On The RISE: Controls in Rare Disease Clinical Trials for Small and Diminishing Populations

September 3, 2025

9:30 am – 4:00 pm ET

Hybrid Public Meeting | National Press Club

Speaker Biographies

Samiah Al-Zaidy is the Global Head of Clinical Development, Gene Therapy Neurology at Alexion Pharmaceuticals. In her role, she oversees the clinical development of the genomic medicine neurology portfolio.

She is a pediatric neurologist and neuromuscular specialist with expertise in clinical AAV gene therapy trials. Dr. Al-Zaidy previously held leadership roles in the gene therapy industry including VP of Clinical Development at Passage Bio, a genetics therapeutics company and senior consultant for Novartis Gene Therapies as well as supporting R&D for early-stage AAV biotech companies. Dr. Al-Zaidy's career in gene therapy began as a post-doctoral fellow at the Center for Gene Therapy and continued as a principal investigator and assistant professor of Pediatrics at The Ohio State University leading the clinical development of various gene therapy clinical trials, including those that ultimately led to the FDA approved product Zolgensma for spinal muscular atrophy.

Ramona Belfiore-Oshan is the Executive Director of the Duchenne Regulatory Science Consortium (D-RSC) at the Critical Path Institute (C-Path). In this role, she provides overall leadership, administrative, and scientific oversight of the Consortium, which focuses on advancing drug development tools for Duchenne muscular dystrophy (DMD) and related neuromuscular diseases.
Dr. Belfiore-Oshan earned her bachelor's degree in biological science, master's in molecular biology, and Ph.D. in neuroscience from the University of Catania in Italy. Her doctoral research centered on neurodegenerative diseases, including amyotrophic lateral sclerosis (ALS) and Alzheimer's disease. She furthered her research during a postdoctoral position in neurology at the Icahn School of Medicine at Mount Sinai, where she also honed skills in science communication, grant writing, and scientific project management.

Joining C-Path in 2021 as the Associate Director for D-RSC, Dr. Belfiore-Oshan played a pivotal role in supporting the implementation of modeling and simulation work for multiple regulatory submissions. Her efforts contributed to the completion of the development of the DMD Clinical Trial Simulator, a tool designed to enhance the design of clinical trials for DMD therapies.

In July 2023, she was appointed as the Executive Director of D-RSC, where she continues to lead initiatives aimed at accelerating therapy development to address the urgent unmet medical needs in DMD and other dystrophinopathies. Dr. Belfiore-Oshan's leadership extends to global collaborative efforts to advance solutions for DMD and other dystrophies.

Elizabeth Berry-Kravis is a Professor of Pediatrics and Neurological Sciences at Rush University Medical Center in Chicago. She established the Fragile X Clinic and Research Program in 1991, through which she has provided care to over 800 patients with fragile X syndrome (FXS). She has studied medical issues, epilepsy and psychopharmacology in FXS, and has been a leader in translational research in FXS for 20 years, including development of clinical outcome measures and biomarkers, natural history studies, newborn screening, and particularly clinical trials of new targeted treatments in FXS, for which she has been PI or Co-PI of 27 trials, both industry and investigator sponsored. Her laboratory studies the cellular role of fragile X messenger ribonucleoprotein (FMRP), relationship between FMRP and clinical function, and optimization of genetic testing methods. More recently she has expanded clinical and translational work to other neurodevelopmental disorders in addition to FXS, including autism spectrum disorders and single gene models of ASD, including Phelan McDermid syndrome, Rett syndrome, and Angelman syndrome. She also is working on translational research in rare neurogenetic disorders including Niemann-Pick type C, Battens disease, pantothenate kinase-associated neurodegeneration, and creatine transporter deficiency, as well as N-of-1 trials of ASOs and gene therapy for early onset epileptic encephalopathies and other neurogenetic conditions. She has received the NFXF Jarrett Cole Clinical Award, FRAXA Champion Award, NFXF William and Enid Rosen Research Award, March of Dimes Jonas Salk Research Award, American Academy of Neurology Sidney Carter Award in Child Neurology, John Merck Fund Sparkplug Award, the FRAXA Ingenuity Award, the FAST Innovation Award, the inaugural Martha Bridge Denckla Award from the Child Neurology Society for work in cognitive disorders of children, and the CureSHANK 2025 PMS Investigator of the Year Award.

Allyson Berent is a veterinary internal medicine specialist/interventionalist who graduated from Cornell University and completed her residency at the University of Pennsylvania, where she served as an Adjunct Assistant Professor before joining the Animal Medical Center in NYC. She is the Director of the Interventional Endoscopy Service, focusing on clinical trials researching medical devices particularly for ureteral and biliary obstructions in animals with naturally occurring diseases. In 2014 Dr. Berent's daughter, Quincy, was diagnosed with Angelman syndrome. In 2015 she joined the Board of Directors for the Foundation for Angelman Syndrome Therapeutics (FAST), becoming the Chief Science Officer. Dr. Berent serves as the co-director of the Angelman Syndrome Biomarker and Outcome Measure Consortium, to co-director for the International Angelman Syndrome Research Council (INSYNC-AS), and is an advisor to numerous pharmaceutical companies working on therapeutic candidates for rare neurodevelopmental disorders. Dr. Berent co-founded GeneTx Biotherapeutics, a company focused on advancing an antisense oligonucleotide therapy for AS, where she was the Chief Operating Officer. GeneTx was acquired in 2022 by Ultragenyx Pharmaceuticals, after launching the Phase1/2 clinical trial, and she now serves as a consultant for Ultragenyx. Dr. Berent is currently the Chief Development Officer at AS^{2}Bio helping to accelerate numerous therapeutic programs for Angelman syndrome including an AAV gene therapy, and HSC autologous ex-vivo gene therapy and a CRISPR gene editor.

Najat Bouchkouj is a pediatric hematologist-oncologist who serves as Associate Director for Pediatrics in the Office of Clinical Evaluation (OCE) at the FDA's Center for Biologics Evaluation and Research (CBER). In this role, Dr. Bouchkouj spearheads strategic regulatory, scientific, and policy initiatives that advance cellular, tissue, and gene therapy development for pediatric patients. Since joining the FDA in 2016, Dr. Bouchkouj has played a pivotal role in developing regulatory frameworks for hematologic malignancies, rare diseases, and patient engagement initiatives. Her expertise has contributed to the approval of numerous innovative cellular, tissue, and gene therapy products. She also led the development of several FDA guidance documents and serves on multiple Agency committees. Dr. Bouchkouj earned her medical degree from Damascus University and completed pediatric residency at SUNY Downstate Medical Center, followed by fellowship training in Pediatric Hematology Oncology at Children's National Medical Center. Prior to her FDA roles, Dr. Bouchkouj served as an attending physician at Boston Children's Hospital and MedStar Georgetown University Hospital. She continues to provide clinical care as a consulting oncologist at Children's National Medical Center.

Rebecca Rothwell Chiu is a Supervisory Mathematical Statistician in the Division of Biometrics IV in the Office of Biostatistics at the Center for Drug Evaluation and Research (CDER) at the U.S. Food and Drug Administration (FDA). Her team provides statistical support for the Division of Rare Diseases and Medical Genetics, focused on developing drugs and biologics intended for the prevention and treatment of rare inborn errors of metabolism. She currently chairs the Office of Biostatistics Rare Disease Committee and is active in CDER working groups dedicated to the design of clinical trials for rare disease drug development. Her prior experience at the FDA includes drug development in rheumatology, COVID-19, and pediatrics. Prior to joining the FDA, Dr. Chiu received her PhD in Biostatistics from the University of Michigan at Ann Arbor.

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. He received a Bachelor of Science in biology from the University of Texas at Austin before going on to medical school at the University of Texas Health Science Center in San Antonio. He completed his residency in Pediatrics and Child Neurology at Children's National Health System in Washington, DC. and his Epilepsy fellowship at Children's Hospital Colorado.

Mary Dwight is the Senior Vice President and Chief Policy & Advocacy Officer for the Cystic Fibrosis Foundation and directs the Foundation's public policy agenda and *Compass*, a team of CF patient advocates who provide assistance services to help navigate insurance, financial, legal, or other complex issues. Dwight also leads the strategic development of the Foundation's efforts to enable and expand access to CF care, integrating the organization's public policy, advocacy, strategic communications, and medical research and care delivery programs. In the same spirit of the CF Foundation's pioneering work in drug development, she leads efforts to explore innovative care delivery mechanisms and alternative payment models that efficiently, effectively, and consistently provide high-quality cystic fibrosis care.

Stacey Frisk is a policy and regulatory affairs leader dedicated to advancing evidence-based policies that promote innovation in rare diseases. She currently serves as Executive Director of the Rare Disease Company Coalition (RDCC), the leading voice of rare disease innovators. In this role, she advocates for policies that promote efficient, patient-centered, and fit-for-purpose approaches to the development and review of rare disease therapies.

With more than a decade of experience spanning industry, patient advocacy, and clinical research, Stacey has developed expertise in translating complex regulatory challenges into actionable policy solutions. At Sarepta Therapeutics, she served as a global regulatory strategist focused on gene therapies to treat ultra-rare neuromuscular disorders, directed regulatory policy and intelligence, and led initiatives to integrate patient data and perspectives into development and regulatory strategies.

Stacey is most passionate about shaping health policies that improve the lives of people with small and complex conditions and ensuring the voices of patients and families remain at the heart of drug development and regulatory decision-making.

Nicole Mayer Hamblett is a Professor of Pediatrics and Adjunct Professor of Biostatistics at the University of Washington. She is Co-Executive Director of the Cystic Fibrosis Therapeutics Development Network (CF TDN) Coordinating Center established at Seattle Children's Research Institute and funded by the Cystic Fibrosis Foundation. She has led the design and analysis of numerous large multicenter clinical studies which have advanced clinical care and outcomes in CF. Dr. Hamblett's research interests include the development of regulatory strategy to advance complex drug development for rare diseases. She is currently a principal investigator on therapeutic trials optimizing treatment regimens in CF, studies to advance biomarkers supporting therapeutic development for novel CFTR modulator therapies, and methodologic work to innovate clinical trials for rare diseases, in particular to support a pipeline of nucleic acid-based therapies for people with CF ineligible for CFTR modulators. She serves on international and national scientific advisory committees for the CF Foundation and is an advisory committee member for the Food and Drug Administration.

Annie Kennedy is the Chief of Policy, Advocacy & Patient Engagement at the EveryLife Foundation for Rare Diseases. A veteran leader in the patient focused drug development movement, Annie joined the EveryLife Foundation in 2018, where she's led numerous community-driven evidence development efforts including the National Economic Burden of Rare Disease study, the Guide to Patient Involvement in Rare Disease Therapy Development, and The Cost of Delayed Diagnosis in Rare Disease: A Health Economic Study. Her advocacy efforts and community activation contributed to new federal infrastructure for rare disease including the Muscular Dystrophy Coordinating Committee (MDCC) and the Rare Disease Innovation Hub at the Food and Drug Administration (FDA). Annie previously held leadership roles at Parent Project Muscular Dystrophy (PPMD) and the Muscular Dystrophy Association (MDA) where she led landmark legislative, regulatory, newborn screening, transitions, and access policy efforts including the MD CARE Act, and the Patient Focused Impact Assessment Act (PFIA), which became the Patient Experience Data provision of the 21st Century Cures Act.

Annie's community roles have included service on the Board of Directors of Cure SMA, the National Duchenne Newborn Screening Steering Committee, the NIH Strategic Planning Working Group on Engaging the Public as Partners in Clinical Research (NexTRAC), and as a member of the NIH NCATS Advisory Council.

Vijay Kumar is a fellowship trained, board-certified adult nephrologist. He joined FDA in 2020 as a Medical Officer in Center for Biologics Research (CBER). He has been involved in review of variety of biological products, including gene and cell therapies, xenotransplantation, and biologic devices. He currently serves as acting director of Super Office of Therapeutic Products (OTP) at CBER.

During his tenure at FDA, he has participated in several working groups for advisory committees, guidance documents and reviewed NIH, FDA-OOPD grants. His external outreach includes FDA representation at several patient focused drug development (PFDD) meetings, patient listening sessions (PLS), invited speaker, panelist, and moderator at national and international scientific meetings on rare pediatric disease and novel cell and gene therapeutics. He continues to be a CBER representative / liaison for public private partnerships including Kidney Health Initiative (KHI), C-PATH Institute, and Lupus ABC Consortium.

Prior to joining the Agency, he practiced clinical nephrology for 23 years. During his clinical practice he held several leadership roles including Medical Director of Home Dialysis, and Chairman of Internal Medicine at a tertiary care hospital. He holds a master's degree in medical management (MMM) from USC Marshall School of Business.

Mark Levenson is currently the Deputy Office Director of the Office of Biostatistics at the Center for Drug Evaluation at the US FDA. He has led many major pre-market and post-market statistical reviews, leading to approvals of novel drugs and to important safety warnings. He contributes to statistical policy and guidance development in the areas of regulatory evidence and real-world evidence. He is a member of the CDER Medical Policy and Program Review Council, the FDA Real-World Evidence Committee, the CBER-CDER Rare Disease Policy and Portfolio Council, and the Office of Biostatistics Statistical Policy Council. Dr. Levenson received a Ph.D. in Statistics from the University of Chicago, a B.A. in Mathematics

from Cornell University, and graduated from the Bronx High School of Science. Mark is an elected fellow of the American Statistical Association.

Jenn McNary is a trusted voice in the rare disease community, as a mother, public speaker and fierce advocate. Her work in the rare disease space as a thought leader earned her the Ryan's Quest Ryan's Hero award in 2013, and the prestigious 2017 Meyer- Whalley instrument of change award. Formerly as the director of outreach and advocacy at a Massachusetts based non-profit foundation, she was responsible for the organization of the largest FDA advisory committee hearing in history, with over 1000 duchenne advocates, families, clinicians and researchers in attendance. Jenn has unique experience in the drug development field, as a parent of children enrolled in clinical trials, an advocate engaging with the regulators and as a patient engagement professional in the life sciences.

Currently, Jenn is the Co-Founder and Principal of Canary Advisors, a boutique patient advocacy consulting firm with a focus on regulatory and access patient engagement. Her other activities include serving as the Founder of One Rare, a non-profit formed to meet the needs of young adults with rare and chronic conditions and raising her children in Massachusetts.

Adora Ndu is a seasoned biopharmaceutical executive with two decades of experience in drug development, regulatory affairs, and health policy. She is the Chief Regulatory Officer and Executive Vice President of Portfolio Strategy and Management at BridgeBio Pharma (NASDAQ: BBIO), where she oversees regulatory, quality, government affairs, and program management. She also served previously as BridgeBio's interim Chief Legal Officer.

Prior to BridgeBio, Dr. Ndu was Group Vice President at BioMarin Pharmaceutical, leading R&D strategy, scientific collaborations, and policy. Earlier in her career, she held key regulatory leadership roles at the U.S. Food and Drug Administration (FDA), including Director of Medical Policy Development at CDER, and served as a U.S. Public Health Service officer.

Dr. Ndu serves on the boards of Acadia Pharmaceuticals and DBV Technologies, and actively contributes to the healthcare community through board roles at the Alliance for Regenerative Medicine, the Food and Drug Law Institute, and Howard University's College of Pharmacy. She has received numerous honors, including awards from the U.S. Public Health Service and recognition by the Maryland Daily Record and Howard University. In 2024, she joined the Economic Club of Washington, D.C.

Dr. Ndu holds a Pharm.D. from Howard University, completed a residency at Georgetown University Hospital, and earned her J.D. from the University of Maryland.

Cara O'Neill is the Chief Science Officer and Co-Founder of the Cure Sanfilippo Foundation. Her daughter, Eliza, lives with Sanfilippo syndrome type A, a rare neurodegenerative metabolic disease. Before shifting her primary focus to advocacy and research, Dr. O'Neill practiced community and academic pediatrics for over a decade, specializing in the primary care of children with complex medical needs.

Her unique combination of professional and personal experience enables her to bridge gaps between scientists, clinicians, industry, and families. She collaborates extensively with stakeholders across the rare disease community to advance patient-centered research, the use of surrogate biomarkers, clinical trial design, and advocacy initiatives. Her contributions include the publication of the first clinical care guidelines for Sanfilippo syndrome, caregiver preference and drug repurposing clinical research, and the development of fit-for-purpose clinical outcome measures.

Todd Paporello is currently the Vice President & Global Head of Regulatory Affairs for the Specialty Care Global Business Unit at Sanofi. Todd has tremendous expertise in global drug development and regulatory strategy in both pharmaceuticals and consumer health organizations covering multiple therapeutic areas and has been responsible for successfully filing and securing worldwide approvals for multiple investigational, new and supplemental drug applications. In addition to his considerable technical regulatory and drug development skillsets, Todd has led and managed diverse Regulatory and Research & Development (R&D) organizations over numerous sites and regions. Todd is a highly-regarded and well-recognized industry leader, routinely called upon to speak at industry and U.S. Food and Drug Administration (FDA) public meetings. In addition, Todd served as the elected Chair of PhRMA's Regulatory Steering Group and was actively engaged in the most recent PDUFA VII negotiations with the U.S. FDA.

Prior to Sanofi, Todd was employed at Bayer where he most recently held the position of Vice President & Head, Regulatory Affairs Americas and Chief U.S. Regulatory Officer for the Pharmaceuticals Division. In addition to his responsibilities in Regulatory Affairs, Todd was also Bayer Pharmaceuticals U.S. R&D Site Head. Prior to Bayer, Todd held senior Regulatory and R&D leadership positions at both Roche / Genentech and Merck / Schering-Plough.

Todd began his pharmaceutical industry career as a Rutgers University Post-Doctoral Pharmaceutical Industry Fellow working both at Roche and the U.S. FDA. He earned his Bachelor of Science and Doctor of Pharmacy degrees from Rutgers University and his Masters of Business Administration degree from Fairleigh Dickinson University.

Vinayak (Vinay) Kashyap Prasad serves as the Director of the Center for Biologics Evaluation and Research (CBER) at the U.S. Food and Drug Administration (FDA).

In his role as director of CBER, Dr. Prasad supervises the FDA's work regulating biological products for human use under applicable federal laws. CBER works to advance the public health by ensuring that biological products are safe and effective and available to those who need them and to provide the public with information to promote their safe and appropriate use.

Dr. Prasad came to the FDA from The University of California at San Francisco, where he has served since 2020 as a professor in the Department of Epidemiology and Biostatistics. Before that, Dr. Prasad was a Professor of Medicine in the Division of Medical Oncology and the Department of Public Health and Preventive Medicine at Oregon Health & Science University. His specialty is hematology and oncology. Before entering academia, Dr. Prasad had a Fellowship in Cancer Prevention at the National Cancer Institute and prior to that he was a Fellow in Oncology at the National Institutes of Health.
Dr. Prasad has published more than 500 academic articles, done extensive research in the field of oncology, and has presented at hundreds of scientific and medical conferences. He is the author of the books, "Malignant: How Bad Policy and Bad Evidence Harm People with Cancer" and "Ending Medical Reversal: Improving Outcomes, Saving Lives." He was the host of the oncology podcast "Plenary Session" and ran The Drug Development Letter.

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.

Tracey Sikora is Vice President of Research and Clinical Programs at the National Organization for Rare Disorders (NORD), where she leads NORD's Rare Disease Centers of Excellence network, IAMRARE patient registry platform, and research granting program. Prior to joining NORD, her 15-year career at the University of Pennsylvania included pre-clinical and clinical research in multiple rare diseases, including MPS I, VI, and VII, Duchenne Muscular Dystrophy, Krabbe disease, Niemann-Pick Type C, Familial Hypercholesterolemia, and Castleman disease. In 2022, Tracey co-founded Every Cure, a nonprofit biotech organization dedicated to finding additional uses for existing drugs using novel technical approaches and artificial intelligence.

Arup Sinha is a Senior Staff Fellow in the Division of Biometrics V, Office of Biostatistics which supports Office of Oncology Drugs at the Center for Drug Evaluation and Research (CDER). He received his PhD in Biostatistics at the University of Texas Health Center at Houston where his research focused on adaptive clinical trial designs. After earning his PhD, he pursued a postdoctoral fellowship at the Yale School of Public Health before joining FDA in 2018. His research interests include innovative clinical trial designs, rare disease drug development, use of real-world data, and externally controlled trials. He is heavily involved in research and evaluation of pediatric and rare cancer drug development.

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.

George Tidmarsh is the Director of the Center for Drug Evaluation and Research at the U.S. Food and Drug Administration (FDA). The Center's mission is to ensure that safe, effective, and high-quality drugs are available to the public. To achieve this, CDER regulates the medical products under its jurisdiction throughout their lifecycle, oversees the development of new and generic drugs, evaluates applications to determine whether drugs should be approved, monitors the safety of drugs after they are marketed, conducts research to advance regulatory science, and takes enforcement actions to protect the public from harmful products.

Dr. Tidmarsh is an accomplished physician-scientist and leader whose experience spans the full arc of drug development. He brings to the FDA over 30 years of experience in biotechnology, clinical medicine, and regulatory science and has authored 143 scientific publications and patents. Dr. Tidmarsh joined the FDA from Stanford University School of Medicine where he was Adjunct Professor, Pediatrics and Neonatology. He served as clinical faculty at Stanford for a number of years prior to devoting his career to clinical research and development.

As the founding co-director of Stanford's Master of Translational Research and Applied Medicine (M-TRAM) program, Dr. Tidmarsh helped train students and researchers to bridge academic research and clinical development to translate scientific discoveries into real-world medical solutions. He has led the successful clinical development of seven FDA-approved drugs and served as founder and CEO of multiple biopharmaceutical companies focused on oncology and critical care medicine and is widely recognized for his ability to bring forward innovative treatments that address serious unmet medical needs.

Karmen Trzupek currently serves as the Sr Director of Scientific Programs at Global Genes, a position that enables her to bring together her passion for supporting rare disease communities and her experiences with academia, telemedicine, advocacy, and industry. Karmen began her career in rare diseases nearly 25 years ago at Oregon Health & Science University. At InformedDNA, she developed the first nationwide telemedicine program for rare disease genetic counseling and genetic testing. She co-developed multiple pharma- and advocacy-sponsored genetic testing programs across a wide range of diseases and served as an expert responder to the FDA. She has volunteered with several patient advocacy organizations: she served more than 10 years on the Board of Directors for the Usher Syndrome Coalition and the Hear See Hope Foundation, and currently serves on the Scientific Advisory Board for the Frontotemporal Dementia Registry. At Global Genes, Karmen drives collaborative programming and partnerships to maximize the RARE-X data platform for the advancement of patient-driven research.

Tingting Zhou is a Lead Mathematical Statistician in the Office of Biostatistics and Pharmacovigilance at the Center for Biologics Evaluation and Research (CBER), U.S. Food and Drug Administration (FDA). She provides regulatory statistical support to the Office of Therapeutic Products (OTP), focusing on clinical trials involving tissue, cellular, and gene therapies across therapeutic areas such as neurology, ophthalmology, cardiology, nephrology, and malignant hematology. Dr. Zhou holds a B.A. in Economics and Applied Mathematics from Johns Hopkins University and a Ph.D. in Biostatistics from the University of Michigan, Ann Arbor.

Moderators Biographies

Steve Berman serves as VP of Science and Regulatory Affairs for the Biotechnology Innovation Organization (BIO). Steve joined BIO after serving as a Director, Translational Regulatory Affairs, at AstraZeneca. Steve joined AZ after eight years at FDA, working in both CDER and OC. He began his federal service focusing on regulatory operations, including the stand-up of the Oncology Center of Excellence and the creation and adoption of the 21st Century Cures Workplan, and concluded his time at FDA focused on speeding new therapies to market through drug development tool qualification. Prior to joining FDA, Steve held a leadership role in Medical Affairs at Sucampo Pharmaceuticals.

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.

Rachele Hendricks-Sturrup is the Research Director of Real-World Evidence (RWE) at the Duke-Margolis Institute for Health Policy in Washington, DC, strategically leading and managing the Institute's RWE Collaborative and RWE policy research portfolio and education. As an engagement expert, biomedical researcher, bioethicist, and policy practitioner with nearly 20 years of experience, her work directly addresses and serves key regulatory/legal, ethical, and social implications at the intersection of health policy and innovation implementation. Recently, she led groundbreaking work within the Institute's RWE Collaborative that resulted in the receipt of a 2024 Innovation Award from the Reagan-Udall Foundation for the United States Food and Drug Administration (FDA).

She presently serves on the Board of Directors for Public Responsibility in Medicine and Research (PRIM&R), Professional Society for Health Economics and Outcomes Research (ISPOR) Steering Committee, has served since 2023 on the Program Committee as both a member and Co-Chair for the Drug Information Association's annual RWE Conference.

Before joining Duke-Margolis, Dr. Hendricks-Sturrup served as Health Policy Counsel and Lead at the Future of Privacy Forum (FPF), leading the organization's health and genetic data initiatives and workgroup. Prior to FPF, she worked in several administrative and scientific roles at various industry, health care, academic, and government research institutions. Dr. Hendricks-Sturrup is also an accomplished health science journalist, having completed a comparative effectiveness research fellowship with the Association of Health Care Journalists in 2017 at the Patient-Centered Outcomes Research Institute in Washington, DC.

Rachel Sher is a Food and Drug Administration (FDA) regulatory lawyer with extensive experience in policy and legislative strategy who helps support life sciences companies as they navigate the broader FDA regulatory process across all stages of a product's life cycle.

Rachel previously served as the Vice President of Policy and Regulatory Affairs for the National Organization for Rare Disorders (NORD), where she led its rare disease-related federal policy and regulatory work with respect to the FDA and other federal agencies, as well as on Capitol Hill and in the states. Prior to her time at NORD, Rachel worked in the Commissioner's Office at the FDA where she led the agency's engagement on the 21st Century Cures Act legislative process, which involved working directly with FDA leadership and medical product centers to give strategic advice and guidance. Before heading to the FDA, Rachel spent ten years on Capitol Hill working as FDA Counsel for the Committee on Energy and Commerce, where she drafted and negotiated all major FDA legislation enacted during her tenure.

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.