Rare Disease Endpoint Advancement Pilot Program Workshop:
Novel Endpoints for Rare Disease Drug Development

Virtual Public Event
June 7-8, 2023

Speaker Biographies

Nancy M. Allen LaPointe is an Adjunct Associate Professor in Medicine at Duke University and Faculty Fellow at the Duke-Margolis Center for Health Policy. She is a researcher and cardiovascular clinical pharmacist with extensive experience in health outcomes research, health services research, evidence synthesis, medication management, and the protection of human research subjects. Her clinical and research work has been focused on patient safety, predominately in patients with cardiovascular disease. This includes work in reducing medication errors, improving medication adherence, safely and effectively translating evidence into clinical practice, comparing safety and effectiveness of therapeutics, evaluating risk communication and mitigation strategies, and exploring the interface between health policy and patient safety. Prior to working with Duke-Margolis, she was an Associate Professor in Medicine at Duke University and the Duke Clinical Research Institute, Director of the Duke Heart Center Distinguished Research Center Program, Chair in the Duke University Health System IRB, Program Director of the Duke Center for Education and Research on Therapeutics, Principal in Applied Research and Analytics at Premier Inc, and Cardiovascular Clinical Pharmacist with the Duke Heart Center. She was also a Clinical Associate Professor at UNC School of Pharmacy and Adjunct Professor of Pharmacy Practice at Campbell University School of Pharmacy and Health Sciences. Dr. Allen LaPointe received her BS in Pharmacy and Doctor of Pharmacy degrees at Purdue University and completed her pharmacy residency at the Duke University Medical Center, Department of Pharmacy, and her clinical pharmacy fellowship in cardiology at the Duke University Medical Center, Division of Cardiology. She then received a MHS with focus on comparative effectiveness research at Duke University.

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

Damien Eggenspieler is a driven professional at the forefront of digital health technology. Trained as an MIT engineer, Damien served several roles in the healthcare industry, before honing an expertise in digital health tools for the last five years as the head of healthcare activities in Sysnav. Endpoints, or the measure of drug efficacy, is the Achilles heel of drug development and limits development and approval of more effective drugs for patients suffering from a wide variety of neurological diseases. With his team, Damien puts technology at the service of clinicians, regulators & all stakeholders of the drug development process. By combining the power of digital technology and enabling collection of meaningful real-world data, they are committed to pushing the boundaries of healthcare innovation to transform drug development and improve patient care. They developed the SV95C, the first digital endpoint qualified by the European Medicines Agency (EMA), by collaborating with world renowned clinicians like Pr. L. Servais from Oxford University, as well as patients' associations and industry partners. Now is the time to address patient needs beyond children affected by Duchenne Muscular Dystrophy.

Hussein Ezzeldin joined the FDA, Office of Biostatistics and Pharmacovigilance (OBPV) in the Center for Biologics Evaluation and Research (CBER) in 2013. Dr. Ezzeldin works on advancing the science of patient input as part of the FDA regulatory-science strategic goals, and he is leading the natural history study for metachromatic leukodystrophy, HOME. Currently, Dr. Ezzeldin co-leads the Biologics Effectiveness and Safety Innovative Methods Initiative (BEST IM), which aims to develop new and innovative methods for a semi-automated adverse events (AEs) reporting system for CBER-Regulated Biological Products. Dr. Ezzeldin is the acting CBER Lead for the digital health technology review team (DHT-RT), supporting the use of DHTs in regulatory submissions.

Kathleen Fritsch is a Master Mathematical Statistician in the Center for Drug Evaluation and Research (CDER) at FDA, reviewing a wide variety of drug product applications in the Dermatology and Dentistry and COVID-19 therapeutic areas. She was member of the working group revising the Multiple Endpoints in Clinical Trials final guidance. Her additional interests related to clinical trials include study design, drug product labeling, multiplicity, and subgroup analysis.

Lili Garrard is a Master Scientist and the technical lead of the Patient-Focused Statistical Scientists (PFSS) group within the Division of Biometrics III, Office of Biostatistics (OB), Office of Translational Sciences (OTS), Center for Drug Evaluation and Research (CDER) at the U.S. Food and Drug Administration (FDA). Dr. Garrard's team provides statistical and psychometrics reviews on the development and use of clinical outcome assessments (COAs) across multiple therapeutic areas. Prior to joining FDA, Dr. Garrard was the RN (registered nurse) survey director at the National Database of Nursing Quality Indicators (NDNQI). Dr. Garrard received a PhD in Biostatistics from the University of Kansas Medical Center, and both an MS degree and a BS degree in Mathematics from the Michigan Technological University.

Sepideh Haghpanah is a team lead at the Rare Diseases Team in Center for Drug Evaluation and Research (CDER) and the clinical lead for the PDUFA VII Rare Disease Endpoint Advancement (RDEA) pilot program. She joined the FDA in 2017 as a medical officer and has previously worked in the Office of New Drugs (OND) and the Office of the Commissioner /Office of Orphan Products Development (OC/OOPD). Dr. Haghpanah received her medical degree from Shiraz University of Medical Sciences in Iran and completed her residency training in physical medicine and rehabilitation (PM&R) at MetroHealth System/ Case Western Reserve University in Cleveland OH. Prior to joining FDA, she was in clinical practice at Cleveland Clinic in Cleveland, OH and MedStar National Rehabilitation Network in Washington, DC.

Laura Lee Johnson is a division director in the Office of Biostatistics at the U.S. Food and Drug Administration (FDA) Center for Drug Evaluation and Research (CDER). Her division supports a wide variety of therapeutic areas many of which rely on less traditional methods to assess evidence. She also works across FDA on patient focused drug development (PFDD), rare disease initiatives, COVID-19, and master protocol programs. Prior to working at the FDA, Dr. Johnson spent over a decade at the U.S. National Institutes of Health working on and overseeing clinical research and research support programs including the CTSAs, PROMIS, and NIH Collaboratory. She worked at both NCI and what is now known as NCCIH. Dr. Johnson co-directs the NIH Principles and Practice of Clinical Research course.

Naomi Knoble is a pediatric neuropsychologist and Associate Director of Rare Disease Measurement Science in the Division of Clinical Outcome Assessment (DCOA), Office of Drug Evaluation Science (ODES), Office of New Drugs (OND), Center for Drug Evaluation Research (CDER), with the US Food and Drug Administration (FDA). Dr. Knoble has a PhD in Counseling Psychology from the University of Oregon and completed clinical training in pediatric neuropsychology at the Oregon Health & Science University and University of Minnesota Medical School. Prior to joining FDA in 2020, Dr. Knoble was a research scientist focused on patient-centered outcomes in global clinical trials and post-market evidence generation. Dr. Knoble is the FDA liaison for the Critical Path Institute's Rare Disease Clinical Outcome Assessment Consortium.

Stefanie Kraus is a Senior Regulatory Counsel in the Office of Regulatory Policy (ORP) within CDER. After practicing pharmaceutical antitrust litigation for ten years at Proskauer Rose, LLP, a large international law firm, Stefanie earned her MPH at the Harvard School of Public Health. Stefanie's work focuses on policy and legal and regulatory issues relating to drug development, clinical trials, clinical research (including diversity and access), digital health technologies, decentralized clinical trials, human subject protections, real-world evidence, emergency use authorization and emergency preparedness, and evidentiary standards for marketing approval. Stefanie serves on the established steering committees relating to the topics above and serves as the lead for disclosure for PDUFA pilot programs relating to evidence generation.

Kerry Jo Lee is the Associate Director for Rare Diseases in the Division of Rare Diseases and Medical Genetics, Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER). In this role she leads the Rare Diseases Team, a multidisciplinary rare disease programming and policy team that works to promote their mission to facilitate, support, and accelerate the development of drugs and therapeutic biologics for rare diseases and serves as the program management office for CDER's Accelerating Rare diseases Cures (ARC) Program. Dr. Lee joined the FDA as a medical officer in 2014 with the former Division of Gastroenterology and Inborn Errors Products, OND, CDER. Dr. Lee then moved to a position as a clinical advisor for the Office of New Drug Policy, CDER, where she served as a lead in the areas of benefit-risk assessment, modernization efforts (including the integrated review for marketing applications), and real- world data/evidence programming before serving in her current position. Dr. Lee is a pediatric gastroenterologist/hepatologist and a graduate of Princeton University and the New York University School of Medicine with an honors degree conferred in microbiology. She completed her residency in pediatrics at the Children's Hospital of Los Angeles followed by a post-doctoral clinical fellowship in Pediatric Gastroenterology, Hepatology, and Nutrition at Columbia University College of Physicians and Surgeons in New York. Dr. Lee maintains a steadfast interest in international policy and bioethics and worked for several years at the former National Bioethics Advisory Commission on reports advising the executive branch on ethical and policy issues in both international and domestic clinical trials.

Yuqun Abigail Luo is a mathematical statistician in the Division of Biostatistics within Office of Biostatistics and Pharmacovigilance, at the Center for Biologics Evaluation and Research (CBER), Food and Drug Administration (FDA). She is responsible for reviewing protocols and marketing applications of cell and gene therapy products for a wide range of indications, including benign hematology, GI, pulmonary, cardiovascular, and others, as well as CBER products for allergy/immunology, infectious disease, inborn error of metabolism. She has also been involved in FDA guidance development, including Human Gene Therapy for Hemophilia (2020), Interacting with the FDA on Complex Innovative Trial Designs for Drugs and Biological Products (2020), Patient-Focused Drug Development: Incorporating Clinical Outcome Assessments Into Endpoints for Regulatory Decision-Making (Draft) (2023). She taught educational short courses and seminars for non-statistician colleagues and been involved in external outreach through participation in topic-focused working groups. Her educational background includes a B.S. in Information Science (Minor: Chemistry) from the University of Science and Technology of China, a Ph.D. in Biostatistics from The Ohio State University. Prior to joining FDA in 2009, she was a tenure-track faculty in the Department of Epidemiology and Biostatistics at Case Western Reserve University.

Rajanikanth Madabushi is the Associate Director for Guidance and Scientific Policy in the Immediate Office of the OCP. He is involved in the drug development, regulation, research and policy from a clinical pharmacology perspective. He is the CDER Point-of-Contact for the MIDD Meeting Program and is actively involved in various MIDD related activities under PDUFA VI and VII.

Ami Mankodi serves as a primary member of the review team for rare diseases, neurogenetic diseases, and neuromuscular diseases in the Division of Neurology 1, Office of Neuroscience, Office of New Drugs, Center for Drug Evaluation and Research, FDA. She received an MD from Mumbai, India. She completed neurology residency at the Johns Hopkins Hospital, Baltimore, MD. She did a neuromuscular fellowship at University of Rochester, NY and a senior clinical fellowship in neurogenetics at the National Institute of Neurological Disorders and Stroke (NINDS), Bethesda, MD. She was appointed as an Assistant Clinical Investigator and an Investigator in the NINDS. She maintains her special interests in rare neurogenetic and neuromuscular diseases, and has published and presented on these topics.

Mark McClellan is the Robert J. Margolis, M.D., Professor of Business, Medicine and Policy and Director of the Duke-Margolis Center for Health Policy. A physician-economist focused on advancing quality and value in health care, his COVID-19 response work spans virus containment and testing strategies, resilient care delivery, and accelerating therapeutics and vaccine development. Dr. McClellan is a former leader of the Centers for Medicare & Medicaid Services and the U.S. Food and Drug Administration. An independent director on the boards of Johnson & Johnson, Cigna, Alignment Healthcare, and PrognomIQ, Dr. McClellan co-chairs the Guiding Committee for the Health Care Payment Learning and Action Network and serves as an advisor for Arsenal Capital Partners, Blackstone Life Sciences, and MITRE.

Lindsey Murray is Executive Director of the Rare Disease Clinical Outcome Assessment (COA) Consortium. Dr. Murray has more than 15 years of experience in health outcomes research. She specializes in quantifying the patient's perspective of health, illness, and treatment through COA development, psychometric testing, and the design and analysis of clinical trials involving COAs. Prior to joining C-Path, Dr. Murray was an integral part of the EXacerbations of Chronic Pulmonary Disease Tool (EXACT®) – Patient-Reported Outcome Initiative team, taking over as Director of the EXACT PROgram. As Director, she had oversight of all EXACT licensing, translations, and analysis work being conducted on the EXACT and its derivative measure, the Evaluating Respiratory Symptoms in Chronic Obstructive Pulmonary Disease (E-RS®: COPD). Dr. Murray's research activities also included serving as principal investigator to develop patient-reported outcome measures in several rare diseases, including neurotrophic keratosis and hypertrophic cardiomyopathy. She was also involved in adapting the E-RS: COPD for use in idiopathic pulmonary fibrosis (E-RS: IPF) and asthma-COPD overlap syndrome. Dr. Murray holds a PhD from George Washington University in epidemiology. She received her MPH from George Washington University in epidemiology with a certificate in health promotion. Previously, Dr. Murray received a BA from the University of Virginia in Charlottesville, Virginia, where she double majored in anthropology and biology with a minor in German literature and language studies.

Patrick Nachman is director of the Division of Nephrology and Hypertension. He completed his fellowship in nephrology at the University of North Carolina, where was deputy director of the UNC Kidney Center. His research focused on Antineutrophil Cytoplasmic Autoantibody (ANCA) vasculitis. His translational clinical research has been in glomerular diseases and systemic vasculitis. He is particularly interested in fostering clinical research and drug development for kidney diseases in general and glomerular diseases in particular.

Michael Pacanowski is the Director of the Division of Translational and Precision Medicine in FDA's Office of Clinical Pharmacology. He oversees a multidisciplinary team of clinical scientists who lead the Office's regulatory review, research, and policy activities related to pharmacogenomics, biomarkers, targeted therapies, and drug development for rare diseases. Dr. Pacanowski received his Pharm.D. from the Philadelphia College of Pharmacy and his M.P.H. from the University of Florida. He completed a residency in clinical pharmacology at Bassett Healthcare in Cooperstown, NY, and a clinical research fellowship in cardiovascular pharmacogenomics at the University of Florida.

Dionne Price is the Deputy Director of the Office of Biostatistics in the Office of Translational Sciences, Center for Drug Evaluation and Research, FDA. In this role, Dr. Price provides leadership to statisticians involved in the development and application of methodology used in the regulation of drug products. She currently leads cross-cutting, collaborative efforts across FDA to advance and facilitate the use of innovative trial designs in pharmaceutical drug development. Dr. Price received her MS in Biostatistics from the University of North Carolina at Chapel Hill and a PhD in Biostatistics from Emory University. Dr. Price is an active member of the American Statistical Association (ASA) and the Eastern North American Region of the International Biometrics Society. She is a Fellow of the ASA and the 2023 President of the ASA.

David L. Rousso David L. Rousso is the US Medical Affairs Therapeutic Area Lead for Ophthalmology at Spark® Therapeutics. He holds a Ph.D. in Neurobiology from the University of California, Los Angeles (UCLA) and received post-doctoral training in visual neuroscience at Harvard University, where he received a Ruth L. Kirschtein National Research Service Award (NRSA) for his work in retinal neuron development and function. David joined Spark in 2016 as part of the US launch team for LUXTURNA®(voretigene neparvovec-rzyl), the first FDA-approved gene therapy for a genetic disease, and leads numerous activities related to Spark's ocular gene therapy programs.

Leonard Sacks received his medical education in South Africa, moving to the USA in 1987, where he completed fellowships in immunopathology and Infectious Diseases. He worked as an attending physician in Washington DC and South Africa and he joined the FDA in 1998 as medical reviewer in the Office of New Drugs. Subsequent positions included acting director of the Office of Critical Path Programs and associate director for clinical methodology in the Office of Medical Policy in the Center for Drug Evaluation and Research. In this capacity he has led efforts to support novel approaches to clinical trials including the use of electronic technology. Besides his involvement in the design and analysis of clinical trials, he maintains a special interest in tuberculosis and other tropical diseases and has published and presented on these topics. He holds academic appointments as Associate Clinical Professor of Medicine at George Washington University, and at the Uniformed Services University of the Health Sciences.

Mary Jo Salerno works as a science policy analyst on CDER Office of New Drugs' Rare Diseases Team and is the administrative lead for the PDUFA VII Rare Disease Endpoint Advancement Pilot Program. Mary Jo also has broad FDA experience as a policy analyst and regulatory project manager in the Center for Tobacco Products, Office of the Commissioner Office of the Executive Secretariat, Europe Office (in the former Office of International Programs), and CDER Office of Biostatistics. Prior to beginning her FDA career in 2013, Mary Jo worked in the pharmaceutical (vaccine) industry, as a clinician (physical therapist), and as an officer in the United States Navy. Mary Jo received her Bachelor of Science from the U.S. Naval Academy, her Master of Science in Physical Therapy from Washington University School of Medicine (St Louis, MO), and her Master of Public Health from Johns Hopkins Bloomberg School of Public Health.

Laurent Servais is Professor of Paediatric Neuromuscular Diseases at the MDUK Oxford Neuromuscular Centre and Invited Professor of Child Neurology at Liège University. After graduating from Louvain Medical School, Brussels, Belgium in 1999, Dr. Servais completed a PhD in neuroscience (cerebellar electrophysiology in mice) at the Free University of Brussels, Belgium, followed by residencies in child neurology at the Free University of Brussels and Robert Debré Hospital, Paris. In 2008, Dr. Servais took a position in neuromuscular disease and clinical research at the Institute of Myology in Paris, where his interest and expertise in neuromuscular diseases flourished. He was subsequently appointed Head of Clinical Trials and Database Services. Most recently, he served as Head of the Institute of Myology's I-Motion (Institute of Muscle-Oriented Translational Innovation), and Head of the Neuromuscular Centre in Liège, Belgium. Dr. Servais joined the MDUK Oxford Neuromuscular Centre and the University of Oxford in September 2019. Presently, Dr. Servais is the leader of the national newborn screening program for SMA in Southern Belgium and of a pilot in the Thames Valley in the UK, where they are conducting a medico-economic analysis of newborn screening for this disease.

Peter Stein is the Director of CDER's Office of New Drugs (OND). OND is responsible for the regulatory oversight of investigational studies during drug development and decisions regarding marketing approval for new (innovator or non-generic) drugs, including decisions related to changes to already marketed products. OND provides guidance to regulated industry on a wide variety of clinical, scientific, and regulatory matters. A nationally-recognized leader in pharmaceutical research and development, Dr. Stein joined CDER in 2016 as the OND Deputy Director. Before coming to FDA, he served as Vice President for late stage development, diabetes, and endocrinology at Merck Research Laboratories. He also served as Vice President, head of metabolism development at Janssen. He has more than 30 years of academic, clinical, and industry experience. Dr. Stein holds a bachelor's degree in history from the University of Rochester in New York and a medical degree from University of Pennsylvania. He trained at Yale University and Yale-New Haven Hospital in internal medicine and in endocrinology and metabolism.

Lynley Thinnes has more than 30 years of experience in drug regulatory affairs in the pharmaceutical industry, and is currently at Travere Therapeutics, biopharmaceutical company exclusively focused on identifying, developing and delivering life-changing therapies to people living with rare disease. Prior to joining Travere, she held senior regulatory affairs positions with a number of companies as a principal in constructing global regulatory strategies and submissions for the development of products across a range of therapeutic areas, with a current focus on rare diseases using surrogate biomarkers. She has proven success in strategic development leading to NDA/MAA approvals, some including Advisory Committee meetings, for FILSPARI®, ZYNRELEF™, TROXYCA ER®, and MOVANTIK™.

Aliza Thompson is Deputy Director of the Division of Cardiology and Nephrology, Center for Drug Evaluation and Research at the U.S. Food and Drug Administration (FDA). The Division of Cardiology and Nephrology regulates and reviews Investigational New Drug applications and marketing applications for drug and biologic products for the treatment of cardiovascular and kidney diseases. Dr. Thompson joined the FDA in 2007. Prior to her current position, Dr. Thompson served as a clinical team leader for products being developed to treat kidney diseases. Dr. Thompson received her medical degree from Johns Hopkins Medical School and completed her Internal Medicine and Nephrology training at Columbia University/New York-Presbyterian Hospital. She holds a Master of Science in Biostatistics/Patient Oriented Research Track from Columbia University Mailman School of Public Health.

Julienne Vaillancourt is a captain in the United States Public Health Service Commissioned Corps (USPHS CC) and since 2015 has served as a policy advisor and rare disease liaison in the Office of the Director in the Center for Biologics Evaluation and Research (CBER) at the FDA. She coordinates CBER's Rare Disease Program, which involves working on relevant policy development and statutory initiatives, works closely with staff from across FDA on cross-cutting rare disease activities, and frequently engages with external stakeholders on collaborative projects and with outreach. She has extensive prior experience as a Regulatory Project Manager (RPM) and RPM Team Leader in CBER's Office of Vaccines Research and Review, where she served for over 17 years.

Susan (Sue) Warner has held a number of positions over her 20+ yr. career at Eli Lilly and Company, with a focus in Regulatory and Clinical Development. Currently, Sue is an Executive Director in Global Regulatory Affairs, in Lilly's Neuroscience Business Unit. Sue has led US Regulatory strategies for a number of drugs in development. Prior to Regulatory, she held multiple positions in Clinical Development planning and execution, across different phases and therapeutic areas. She has also led process improvement initiatives. Sue achieved her Pharm.D. from the University of Michigan prior to joining Lilly.

Kevin P. Weinfurt is Professor and Vice Chair of Research in the Department of Population Health Sciences at Duke University Medical Center and a faculty member of the Duke Clinical Research Institute. He holds secondary appointment as a Professor of Psychology and Neuroscience, Professor of Psychiatry and Behavioral Sciences, Professor of Biostatistics and Bioinformatics, and a Faculty Associate of the Trent Center for the Study of Medical Humanities and Bioethics. Dr. Weinfurt also co-directs the Center for Health Measurement at Duke and is co-director of the Clinical Research Training Program (Masters degree offered through the School of Medicine). Dr. Weinfurt currently works as Special Governmental Employee for the U.S. Food and Drug Administration, helping to create the Patient-Focused Drug Development guidance series. He is also a member of the Secretary's Advisory Committee for Human Research Protections.

Celia M. Witten is the Deputy Director of the Center for Biologics Evaluation and Research at the Food and Drug Administration (FDA/CBER). Currently she is also the Acting Super Office Director of the Office of Therapeutic Products at CBER. Between 2005 and 2016 she served as the Director of the Office of Cellular, Tissue and Gene Therapy at the FDA/CBER. Between 1996 and 2005 she served as Director of the Division of General, Restorative, and Neurological Devices in the Office of Device Evaluation in the Center for Devices and Radiological Health (CDRH). Previous to FDA, she worked for over 10 years as a practicing physician at the National Rehabilitation Hospital (NRH) in Washington, D.C. Her educational background includes a B.A. earned at Princeton University (Magna Cum Laude), a Ph.D. from Stanford University, and an M.D. from the University of Miami School of Medicine. In addition to her academic achievements she is Board Certified in Physical Medicine and Rehabilitation.

Lei Xu serves as the Chief of General Medicine Brach 2 in the FDA's Division of Clinical Evaluation General Medicine (DCEGM), Office of Clinical Evaluation (OCE), Office of Therapeutic Products (OTP) 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 biologics (e.g., gene therapy, cellular therapy and plasma-derived products) and devices in several clinical areas, including Neurology, Ophthalmology, Dermatology, and burn and wound care. Her Branch reviewed all the clinical data that led to FDA-approval several gene and cellular therapies, including the first two adeno associated virus (AAV) vector-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,

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and the first topical gene therapy: beremagene geperpavec (VYJUVEK) for dystrophic epidermolysis bullosa. 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: Human Gene Therapy for Retinal Disorders, and Guidance for Industry: Human 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.