Duke | Robert J. Margolis, MD Center for Health Policy

Improving Basic Research, Clinical Trial Infrastructure, and Community Engagement to Support Drug Development for ALS

Duke-Robert J. Margolis, MD, Center for Health Policy
Virtual Meeting (Zoom)
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Biographies

Jinsy Andrews is an Associate Professor of Neurology and serves as the Director of Neuromuscular Clinical Trials at Columbia University. She currently oversees neuromuscular clinical trials and cares for patients with neuromuscular disease, primarily with Amyotrophic Lateral Sclerosis (ALS). Dr. Andrews has extensive experience in all phases of human clinical trials and drug development in both academic and industry settings. Dr. Andrews is the elected co-chair of the Northeastern ALS (NEALS) Consortium, which is a network of over 100 ALS clinical research centers internationally. She is also elected to the National Board of Trustees of the ALS Association and is a Fellow of the American Academy of Neurology (FAAN). Dr. Andrews has also received the Diamond Award for ALS clinical research from Wings Over Wall Street and the Muscular Dystrophy Association. Dr. Andrews received her B.S. from Union College, M.Sc. in Biostatistics (Patient-Oriented Research) from Columbia University's Mailman School of Public Health and M.D. from Albany Medical College. She completed her residency training in Neurology at the University of Connecticut and served as the Chief Neurology Resident in her final year. Dr. Andrews completed fellowship training in Neuromuscular Disease/ALS and Clinical Neurophysiology at Columbia University. She is board certified in Neurology, Neuromuscular Disease, and Electrodiagnostic Medicine.

Eric Bastings is the Deputy Director at the Office of Neuroscience (ON), as well as the Director at the Division of Neurology 1 (DN1), Center for Drug Evaluation and Research, Food and Drug Administration. The Office of Neuroscience includes five review divisions: Division of Neurology 1 (DN1); Division of Neurology 2 (DN2); Division of Psychiatry (DP); Division of Anesthesiology, Addiction Medicine, and Pain Medicine (DAAP); and Division of Pharmacology and Toxicology for Neuroscience (DPTN). Dr. Bastings joined FDA in 2000, as a Medical Officer in the Division of Neurology Products. He subsequently served as Clinical Team Leader in the same division from 2003 to 2008, before being appointed Deputy Director in 2008. Prior to joining the FDA, Dr. Bastings was Assistant Professor of Neurology at Wake Forest University School of Medicine, Winston-Salem, North Carolina.

Richard Bedlack is currently a Professor of Neurology at Duke and Director of the Duke ALS Clinic. Dr. Bedlack grew up in a small town in central Connecticut. He went to college at William and Mary in Virginia, then back to Connecticut for an MD and PhD in Neuroscience at UConn. Finally, he came to Duke where he completed his Medicine Internship, Neurology Residency, Neuromuscular Fellowship, and Masters in Clinical Research Science. He has won awards for teaching and patient care, including best Neurology teacher at Duke, Health Care Hero, Strength Hope and Caring Award, America's Best Doctor, the American Academy of Neurology Patient Advocate of the

Year and the Rasmussen ALS Patient Advocate of the Year. He has received ALS research grants, participated in ALS clinical trials, published more than 120 ALS articles. He is leader of the international ALSUntangled program which utilizes social networking to investigate alternative and off label treatment options for patients with ALS, and leader of the ALS Reversals program which attempts to understand why some people with ALS recover from it, and to make this happen more often. He lives in Durham, North Carolina with his wife Shelly and two mischievous cats.

Michael Benatar is a Professor of Neurology and Walter Bradley Chair in ALS Research at the University of Miami. He is Executive Director of the University of Miami ALS Center and also serves as Chief of the Neuromuscular Division, and Vice Chair of Clinical & Translational Research in the Department of Neurology. He obtained his medical degree at the University of Cape Town in South Africa and is also trained in both basic neuroscience (DPhil, Oxford) and clinical research methods (Masters in the Science of Clinical Research Emory). He leads an active clinical and translational research program focused on biomarker and therapy development for ALS. He is the principal investigator of the ongoing Pre-Symptomatic Familial ALS (Pre-fALS) study as well as the CReATe Consortium, a rare diseases clinical research consortium focused on ALS and related neurodegenerative diseases.

Kristina Bowyer is currently the Vice President of Patient Centric Drug Development at Ionis. She joined Ionis in 1992 and she founded Ionis' Patient Advocacy program 2012 to ensure that the patient perspective is incorporated into every aspect of drug development including market authorization. Kristina's expertise is in rare and neurological diseases where she has focused on methods to capture burden of disease from both the patient and the caregiver perspectives through innovative partnerships across multiple programs and organizations, such as ALS, amyloidosis, myotonic dystrophy and spinal muscular atrophy. Ionis currently has an extensive rare disease pipeline of RNA targeted therapeutics and has established strategic alliances with global pharmaceutical companies with the expertise to successfully launch rare disease treatments and ensure broad patient access.

Wilson Bryan is Director of the Office of Cellular, Tissue, and Gene Therapies (OTAT) in the Center for Biologics Evaluation and Review (CBER), at the U.S. Food and Drug Administration (FDA). Dr. Bryan is a neurologist who graduated from the University of Chicago Pritzker School of Medicine. He served on the neurology faculty of the University of Texas Southwestern Medical School for 13 years. He has been an investigator on clinical trials in cerebrovascular disease and neuromuscular disorders, particularly amyotrophic lateral sclerosis. Dr. Bryan joined the FDA in 2000.

Teresa Buracchio is the Deputy Director for Division of Neurology 1 in the Office of New Drugs, Center for Drug Evaluation and Research, Food and Drug Administration. Dr. Buracchio is a neurologist with subspecialty training in geriatric neurology. 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, Dr. Buracchio worked at AbbVie as an Associate Medical Director for Neuroscience Clinical Development. She joined FDA in 2013 and the Division of Neurology Products in 2014 where she has worked as a clinical reviewer and team leader for Alzheimer's disease and dementia, epilepsy, and neuromuscular and rare diseases.

Patrizia Cavazzoni is the Acting Director at the Center for Drug Evaluation and Research (CDER) at the Food and Drug Administration (FDA). Dr. Cavazzoni received her medical degree at McGill University and completed a residency in Psychiatry and a fellowship in mood disorders at the University of Ottawa. She subsequently joined the faculty of medicine at the University of Ottawa as an assistant professor, where she was engaged in clinical work, teaching, and research on genetic predictors of mood disorders, authoring numerous peer-reviewed scientific publications. Following this, Dr. Cavazzoni worked in the pharmaceutical industry for several years, and held senior leadership positions in clinical development, regulatory affairs and safety surveillance. Dr. Cavazzoni is certified by the American Board of Neurology and Psychiatry, a Fellow of the Canadian Royal College of Physician and Surgeons, a member of the Canadian College of Neuropsychopharmacology and recipient of the American College of Psychiatrists' Laughlin Fellowship.

Merit Cudkowicz is the Chief of the Massachusetts General Hospital Neurology Service, Director, Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital, and the Julieanne Dorn Professor of Neurology at Harvard Medical School in Boston. Dr. Cudkowicz directs the Massachusetts General Hospital ALS Program and the Massachusetts General Hospital Neurological Clinical Research Institute. She is one of the founders and former co-directors of the Northeast ALS Consortium (NEALS), a group of over 100 clinical sites in the United States, Canada, Europe and the Middle East dedicated to performing collaborative academic led clinical trials and research studies in ALS. She is Principal Investigator of the Clinical Coordination Center for the National Institute of Neurological Disorders and Stroke's Neurology Network of Excellence in Clinical Trials (NeuroNEXT). The NeuroNEXT network is an efficient phase II network to develop innovative and new treatments for people with neurological disorders.

Billy Dunn is the Director of the Office of Neuroscience, Center for Drug Evaluation and Research, at the U.S. Food and Drug Administration. Dr. Dunn oversees five review divisions within ON: Division of Neurology 1 (DN1); Division of Neurology 2 (DN2); Division of Psychiatry (DP); Division of Anesthesiology, Addiction Medicine, and Pain Medicine (DAAP); and Division of Pharmacology and Toxicology for Neuroscience (DPTN). The five review divisions regulate and review Investigational New Drug applications and marketing applications for drug and biologic products for neurodegenerative, movement, and neuromuscular disorders (such as Alzheimer's

disease, Parkinson's disease, Huntington's disease, muscular dystrophy, and amyotrophic lateral sclerosis); for seizures, epilepsies, migraine and other headaches, traumatic brain injury, stroke, inner ear disorders, and multiple sclerosis and other neuroimmunologic disorders; for psychiatric and sleep disorders (such as bipolar disorder, schizophrenia, major depressive disorder, attention deficit hyperactivity disorder, obsessive-compulsive disorder, posttraumatic stress disorder, anxiety disorders, autism spectrum disorder, narcolepsy, and insomnia); and for acute pain, chronic pain, addiction, and surgical anesthetics, sedatives, and neuromuscular-blocking agents. More information can be found here: https://www.fda.gov/about-fda/center-drug-evaluation-and-research-cder/office-neuroscience.

Toby Ferguson is Head of the Neuromuscular Development Unit at Biogen and leads the neuromuscular clinical development group. Dr. Ferguson is a neuromuscular neurologist and neuroscientist who joined Biogen in 2013. His professional experience has been focused on developing treatments for neuromuscular disease, with a focus on ALS. Dr. Ferguson plays a key role in developing ALS clinical trials, and in driving preclinical strategy within neurodegenerative and neuromuscular disease. His group also works closely with the scientific and biomarker, and commercial teams at Biogen, Ionis, and other external collaborators to identify novel disease targets and to develop the needed tools for efficient clinical development. At Biogen, Dr. Ferguson has advanced multiple programs into the clinic for ALS, DM1, and Parkinson's disease. In ALS, one program (tofersen for SOD1 ALS) is in the final stages of clinical testing, and multiple additional programs are in early clinical testing including a program for C9ORF72 ALS and two programs for the broader ALS population. He is dedicated to the successful development of meaningful therapeutics for ALS and strongly believes that collaboration across industry, academia, and advocacy organizations is crucial to providing people with ALS the therapies they need and deserve. Prior to Biogen, Dr. Ferguson had a clinical neuromuscular neurology practice and a lab focused on peripheral axon injury and regeneration at Shriners Research Center and Temple University in Philadelphia. Toby trained in neurology and neuromuscular disease at the University of Pennsylvania. He obtained an MD and PhD (Neuroscience) at the University of Florida and maintains an interest in axon regeneration and degeneration.

Erin Fleming is Director of Research Operations at Project ALS, a non-profit 501(c)3 organization that identifies and funds promising scientific research toward the first effective treatments and a cure for ALS. Project ALS recruits the world's best scientists and doctors to work together—rationally and aggressively—to develop a better understanding of the ALS disease process and, in parallel, better therapeutic strategies. Previously, Erin was associate director at Project ALS, project manager at the biotech Applied Therapeutics, and project director at the boutique life sciences consulting firm Clearpoint Strategy Group. She holds a BA in English and Comparative Literature from Columbia University.

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.

Philip Green is an ALS Research Ambassador. Phil is a graduate of the University of Washington where he was on the 1991 National Championship UW Football team. He has spent the past 25 years building a career in developing technology solutions for brands such as Sony, Hewlett Packard, and Home Depot. A loving husband to his wife Jennifer, Phil is also a dedicated father to four children (Arianne-18, Hunter-17, Parker-13, and Whitney-10). Phil was diagnosed with ALS in August of 2018 and immediately dedicated himself to making a difference in the fight against this horrific disease. Phil is active in helping multiple ALS organizations with promoting ALS legislation and policy issues, increasing awareness and raising funds to find effective treatments and cures, and providing much needed support services for ALS patients and families. The following is a list of the various organizations that he works with and the role he serves with each: Augie's Quest, Leadership Council; Team Gleason, Board of Directors; ALS CURE Proct, Board of Directors; International Alliance of ALS/MND Associations, PCAC (PALS & CALS Advisory Council); International Alliance of ALS/MND Associations, Innovation and Technology Council; ALS Association, PCAC (PALS & CALS Advisory Council); Cytokinetics, Patient Advisory Board; Amylyx, Patient Advisory Council; Orphazyme, Patient Advisor; Clara Health, Breakthrough Crew; MT Pharma, Technology Advisory Board; HEALEY Platform Trial, Patient Advisory Committee; HEALEY Platform Trial, Recruitment and Retention Committee; UW Medicine Center for Translational Muscle Research, Patient Advisor; CDMRP ALS Research Program, Consumer Reviewer; I AM ALS, Clinical Trials Committee Co-chair; I AM ALS, Legislative Affairs Committee; I AM ALS, Community Advisory Committee; NEALS, Research Ambassador; International Symposium on ALS/MND, Patient Fellow (2019, 2020)

Amelie Gubitz is a Program Director in the Division of Neuroscience at the National Institute of Neurological Disorders and Stroke. Her portfolio includes basic, translational, and clinical research on amyotrophic lateral sclerosis, X-linked spinal and bulbar muscular atrophy, hereditary spastic paraplegia and Friedreich's ataxia. Dr. Gubitz earned a Ph.D. in pharmacology from the University of Cambridge, UK, and then completed a Wellcome Trust Fellowship at Harvard Medical School in circadian biology. She has also worked as a Senior Scientist in the target discovery group for neuropathic pain at Parke Davis, UK, and as a Research Assistant Professor at the University of Pennsylvania where she studied the molecular pathogenesis of spinal muscular atrophy.

Jane Larkindale is the Executive Director of both the Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP) and the Duchenne Regulatory Science Consortium (D-RSC) at the Critical Path Institute and runs several other rare disease projects. She has dedicated the past decade to accelerating drug development for rare diseases, through promoting increased efficiency, increased cooperation, shared infrastructure and supporting high quality science. She launched the RDCA-DAP with colleagues from the Food and Drug Administration and the National Organization for Rare Disorders in September of 2019 with the goal of aggregating data across rare diseases to inform on natural history, biomarkers and outcome measures. Through D-RSC, she leads an international consortium dedicated to developing regulatory-ready drug development tools for Duchenne Muscular Dystrophy, specifically developing a clinical trial simulation tool, data standards and an integrated database of clinical data that can be used by the community. She has also

worked with several non-profit organizations and consulted with industry on projects to support research, develop infrastructure and help researchers source the tools and collaborators they need. She is a molecular biologist by training, having completed her D.Phil. (Ph.D.) in the Department of Plant Sciences at Oxford University in 2001, which she attended on a Rhodes Scholarship.

Peter Marks is Director of the Center for Biologics Evaluation and Research at the U.S. Food and Drug administration. Dr. Marks received his graduate degree in cell and molecular biology and his medical degree at New York University and completed Internal Medicine residency and Hematology/Medical Oncology training at Brigham and Women's Hospital in Boston. He has worked in academic settings teaching and caring for patients and in industry on drug development. He joined the FDA in 2012 as Deputy Center Director for CBER and became Center Director in January 2016.

Paul Mehta is the Principal Investigator for the United States congressionally-mandated National Amyotrophic Lateral Sclerosis (ALS) Registry which resides within the Centers for Disease Control and Prevention and is responsible for providing medical, scientific, and epidemiological expertise on matters related to ALS.

Paul Melmeyer is the Director of Regulatory Affairs at the Muscular Dystrophy Association (MDA). In this role, Paul leads MDA's policy and advocacy initiatives pertaining to therapeutic development with a particular focus on regulatory efforts. Prior to joining MDA, Paul spent over six years with the National Organization for Rare Disorders (NORD). At NORD, Paul led the Federal policy operations in developing and advocating for the enactment and implementation of pro-rare disease patient policy. Paul also holds a Master of Public Policy (MPP) from the George Washington University. Paul is constantly seeking opportunities to make a difference in the lives of the less fortunate through compassionate and effective policy change.

Carolina Mendoza-Puccini Carolina Mendoza-Puccini is a Program Officer at the National Institute of Neurological Disorders and Stroke (NINDS), Division of Clinical Research (DCR). Dr. Mendoza-Puccini received her medical degree from Universidad del Norte Medical School in Colombia. Before joining NINDS, she was a Research Fellow at the University of Miami Miller School of Medicine, Department of Neurology, Division of Cognitive Disorders. Carolina is the Program Officer for the Strategies to Innovate Emergency Care Clinical Trials Network (SIREN) and the Clinical Trials Readiness Project. In addition, Carolina manages the NINDS Common Data Elements (CDE) project and participates in various trans-NIH data harmonization working groups. Her main interests are data standards, data sharing and human subjects.

Sandy Morris is an ALS Patient Advocate. Sandy worked for Hewlett-Packard for almost 30 years. She was diagnosed with ALS on January 6, 2018. Sandy became a fierce ALS advocate after her clinical trial experience and vowed to make changes in this destructive disease. She partnered with Bryan Wallach, founder of I Am ALS, to lead the patient advisory council and clinical trials committee. Standing on the shoulders of giants, they worked with the FDA leaders to get the guidance document for clinical trials updated in September 2019. They met with over 30 ALS drug sponsors to introduce the Patient centric trial design (PaCTD) ratings. They created an ALS Caucus which currently has over 150 members. Recently they have been involved with getting over 265 co-sponsors for the bill H.R.8662/S.4867 Accelerating Access to Critical Therapies for ALS Act (ACT), which ensures expanded access in trials, as well as establishing a Consortium at the FDA level. Sandy has also had the privilege of working on the Patient Advisory Council and Recruitment and Retention Team for the Sean Healey Platform Trial. Sandy was also honored to receive the 2020 Stephen Heywood Award from ALS TDI.

Melanie Quintana is a Director and Senior Statistical Scientist at Berry Consultants, where she specializes in designing Bayesian adaptive clinical trials across a wide range of therapeutic areas. Her work includes numerous examples in designing platform trials and clinical trials in rare and progressive disease with a focus on developing models of disease progression to design better and more powerful clinical trials. Before joining Berry Consultants, she earned her Ph.D. in Statistics from Duke University and went on to pursue a Postdoc in Biostatistics at The University of Southern California. While at Duke and USC, she worked closely with collaborators within multiple large studies and consortiums around the nation to develop and implement analytical strategies to assess genetic risk factors for various complex diseases.

Jeremy Shefner is the Kemper and Ethel Marley Professor and Chair of Neurology and is Senior Vice President of Barrow Neurological Institute. Dr. Shefner joined Barrow in the fall of 2014. Previously, he was Chair of Neurology at SUNY Upstate Medical University in Syracuse, N.Y. Dr. Shefner earned his medical degree from Northwestern University Medical School and his doctorate in sensory physiology from the University of Illinois. He completed his residency at Harvard Longwood Neurology Training Program and his fellowship in clinical neurophysiology at Brigham and Women's Hospital before joining the faculty at Harvard Medical School. Dr. Shefner's research focuses on biomarker development and clinical therapeutics of amyotrophic lateral sclerosis (ALS) and spinal muscular atrophy. He is co-founder and former co-chair of the Northeast ALS (NEALS) Clinical Trials Consortium, the largest ALS-dedicated group of its kind in the world. He continues to direct NEALS outcomes and clinical monitoring cores, currently involved in multiple clinical trials. His main research focus is the development of novel outcome measures for ALS trials. Dr. Shefner has published more than 200 chapters and papers in peer- reviewed journals and has served on multiple grant review panels. He has also participated in committees organized by the Institute of Medicine to investigate the relationship between military service and ALS, as well as the health effects of Agent Orange on Vietnam War veterans. In 2014, Dr. Shefner received the Sheila Essey Award for ALS Research, the major award given annually by the ALS Association and the American Academy of Neurology. He is a Fellow of the American Academy of Neurology and the American Neurological Association, on the editorial boards of both the ALS Journal and Neurotherapeutics and is the neuromuscular section editor of UpToDate..

Alexander Sherman Alex is the Director, Center for Innovation and Biomedical Informatics (CIB) at the NCRI and a Principal Associate in Neurology at Harvard Medical School. The focus of Alex's work and research is conception, design, development of technology, platforms, infrastructure, and methods for collaborative clinical research and optimization of clinical research in disease-specific networks. Alex's CIB team has introduced such innovative and break-through concepts and products as NeuroBANK®, a patient-centric research platform and accelerated research environment, utilized by hundreds of investigators at 80+ clinical sites in 14 countries; PRO-ACT™, a harmonized anonymized dataset from 23 ALS clinical trials that is an invaluable resource for the design of future clinical trials and the identification of unique observations, novel correlations, and biomarkers of ALS; and The SigNET™ (NeuroGUID) Platform for unique and secure patient identification across research continuum. All three platforms have won the Best Practice Award from Bio-IT World Congress in Precision and Translational Medicine category in 2013, 2016, 2018, and 2020, as "outstanding example of how technology innovations and strategic initiatives can be powerful forces for change in the life sciences, from basic biomedical research to drug development and beyond." Other aspects of Alex's research are patient empowerment, developing a system of incentives and supporting technologies to secure collaboration, integration, harmonization and sharing of clinical and research information by all clinical research enterprise participants. Alex served on the NINDS Common Data Elements committee, is the Chair of the Big Data committee of NEALS ALS consortium and is a founding Board Member of ALD Connect consortium. Mr. Sherman holds Master of Science degree in Nuclear Engineering and graduated from a doctorate program in Nuclear Engineering. For more information, please visit https://www.data4cures.org

Neil Thakur is Chief Mission Officer at the ALS Association. Dr. Thakur brings more than two decades of experience as a public health expert to the fight against ALS. He has led The ALS Association's mission programs – research, care services, and advocacy – since 2018. Before joining the Association, Dr. Thakur served in the National Institutes of Health (NIH) Office of the Director, making NIH research more impactful. He managed the world's largest policy to make biomedical research papers publicly accessible and co-chaired the White House taskforce that lead to the requirement that all federal science agencies adopt similar policies. He also spent a year on detail to the US Senate Special Committee on Aging, focusing on effective long-term health care. Prior to his time at NIH, he was Assistant Director of Health Services Research and Development at the Department of Veterans Affairs. Dr. Thakur has received numerous federal awards, including the Secretary for Health and Human Services' award for Meritorious Service, the second highest award that the Secretary can bestow. Dr. Thakur holds a Ph.D. in Health Policy from Yale University School of Public Health and completed a NIMH postdoctoral fellowship in mental health services research at the Cecil G. Sheps Center for Health Services Research at the University of North Carolina at Chapel Hill.

Julia (Julie) Tierney is the Chief of Staff for FDA's Center for Biologics Evaluation and Research (CBER). In that capacity, she serves as the principal advisor to the CBER Center Director and facilitates planning and implementation of Center priorities. Ms. Tierney joined CBER in early 2017 as a Senior Policy Advisor for Strategic Planning & Legislation. From 2015 to 2016, Ms. Tierney served as FDA's detailee to the U.S. Senate Health, Education, Labor & Pensions (HELP) Committee as a Senior Health Policy Advisor. She joined FDA in 2008, as an Associate Chief Counsel for Drugs in FDA's Office of Chief Counsel, providing ongoing legal counsel to FDA leadership and program staff on drug and biologic-related legal issues. Prior to working at FDA, Ms. Tierney practiced food and drug law at private law firms. She received her J.D. from Georgetown University Law Center and her undergraduate degree in Biology and History from Johns Hopkins University.

Bryan Traynor is a neurologist and Senior Investigator at the National Institute on Aging, and adjunct professor at Johns Hopkins University. Dr. Traynor is best known for his work aimed at understanding the genetic etiology of amyotrophic lateral sclerosis (ALS) and frontotemporal dementia (FTD). He led the international consortium that identified pathogenic repeat expansions in C9ORF72 as a common cause of ALS and FTD. He has over 200 publications in professional journals, including Neuron, New England Journal of Medicine, and Nature Neuroscience, and has received numerous awards for his work including the NIH Director's award, the Derek Denny-Brown award, the Sheila Essey award for ALS Research, and the Potamkin Prize for Research in Pick's, Alzheimer's, and Related Diseases. He has sat on the editorial boards of JAMA Neurology, JNNP, and Neurobiology of Aging, and is currently an associate editor of Brain. He received his medical degree, a Medical Doctorate, and a Doctor of Philosophy from University College Dublin. He also received a Masters in Medical Science from HST Harvard-MIT. He completed a Neurology residency and fellowship training at Massachusetts General Hospital and Brigham and Women's Hospital, Boston, and was a Staff Neurologist at Harvard Medical School and Massachusetts General Hospital before moving to Boston.

Fernando Vieira is the Chief Scientific Officer at the ALS Therapy Development Institute where he leads a multidisciplinary team of scientists and research in their efforts to discover and develop effective treatments and biomarkers for amyotrophic lateral sclerosis (ALS). He has been focused on ALS research since 2001. His research findings – spanning basic discovery, preclinical discovery and optimization, clinical development, and translational ALS research have been widely cited. Specifically, he has focused on optimizing animal models for ALS preclinical pharmacology and drug screening. He has led or contributed to the identification and preclinical validation of four drugs that have been advanced into human clinical assessment and has been awarded multiple patents for those drugs. His teams have contributed important basic research findings to the study of genetic ALS, focusing on SOD1 mutation biology and C9orf72 mutation biology. Dr. Vieira also serves on the Scientific Advisory Board of the ALS Investment Fund. Dr. Vieira received his medical degree from Harvard Medical School and a Bachelor's degree in Biological Engineering from the University of Florida.

Richard White is a Policy Analyst at National Organization for Rare Diseases (NORD). Richard White joined NORD in mid-2020 and handles a portfolio that includes FDA, NIH, and CDC issues- specifically, issues relating to drug development and review as well as regulatory and scientific innovation. He also advocates for NORD's policies on Capitol Hill as well as various federal agencies. Prior to joining NORD, Richard spent time at the Biotechnology Innovation Organization working on rare and orphan disease initiatives as well as other regulatory issues in the drug development and approval life-cycle.

Lei Xu is the Chief of General Medicine Brach 2 in the FDA's Division of Clinical Evaluation and Pharmacology/Toxicology (DCEPT) of Office of Tissue and Advanced Therapies (OTAT) at Center for Biologics Evaluation and Research (CBER). Her Branch is responsible for reviewing clinical trial protocols, overseeing clinical trial conducts and evaluating trial data of investigational biological products (e.g., gene therapy, cellular therapy and plasma-derived products) in several clinical areas, including Neurology, Ophthalmology, Pulmonology, Dermatology, and burn and wound care. Her Branch reviewed all the clinical data that led to FDA-approval of the first two adeno associated virus-based gene therapy products: voretigene neparvovec (Luxturna) for the treatment of retinal dystrophy due to RPE65 mutation, and onasemnogene abeparvovec (Zolgensma) for the treatment of spinal muscular atrophy. In addition to the regulatory responsibilities, she is actively involved in FDA guidance development, including the Guidance for Industry: Expedited Programs for Serious Conditions, Guidance for Industry: Gene Therapy for Retinal Disorders, and draft Guidance for Industry: Gene Therapy for Neurodegenerative Diseases. Dr. Xu received her M.D. from Central South University Xiangya School of Medicine in China, and her Ph.D. in neuroscience from Yale University. She completed residency training in Neurology at Loyola University Chicago. She is board-certified in Neurology by the American Board of Psychiatry and Neurology.

Duke-Margolis Moderators

Mark McClellan is Director of the Margolis Center for Health Policy at Duke University and the Robert J. Margolis Professor of Business, Medicine, and Policy. He is a physician economist who focuses on quality and value in health care including payment reform, real-world evidence and more effective drug and device innovation. He is former administrator of the Centers for Medicare & Medicaid Services and former commissioner of the U.S. Food and Drug Administration, where he developed and implemented major reforms in health policy. He was previously Senior Fellow at the Brookings Institution and a faculty member at Stanford University.