REAGAN-UDALL FOUNDATION FOR THE FDA

Natural History Studies and Registries in the Development of Rare Disease Treatments

Hybrid Public Meeting
May 13, 2024; 10AM - 4PM (eastern time)

Presenter & Reactor Panelist

Philip J. Brooks, PhD
National Center for Advancing Translational Sciences, NIH

Dr. Philip J. (P.J.) Brooks is the Deputy Director of NCATS' Division of Rare Diseases Research Innovation. He also is the working group co-coordinator for the NIH Common Fund program on Somatic Cell Genome Editing, one of the leaders of the Platform Vector Gene Therapy (PaVe-GT) pilot project and the co-chair of the Bespoke Gene Therapy Consortium. He also represents NCATS in the International Rare Diseases Research Consortium (IRDiRC).

In May 2022, Dr. Brooks was selected as the recipient of the 2022 Sonia Skarlatos Public Service Award by the American Society of Gene & Cell Therapy for consistently fostering and enhancing the field of gene and cell therapy.

Dr. Brooks received his doctorate in neurobiology from The University of North Carolina at Chapel Hill. After completing a postdoctoral fellowship at The Rockefeller University, he became an investigator in the NIH intramural program, where he developed an internationally recognized research program, including research on rare neurologic diseases resulting from defective DNA repair.

Patrizia Cavazzoni, MD
Center for Drug Evaluation and Research, FDA

Dr. Patrizia Cavazzoni is the director of the Center for Drug Evaluation and Research (CDER) at the U.S. Food and Drug Administration. The Center's mission is to ensure that safe, effective and high-quality drugs are available to the public. To achieve this, CDER regulates the medical products under its jurisdiction throughout their lifecycle, oversees the development of new and generic drugs, evaluates applications to determine whether drugs should be approved, monitors the safety of drugs after they are marketed, conducts research to advance regulatory science and takes enforcement actions to protect the public from harmful products. Dr. Cavazzoni joined the FDA in January 2018 as CDER's Deputy Director for Operations where she has led several key initiatives on behalf of the organization. She also served as Acting Principal Deputy Commissioner of Food and Drugs from January 2019 to February 2019.

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. During her training, she was an investigator in clinical trials of novel antipsychotic and antidepressant medications and became a research collaborator within the International Group for The Study of Lithium Treated Patients. She subsequently received a full-time appointment to the Faculty of Medicine at the University of Ottawa and joined the Mood Disorders Program at the Royal Ottawa Hospital, where she treated patients suffering from severe mood disorders, taught students and conducted research on genetic predictors of bipolar disorder as part of a multidisciplinary international collaborative effort, authoring numerous peer-reviewed scientific publications.

Dr. Cavazzoni obtained certification by the American Board of Neurology and Psychiatry in 1997 and 2008 and was a fellow of the Canadian Royal College of Physicians and Surgeons from 1997 until 2023. She is a fellow of the Canadian College of Neuropsychopharmacology and a recipient of the American College of Psychiatrists' Laughlin Fellowship.

Jennifer Farmer, MS
Friedreich's Ataxia Research Alliance

Jennifer Farmer is the Chief Executive Officer of the Friedreich's Ataxia Research Alliance (FARA). Jennifer has a master's degree in Genetic Counseling and before joining FARA in 2006, she worked at the University of Pennsylvania and Children's Hospital of Philadelphia. As the CEO, she helps to carry out the strategic mission of the organization through leading FARA's research and partnership initiatives.

Benjamin Forred, MBA, ACRP-CP
Sanford Research

Benjamin Forred has worked in the field of biomedical research since 2009. His experience ranges from nearly a decade working at the bench in basic science, to five years working in the clinical research space. Currently, he is responsible for translational research projects at Sanford Research. His team maintains a colony of transgenic mice and a vast number of patient and rodent cell lines that model a number of rare disorders. His group then partners with for-profit biopharma companies to screen promising therapeutics. Additionally, Forred and his team assist basic scientists with human subject research projects and coordinate clinical sample collection. He has also been responsible for the growth of the CoRDS Registry since 2016. CoRDS is an international, disease agnostic, rare disease registry offered at no cost to people living with rare conditions or to the researchers investigating rare disease. Through this endeavor, Forred has built strong relationships with a number of advocacy organizations, and he constantly works to put the wellbeing of rare patients at the forefront of all phases of his career.

Collin Hovinga, PharmD, MS, FCCP
Critical Path Institute

Dr. Collin Hovinga serves as Vice President of the Rare and Orphan Disease Programs at the Critical Path Institute overseeing the Critical Path for Rare Neurodegenerative Diseases public-private partnership and C-Path's Rare Disease Cures Accelerator-Data and Analytics Platform. He completed his bachelor's degree in Biology and doctoral degree in Pharmacy from Creighton University in Omaha, Nebraska. After which he pursued a Residency and Fellowship in Pediatric Pharmacotherapy with emphasis in Pediatric Neuroscience at the University of Tennessee, Memphis, LeBonheur Children's Medical Center. He has a master's degree in Epidemiology from the University of Tennessee Health Science Center. Dr. Hovinga has been active in studying factors that influence the efficacy and safety of medications in children and in rare/orphan diseases. Dr. Hovinga is recognized as an expert in trial design, real world data and clinical pharmacology and has served as an advisor to NIH/NINDS and FDA SGE (CNS/PNS and DSRM).

Leslie Gordon, MD, PhD
The Progeria Research Foundation

Dr. Leslie Gordon is the mother of a child with Progeria, co-founder of The Progeria Research Foundation (PRF), and serves as PRF's volunteer Medical Director. Dr. Gordon is the Principal Investigator for ongoing Progeria programs, including the PRF International Progeria Registry, Medical and Research Database, Cell and Tissue Bank, and the Diagnostics Program. She has organized 11 National Institutes of Health-funded, international scientific meetings on Progeria, and has served as trial investigator in four Progeria clinical drug trials at Boston Children's Hospital.

She is Professor of Pediatrics at Hasbro Children's Hospital and the Alpert Medical School of Brown University in Providence, RI. She is a Research Associate in Anaesthesia at Harvard Medical School and Senior Staff Scientist at Boston Children's Hospital.

Dr. Gordon was co-author on the 2003 gene discovery and 2021 genetic editing publications (Nature), as well as lead author of the 2018 Progeria treatment discovery study (JAMA) and 2023 Progeria biomarker discovery.

Henry Kaminski, MD
George Washington University

Dr. Henry J. Kaminski serves as the Meta A. Neumann Professor of Neurology at George Washington University. Dr. Kaminski has performed clinical, translational, and basic investigations related to the rare, autoimmune disease, myasthenia gravis, for over 25 years with support of the NIH, Department of Veterans Affairs, the Muscular Dystrophy Association and the Myasthenia Gravis Foundation of America. He was instrumental in establishment of the MG Patient Registry, which was utilized to better define treatment and its complications for MG patients. In 2019, Dr. Kaminski established as principal investigator the NIH Rare Disease Clinical Research Network site dedicated to myasthenia gravis, MGNet. Recently, he has started work towards objective quantitation of the neuromuscular examination via telemedicine using artificial intelligence in order to enhance clinical outcome measures and enhance clinical trial performance.

Eileen King, PhD
Cincinnati Children's Hospital Medical Center

Dr. Eileen King is a Research Professor within the Division of Biostatistics and Epidemiology at Cincinnati Children's Hospital Medical Center. Dr. King serves as PI on two NIH-funded administrative and data coordination research networks including the Data Management and Coordinating Center for the Rare Disease Clinical Research Network and the Administrative Coordinating Center and data hub for the Bench to Bassinet Program. She has 14 years of experience leading statistics and data coordination teams in the academic sector in addition to 20 years of industry experience in pharmaceutical and health care research where she was head of the Biometrics and Statistical Sciences Department at The Procter & Gamble Company. Dr. King received her doctoral degree in Statistics from Texas A&M University and her master's degree in Statistics from the University of Wyoming and is a Fellow of the American Statistical Association.

Kerry Jo Lee, MD
Center for Drug Evaluation and Research, FDA

Dr. Kerry Jo Lee is the Associate Director for Rare Diseases in the Division of Rare Diseases and Medical Genetics (DRDMG), Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine (ORPURM), Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER). In this role she leads CDER's Rare Diseases Team, a multidisciplinary rare disease programming and policy team that works across CDER to promote their mission to facilitate, support, and accelerate the development of drugs and therapeutic biologics for rare diseases. Dr. Lee also serves as Program Manager of CDER's Accelerating Rare disease Cures (ARC) Program. Dr. Lee is a pediatric gastroenterologist/hepatologist who joined the FDA as a medical officer in 2014. Through her previous roles in CDER/OND, Dr. Lee has 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 in CDER drug review and policy.

Catherine Lerro, PhD, MPH
Oncology Center for Excellence, FDA

Dr. Catherine Lerro is a Senior Pharmacoepidemiologist in the Oncology Center of Excellence's (OCE) at the US Food and Drug Administration (FDA). Prior to joining the OCE, Dr. Lerro was an epidemiology reviewer and team lead in the Office of Surveillance and Epidemiology in the FDA's Center for Drug Evaluation and Research. She earned her doctoral degree in Cancer Epidemiology from Yale, her bachelor's degree in Public Health studies from Johns Hopkins and her master's degree in Public Health in chronic disease epidemiology from Yale. She completed a post-doctoral fellowship at the National Cancer Institute in the Division of Cancer Epidemiology and Genetics. She has also previously worked as an epidemiologist in the Surveillance and Health Services Research group at the American Cancer Society.

John Lieske, MD
Mayo Clinic Hospital – Rochester

Dr. John Lieske is a nephrologist and Professor of Medicine at Mayo Clinic in Rochester, Minnesota and has a long-standing interest in the pathogenesis and treatment of urinary stone disease. Over the last decade he has completed clinical trials evaluating the effect of diet, probiotics, phosphate binders, vasopressin antagonism, and small inhibitory RNA in stone disease patients with and without primary or enteric hyperoxaluria. Dr. Lieske also studies the genetics of monogenic and common kidney stones as well as the role of intrarenal macrophages/inflammation in urinary stone pathogenesis. Dr. Lieske is medical director of the Renal Testing Laboratory in the Department of Laboratory Medicine that performs all kidney related testing including those related to the diagnosis and treatment of kidney stone patients. He is also director of the Rare Kidney Stone Consortium, site PI for the Urinary Stone Disease Research Network site at Mayo Clinic and has NIDDK grants to study the genetics of urinary stone disease and the role of intrarenal macrophages in renal calcification and stone disease.

Kirtida Mistry, MBBCh, DCH, MRCPCH
Center for Drug Evaluation and Research, FDA

Dr. Kirtida Mistry is a senior physician and clinical reviewer in the Division of Cardiology and Nephrology, Center for Drug Evaluation and Research at the U.S. Food and Drug Administration. Dr. Mistry is a pediatric nephrologist and board certified in Pediatrics and Pediatric Nephrology. Before joining FDA, Dr. Mistry was a Clinical Associate Professor of Pediatrics at the George Washington University, and an attending physician at the Children's National Health System in Washington, DC, where she served as Medical Director of Dialysis. Dr. Mistry completed her training in pediatrics at St. Louis Children's Hospital, Washington University School of Medicine, St. Louis, MO, and in pediatric nephrology at Children's Hospital Boston, Harvard Medical School, Boston, MA.

Jill Morris, PhD
National Institute of Neurological Disorders and Stroke, NIH

Dr. Jill A. Morris is a NINDS Program Director. She is responsible for a disease research portfolio including multiple rare neurological disorders (lysosomal storage disorders, leukodystrophies, inborn errors of metabolism, neurofibromatoses, congenital disorders of glycosylation (CDGs), mitochondrial disorders, peroxisomal disorders, and metal metabolism disorders) as well as hydrocephalus, neural tube defects, and Tourette Syndrome. She is also responsible for grants on technology development for gene-targeted therapies including gene, ASOs and RNAi therapies as well as delivery methods. Dr. Morris is the NINDS Liaison for the Rare Disease Clinical Research Network (RDCRN) an initiative of the Division of Rare Diseases Research Innovation (DRDRI) at NCATS in collaboration with NINDS. NINDS co-funds 11 of the 20 consortia as well as the Data Management Coordinating Center. Her prior experience includes appointments as an Assistant Professor at Northwestern University and a Senior Research Biologist at in the Department of Neuroscience at Merck Research Laboratories.

Katherine Needleman, PhD, RAC
Office of Orphan Products Development, FDA

Dr. Katherine Needleman serves as the Director for the Orphan Products Grants Program in the Office of Orphan Products Development (OOPD) at the Food and Drug Administration (FDA). She manages the $19 million OOPD extramural research budget that is used to support grants in the Orphan Products Clinical Trials Grants Program and the Orphan Products Natural History Grants Program. She also Directs the new FDA Rare Neurodegenerative Disease Grant Program established by the Accelerating Access to Critical Therapies for Amyotrophic Lateral Sclerosis Act (ACT for ALS) which had a budget of $5 million in fiscal year 2023. She works closely with project officers, researchers, and organizations to advance promising medical products for rare diseases or conditions to market approval, to increase publications of significant findings in the scientific literature, and to oversee the responsible use of federal funds.

Dr. Needleman joined FDA's Center for Biologics Evaluation and Research (CBER) in 2002 in the area of therapeutic proteins and moved to the Center for Drug Evaluation and Research (CDER) in the Division of Neurology Products in 2005. In her positions at both CBER and CDER, she served as a regulatory expert throughout the entire review process from pre-IND/discovery through post-marketing approval for numerous products including many orphan products. She has been involved in various initiatives to improve efficiency of the review and management process and serves on multiple international and domestic working groups to encourage research and development of rare disease products. She earned a bachelor's degree from Bowdoin College, a master's degree in Pharmacology and Molecular Sciences from the Johns Hopkins University School of Medicine, and a doctoral degree in Experimental and Clinical Pharmacology from the University of Minnesota. She has also obtained the Regulatory Affairs Certification (RAC) from the Regulatory Affairs Professional Society (RAPS).

Suzanne Pattee
Office of the Commissioner, FDA

Suzanne Pattee is a regulatory counsel with the Office of Clinical Policy in the Office of the Commissioner. She previously worked for the Office for Medical Policy and the Office for Pharmaceutical Quality in the Center for Drug Evaluation and Research (CDER).

Before joining FDA in 2009, Ms. Pattee was a vice president at a rare disease foundation where she led policy initiatives in clinical trials, patient affairs, among others. She also led bioethics issues for a biotechnology trade association. Suzanne was a member of the Secretary's Advisory Committee for Human Research Protections and served on the board of an accreditation association. Suzanne earned her law degree from The George Washington University, and her bachelor's degree from The College of William and Mary.

Dominique Pichard, MD, MS
National Center for Advancing Translational Sciences, NIH

Dr. Dominique C. Pichard is the director of NCATS' Division of Rare Diseases Research Innovation, where she leads rare disease research efforts. In this capacity, she also collaborates with other divisions and offices across NCATS and, more broadly, with other NIH institutes and centers regarding rare diseases research. Before joining NCATS in September 2023, Pichard was the chief science officer at the International Rett Syndrome Foundation — the largest patient advocacy and research foundation for Rett syndrome in the United States. Rett syndrome is a rare genetic neurological disorder that affects 1 in 10,000 females (and even fewer males) and leads to severe impairments affecting nearly every aspect of life, including speaking, walking, eating and the ability to breathe easily. In her capacity at the International Rett Syndrome Foundation, Pichard engaged in patient advocacy and created several initiatives to improve translational research in Rett syndrome, providing the grounds for successful drug development. Prior to her tenure at the International Rett Syndrome Foundation, Pichard was a clinical researcher at the National Cancer Institute and the National Institute of Arthritis and Musculoskeletal and Skin Diseases, where she studied many different rare diseases and gained expertise in rare disease clinical trial development.

In addition to her work in rare disease research, Pichard is mother to a daughter who lives with Rett syndrome, so she has a deep understanding of the many challenges associated with having a rare disease, including the diagnostic odyssey, the lack of treatments and the many untreated medical complications related to the disease. She is passionate about improving the system for all those affected by rare diseases, which aligns with NCATS' vision of bringing more treatments to all people more quickly.

Pichard received her medical degree from Georgetown University School of Medicine and her master's degree from the University of Maryland School of Medicine.

Catherine Pilgrim-Grayson, MD, MPH
Center for Drug Evaluation and Research, FDA

Dr. Catherine Pilgrim-Grayson is the Acting Director of the Division of Rare Diseases and Medical Genetics at the US FDA, providing scientific, clinical, and technical direction on all medical and scientific decisions and judgment in connection with the review and evaluation of drugs in that arena. She advances OND's policies and research agenda for rare diseases, liaising with other FDA offices and other regulatory agencies, industry, professional organizations, academia, and the public. In prior roles at FDA she was a Clinical Reviewer, acting Clinical Team Leader, acting Deputy Director and the Deputy Director for Safety. Dr. Pilgrim-Grayson is a board-certified obstetrician/gynecologist. She graduated from Swarthmore College with Honors, the University of Pennsylvania Perelman School of Medicine and the Johns Hopkins Bloomberg School of Public Health with Honors. She completed her Gynecology and Obstetrics residency at Johns Hopkins and subsequently had a varied medical career, focused on the practice of academic medicine at Johns Hopkins, leading the Johns Hopkins Fibroid Center and teaching, conducting research and international public health outreach. Dr. Pilgrim-Grayson has also previously held a faculty position at Thomas Jefferson University, leading the Obstetrics and Gynecology clinic, and has served as the Chief of the Department of Obstetrics and Gynecology at the University of Maryland St. Joseph Medical Center.

Donna Rivera, PharmD, MSc
Oncology Center of Excellence, FDA

Dr. Donna R. Rivera is the Associate Director for Pharmacoepidemiology in the Oncology Center of Excellence at the U.S. Food and Drug Administration. She leads the Oncology Real World Evidence (RWE) Program, focused on the use of Real-World Data (RWD) and RWE for regulatory purposes, as well as management of the RWD research portfolio strategy and development of regulatory policy to support the OCE mission. As a pharmacist and pharmacoepidemiologist, Dr. Rivera has interests in the use of RWD to advance health equity, observational study designs and methodological approaches, and appropriate uses of RWD for drug development to increase access of effective therapies to patients.

In her previous role at the National Cancer Institute (NCI), she led a strategic RWD initiative to facilitate large scale, longitudinal treatment data linkages with SEER through collaborative public private partnerships.

Kimberly Smith, MD, MS
Center for Drug Evaluation and Research, FDA

Dr. Kimberly Smith is a nephrologist and Senior Medical Advisor with the Real-World Evidence Analytics team in the Office of Medical Policy (OMP) within the Center for Drug Evaluation and Research (CDER) at the U.S. Food and Drug Administration (FDA). In her current role, she develops and implements programs and policies related to the use of real-world evidence in drug development. Dr. Smith previously served at FDA as the team leader for OMP's Division of Clinical Trial Quality and as the nephrology team leader in the Division of Cardiology and Nephrology in CDER's Office of New Drugs. Before joining the FDA, she was with the Coverage and Analysis Group at the Centers for Medicare and Medicaid Services.

Ronen Spiegel, MD
Emek Medical Center

Dr. Ronen Spiegel serves as the Director of Pediatric Department and Head of the Center for Rare Diseases at Emek Medical Center, Afula, Israel. He received his MD degree at the Technion University, Haifa, Israel. Dr. Spiegel completed his residency in general pediatrics at Emek Medical Center and did fellowship in medical genetics and metabolic diseases at Bnei Zion hospital and Emek Medical Center. Prof. Spiegel main research interests include mitochondrial diseases, lysosomal storage diseases and neurometabolic diseases. Key example of his main research include the first description of ACO2 gene associated diseases and the future clinical delineation of disease spectrum. Prof. Spiegel is the leading PI of several industry sponsored multicenter, multinational interventional clinical trials including Niemann Pick type C disease, molybdenum cofactor deficiency, and various mucopolysaccharidoses. Main examples include the natural history study of molybdenum cofactor deficiency which served as the starting point for the future phase 2/3 open label study in which he played a major role.

Liza Squires, MD
Sentynl Therapeutics

Dr. Liza Squires began her career in academic, hospital-based child neurology where the unmet need for therapeutic options drew her to a career in drug development. Over the past 20 years, she has held positions of increasing responsibilities in both early and late-stage development at companies including Johnson and Johnson, Shire Pharmaceuticals, Lumos Pharma, and Origin Biosciences where she played a critical role in the transition of Nulibry^{tm} to Sentynl Therapeutics. Currently, she is the Chief Medical Officer at Neuren Pharmaceuticals. She has led and contributed to multiple NDAs and sNDAs resulting in global regulatory approvals. She received her bachelor's degree from the University of Michigan and medical degree from Michigan State University. Dr. Squires trained in general pediatrics at Yale University and did her residency in Child Neurology at Massachusetts General Hospital and is board certified in General Pediatrics and Neurology with Special Competence in Child Neurology.

Zohreh Talebizadeh, PhD
Global Genes

Dr. Zohreh Talebizadeh leads the RARE-X Research Program at Global Genes, serving as Senior Director and the Principal Investigator. Her research endeavors, spanning over two decades, encompass both rare and common diseases, with a particular focus on neurodevelopmental disorders. Dr. Talebizadeh's broad skill set spans genetics, epigenetics, data science, and patient-centered outcomes research. Her collaborative spirit shines through her strong partnerships with diverse stakeholders, including patient advocates. Her scientific accomplishments include about 40 publications and 3 book chapters. Notably, her passion for patient-centered research led her to launch a unique initiative promoting the integration of patient perspectives in genetics research. Prior to her current role at Global Genes, she served as a Translational Research Manager at the American College of Medical Genetics and Genomics in Bethesda, MD, contributing to the NICHD-funded Newborn Screening Translational Research Network program. She earned her doctoral degree in Genetics from the University of Nebraska Medical Center in Omaha, NE, where she studied the genetics of hearing loss.

Tiina Urv, PhD
National Center for Advancing Translational Sciences, NIH

Dr. Tiina Urv is the program director for the Rare Diseases Clinical Research Network (RDCRN), a multidisciplinary international program in the Division of Rare Diseases Research Innovation. As the lead for the RDCRN program, Dr. Urv collaborates with 10 NIH Institutes to manage 22 consortia and a central Data Management Coordinating Center. The RDCRN has more than 200 participating sites in 17 countries and more than 100 Patient Advocacy Groups as research partners and conducts research on about 200 rare diseases. Before joining the division, she was a program director in the Division of Clinical Innovation where she provided stewardship for multiple Clinical and Translational Science Awards Program and worked with the Trial Innovation Network as well as NCATS' Division of Rare Diseases Research Innovation.

Dr. Urv came to the National Institutes of Health (NIH) in October 2006, working as a program director at the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD) in the Intellectual and Developmental Disabilities Branch. Prior to joining NIH, she was an assistant professor at the University of Massachusetts Medical School's Eunice Kennedy Shriver Center and a research scientist at the New York State Institute for Basic Research in Developmental Disabilities. At NICHD, Dr. Urv coordinated the Hunter Kelly Newborn Screening Research Program, chaired the trans-NIH Fragile X research program, and managed a diverse portfolio of basic, behavioral and bio-behavioral research related to developmental disabilities and rare diseases.

Dr. Urv is a developmental disabilities specialist with a doctoral degree. from Columbia University. She earned her undergraduate degree from the University of Washington.

Michael Wagner, PhD
Cincinnati Children's Hospital Medical Center

Dr. Michael Wagner is an Associate Professor of Pediatrics in the Division of Biomedical Informatics at Cincinnati Children's Hospital Medical Center. Dr. Wagner serves as MPI on the NHLBI-funded Administrative Coordinating Center for the Bench to Bassinet Consortium as well as the NCATS-funded Data Management and Coordinating Center for the Rare Diseases Clinical Research Network. His team have provided cloud-based computational infrastructure and informatics expertise for various NIH-funded research networks for the past 12 years. Dr. Wagner earned a doctoral degree in Operations Research from Cornell University and an undergraduate degree in industrial and systems engineering from the University of Karlsruhe (now KIT) in Germany.

Kristen Wheeden, MBA
United Porphyrias Association

Kristen Wheeden is a fierce advocate for the porphyria community. When her youngest son was diagnosed with erythropoietic protoporphyria (EPP), Wheeden pivoted into the world of rare diseases. Understanding that advancing awareness, research, and therapies lies in uniting patients, physicians, industry, and regulators, she co-launched the United Porphyrias Association (UPA). Driven by the notion that hope is ineffective without action, she has delivered new hope for patients to enjoy improved health outcomes. Wheeden serves as President of the UPA, is a member of the NIH-supported Porphyrias Consortium. She serves on the Boards for the American Porphyrias Expert Collaborative (APEX), Shadow Jumpers Inc., the Coalition of Skin Diseases (CSD) and on the NIH Cures Acceleration Network. Her column, *Hope in Action*, is published by BioNews. Wheeden earned her bachelor's degree and MBA from The George Washington University, a master's degree in Public Health from the University of Maryland; and is currently pursuing a doctoral degree in Public Health.

Moderator

Susan C. Winckler, RPh, Esq.
Chief Executive Officer, Reagan-Udall Foundation for the Food and Drug Administration

Susan C. Winckler is CEO of the Reagan-Udall Foundation for the Food and Drug Administration. The Foundation is the non-profit organization created by Congress to advance the mission of the FDA. Prior to accepting the Foundation post in May of 2020, Ms. Winckler served as President of Leavitt Partners Solutions. As President and Chief Risk Management Officer for the Leavitt Partners family of businesses, Ms. Winckler advised corporate executives on policy and business matters. As CEO of the Food & Drug Law Institute, she provided attorneys, regulators, industry leaders, and consumers with a neutral forum to address domestic and global issues. As FDA Chief of Staff from 2007-2009, Ms. Winckler managed the Commissioner's office; served as his/her senior staff adviser; analyzed policies; and represented FDA before myriad government and external stakeholders. She simultaneously led FDA's Offices of Legislation, External Relations, Public Affairs, and Executive Secretariat. As APhA Vice President Policy/Communications and Staff Counsel, she served as the association's lead spokesperson and senior liaison to Congress, the executive branch, state associations, and allied groups. Ms. Winckler earned a bachelor's degree from the University of Iowa College of Pharmacy and her juris doctorate *magna cum laude* from Georgetown University Law Center. She is an APhA Fellow, an elected member and Chair of the United States Pharmacopeial Convention (USP) Board of Trustees (2015-2020, 2020-2025), a member of the Purgo Scientific, LLC board, and a member of the Virginia Commonwealth University School of Pharmacy National Advisory Council.