Speakers

EVERYLIFE FOUNDATION FOR RARE DISEASES | Scientific Workshop

Lynn Albizo, JD

Lynn H. Albizo is the Chief Public Policy Officer for the Immune Deficiency Foundation. She is an accomplished healthcare policy strategist experienced in legislative and regulatory advocacy at the state and federal level. Her professional experience includes work as a Senior Analyst for the Maryland Health Care Commission on telehealth issues, Director of Public Affairs for the Maryland Addictions Directors Council, Executive Director of NAMI Maryland and extensive government relations consulting experience on issues related to mental health, medication access and Medicare and Medicaid issues. She has proven policy expertise in telehealth and health technology policy, newborn screening, behavioral health, and rare and chronic diseases. She has a Bachelor of Arts degree from Union College and a Juris Doctorate from the George Washington University National Law Center. She is also licensed to practice law in the State Of Maryland.

A.J. Allen, MD, PhD

Albert John "A.J." Allen, M.D., Ph.D. is a child and adolescent psychiatrist and a pharmacologist with over 75 publications in pharmacology, child psychiatry, human abuse potential assessment, pediatric drug development, drug safety and bioethics. A.J. received an S.B in chemistry with general honors and an S.M in biochemistry from the University of Chicago in 1980. He received his M.D. and a Ph.D. in pharmacology from the University of Iowa in 1988. He completed residencies in General Psychiatry and Child Psychiatry at the University of Iowa (1988-1992), along with a Research Fellowship in Child Psychiatry at the National Institute of Mental Health in Bethesda, MD (1992-1995).He was an Assistant Professor at the Institute for Juvenile Research, University of Illinois at Chicago (UIC, 1995-2000) where he ran a pediatric psychopharmacology clinic and a pediatric anxiety and tic disorders clinic, and also served on one of the UIC Institutional Review Boards (IRBs).

A.J. joined Lilly as a global research physician during the phase 3 development of atomoxetine for ADHD and in 2004 he was promoted to global medical director, and subsequently senior medical director, for development of atomoxetine and other medications for ADHD and related disorders. In 2009, he became a member of the Lilly Bioethics Committee and he chaired the committee from 2011-2013, and remained a member representing pediatrics until he retired from Lilly in December, 2021. Amongst his many activities, A.J.'s primary responsibilities from 2011 until his retirement in 2011 were related to the development of policies, programs and infrastructure to broadly improve pediatric drug development efforts across Lilly's portfolio in neuroscience (psychiatry and later neurology), pain, diabetes, cardiovascular disease, oncology, immunology, and COVID. During this time A.J. co-chaired Lilly's Pediatric Steering Committee and was medical lead for Lilly's Pediatric Capabilities Function, he also served on Lilly Global Patient Safety's Reproduction, Pregnancy and Pediatric Safety Advisory Committee.

A.J. was a member of BIO's pediatric committee from 2011 to 2021 and chair of the committee from 2013 to 2015. A.J. is also a past-member of the Secretary's Advisory Committee on Human Research Protections (SACHRP, 2011 to 2015) and current member of SACHRP's Subcommittee on Harmonization, a member of the EPA's Human Subjects Research Board (HSRB), a past-member of Public Responsibility In Medicine and Research's (PRIM&R's) board of directors and member and chair of its Advancing Ethical Research Core Conference Planning Committee, as well as a member of the American Academy of Child and Adolescent Psychiatry's (AACAP's) ethics committee and a corresponding member of AACAP's Psychopharmacology and Device Committee. Though retired from Lilly, he continues his professional activities through these means and several others, as well as mentoring and consulting.

In 2013 he was diagnosed with multiple myeloma and several secondary conditions and underwent a stem cell transplant. His treatments have continued off and on and he is currently doing very well. His experiences with this chronic illness and related chronic illnesses has had a significant impact on his thinking concerning bioethics and pediatric drug development. He has a weakness for kids (the human kind) and pets (especially cats).

A.J. was involved at several points in events leading up to and including the creation of the Institute for Advanced Clinical Trials for Children (I-ACT4C) and served as Lilly's observer on I-ACT4C's board through 2021. A.J. also was involved in events leading up to and including the creation of I-ACT4C's sister organization in Europe, Conect4Children (C4C). He became I-ACT4C's chief medical officer in August of 2023. A.J.'s thoughts regarding I-ACT4C and its mission, "Nothing is more important than our children. They are our future, yet they are often amongst the most vulnerable, neglected, forgotten, and abused in our society. We must do better. In medical drug and device development, this means that children and their families have the same right to benefit in a timely manner from modern medical advances as adults, with the same expectations regarding scientific data supporting safety, efficacy, and dosing. Put another way, when it comes to medical drug and device development, children and their families should be treated as first class citizens, not relegated to second class status."

Matthew Ellinwood, DVM, PhD

Dr. Matthew Ellinwood, holding a DVM and PhD in physiology from Colorado State University, has extensive expertise in molecular biology, reproductive physiology, innate immunity, and comparative medical genetics. Currently serving as the Chief Scientific Officer at the National MPS Society, Inc., he guides research efforts for mucopolysaccharidosis and mucolipidosis disorders, contributing to clinical and public health advancements.

Teresa Fecteau, PhD

Dr. Teresa Fecteau is Director of Medical Affairs with the Biogen US Medical Rare Disease Team. She leads Amyotrophic Lateral Sclerosis (ALS) strategy, planning, and implementation efforts, and supports a range of other Rare Disease Medical activities for Friedreich Ataxia (FA) and Spinal Muscular Atrophy (SMA). Dr. Fecteau received her Doctorate in Clinical Psychology from the University of Maine and completed a specialization in Behavioral Medicine/Medical Psychology during residency and post-doctoral fellowship at the Duke University School of Medicine. She has extensive experience as a clinician, medical educator, researcher, medical

writer, and leader within the pharmaceutical industry, academic medicine, and VA/DoD environments, with the past decade largely focused in Neurology, Rare Neuromuscular Diseases, and driving awareness of the overarching importance of equitable access to empirically driven multidisciplinary care for chronic, intractable illnesses.

Tim Franson, MD

Professional employment: Dr. Franson currently serves as a Principal of Faegre Drinker Consulting in the firm's life sciences regulatory consulting practice (2019-present) and was previously Chief Medical Officer for YourEncore (2014-19). He spent the majority of his professional career with Eli Lilly and Co. (Lilly Research Laboratories) from 1986 until 2008, retiring from the leadership role of Global Vice President for Regulatory Affairs and Drug Safety (2003-2008), and was directly responsible for Lilly's regulatory submissions including over 25 major approvals, as well as regulatory compliance and policy matters. Prior to his work at Lilly, Dr. Franson was an Assistant Professor of Medicine (Infectious Diseases) and Hospital Epidemiologist at Medical College of Wisconsin (Milwaukee).

Pharmaceutical development capabilities: He has extensive experience in early and late phase drug development relating to clinical, regulatory, quality and related disciplines for small molecule, biological, cell and gene therapies. His consulting practice has served over 100 client firms with involvement in over 20 major product approvals.

Leadership experience: Franson has represented industry in policy matters including Congressional testimony for several renewals of the Prescription Drug User Fee Act (PDUFA), notably as co-chair of industry negotiations for PDUFA-3. Franson was a member of the Steering Committee for the independently developed draft guidance for FDA consideration regarding development of drugs for Duchenne Muscular Dystrophy and continues to collaborate in providing rare disease drug development advice for multiple companies and advocacy groups, with emphasis in neurological diseases. He also served from 2016-2021 as Chair of the Board of Directors for the Critical Path Institute (funded primarily by FDA and the EMA/IMI for biomarker and data consortia endeavors) and remains on that Board. He was the President of the U.S. Pharmacopeial Convention from 2010- 2015 and served on their Board of Trustees until 2020. He now serves as a Director for Paratek Pharmaceuticals and Cidara Therapeutics. He is Adjunct Professor of Clinical Pharmacology at Indiana University School of Medicine.

Professional service: Dr. Franson served on the Xavier University of Louisiana Board of Trustees from 2002-2008; Regulatory Advisory Board–Center for Medicines Research International from 2003-2008; Pharmaceutical Research and Manufacturers Association-PhRMA from 1995-2007 (past chair of Clinical Steering Committee, past chair of GMP Task Force, past chair of FDA Staff Work Group, member-Regulatory Affairs Committee and co-chair of PDUFA-3 coordination/negotiations committee); Auburn University Harrison School of Pharmacy Dean's Advisory Committee; and Drake College of Pharmacy National Advisory Committee. Dr. Franson also served on American Association of Colleges of Pharmacy's Pharmaceutical Education Advisory Committee and the Association of American Medical Colleges-PhRMA Clinical Trials Forum. He previously served on the NIH-NCATS Treatment of Rare and Neglected diseases (TRND) review panel.

Recognitions: Dr. Franson was named James Scholar–University of Illinois College of Medicine (1977-78); is a member of Rho Chi Honor Society– Drake University College of Pharmacy; earned

Lilly Chairman's Ovation Award (2002); and was awarded the Sagamore of the Wabash by Indiana Governor Mitchell E. Daniels Jr. in 2008 for community service. Franson was Commencement Speaker (2017) for the University of Iowa College of Pharmacy.

Dr. Franson's professional interests include infectious diseases in the elderly (having served as former NIA/NIH RO-1 grant principal investigator); hospital epidemiology (device associated nosocomial infections); and antibiotic drug utilization review, as well as rare disease policy development. He has published over 50 articles in peer reviewed journals and is board certified in Internal Medicine and Infectious Diseases.

Aaron Goldenberg, PhD, MPH

Aaron Goldenberg is a Professor and Vice-Chair in the Department of Bioethics at Case Western Reserve University. He is also Director for the CWRU Bioethics Center for Community Health and Genomic Equity. Dr. Goldenberg has a background in bioethics, health behavior/health education, public health ethics, and public health genetics. He has focused his work on the ethical, legal, and social issues associated with the integration of new genomic technologies into research, clinical and public health settings. Dr. Goldenberg's research program also focused on the intersection between ethics, genetics, rare diseases, newborn screening, and health equity. He was the Co-Chair the NBSTRN Bioethics and Legal Workgroup, and with this group, led a recent effort to develop a set ELSI questions that can be integrated into NBS Pilots to help assess the potential benefits and harms of adding new conditions to NBS panels. He is also Co-Chair for Ethical, Legal, Social and Policy Issues In Newborn Screening Subcommittee for the Association of Public Health Laboratories.

Kishore Hari

Kishore Hari is a Senior Program Manager on the Science in Society team at the Chan Zuckerberg Initiative, managing a portfolio of projects that bolsters patient-driven research in rare disease. Previously, he worked at UCSF working on numerous research participation, science communication, and public engagement projects. He also served as the science correspondent for Adam Savage's Tested.com and co-hosted the award winning Science in Society podcast "Inquiring Minds" at Mother Jones.

Justin Hopkin, MD

Justin Hopkin is a physician, advocate and rare disease parent. As chief of the Hospital Medicine Division at the University of Rochester, he provides strategic and operational leadership with responsibility across the division's missions. He is a board member for Uplifting Athletes and an emeritus board member for the National Niemann Pick Disease Foundation (NNPDF), the patient support organization for patients and families with Niemann Pick Disease. He is on the scientific advisory board for the NNPDF and International Niemann Pick Disease Registry. As an advocate, his works to support and empower patient communities. He is particularly interested in promoting collaboration between patients, industry and regulators with a focus on drug development, clinical trial design, patient-owned registries and newborn screening.

Annie Kennedy

Annie Kennedy is chief of policy, advocacy, and patient engagement, at EveryLife Foundation for Rare Diseases. Focused on improving health outcomes for people living with rare diseases by advancing the development of treatment and diagnostic opportunities for rare disease patients through science-driven public policy, Kennedy's work includes building strong partnerships with policy makers, federal agencies, industry, and alliances. Kennedy has served within the community for nearly three decades through her roles with Parent Project Muscular Dystrophy (PPMD) and the Muscular Dystrophy Association (MDA). In that time, she helped lead legislative efforts around passage and implementation of the MD-CARE Act (2001, 2008, 2014) and the Patient Focused Impact Assessment Act (PFIA), which became the Patient Experience Data provision within the 21st Century Cures Act (section 3001). She has engaged with the Food and Drug Administration and industry around regulatory policy and therapeutic pipelines, led access efforts as the first therapies were approved in Duchenne, and engaged with the Institute for Clinical and Economic Review around the development of the modified framework for the valuation of ultra-rare diseases.

Kennedy's community roles include service on the board of directors of Cure SMA, the PFDD Works coalition, the Patient Driven Values in Healthcare Evaluation (PAVE) Steering Committee, FasterCures Cures for Life initiative, the National Health Council's PCORI Valuation Group, the Innovation and Value Initiative Patient Advisory Committee, the National Duchenne Newborn Screening Pilot Program Steering Committee, the Institute for Gene Therapies Patient Advocacy Advisory Council, the State Rare Disease Education Initiative (STRiDE) National Steering Committee, and as a member of the National Institutes of Health's National Center for Advancing Translational Sciences Advisory Council and the Cures Accelerator Network Advisory Board.

Sharon King

Sharon is a thought leader who has united public officials, researchers, biotech and industry representatives, and patient advocates to gain real progress in rare disease treatment development. She is a state-appointed member of the North Carolina Advisory Council on Rare Diseases and chair of the North Carolina Rare Disease Coalition. By day, Sharon also serves as manager of advocacy and community engagement at Aldevron, a leader in advancing biological science.

A graduate of Leadership Charlotte Class XVI and a past member of the Charlotte Rotary, Sharon was honored as one of Charlotte's 50 Most Influential Women in 2016 and received the Elon Homes and Schools Caring for Children Award in 2013. She has also been nominated for the Global Genes RARE Champion of Hope award.

Sharon is past president of the Charlotte Piano Teachers' Forum, Symphony Guild of Charlotte and Junior League of Charlotte, and she served on the Boards of the Council for Children's Rights and the Association of Junior Leagues International. She was a North Carolina Delegate to the 1997 President's Summit for America's Future.

Sharon is a graduate of Meredith College with a bachelor of music in applied piano.

Esther Krofah, MPP

Esther Krofah is the executive vice president of MI Health, leading FasterCures, Public Health, the Future of Aging and Feeding Change. She has extensive experience managing efforts to unite diverse stakeholders to solve critical issues and achieve shared goals that improve patients' lives. Most recently, Krofah was the director of public policy at GlaxoSmithKline (GSK), where she led engagement with the US Department of Health and Human Services (HHS) and relevant executive branch agencies on broad healthcare policy issues. Prior to GSK, Krofah was a deputy director of HHS' Office of Health Reform. She also served as program director at the National Governors Association (NGA) healthcare division and worked in consulting at Deloitte Consulting LLP. Krofah received a BA from Duke University and a master's in public policy from the Harvard University John F. Kennedy School of Government.

Peter Marks, MD, PhD

Peter Marks, M.D., Ph.D. is the director of the Center for Biologics Evaluation and Research (CBER) at the Food and Drug Administration. The center is responsible for assuring the safety and effectiveness of biological products, including vaccines, allergenic products, blood and blood products, and cellular, tissue, and gene therapies.

Dr. Marks and center staff are committed to facilitating the development of biological products and providing oversight throughout the product life cycle. Examples of these activities include:

- reviewing and providing advice during product development
- evaluating applications and making approval decisions based on safety and effectiveness data
- monitoring the safety of biological products
- conducting research that supports product development and characterization

"The center regulates and does research on complex biologic products that touch people's lives on a daily basis," says Dr. Marks. "Many of the products that we regulate are vital for promoting and protecting the public health, including vaccines, blood products, and tissues for transplantation. I'm very proud to lead a team of highly committed individuals whose efforts help to ensure the timely development of safe and effective products to meet important medical needs."

Dr. Peter Marks received his graduate degree in cell and molecular biology and his medical degree at New York University. Following this, he completed an Internal Medicine residency and Hematology/Medical Oncology fellowship at Brigham and Women's Hospital in Boston, where he subsequently joined the attending staff as a clinician-scientist and eventually served as Clinical Director of Hematology.

He then moved on to work for several years in the pharmaceutical industry on the clinical development of hematology and oncology products prior to returning to academic medicine at Yale University where he led the Adult Leukemia Service and served as Chief Clinical Officer of Smilow Cancer Hospital. He joined the FDA in 2012 as Deputy Center Director for CBER and became Center Director in 2016. Dr. Marks is board certified in internal medicine, hematology and medical oncology, and is a Fellow of the American College of Physicians. In 2022, he became a Member of the National Academy of Medicine, one of the highest honors in the fields of health, science and medicine.

Janet Maynard, MD, MHS

Dr. Janet Maynard is the Director of the Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine (ORPURM) within the Food and Drug Administration's Center for Drug Evaluation and Research (CDER). In this role, she oversees the development, review, and regulation of applications for drugs and biologic products reviewed within the divisions in ORPURM: The Division of Pediatrics and Maternal Health, the Division of Rare Diseases and Medical Genetics, the Division of Urology, Obstetrics and Gynecology, and the Division of Pharmacology-Toxicology for Rare Diseases, Pediatrics, Urologic and Reproductive Medicine/Specialty Medicine. Prior to serving as Director, she was the Deputy Director of ORPURM.

Prior to ORPURM, Dr. Maynard was the Director of the Office of Orphan Products Development, and oversaw legislatively mandated designation and grant programs intended to promote the development of products for rare diseases including, orphan drug, rare pediatric disease, and humanitarian use device designation programs, as well as clinical trial, natural history study, and pediatric device consortia grant programs. Before directing OOPD, she was a clinical team leader in CDER's Division of Anesthesia, Analgesia, and Addiction Products. She originally joined FDA in 2011 as a Medical Officer in the Division of Pulmonary, Allergy, and Rheumatology Products (DPARP), before becoming a clinical team leader in DPARP.

Dr. Maynard received her medical degree from Vanderbilt University and completed a residency in internal medicine at Duke Hospital. Subsequently, she completed a fellowship in rheumatology at Johns Hopkins Hospital. During her fellowship, she completed a Master of Health Science at the Johns Hopkins Bloomberg School of Public Health in the Graduate Training Program in Clinical Investigation.

Reenie McCarthy, JD

Reenie McCarthy is the Chief Executive Officer and a member of the Board of Stealth BioTherapeutics, a biopharmaceutical company developing investigational therapies to treat diseases of mitochondrial dysfunction. Stealth is in Phase 3 development for mitochondrial myopathy, a rare genetic disease, and dry age-related macular degeneration, the leading cause of progressive blindness in older individuals, and has a new drug application under review by the FDA for the ultra-rare genetic disease, Barth syndrome. In addition to leading Stealth, Reenie also serves on the board of Biotechnology Innovation Organization (BIO) as a member of the Emerging Companies Section Governing Board and as Chair of the Board of Directors of Cognoa, a privately held company which has developed the first FDA-approved diagnostic for autism spectrum disorder. Reenie previously led the US-based investment team of Morningside Ventures from 1993 through 2015, providing operational and Board-level management oversight to venture- and private-equity backed companies in the United States, Canada, and Europe. Reenie earned her J.D. from the University of Pennsylvania Carey School of Law and her B.A. from Bates College, cum laude, Phi Beta Kappa.

Paul Melmeyer, MPP

Paul serves as the Executive Vice President, Public Policy and Advocacy, at the Muscular Dystrophy Association (MDA). In this role, Paul leads MDA's policy and advocacy initiatives pertaining to public health, therapeutic development, access to care, and disabilities. Prior to joining MDA, Paul spent over six years with the National Organization for Rare Disorders (NORD). At NORD, Paul led the Federal policy operations in developing and advocating for the enactment and implementation of pro-rare disease patient policy. Paul also holds a Master of Public Policy (MPP) from the George Washington University.

Emily Milligan, MPH

Emily is the Executive Director of the Barth Syndrome Foundation. She is an advocate dedicated to improving the lives of people through better health and social equity. With a background in public health and international relations, she brings decades of experience in managing complex research and product development (R&D) portfolios for medically underserved populations. Previously, Emily held instrumental roles at renowned institutions, including the United Nations in Brazil and Nicaragua, as well as key positions at Columbia University and New York University.

Her expertise in R&D management was further honed during her tenure at JDRF, where she led research operations and scientific teams, overseeing an average annual $100 million research portfolio. At JDRF (formerly known as "Juvenile Diabetes Research Foundation"), Emily spearheaded the launch of a $200 million venture fund dedicated to investing in companies developing life-saving products for individuals living with type one diabetes. Her strategic vision and leadership were instrumental in driving impactful research initiatives and fostering innovation in the sector.

Since joining the Barth Syndrome Foundation in 2018, Emily has emerged as a prominent thought leader in the ultra-rare disease space, driving the organization to the forefront in the eyes of the U.S. Congress, the FDA, and peer coalitions. She has navigated the foundation through complex dynamics with the FDA and augmented interest from academia and industry in ultra-rare drug opportunities. Emily believes in the rare disease community's duty to help each other, stating that advancing the mission of one progresses the missions of all. Her commitment to the cause has deepened over time, evolving into a personal loyalty.

Adora Ndu, PharmD, JD

Adora Ndu is the Chief Regulatory Affairs and Interim Legal Officer for BridgeBio Pharma, joining the ML Bio board in October 2022. Prior to joining BridgeBio, she held various leadership positions at BioMarin Pharmaceutical, including as Group Vice President of Worldwide Research and Development Strategy, Scientific Collaborations and Policy, and also as Vice President of Regulatory Affairs Policy, Research, Engagement, and International. Dr. Ndu served at the U.S. Food and Drug Administration (FDA) in several roles of increasing responsibility, including as Director for the FDA's Division of Medical Policy Development, team leader at the FDA's Office of Prescription Drug Promotion (OPDP), and post-market safety reviewer with the FDA's Division of

Pharmacovigilance, additionally, she was a Commander in the Commissioned Corps, United States Public Health Service. Dr. Ndu is a member of the board of directors for Acadia Pharmaceuticals and DBV Technologies. Dr. Ndu holds Doctor of Pharmacy degree from Howard University College of Pharmacy and a Juris Doctor degree from the University of Maryland Carey Law School.

Ethan Perlstein, PhD

Ethan O. Perlstein received a PhD in 2006 from Harvard University (Department of Molecular and Cell Biology) while working in the laboratory of Professor Stuart Schreiber. He completed an independent postdoctoral fellowship at the Lewis-Sigler Institute at Princeton University from 2007 to 2012. Since its founding in 2014, he is CEO of Perlara PBC, the first biotech Public Benefit Corporation partnering with highly motivated families to develop treatments and cures for genetic diseases. Perlara's first joint venture Maggie's Pearl is the commercial sponsor of a pivotal Phase 3 trial of epalrestat for the treatment of PMM2-CDG. In 2019, he joined the Christopher & Dana Reeve Foundation as their first Chief Scientific Officer, but due to unexpected COVID-related financial constraints his team was let go in Spring 2020. In Summer 2020, Ethan became CEO of the EveryONE Foundation (EOF), cofounded by Dr. Tim Yu and Julia Vitarello, whose mission is to make individualized medicines safe and accessible to everyone.

Sal Rais, MD, MBA

Sal Rais is currently an Investment Analyst at T. Rowe Price, where he has focused on the biotechnology space for the past 3 years. Sal has 13 years of experience across the healthcare and investment space. He obtained his undergraduate degree in Neuroscience and Economics at William & Mary followed by a joint MD/ MBA at the University of Virginia.

Joni Rutter, PhD

Joni L. Rutter, Ph.D., is the director of the National Center for Advancing Translational Sciences (NCATS) at the National Institutes of Health (NIH). She oversees the planning and execution of the center's complex, multifaceted programs that aim to overcome scientific and operational barriers slowing the development and delivery of new treatments and other health solutions. Under Rutter's direction, NCATS supports new tools and approaches to make each step in the translational process more effective and efficient. A key goal is to speed research across a range of diseases, with a particular focus on rare diseases. By advancing the science of translation, NCATS helps turn promising research discoveries into real-world applications that improve people's health.

In her prior role as the NCATS deputy director, Rutter worked with colleagues from government, academia, industry and nonprofit patient organizations to create robust interactions with NCATS programs.

Before joining NCATS, Rutter was the director of scientific programs within the All of Us Research Program. She led scientific programmatic development and implementation efforts to

build a national research cohort of 1 million or more U.S. participants to advance precision medicine.

While at NIH, she also led the Division of Neuroscience and Behavior at the National Institute on Drug Abuse (NIDA). In this role, she developed and coordinated research on basic and clinical neuroscience, brain and behavioral development, genetics, epigenetics, computational neuroscience, bioinformatics, and drug discovery. Rutter also coordinated the NIDA Genetics Consortium and biospecimen repository.

During her career, Rutter has earned a national and international reputation for her diverse and unique expertise via her journal publications. She has received several scientific achievement awards, including the 2022 Rare Disease Legislative Advocates–RareVoice Award for Federal Advocacy and the 2022 FedHealthIT–Women in Leadership Impact Award.

Rutter received her Ph.D. from the Department of Pharmacology and Toxicology, Dartmouth Medical School, Hanover, New Hampshire, and completed a fellowship at the National Cancer Institute within the Division of Cancer Epidemiology and Genetics.

Frank Sasinowski, MS, MPH, JD

Mr. Sasinowski, M.S., M.P.H., J.D., assists sponsors in developing new medicines and has helped secure FDA approval for hundreds of new drugs, including more than 45 new molecular entities, often for serious and/or rare diseases. Mr. Sasinowski joined FDA in 1983 as regulatory counsel in the Center for Drugs and Biologics, where he was key to implementing both the 1983 Orphan Drug law and the 1984 Hatch-Waxman law. Mr. Sasinowski twice received the FDA Award of Merit. In 1987, he left the FDA as Deputy Director of the health policy staff in the Commissioner's office and joined Hyman, Phelps & McNamara P.C. In December 2014, Frank was appointed an Adjunct Professor of Neurology at the University of Rochester Medical Center. Mr. Sasinowski's work has been widely recognized by industry and political leaders, as well as notable organizations. For example, Mr. Sasinowski was asked by both political parties to testify at the May 2014 inaugural hearing of Congress 21st Century Cures Initiative. He was a member of the NORD Board of Directors for 16 years and was given a lifetime achievement award in 2013. In October 2012, President Obama recognized Mr. Sasinowski's contributions to the President's Council of Advisors on Science and Technology (PCAST) report, "Propelling Innovation in Drug Discovery, Development and Evaluation." Mr. Sasinowski has been Chair of the Food and Nutrition Section of the American Public Health Association (APHA) and has taught health law at American University. Mr. Sasinowski has also served on the Board of Directors of the United States Pharmacopeia (USP).

Jamie Sullivan, MPH

Jamie Sullivan joined the EveryLife Foundation for Rare Diseases in July 2020 where she serves as the Vice President of Policy. Previously, Jamie served in a variety of roles for the COPD Foundation for more than a decade. Jamie's work has focused on achieving patient-centered federal, state and regulatory policy changes in the areas of health appropriations, public health and regulatory infrastructure and access to care. Jamie has experience building programs to engage and train patient advocates as well as advocating for the robust inclusion of patient and caregiver perspectives in all aspects of treatment development and review. Prior to joining the COPD Foundation, Jamie supported the research and clinical programs of the Alpha-Foundation. She obtained her master's in public health policy and management at the Florida International University.

Kevin Thorneloe, PhD

Kevin Thorneloe, PhD is a Senior Medical Director in Clinical Research at Pharming Healthcare. Kevin received his PhD in Biochemistry and Molecular Biology from the University of Calgary and completed a post-doc in Pharmacology at the University of Vermont, prior to working in the pharmaceutical industry for the past 20 years. He was initially a research lab head at Merck and GSK, where he led drug discovery teams. The majority of his career, at GSK and then Aeglea Biotherapeutics, has been focused on the development of small molecule and biologic agents, to deliver life changing new medicines to rare disease patients. For the last 2 years, Kevin has been at Pharming Healthcare where he is currently responsible for the development of leniolisib in new disease states.

Janet Woodcock, MD

Janet Woodcock recently completed a long career at FDA. She served as Director of the Center for Drug Evaluation and Research for over twenty years in several stretches. Most recently she served as Principal Deputy Commissioner and prior to that as Acting FDA Commissioner. She held multiple other senior positions at FDA including at the Center for Biologics Evaluation and Research. She was the therapeutics lead for “Operation Warp Speed” during the COVID pandemic. Her most recent effort was spearheading a major reorganization of FDA’s foods program and the Office of Regulatory Affairs. Dr. Woodcock completed many major regulatory initiatives during her FDA tenure. She was instrumental in getting the biosimilars legislation enacted and worked to ensure adoption in the clinical community. Additionally, she worked with industry and Congress to bring about the first GDUFA. After passage of the legislation, she oversaw an extensive reorganization of the generic drug review program at CDER, that successfully met the aggressive targets of the legislation and led to eventual reauthorizing, with elimination of backlogs and approval of thousands of generic drugs.