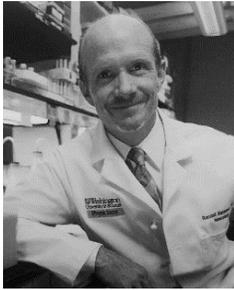# Duke | Robert J. Margolis, MD Center for Health Policy

**Public Meeting: Utilizing Innovative Statistical Methods
and Trial Designs in Rare Disease Settings**
DoubleTree by Hilton • Silver Spring, MD
March 19, 2018

Biographies

Randall Bateman, MD, is the Charles F. and Joanne Knight Distinguished Professor of Neurology, Director of the Dominantly Inherited Alzheimer Network (DIAN), and Director of the DIAN Trials Unit (DIAN-TU). Dr. Bateman's research focuses on the pathophysiology and development of improved diagnostics and treatments of Alzheimer's disease. His lab recently reported on an accurate blood test for Alzheimer's disease plaques. Dr. Bateman's research in DIAN has provided evidence for a cascade of events beginning decades before symptom onset that leads to AD dementia, supporting development of Alzheimer's disease prevention trials.

Julie Beitz has had a 23-year career at FDA involving both pre- and post-approval regulatory activities. Since 2006, she has served as the Director, Office of Drug Evaluation III. In this capacity, she oversees the review activities of the Division of Gastroenterology and Inborn Errors of Metabolism, the review division responsible for advising manufacturers on the development and approvability of new drugs and biologic products intended to treat rare metabolic disorders. She has served on several working groups involved in the implementation of the new regulatory authorities provided for under the FDA Amendments Act of 2007 (FDAAA), as the CDER liaison for FDA's Office of Women's Health, and as a member of CDER's Medical Policy Council, and Drug Development Tools Committee.

Scott Berry, PhD, is President and a Senior Statistical Scientist at Berry Consultants, LLC, where he works on the design of innovative clinical trials, Bayesian statistics, and clinical trial simulation. He earned his PhD in statistics from Carnegie Mellon University and was an Assistant Professor at Texas A&M University before co-founding Berry Consultants in 2000. He is adjunct faculty in the Department of Biostatistics at the University of Kansas Medical Center. Dr. Berry was elected as a Fellow of the American Statistical Association in 2013.

Abby Bronson is the Senior Vice President of Research Strategy at Parent Project Muscular Dystrophy where she develops research strategies and tools that will accelerate drug development in the Duchenne space. She brings experience from a variety of areas, including industry, academic medical centers and NIH. Previously, she was responsible for Director of Operations at the Division of Clinical Innovation at the National Center for Accelerating Translational Science at NIH. Previous to that she was Manager of Special Projects at Children's National Medical Center, focusing on rare disease drug development in the neuromuscular space. While at Children's, Abby facilitated interactions and negotiations with research institutions, industry, patient advocates and federal funders involved in therapeutic development for products to treat

Duchenne Muscular Dystrophy. Previous to Children's National, Abby held positions of increasing responsibility at MedImmune, most recently being the Director of International Marketing. She also worked in New Product Planning and US Marketing while at MedImmune. Abby started her career at Ciba-Geigy and subsequently worked at Medtronic. She holds an MBA from the Wharton School of Business at the University of Pennsylvania and a BA from the University of Vermont.

Aloka Chakravarty is the Acting Director of the Office of Biostatistics in CDER, FDA. Dr. Chakravarty joined CDER in 1992 and brings to her current position considerable experience in CDER. She is an internationally recognized thought leader in multi-regional clinical trials, safety evaluation, surrogate markers and biomarkers in drug development and has presented and published widely on it. Her research interests include MRCTs, surrogate endpoint methodology, biomarkers, interim analysis, meta-analysis, Bayesian methodology, safety evaluation and statistical computing. Dr. Chakravarty served as an Adjunct Faculty in Department of Statistics, Foundation for Advanced Education in the Sciences, National Institutes of Health. Dr. Chakravarty has received numerous awards, including the FDA Award of Merit in 2008 and Dr. Frances O. Kelsey Drug Safety Excellence Award in 2012. Aloka received her Ph.D. in Statistics from Temple University, and M.Stat from Indian Statistical Institute. Dr. Chakravarty is a Fellow of the American Statistical Association and an Associate Editor of Statistics in Biomedical Research.

Dr. Yeh-Fong Chen is a statistical team leader, supporting the Division of Gastroenterology and Inborn Errors Products at CDER of the FDA. She joined the FDA in 2000 immediately after graduation, with a Ph.D. in Statistics from the University of Iowa. She has had years' experience in reviewing drug applications for psychiatry products and has performed reviews on drug products in the cardiovascular and renal disease areas for more than five years. In recent three years, she has reviewed many applications for gastrointestinal, liver, and rare diseases. In addition to performing IND/NDA reviews, she devotes herself to research and collaborates with FDA statistical and clinical colleagues as well as statisticians in academia. Her research interests include clinical trial designs, especially adaptive designs and two-stage designs, multi-regional clinical trials, and statistical approaches handling missing data. Moreover, she has had experience in organizing sessions for various conferences including DIA, ENAR, ICSA, and FDA/Industry workshop.

Gregory Daniel is a Clinical Professor in Duke's Fuqua School of Business and Deputy Director in the Duke-Robert J. Margolis Center for Health Policy at Duke University. Dr. Daniel directs the DC-based office of the Center and leads the Center's pharmaceutical and medical device policy portfolio which includes developing policy and data strategies for improving development and access to innovative pharmaceutical and medical device technologies. This includes post-market evidence development to support increased value, improving regulatory science and drug development tools, optimizing biomedical innovation, and supporting drug and device payment reform. Dr. Daniel is also a Senior Advisor to the Reagan-Udall Foundation for the FDA and Adjunct Associate Professor in the Division of Pharmaceutical Outcomes and Policy at the UNC Eshelman School of Pharmacy. Previously, he was Managing Director for Evidence Development & Biomedical Innovation in the Center for Health Policy and Fellow in Economic Studies at the Brookings Institution and Vice President, Government and Academic Research at HealthCore (subsidiary of Anthem, Inc). Dr. Daniel's research expertise includes utilizing electronic health data in designing research in health outcomes and pharmacoeconomics, comparative effectiveness, and drug

safety and pharmacoepidemiology. Dr. Daniel received a PhD in pharmaceutical economics, policy and outcomes form the University of Arizona, as well as an MPH, MS, and BS in Pharmacy all from The Ohio State University.

Billy Dunn is the Director of the Division of Neurology Products at the U.S. Food and Drug Administration's Center for Drug Evaluation and Research. The Division of Neurology Products (DNP) regulates and reviews Investigational New Drug (IND) applications and marketing applications for drug and biologic products for the treatment of neurological diseases and conditions, such as Alzheimer's disease, stroke, Parkinson's disease, Huntington's disease, epilepsy, migraine headaches, muscular dystrophy, amyotrophic lateral sclerosis, multiple sclerosis, cerebral palsy, dementia, narcolepsy, Lennox-Gastaut syndrome, and insomnia.

Cartier Esham serves as Executive Vice President for Emerging Companies at the Biotechnology Innovation Organization (BIO). In this role, Dr. Esham manages and directs BIO's policy development, advocacy, research and educational initiatives for BIO's emerging companies, which comprise approximately 90% of BIO's membership. This includes capital formation policy and health policy impacting emerging companies, as well as research and analysis of the biopharmaceutical industry and life-science investment and financing. Among the priorities of BIO's Emerging Companies Section are: promoting a science-based FDA regulatory environment; supporting NIH funding and programs/initiatives such as SBIR and NCATS that promote the effective transfer of technology; and working to create a public and private market environment that incentivizes the research and development of innovative treatments and therapies. Prior to joining BIO, Dr. Esham was a Vice President and Director of Research at Dutko Worldwide, a private consulting firm in Washington, D.C. There she worked on a variety of environmental, education, science, technology and health care related issues both on the federal and state/local levels. Esham has a Ph.D. in Microbiology from the University of Georgia, a Master's degree in Marine Biology from the University of North Carolina at Wilmington and a Bachelor of Science Degree from the University of Kentucky. She has published papers in peer-reviewed science journals on water quality, marine microbial ecology and bacterial phylogeny.

Telba Irony is Deputy Director of the Office of Biostatistics and Epidemiology at CBER. She joined FDA to implement the use of Bayesian statistics for the regulation of medical devices, and led the Decision Analysis initiative at CDRH including Bayesian statistics, benefit-risk determinations, and the science of patient input. Telba received the 2014 FDA Excellence in Analytical Science Award for spearheading innovative regulatory science studies culminating in the release of novel guidance documents, supporting complex policy decision making and changing the submission review paradigm. She has a PhD from Berkeley, is a fellow of the American Statistical Association, and an elected member of the International Statistical Institute.

Laura Lee Johnson is the Acting Director of the U.S. Food and Drug Administration (FDA) Center for Drug Evaluation and Research's (CDER) Division of Biometrics III and the Clinical Outcome Assessment liaison for CDER's Office of Biostatistics. She provides guidance on design, logistics, implementation, and analysis of research studies ranging from person reported outcome (PRO) measure qualification to safety and randomized studies of all sizes. She works across CDER and other parts of FDA on patient focused drug development initiatives. Prior to working at the FDA she spent over a decade at the U.S. National Institutes of Health (NIH) working on and overseeing clinical research and research support programs. At NIH she contributed to programs such as the CTSAs, PROMIS, and the NIH Collaboratory. She has been involved with numerous projects developing, validating, and using clinical outcome assessments in both patient care and research and received several NIH Director's Awards and an FDA award for her work involving clinical trials in various populations, health related quality of life, and teaching. She has co-authored several articles and book chapters across a variety of disciplines and served on NIH and PCORI review and methods panels. Among her many activities Dr. Johnson serves on the FDA-NIH Interagency Clinical Outcome Assessments Working Group, the IMI PREFER Scientific Advisory Board, co-directs the NIH Principles and Practice of Clinical Research course, and volunteers with the Montgomery County Maryland Science Fair. Dr. Johnson received her Ph.D. in Biostatistics from the University of Washington.

Petra Kaufmann, MD, MSc, FAAN, is the Vice President R & D Translational Medicine at AveXis, a clinical-stage gene therapy company focused on bringing gene therapy out of the lab and into the clinical setting for patients and families devastated by rare and orphan neurological genetic diseases. Prior to joining AveXis, Dr. Kaufmann was director of the NCATS Office of Rare Diseases Research where her work included overseeing the Rare Diseases Clinical Research Network program, Genetic and Rare Diseases Information Center, and the Toolkit for Patient-Focused Therapy Development. Kaufmann focuses on engaging a broad range of rare diseases research stakeholders to accelerate translation from discovery to health benefits through use of innovative methods. While at NCATS, she initiated the Trial Innovation Network, a US network of academic medical centers using a single IRB, pre-negotiated contracts, and participant recruitment strategies based on electronic medical record data. Before joining NCATS, Kaufmann was the director of the Office of Clinical Research at the National Institute of Neurological Disorders and Stroke (NINDS), where she worked with investigators to plan and execute a large portfolio of clinical research studies and trials in neurological disorders, and where she launched NeuroNEXT, a trial network to support scientifically sound, biomarker-informed phase 2 trials for neurological diseases in partnership with academia, industry and patients groups. Kaufmann spent most of her career at Columbia University, where she conducted postdoctoral research in molecular genetics and where she trained in Neurology prior to becoming a faculty member in 2000, and receiving tenure in 2009. While at Columbia University, her research focused on observational studies and trials in mitochondrial diseases and neuromuscular diseases including SMA and ALS. She has served on scientific advisory boards for numerous national and international organizations such as the Clinical Trial Transformation Initiative (CTTI), the Italian Telethon, the Treat NMD Advisory Committee for Therapeutics, the Great Ormond Street/U of London Biomedical Research Centre, the European Reference Network for Rare Neurological Disorders, and the International Rare Diseases Research Consortium (IRDiRC) for which she chairs the Interdisciplinary Science Committee. Her research has resulted in over 120 publications and numerous presentations at scientific meetings. A native of Germany, Kaufmann earned her M.D. from the University of Bonn and her Masters in Biostatistics from Columbia University's Mailman School of Public Health. Kaufmann is board certified in neurology,

neuromuscular medicine and electrodiagnostic medicine. She currently volunteers to see patients in the Muscular Dystrophy Association Clinic at Children's National Medical Center in Washington, D.C. In her free time, she enjoys yoga, skiing, cooking and most of all spending time with her husband and their four children.

Lucas Kempf is the Acting Associate Director for the Rare Disease program in the OND immediate office. Prior to joining FDA in 2012, Lucas spent 8 years at NIH with a focus on neuroscience research, working to understand the genetics of neuropsychiatric disease and developing translational approaches and therapeutics to study these disorders. Lucas was trained at UC-Berkeley in molecular biology and genetics, received his MD degree from the University of Kansas, and did his post-graduate training in psychiatry at Georgetown and Johns Hopkins before moving to the NIH for fellowship.

Lisa LaVange, PhD, is Professor and Associate Chair of the Department of Biostatistics in the Gillings School of Global Public Health at the University of North Carolina at Chapel Hill. She is also director of the department's Collaborative Studies Coordinating Center (CSCC), overseeing faculty, staff, and students involved in large-scale clinical trials and epidemiological studies coordinated by the center. From 2011 to 2017, Dr. LaVange was director of the Office of Biostatistics in the United States Food and Drug Administration (FDA) Center for Drug Evaluation and Research (CDER). There, she oversaw more than 200 statisticians and other staff members involved in the development and application of statistical methodology for drug regulation. She was a leader in developing and assessing the effectiveness and appropriateness of innovative statistical methods intended to accelerate the process from drug discovery to clinical trials to FDA approval and patients' benefit, with a particular focus on rare diseases. Prior to her government and academic experience, she spent 16 years in non-profit research and 10 years in the pharmaceutical industry. Dr. LaVange is an elected fellow of the American Statistical Association (ASA) and is the 2018 ASA President.

Nicole Mayer Hamblett, PhD, is a Professor of Pediatrics and Adjunct Professor of Biostatistics at the University of Washington, and Co-Executive Director of the Cystic Fibrosis Therapeutics Development Network Coordinating Center at Seattle Children's Hospital. She is a biostatistician who has led the design and analysis of numerous clinical studies which have advanced clinical care and outcomes in CF. Dr. Hamblett directs the TDN Consulting Program which partners with industry sponsors to strategize on complex drug development issues relevant to rare diseases. She is currently a principal investigator on therapeutic trials optimizing antibiotic treatment regimens in CF and studies to advance biomarkers supporting therapeutic development for novel CFTR modulator therapies. Dr. Hamblett is a member of the Cystic Fibrosis Foundation's Patient Registry Committee and Clinical Research Advisory Board.

Mark McClellan is the Robert J. Margolis Professor of Business, Medicine, and Policy, and Director of the Duke-Margolis Center for Health Policy at Duke University with offices at Duke and in Washington DC. The new Center will support and conduct research, evaluation, implementation, and educational activities to improve health policy and health, through collaboration across Duke University and Health System, and through partnerships between the public and private sectors. It integrates the social, clinical, and analytical sciences to integrate technical expertise and practical capabilities to develop and apply policy solutions that improve health and the value of health care locally, nationally, and worldwide. Dr. McClellan is a doctor and an economist, and his work has addressed a wide range of strategies and policy reforms to improve health care, including such areas as payment reform to promote better outcomes and lower costs, methods for development and use of real-world evidence, and more effective drug and device innovation. Before coming to Duke, he served as a Senior Fellow in Economic Studies at the Brookings Institution, where he was Director of the Health Care Innovation and Value Initiatives and led the Richard Merkin Initiative on Payment Reform and Clinical Leadership. He also has a highly distinguished record in public service and in academic research. Dr. McClellan is a former administrator of the Centers for Medicare & Medicaid Services (CMS) and former commissioner of the U.S. Food and Drug Administration (FDA), where he developed and implemented major reforms in health policy. These include the Medicare prescription drug benefit, Medicare and Medicaid payment reforms, the FDA's Critical Path Initiative, and public-private initiatives to develop better information on the quality and cost of care. Dr. McClellan is the founding chair and a current board member of the Reagan-Udall Foundation for the FDA, is a member of the National Academy of Medicine and chairs the Academy's Leadership Council for Value and Science-Driven Health care, co-chairs the guiding committee of the Health Care Payment Learning and Action Network, and is a research associate at the National Bureau of Economic Research. He has also previously served as a member of the President's Council of Economic Advisers and senior director for health care policy at the White House, and as Deputy Assistant Secretary for Economic Policy at the Department of the Treasury. He was previously an associate professor of economics and medicine with tenure at Stanford University, and has twice received the Kenneth Arrow Award for Outstanding Research in Health Economics.

Gigi McMillan is the Graduate Program Coordinator for the Bioethics Institute at Loyola Marymount University. She received her Master's in Bioethics at LMU and is currently a doctoral student at Loyola Chicago. Mrs. McMillan has extensive experience as a Subject/Patient Advocate on local and national IRBs, was a member of the Subpart A Subcommittee for SACHRP and is a patient representative for the FDA. She served on the board of the American Society for Bioethics and Humanities, has been a faculty member at PRIM&R (Public Responsibility in Medicine and Research) since 2004, and is the Director of Community Engagement for Narrative Inquiry in Bioethics.

Richard A. Moscicki, MD, is the Executive Vice President for Science and Regulatory Advocacy and the Chief Medical Officer at Pharmaceutical Research and Manufacturers of America (PhRMA). Dr. Moscicki came to PhRMA in 2017 after serving as the Deputy Center Director for Science Operations for the U.S. Food and Drug Administration's (FDA) Center for Drug Evaluation and Research (CDER) since 2013. While at FDA, Dr. Moscicki brought executive direction of Center operations and leadership in overseeing the development, implementation, and direction of CDER's programs. Previous positions include serving as Chief Medical Officer at Genzyme Corporation from 1992 to 2011 where he was responsible for worldwide

global regulatory and pharmacovigilance matters, as well as all aspects of clinical research and medical affairs for the company. He served and a senior vice president and Head of Clinical Development at Sanofi-Genzyme from 2011-2013. Dr. Moscicki received his medical degree from Northwestern University Medical School. He is board certified in internal medicine, diagnostic and laboratory immunology, and allergy and immunology. He completed his residency in Internal Medicine, followed by a fellowship at Massachusetts General Hospital (MGH) in clinical immunology and immunopathology. He remained on staff at MGH and on the faculty of Harvard Medical School from 1979 until 2013.

Karen Price received her PhD in Statistics from Baylor University in 2001, and joined Eli Lilly and Company at that time. She is currently Senior Research Advisor at Eli Lilly and Company where she leads the Statistical Innovation Center, a team that focuses on innovative design and analysis of clinical trials. In 2012, Karen formed the DIA Bayesian Scientific Working Group and currently serves as past-chair. This group includes members in Industry, Regulatory, and Academia. The group's mission is to ensure that Bayesian methods are well-understood, accepted, and broadly utilized throughout medical product development. Her research interests include Bayesian meta-analysis, Bayesian methods for safety signal detection and evaluation, and Bayesian design and analysis of clinical trials. In 2016, Karen was elected a Fellow of the American Statistical Association.

Michael Proschan has been a Mathematical Statistician in the Biostatistics Research Branch at the National Institute of Allergy and Infectious Diseases since January of 2006. Prior to coming to NIAID, he spent about 16 years at the National Heart, Lung, and Blood Institute. He co-authored the books: Statistical Monitoring of Clinical Trials: A Unified Approach, with Gordon Lan and Janet Wittes (Springer, 2006) and Essentials of Probability Theory for Statisticians, (CRC Press, 2016) with Pamela Shaw. Dr. Proschan received his Ph.D. in Statistics from Florida State University in 1989, M.S. in Statistics from Stanford University in 1982, and B.S. in Statistics and Mathematics (double major) from Florida State University in 1980. He became a Fellow of the American Statistical Association in 2005.
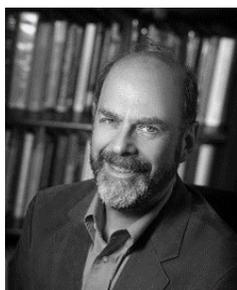
Gary Rosner is the Eli Kennerly Marshall Jr. Professor of Oncology at the Johns Hopkins University School of Medicine and Professor of Biostatistics, Bloomberg School of Public Health, Johns Hopkins University. He directs the Division of Biostatistics and Bioinformatics in the Department of Oncology and heads the Research Program in Quantitative Sciences at the Sidney Kimmel Comprehensive Cancer Center at Johns Hopkins. Dr. Rosner received his Sc.D. in Biostatistics from the Harvard School of Public Health in 1985. He was a member of the faculties of Yale University, Duke University, and The University of Texas M. D. Anderson Cancer Center. His collaborations include the design and analysis of clinical studies in cancer and population modeling in pharmacokinetics and pharmacodynamics. Dr. Rosner carries out research on Bayesian statistical methods to improve the design and analysis of complex cancer research studies. He is a Fellow of the American Statistical Association and an Associate Editor for the journals Biometrics and Clinical Trials.

Jerald Schindler has retired from Merck Research Laboratories. During his career at Merck he served in various leadership roles including Vice President, Global Late Development Statistics. This global group of 140 statisticians was responsible for the design and analysis of clinical trials for all therapeutics areas under development at Merck. Dr. Schindler has also led the adoption of adaptive trial design at Merck which has led to dramatic efficiencies in terms of cost and time. Dr. Schindler is also Adjunct Professor of Biostatistics at the Harvard T.H. Chan School of Public Health and is on the faculty of the Post Graduate Course in Pharmacology at the Tufts University Center for the Study of Drug Development. Prior to joining Merck, Dr. Schindler was President of the Pharmaceutical Research Division at Cytel. Previously, he was the Chief Biostatistician and Global Head of Biostatistics and Clinical Technology at Wyeth Research.

John Scott is Acting Director of the Division of Biostatistics in the FDA's Center for Biologics Evaluation and Research, where he has also served as Deputy Director and as a statistical reviewer for blood products and for cellular, tissue and gene therapies. Prior to joining the FDA in 2008, he worked in psychiatric clinical trials at the University of Pittsburgh Medical Center and did neuroimaging research with the Neurostatistics Laboratory at McClean Hospital, Harvard Medical School. His research interests include Bayesian and adaptive clinical trial design and analysis, drug and vaccine safety, data and text mining, and benefit-risk assessment. He holds a Ph.D. in Biostatistics from the University of Pittsburgh and an M.A. in Mathematics from Washington University in St. Louis, and is an editor of the journal, Pharmaceutical Statistics.

Patroula Smpokou, MD, is a clinical team leader (lead medical officer) in the Division of Gastroenterology and Inborn Errors Products (DGIEP) at FDA's Center for Drug Evaluation and Research. She leads the clinical review of products intented for the treatment of rare genetic diseases called inborn errors of metabolism. She completed her general pediatrics training at Yale Univeristy and her fellowship in clinical genetics at Harvard Medical School. She previously practiced clinical pediatric genetics at Children's National Health System in Washington, DC and held an academic appointment as assistant professor of Pediatrics at The George Washington University School of Medicine and Health Sciences. Her professional interest and expertise include regulatory, clinical, and statistical considerations in drug development in rare diseases such as lysosomal storage diseases and other inborn errors of metabolism**.**

Rajeshwari Sridhara is the Division Director of Division of Biometrics V, Office of Biostatistics which supports Office of Hematology Oncology Products at the Center for Drug Evaluation and Research (CDER). She joined the Food and Drug Administration (FDA) in 1999. Dr. Sridhara has contributed in the understanding and addressing the statistical issues that are unique to the oncology disease area such as evaluation and analysis of time to disease progression. Her research interests also include evaluation of surrogate markers and design of clinical trials. She has organized, chaired and given invited presentations at several workshops. She has worked on many regulatory guidance documents across multiple disciplines. She has extensively published in refereed journals and presented at national and international conferences. She is an elected fellow of the American Statistical Association. Prior to joining FDA, Dr. Sridhara was a project statistician for the AIDS vaccine evaluation group at EMMES Corporation, and she was an assistant professor at the University of Maryland Cancer Center.

Roy Tamura, PhD, is an associate professor of biostatistics in the Health Informatics Institute at the University of South Florida. His research interests are in clinical trial design and in the statistical properties of multi-stage, re-randomization clinical trials. These designs hold promise for improved efficiency and are important in rare diseases and diseases with high placebo response. Dr. Tamura was previously a Research Fellow at Eli Lilly and Company and is a Fellow and a Certified Professional Statistician in the American Statistical Association.

P. K. Tandon, PhD, joined Ultragenyx Pharmaceutical in January 2017 as Senior Vice President of Biometrics and Development Strategy. Most recently, he was appointed as the Site Head of the newly established gene therapy unit of Ultragenyx based in Cambridge, MA. Prior to joining Ultragenyx, he was Clinical Science Officer and SVP at Sanofi-Genzyme and Genzyme Corporation. During his 20 year career at Genzyme, he was actively involved in developing rare disease, neurology and oncology products. His area of expertise include clinical development strategies, interim monitoring of the data, adaptive designs, and health outcomes clinical trials. He received his Ph.D. from The Ohio State University and holds an Executive Program diploma from the Sloan School of Management, MIT. Dr. Tandon also serves as an Associate Professor, Department of Biostatistics and Epidemiology, School of Public Health, Boston University.

Ellen Werner is a Program Officer in the Blood Epidemiology and Clinical Therapeutics Branch at the National Heart, Lung, and Blood Institute, National Institutes of Health. Dr. Werner joined the NIH in August, 2000 as a Program Director in training and career development programs. Since then, Dr. Werner has established programs in clinical and epidemiologic research, and development of resources for patient-reported outcomes, common data elements and standard measures in non-malignant hematology. She is the Senior Science Officer on the Adult Sickle Cell Quality of Life Measurement Information System (ASCQ-Me), the Patient-Reported Outcomes Measurement Information System (PROMIS), the Validation of Pediatric Patient Reported Outcomes in Chronic Diseases (PEPR) Consortium, the Sickle Cell Disease and Hemophilia projects in the National Human Genome Research Institute's consensus measures for Phenotypes and eXposures (PhenX) Toolkit. Dr. Werner holds the IND with the FDA for the hydroxyurea BABY HUG studies in pediatric sickle cell anemia. Dr. Werner serves on the HHS Sickle Cell Disease Data Subgroup, the NIH Common Data Elements Task Force, the NHLBI Data Science Working Group Core Committee, the PhenX Steering Committee as the NHLBI Liaison, the NIH Data Dictionary Infrastructure Working Group, the TOPMed Phenotype Harmonization Work Group, and the American Society of Hematology's Sickle Cell Disease Coalition. Prior to coming to NIH, Dr. Werner was a Senior Health Scientist at the Institute for Survey Research at Temple University, and at several survey research organizations. She has a Master's Degree in Educational Administration and a Ph.D. in Public Health Education with a concentration in Epidemiology.

Issam Zineh is Director of the Office of Clinical Pharmacology (OCP) at the U.S Food and Drug Administration (FDA). From 2008-2012, Dr. Zineh was the Associate Director for Genomics in OCP. He also served as Co-Director of the CDER Biomarker Qualification Program until 2015. He is an experienced applied clinical pharmacologist who was formerly on the faculty of the University of Florida (UF) Colleges of Pharmacy and Medicine and Associate Director of the UF Center for Pharmacogenomics. Dr. Zineh received his PharmD from Northeastern University and completed his residency at Duke University Medical Center. He completed a fellowship in cardiovascular pharmacogenomics at UF where he also obtained his MPH in Health Policy and Management. He is a recognized expert in the fields of drug development and evaluation, clinical pharmacology, pharmacotherapy, and precision medicine. As Director of OCP, Dr. Zineh leads a staff of 240 regulatory, research, project management, and administrative staff in FDA's efforts to enhance drug development and promote regulatory innovation through clinical pharmacology and experimental medicine.