in Cell and Molecular Biology from Tulane University and a Ph.D. in Microbiology and Immunology from Louisiana State University.



Michael Lehmicke joined ARM in 2019 as its first Director of Science and Industry Affairs. Michael has over 20 years of R&D experience in biomaterials, medical devices, and regenerative medicine. He has led product development teams for class II devices, human cell and tissue-based products, and drug/device combination products. He is a creator and an inventor with multiple U.S. patents to his name. Michael has an MSc in /Biomedical Engineering, with a focus on tissue engineering, from Drexel University. Michael's areas of expertise include cell-based tissue engineering, bioceramics, biodegradable polymers, project management, strategic pipeline development, and business development. He is passionate about

regenerative medicine and believes that it represents our best hope for meeting many unmet clinical needs and thereby improving patients' lives.



Ashley Munchel is a board-certified pediatrician and pediatric hematologist/oncologist currently serving as a clinical reviewer in the Benign Hematology Branch in the Center for Biologics Evaluation and Research at the FDA. In this role, she has served as the primary clinical reviewer for multiple gene and cell therapies targeting hemoglobinopathies and bone marrow failure syndromes. Prior to joining the FDA in January 2023, Dr. Munchel was an Assistant Professor of Pediatrics at the University of Maryland School of Medicine and an attending physician in pediatric hematology/oncology at the University of Maryland Children's Hospital from 2013 to 2023. She completed her pediatric hematology/oncology fellowship training through the combined program at Johns

Hopkins and the Pediatric Oncology Branch at the National Institutes of Health.



Valerie Myers joined the Office of Pharmacology/Toxicology (OPT) within the Office of Therapeutic Products (OTP)/CBER in April 2023. She brought with her an extensive expertise from the private sector and academia of gene therapy products for cardiomyopathy and a deep knowledge of the genetic underpinnings of heart failure. Since joining OPT, Dr. Myers has reviewed files for cell and gene therapies, xenograft products, and other advanced therapeutics. Her reviews have spanned the full development lifespan of OTP products, from INTERACT stage, pre-IND, IND and BLA. She serves as a subject matter expert for cardiomyopathy files,

is on several working groups within the agency, across HHS agencies, and with external stakeholders. Dr. Myers is one of OPT's go-to reviewers for rare disease files because of her comprehensive reviews, regulatory knowledge, and flexibility in working with sponsors.



Dan O'Connor is currently the Director Regulatory and Early Access Policy at The Association of the British Pharmaceutical Industry (ABPI). He joined the ABPI from the Medicines and Healthcare products Regulatory Agency in 2023 where he was Deputy Director of the Innovation Accelerator and Regulatory Science. Dan has special interests in drug development, rare diseases, regulatory science, early access, health innovation, patient engagement and drug repurposing. He completed higher medical training in Pharmaceutical Medicine and is Editor-Author of the Oxford Specialist Handbook in Pharmaceutical Medicine. He is a co-leading the work

of the UK Rare Therapies Launch Pad (The Rare Therapies Launchpad: a pilot program for individualized medicines in the UK | Nature Medicine)



Michael Pacanowski is the Director of the Division of Translational and Precision Medicine in FDA’s Office of Clinical Pharmacology. He oversees a multidisciplinary team of clinical scientists who lead the Office’s regulatory review, research, and policy activities related to pharmacogenomics, biomarkers, targeted therapies, and drug development for rare diseases.



Michael Panzara is Chief Medical Officer at Neurvati Neurosciences and GRIN Therapeutics. He joined Neurvati in 2022, bringing more than 20 years of experience developing therapies for neurological disorders having served in leadership roles at Wave Life Sciences, Sanofi, Genzyme, and Biogen and currently serves on the Boards of Directors of Athira Pharma, Inc and Cadenza Bio, Inc. Prior to joining Neurvati, Dr. Panzara was Chief Medical Officer, Head of Therapeutics Discovery and Development at Wave Life Sciences, overseeing the company’s therapeutic R&D portfolio with a focus on genetically defined diseases. Before that he held leadership positions at Genzyme (later Sanofi Genzyme) eventually serving as Head of Multiple Sclerosis, Neurology and Ophthalmology Development. He previously served as Vice President and Chief Medical Officer of Neurology at Biogen, where he was clinical lead for TYSABRI (natalizumab), overseeing its clinical program and global approvals and managed clinical development activities for late-stage MS products. Dr. Panzara received his BA in biology from the University of Pennsylvania, MD from Stanford University, neurology training at Massachusetts General Hospital, postdoctoral training in immunology and rheumatology at Brigham and Women’s Hospital, and earned his MPH from Harvard School of Public Health.



Bart Rogers is a reviewer in the Division of Translational and Precision Medicine, Office of Clinical Pharmacology (OCP) within CDER at the U.S. Food and Drug Administration (FDA). He also serves as an active-duty officer in the U.S. Public Health Service. Dr. Rogers serves as a Genomics and Targeted Therapy reviewer across multiple non-oncologic therapeutic areas and is the lead reviewer for all RNA-therapeutics within OCP. His work focuses on integrating pharmacogenomic and molecular data to advance precision medicine approaches in regulatory review. Prior to joining the FDA, CAPT Rogers earned both his Pharm.D. and Ph.D. from the University of Maryland School of Pharmacy, where his doctoral research explored the pharmacogenomics of beta-blockers in heart failure. His current research interests include the pharmacology of synthetic oligonucleotides, “N-of-1” individualized drug development, and pharmacogenomics.



Amy Comstock Rick is the Director of Strategic Coalitions for the U.S. Food and Drug Administration's (FDA) Rare Disease Innovation Hub (the Hub). She serves in a cross-cutting role across FDA's Center for Drug Evaluations and Research (CDER) and Center for Biologics Evaluation and Research (CBER) to facilitate implementation of the Hub. She also works closely with both centers to develop and carry out a rare disease strategic agenda. Most recently, Ms. Rick served as Principal Consultant at Leavitt Partners, focusing on health policy matters, with a primary focus on rare disease and medical product development. Before Leavitt Partners, she served as President and Chief Executive Officer of the Food and Drug Law Institute (FDLI), a non-profit organization dedicated to providing an innovative, open, balanced exchange of ideas and viewpoints across the field of food and drug law. Before joining FDLI, Ms. Rick was Chief Executive Officer of the Parkinson's Action Network. Ms. Rick also served as President of the Coalition for the Advancement of Medical Research and on the Boards of Directors for Research America, the National Health Council, and the American Brain Coalition. Ms. Rick had previous federal service as a career attorney at the U.S. Department of Education in 1988, focusing primarily on the field of government ethics. She was the Senate-confirmed Director of the U.S. Office of Government Ethics from 2000 to 2003 and Associate Counsel to the President in the White House Counsel's Office from 1998 to 2000.



Klaus Romero is a prominent clinician scientist and scholar, who serves as both the Chief Executive Officer and Chief Science Officer at Critical Path Institute. As a recognized thought-leader, Dr. Romero established C-Path's Quantitative Medicine Program and has been an instrumental leader in the growth of the organization's portfolio of transformative consortia and public-private-partnerships across more than 16 therapeutic development areas. As both a scientist and an executive, Dr. Romero led the generation of actionable drug development tools in Alzheimer's disease, which introduced a transformation in the drug development process for this

indication. In tuberculosis, Romero's leadership was instrumental in generating a drug development infrastructure that allowed the approval of the first new individual drug and the first new regimen for this disease, in more than 50 years. Dr. Romero's leadership has also resulted in the transformation of therapeutic development paradigms for many other diverse areas, like polycystic kidney, Parkinson's and Huntington's diseases, as well as type 1 diabetes prevention, kidney transplantation, Duchenne muscular dystrophy, and several other rare and orphan indications. As a trained clinical pharmacologist and epidemiologist, 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 of Pharmacoepidemiology. He is also an Associate Research Professor at the University of Arizona, as well as an Adjunct Professor at the University of Southern California and Arizona State University.



Neil Shneider serves as the Claire Tow Professor of Motor Neuron Disorders in Neurology, and the Director of the Eleanor and Lou Gehrig ALS Center at Columbia University. He is also co-director of the Claire Tow Center for Motor Neuron Biology and Disease where his lab focuses on the study mechanisms of neurodegeneration in ALS and the discovery and development of novel therapeutics for ALS and related disorders. Dr. Shneider worked with Ionis Pharmaceuticals to develop ION363 (jacifusen), an anti-sense oligonucleotide (ASO) for ALS patients with rare mutations in the FUsed in Sarcoma (FUS) gene. Dr. Shneider is a graduate of Harvard College and

earned his M.D. and Ph.D. degrees at the Columbia University College of Physicians and Surgeons. In partnership with the n-Loxam Foundation, Dr. Shneider founded Silence ALS, an initiative to develop individualized ASOs for ALS patients with ultra-rare, pathogenic mutations in ALS genes. Dr. Shneider was co-chair of the Translating Fundamental Research into Potential ALS Therapies Working Group for the NIH ALS Strategic Planning Workshop, and is a multi-PI on the NIH-funded ALL ALS Clinical Research Consortium.



Amy Simon is Chief Medical Officer of Beam. Dr. Simon brings more than 20 years of clinical experience to Beam, serving in roles as a physician-scientist in academia and the biotechnology industry. Dr. Simon joins Beam from Alnylam Pharmaceuticals, where she spent over a decade in various roles with increasing responsibility for the clinical development of RNAi-based medicines, most recently serving as Vice President, Clinical Development. During her tenure at Alnylam, she led the successful execution of clinical programs from natural history studies to Phase 1 through Phase 4 studies, regulatory interactions with both U.S. and ex-U.S. authorities, and drug approvals in the U.S. and EU. Dr. Simon was the lead clinician

developing GIVLAARI® (givosiran) for patients with acute hepatic porphyria, which was approved by the Food and Drug Administration in 2019. Prior to entering the biotech industry, Dr. Simon worked in academia at Tufts University, serving as a professor and a director of the Asthma Center in the Pulmonary and Critical Care Division at Tufts University School of Medicine and as a professor at Tufts Graduate School of Biomedical Science where her laboratory conducted basic science research on asthma. She began her career in clinical practice, training as a resident in internal medicine and as a fellow in pulmonary and critical care medicine at Tufts Medical Center. Dr. Simon holds a B.A. in History and Science from Harvard University, and an M.D. from Tufts University School of Medicine.



Charlene Son Rigby is the Chief Executive Officer of Global Genes. She has spent her career building organizations at the intersection of data, technology, and life sciences. Charlene was previously Chief Business Officer at Fabric Genomics and held executive roles at enterprise software and genomics companies, including Oracle and DoubletWist. She started her career in neuroscience research at Roche. When Charlene’s daughter was diagnosed with a rare genetic disease, she co-founded the STXBP1 Foundation. She is committed to finding a cure for her daughter’s disorder. Charlene’s unplanned connection between her personal life and profession has helped push forward the search for a cure for her daughter and

kids like her, and given her work deeper meaning. She holds a B.A. in Human Biology from Stanford University and an M.B.A. from the Haas School of Business at U.C. Berkeley.



Brian Stultz is the Chief of Gene Therapy Branch 3 located in the Office of Gene Therapy CMC, Office of Therapeutic Products, Center for Biologics Evaluation and Research. Brian has over 20 years of experience with cell and gene therapy CMC review including expertise in plasmids, peptides, mRNA, genome editing, microbial vector, and AAV based products. He received a Master of Science with training in Biochemistry and Molecular Biology from the University of Virginia. Brian joined the FDA in 2000 working in the lab of Dr. Hursh publishing multiple papers on the regulation of the TGF-β signaling pathway and identifying critical quality attributes of MSCs. In addition to lab research, he participated in reviewing gene therapy CMC

and eventually transitioned to full time review of gene therapy products. In 2023 he took on the role of branch chief in the new super office of therapeutic products.



Marshall Summar is the Chief Executive Officer of Uncommon Cures, LLC, a company focused on consolidating rare disease clinical trials and utilizing innovative technologies to reduce costs and timelines. Before co-founding Uncommon Cures in 2022, Dr. Summar led the Rare Disease Institute at Children’s National Medical Center, the first clinical home for patients with genetic rare diseases, and the first NORD-designated Rare Disease Center of Excellence. Dr. Summar’s research has resulted in over 180 peer-reviewed publications and more than 100 international patents spanning therapies, software, and devices. His work has contributed to new treatments for sickle cell anemia, organic acidemias, congenital heart disease, and premature birth. A board-certified pediatrician and geneticist, Dr. Summar has held leadership roles with NORD, the Society for Inherited Metabolic Disorders, and numerous advisory boards, earning NORD’s Lifetime Achievement Award in 2022.



Mark Trusheim is Senior Strategy Advisor, NEWDIGS at Tufts Medical Center. Mark’s research focuses on the economics of biomedical innovation, especially payment innovations for biopharmaceuticals and gene therapies, biosimilars, and precision medicine. Mark held appointments for 18 years at MIT Sloan in Applied Economics and has served as a Special Government Employee for the FDA’s Office of the Commissioner. Mark is also President of Co-Bio Consulting, LLC. His career has spanned policy as the President of the Massachusetts Biotechnology Council, diagnostics as founder of Cantata Labs, genomics as President of Cereon Genomics, eHealth as Vice President of Monsanto Health Solutions, managed care marketing at Searle Pharmaceuticals, and big data at Kenan Systems. He holds degrees in Chemistry from Stanford University and Management from MIT.



Fyodor Urnov is a Professor of Molecular Therapeutics at UC Berkeley and a Director at its Innovative Genomics Institute (IGI). He co-developed the toolbox of human genome and epigenome editing, co-named genome editing, and was on the team that advanced all of its first-in-human applications to the clinic. He also led the effort that identified the genome editing target for an approved medicine to treat sickle cell disease and beta-thalassemia. A major goal for the field of genome editing and a key focus of Fyodor's work is expanding access to CRISPR therapies for genetic disease. As part of that effort Fyodor directs the Danaher-IGI Beacon for CRISPR

Cures - a first-in-class academia-industry partnership developing and advancing to the clinic CRISPR-based platform approaches to treat severe Mendelian diseases of the immune system. In May 2025 a paper in the New England Journal of Medicine described the world’s first on-demand engineered CRISPR therapy for a newborn with a severe metabolic disorder; Fyodor was the IGI lead of this 4-way collaboration between CHOP, Penn Medicine, Danaher, and the IGI that reduced the “CRISPR as a platform” approach to clinical practice. Fyodor also directs the newly established CZI-IGI Center for Pediatric CRISPR Cures that aims to expand the “CRISPR on-demand” approach exemplified in that effort to multiple additional pediatric patients with severe genetic diseases.



Sonia Vallabh co-runs a prion research laboratory at the Broad Institute of MIT and Harvard along with her husband, Eric Minikel. She earned her PhD in Biological and Biomedical Sciences from Harvard Medical School in 2019. Sonia and Eric left their previous careers to devote their lives to biomedical research after learning in 2011 that Sonia had inherited from her mother a mutation that causes genetic prion disease, a rapidly fatal and currently untreatable neurodegenerative disease that typically strikes in midlife. At the Broad Institute, Sonia works on prion disease drug discovery, with a focus on lowering the amount of prion protein in the brain. She also works on biomarkers, natural history and other tools to enable meaningful

clinical trials, including in presymptomatic people at risk.



Julia Vitarello founded Mila's Miracle Foundation in 2016 upon learning that her 6-yr-old daughter, Mila had Batten disease, a fatal genetic condition with no cure. In a race to save Mila, Julia's collaboration with Dr. Tim Yu from Boston Children's Hospital led to the first-ever medicine designed for one person, named milasen. After initially halting Mila's symptoms, her disease eventually progressed, and in 2021, Mila died at just ten years old. Driven by a sense of hope and responsibility, Julia is on a mission to open up an entirely new field of 'interventional genetics' to meet the new era of individualized medicines which Mila pioneered, helping to solve the global health crisis of genetic disease in children. Julia has been leading

the global movement, "Mila to Millions", focused on convening the right people around the right ideas in the right places to pilot system-wide change. She initiated the Rare Therapies Launch Pad, a UK pilot focused on modeling regulatory and reimbursement change, and is working on a documentary film as part of an impact campaign highlighting the moral imperative. She co-founded EveryONE Medicines, the first biotech focused on individualized medicines, and the N=1 Collaborative, the international hub for rethinking and setting the standards for this new paradigm. Julia speaks regularly around the world, engaging academics, biotechs, governments, regulators, payers and families alike.



Tim Yu is a physician-scientist in the Division of Genetics & Genomics at Boston Children's Hospital and Harvard Medical School. He leads a research group that works at the intersection of genomics, informatics, and neurobiology to better understand, diagnose, and treat rare neurologic disease. He completed MD-PhD training at UC San Francisco and neurology residency at Massachusetts General Hospital and Brigham and Women's Hospital, and postdoctoral training at Boston Children's Hospital. An early pioneer in the application of next generation sequencing to human disease gene discovery, Dr. Yu and his group pivoted to studying new models for interventional genomic medicine, beginning with the 2018

development of a patient-customized antisense oligonucleotide for a young girl with CLN7 Batten disease. This and subsequent cases by his group and others have served as the basis for FDA guidances for individualized drug products under which >30 patients have been treated to date. He continues to work closely with patients, foundations, physicians, and regulators to explore and fortify this pathway. He is founder of the N=1 Collaborative, an independent nonprofit devoted to developing practices for individualized medicine.

Moderators Biographies



Michelle Campbell is the Associate Director for Stakeholder Engagement and Clinical Outcomes in the Office of Neuroscience, Office of New Drugs (OND) in FDA's Center for Drug Evaluation and Research. Dr. Campbell joined the FDA in 2014 and previously was a reviewer on the Clinical Outcome Assessments (COA) Staff and Scientific Coordinator of the COA Qualification Program in OND. Dr. Campbell's focus is in patient-focused drug development and the use of patient experience data in the regulatory setting. Prior to joining FDA, Dr. Campbell spent more than 10 years conducting research in the academic-clinical setting, including five years in a neurology and developmental medicine department. Dr. Campbell earned her BA in

Biology from the College of Notre Dame, her MS in Health Science from Towson University, and her PhD in Pharmaceutical Health Services Research from the University of Maryland School of Pharmacy.



Brian Canter is an Assistant Research Director on the Biomedical Innovation team working on policy solutions to improve development, regulatory review, and evidence generation for broadening access and availability to medical products. He manages one of the Biomedical Innovation team's cooperative agreements with the U.S. Food and Drug Administration (FDA). Brian's portfolio of work spans several key areas within the biomedical innovation space. Supporting the Institute's thought leadership to modernize clinical trials, Brian guided the policy work for the Coalition for Advancing Clinical Trials at the Point of Care. Brian has also managed several projects within the Institute's regulatory science work, including public meetings

convened with FDA to advance clinical trial innovation and premarket safety analytics. In addition to biomedical innovation, Brian has done extensive work within the Institute's 21st Century Public Health and Population Health portfolio. Prior to joining Duke-Margolis, Brian completed a PhD in Biomedical Sciences with a focus in Biomedical Engineering from Rutgers University. Brian also graduated with a Bachelor of Science in Biomedical Engineering from Tufts University.



Charles Gersbach is the John W. Strohbehn Distinguished Professor of Biomedical Engineering at Duke University and the Director of the Duke Center for Advanced Genomic Technologies. His research interests are in genome and epigenome editing, gene therapy, regenerative medicine, biomolecular and cellular engineering, synthetic biology, and genomics. His work has led to new approaches to study genome structure and function, program cell biology, and treat genetic disease. Dr. Gersbach's work has been recognized through awards including the NIH Director's New Innovator Award, the NSF CAREER Award, the Outstanding New Investigator Award from the American Society of Gene and Cell Therapy, and

induction as a Fellow of the American Institute for Medical and Biological Engineering and member of the National Academy of Inventors. He is also the co-founder of several biotechnology companies and an advisor to others.



Gerrit Hamre is a Research Director in Biomedical Regulatory Policy at the Institute. Gerrit has worked for nearly 20 years in the pharmaceutical industry with a focus on clinical research, regulatory, and commercial roles. Central to much of his career work is extensive internal and external stakeholder engagement to advance innovative, evidence-based healthcare solutions. He has often worked in the drug development and approval environment. Highlights of Gerrit's career so far have included his work in the Food and Drug Administration's Office of Legislation and as a Peace Corps Volunteer in South Africa.



Scott Winiecki is currently serving as the Acting Associate Director for Rare Disease at FDA Center for Drug Evaluation and Research (CDER). He received his MD degree from the University of Maryland and completed his pediatric training at the Children's Hospital of Philadelphia. After 12 years in private pediatric practice, he joined the U.S. Food and Drug Administration in 2011. While working on the safety of blood products and vaccines, he received the FDA's "Outstanding New Reviewer" Award and a Public Health Achievement Award. After 5 ½ years working on biologics, he joined CDER in 2016. He spent 6 years managing the Safe Use Initiative, a group whose goal is to reduce preventable harm from medications. In December

2022, he joined the Rare Disease Team where he works collaboratively with external and internal rare disease stakeholders to drive scientific and regulatory innovation and engagement to accelerate the availability of treatments for patients with rare diseases.

This project is supported by the Food and Drug Administration (FDA) of the U.S. Department of Health and Human Services (HHS) as part of a financial assistance award [U19FD006602] totaling \$5,192,495 with 100 percent funded by FDA/HHS. The contents are those of the author(s) and do not necessarily represent the official views of, nor an endorsement, by FDA/HHS, or the U.S. Government.