Welcome to FDA's Rare Disease Day 2022 Virtual Public Meeting

Webcast link:

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Speaker Biographies

FDA's Rare Disease Day 2022 Speakers and Moderator Biographies

Rosalyn Adigun, MD, PharmD, MSc, Medical Officer, Center for Drugs Evaluation and Research (CDER), FDA

Dr. Rosalyn Adigun received her MD and PharmD degrees from University of Texas Medical Branch and University of Houston College of Pharmacy respectively. She completed training in Cardiology, Advanced Echocardiology, and Advanced Heart Failure and Transplant Cardiology at Mayo Clinic Rochester. She also holds a M.Sc. in Clinical and Translational Sciences from Mayo Clinic School of Biomedical Sciences with an area of interest in Cardiac amyloidosis. Dr. Adigun recently joined the Division of Cardiovascular and Renal Products, Office of New Drugs in CDER/FDA.

Victor C. Baum, MD, Reviewer, Medical Officer, Center for Biologics Evaluation and Research (CBER), FDA

Dr. Victor "Vic" Baum joined FDA's Center for Biologics Evaluation and Research's (CBER) Office of Blood Research and Review as a Medical Officer in 2014, reviewing primarily modified blood products. During this period, he also spent time reviewing inborn error submissions. Prior to joining FDA, Vic was the F.A. Berry Professor of Pediatric Anesthesiology, Professor of Pediatrics, Executive-Vice Chair of Anesthesiology and Chief of Cardiac Anesthesia at the University of Virginia. He has been board certified in Anesthesiology, Pediatrics, Pediatric Cardiology and Pediatric Critical Care, and has authored over 100 papers and book chapters and three editions of a book on anesthesia and genetic and dysmorphic syndromes. Vic has lectured widely in the U.S., Europe and Asia and remains on the editorial boards of several journals. At FDA, Vic sits on the Pediatric Review Committee and at CBER he is a member of the Pediatric Working Group and the Rare Disease Coordinating Committee. In addition to reviewing "regular" INDs, in the course of his work Vic handles many emergency INDs.

Diana Bradford, MD, Cross Disciplinary Team Leader on the Neurologic, Pediatric Solid Tumor and Rare Tumor, Center for Drugs Evaluation and Research (CDER), FDA

Dr. Diana Bradford is a pediatric hematologist/oncologist and Cross Disciplinary Team Leader on the Neurologic, Pediatric Solid Tumor and Rare Tumor team in the Division of Oncology 2 within the Center for Drug Evaluation and Research (CDER). Dr. Bradford joined the FDA in 2017 as a clinical reviewer. Dr. Bradford received her bachelor's degree from Dartmouth College, her medical degree from the University of Massachusetts Medical School, and completed residency training in pediatrics at Brown University/Hasbro Children's Hospital. She then completed fellowship training in pediatric hematology/oncology at Children's National Medical Center in Washington, DC. Her fellowship research at the National Cancer Institute was focused on patients with rare tumors and tumor predisposition syndromes. She is board certified in pediatrics and pediatric hematology/oncology.

Julie Breneiser, Gorlin Syndrome Alliance (GSA)

Julie Breneiser is the Executive Director of the Gorlin Syndrome Alliance (GSA). Prior to her position as director, Julie served as volunteer Board President for the GSA. Mrs. Breneiser and her two young adult children are affected with Gorlin syndrome, a rare genetic disorder caused by a tumor suppressant mutation that can affect every organ system. Her early career began as a Physician Assistant followed by teaching preschoolers with disabilities. Mrs. Breneiser's work with the GSA focuses on collaboration with industry to bring new and/or potential treatments to clinical trial. She also provides individual support to affected patients and their caregivers along with educating health care providers about this rare disease. Mrs. Breneiser previously served as a consumer reviewer for the National Institutes of Health and the Department of Defense's Congressionally Directed Medical Research Program. Mrs. Breneiser speaks nationally and internationally raising awareness about Gorlin syndrome. She is a member of the Patient Engagement Collaborative (PEC), a joint effort of the U.S. Food and Drug Administration (FDA) and the Clinical Trials Transformation

Initiative (CTTI).

Dr. Sara Brenner, MD, MPH, Chief Medical Officer for In Vitro Diagnostics and Associate Director for Medical Affairs, CDRH, FDA

Dr. Sara Brenner is a preventive medicine and public health physician serving as the Chief Medical Officer for In Vitro Diagnostics and Associate Director for Medical Affairs in the Center for Devices and Radiological Health at the U.S. Food and Drug Administration (FDA). Dr. Brenner advises leadership on regulatory premarket and post-market compliance and surveillance, as well as broader initiatives to promote and protect public health across the medical technology landscape. She contributes to decisions regarding policy development, program execution, and short- and long-range program goals and objectives. Since February 2020, Dr. Brenner has been supporting the National COVID-19 Response at multiple levels, focused on diagnostics, data, and leveraging technology across the interagency response. Since May 2020, Dr. Brenner has also been serving as the Diagnostic Data Lead on the U.S. Department of Health and Human Services (HHS) Data Strategy and Execution Workgroup.

Prior to FDA, Dr. Brenner served as a Senior Policy Advisor in the White House Office of Science and Technology Policy (OSTP) with a broad portfolio in biomedical science, technology, and human health. Before joining the federal government, Dr. Brenner was a tenured faculty member at the SUNY Polytechnic Institute Colleges of Nanoscale Science & Engineering (CNSE) where she was an Associate Professor of Nanobioscience, the Assistant Vice President for NanoHealth Initiatives, and Director of the MD/PhD Program in Nanomedicine. Dr. Brenner received her MD from the University of Iowa Carver College of Medicine, her MPH from the SUNY UAlbany School of Public Health (Health Policy and Administration), and BS in genetics (minor in philosophy) from Iowa State University. She trained in Internal Medicine at Evanston Northwestern in Chicago and Preventive Medicine and Public Health at the New York State Department of Health and SUNY UAlbany School of Public Health.

Jamie Brewer, MD, Medical Oncologist and Acting Clinical Team Lead, Office of Oncologic Diseases (OOD), FDA

Dr. Jamie Brewer is a medical oncologist and Acting Clinical Team Lead in the Division of Oncology 3 (DO3) in the Office of Oncologic Diseases (OOD) at the Food and Drug Administration (FDA). Dr. Brewer joined the FDA in 2018 and previously served as a clinical reviewer on the Genitourinary Cancer team. Dr. Brewer serves as the Oncology Center of Excellence (OCE) Scientific Liaison for Cancer Disparities for which she actively engages with FDA colleagues and external stakeholders to promote inclusion and representation of diverse patient populations in clinical trials. Dr. Brewer is an active contributor to OCE initiatives such as Project Equity and Project Community. She is also a participant in multiple internal and external scientific working groups. Dr. Brewer completed her medical training at The University of Illinois at Chicago. She completed her residency and a joint fellowship in Medical Oncology and Clinical Pharmacology and Pharmacogenomics at The University of Chicago.

Andrew Byrnes. PhD, Branch Chief and Principal Investigator, Center for Biologics Evaluation and Research (CBER), FDA

Dr. Andrew P. Byrnes is a Branch Chief and Principal Investigator in CBER. He has over 20 years of experience at FDA in reviewing the manufacturing of gene therapies, cell therapies and other products for clinical use. The Gene Transfer and Immunogenicity branch is a group of six laboratories that perform research to improve the safety and efficacy of cell and gene therapies, and members of the Branch also review manufacturing of investigational and licensed cell and gene therapy products. Dr. Byrnes has a background in virology and gene therapy. Dr. Byrnes received his undergraduate degree from Yale University, earned his Ph.D. from the University of Oxford, and then conducted postdoctoral research at Johns Hopkins University before joining FDA in 2000.

Eileen Cadel, PhD, Lead Reviewer, Center for Devices and Radiological Health (CDRH), FDA

Dr. Eileen Cadel is a Biomedical Engineer in the FDA's Center for Devices and Radiological Health. As a Lead Reviewer in DHT6B, Division of Spinal Devices, she is a subject matter expert in devices for pediatric patients and has been recognized for her work on novel anterior non-fusion devices for adolescent idiopathic scoliosis patients. Prior to joining DHT6B, Dr. Cadel worked in the Division of Applied Mechanics in the Office of Science and Engineering Laboratories researching and developing better test methods to assess spinal and orthopedic devices as it applies to premarket review. She earned her Ph.D. in bioengineering from the University of Kansas and her B.S. in biomedical engineering from the University of Virginia.

Robert M. Califf, MD, MACC, Commissioner of Food and Drugs

Dr. Robert M. Califf was confirmed earlier this year as the 25th Commissioner of Food and Drugs.

As Commissioner, Dr. Califf oversees the full breadth of the FDA portfolio and execution of the Federal Food, Drug, and Cosmetic Act and other applicable laws. This includes assuring the safety, effectiveness, and security of human and veterinary drugs, vaccines and other biological products for human use, and medical devices; the safety and security of our nation's food supply, cosmetics, dietary supplements, products that give off electronic radiation; and the regulation of tobacco products.

Dr. Califf has had a long and distinguished career as a physician, researcher, and leader in the fields of science and medicine. He is a nationally recognized expert in cardiovascular medicine, health outcomes research, health care quality, and clinical research, and a leader in the growing field of translational research, which is key to ensuring that advances in science translate into medical care.

This is Dr. Califf's second stint as Commissioner. He also served in 2016 as the 22nd Commissioner. Before assuming the position at that time, he served as the FDA's Deputy Commissioner for Medical Products and Tobacco.

Prior to rejoining the FDA in 2022, Dr. Califf was head of medical strategy and Senior Advisor at Alphabet Inc., contributing to strategy and policy for its health subsidiaries Verily Life Sciences and Google Health. He joined Alphabet in 2019, after serving as a professor of medicine and vice chancellor for clinical and translational research at Duke University. He also served as director of the Duke Translational Medicine Institute and was the founding director of the Duke Clinical Research Institute.

Dr. Califf is a graduate of Duke University School of Medicine. He completed a residency in internal medicine at the University of California, San Francisco and a fellowship in cardiology at Duke.

Michelle Campbell, Senior Clinical Analyst, Center for Drugs Evaluation and Research (CDER), FDA

Dr. Michelle Campbell is the Senior Clinical Analyst 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. Previously, Dr. Campbell 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 on 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.

Denise Casey, MD, MS, Pediatric Oncologist and Former FDA Clinical Reviewer

Dr. Denise Casey is a pediatric hematologist/oncologist by training, a former FDA Medical Officer, and currently works as a clinical consultant in a company that assists pharmaceutical and biotech companies with regulatory submissions. Dr. Casey completed her pediatric hematology/oncology training and obtained a Master of Science in Clinical Investigation at Memorial Sloan Kettering Cancer Center/Weill Cornell Medical Center. She subsequently spent four years as an Assistant Professor in the Division of Pediatric Hematology/Oncology at Golisano Children's Hospital, University of Rochester Medical Center in New York. There she was responsible for clinical care of patients and supervision and education of fellows, residents and medical students, and served as an investigator for Children's Oncology Group and Dana Farber Cancer Institute clinical trials. Dr. Casey joined FDA in 2013 where she spent 7 ½ years as a clinical reviewer, including more than 6 years on the neuro-oncology, pediatric and rare tumors team and approximately one year as an acting clinical Team Leader for the melanoma/sarcoma team in the Office of Oncologic Diseases. Dr. Casey was the primary clinical reviewer for multiple marketing applications for drugs intended to treat rare tumors. She participated in internal and external outreach initiatives focused on trial design, endpoints, and efficient drug development for rare tumor indications. During her time at FDA, Dr. Casey was a member of the Pediatric Oncology Working Group, participated in meetings of the Pediatric Subcommittee of the Oncologic Drugs Advisory Committee, and was an invited speaker to conferences and workshops to present on topics including trial design for rare tumor drug development programs, patient experience data, and pediatric drug development legislative initiatives.

Martha Donoghue, MD, Deputy Director, Division of Oncology 2, Offices of Oncologic Diseases (OOD), CDER; and Acting Associate Director for Pediatric and Rare Cancer Drug Development, Oncology Center of Excellence (OCE), FDA

Dr. Martha Donoghue is a pediatric oncologist and Deputy Director of the Division of Oncology 2 in the Office of Oncologic Diseases at the FDA, where she provides regulatory oversight and advice regarding the development of drugs to treat pediatric solid tumors and other rare cancers. She also serves as the Acting Associate Director of

Pediatric and Rare Cancer Drug Development in the Oncology Center for Excellence(OCE). In this role, she participates in FDA working groups and committees and engages with external stakeholders to facilitate development of drugs to treat rare diseases, including rare cancers. Areas of special interest include the use of innovative clinical trial designs and rare world evidence to optimize drug development. Prior to joining FDA in 2009, Dr. Donoghue completed a fellowship in Pediatric Hematology and Oncology at the Children's National Medical Center after working for several years as a general pediatrician in private practice. She received her medical degree from Emory University and completed a residency in general pediatrics at the Georgetown University Medical Center.

Preston M. Dunnmon, MD, MBA, FACP, FACC, Cardiologist and former FDA Clinical Team Leader

Dr. Preston M. Dunnmon received his medical degree from the Duke University School of Medicine in 1980, following which he completed his internal medicine residency and cardiology fellowship at UT Southwestern Medical Center in Dallas. He was the recipient of an American Heart Association / Bugher Foundation Molecular Cardiology Fellowship, during which his research focus was G-protein mediated transmembrane signal transduction in the heart, and he reviewed manuscripts regarding G-proteins for Circulation. He is a Fellow of the American College of Physicians and a Fellow of the American College of Cardiology. After serving in the US Air Force as Division Chief of Cardiology at Andrews Air Force Base Medical Center and catheterization laboratory staff at both Walter Reed USA Medical Center and Bethesda National Naval Medical Center, Dr. Dunnmon transitioned to private industry where he was the Section Head of late phase clinical development for cardiovascular, GI, and GU development programs globally at Procter & Gamble Pharmaceuticals. In 2010, Dr. Dunnmon returned to government service, most recently as clinical team leader and cross-disciplinary team leader for the Division of Cardiology and Nephrology. Dr. Dunnmon has advanced training and expertise in the evaluation of nanoparticle-based therapeutics and served as an FDA member on multiple DARP and DTRA scientific advisory boards. Dr. Dunnmon is currently Vice President, Data Sciences, Cardiovascular-Metabolic and Pulmonary Hypertension at a large pharmaceutical company. He holds current board certifications in both internal medicine and cardiovascular diseases.

Lewis Fermaglich, MD, MHA, Medical Officer, Office of Orphan Products Development (OOPD), FDA

Dr. Lewis Fermaglich is a board-certified pediatrician is a Medical Officer in the Office of Orphan Products Development (OOPD). While in OOPD, he has been involved in orphan drug and rare pediatric disease (RPD) designations, acted as a Project Officer for several Orphan Products Clinical Trials grants, and is conducting research on trends in orphan designated diseases and drugs since the enactment of the Orphan Drug Act. He received his undergraduate degree at Wesleyan University and then an MD from the University of Kentucky College of Medicine. He completed his pediatric residency at Children's National Medical Center in Washington, DC, where he was Chief Resident. After residency, he was a practicing general pediatrician for 10 years – first as a military physician, and then in private practice in Rockville, MD. Lewis came to FDA in 2017, originally assigned to the Division of Clinical Review (DCR) in the Office of Generic Drugs (OGD) as a Medical Officer.

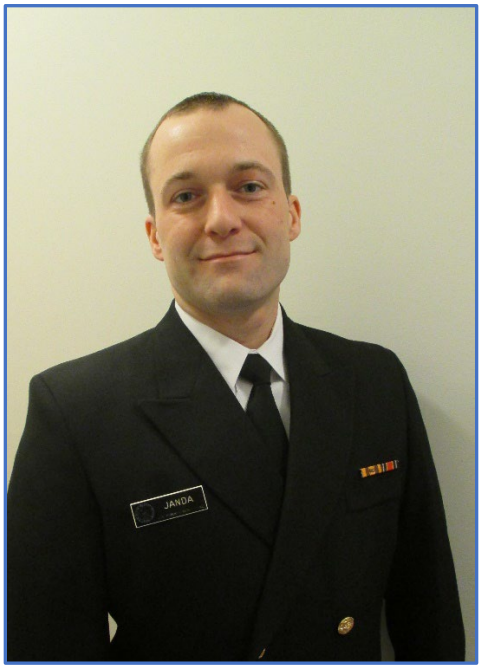
Elizabeth Hart, MD, Branch Chief, Office of Tissues and Advanced Therapies (OTAT), Center for Biologics Evaluation and Research (CBER), FDA

Dr. Elizabeth Hart is the Branch Chief of General Medicine Branch 1 in the Office of Tissue and Advanced Therapies (OTAT) in the Center for Biologics Evaluation and Research (CBER) at the Food and Drug Administration (FDA). Dr. Hart completed her undergraduate medical training at the University of Pennsylvania, a residency in pediatrics at Rainbow Babies and Children's Hospital, and a fellowship in pediatric endocrinology at Boston Children's Hospital. She provided clinical care, taught medical students and residents and conducted clinical and translational research prior to joining the FDA in 2014. At FDA, she initially served as a medical officer in the Division of Gastroenterology and Inborn Errors Products where she developed expertise in innovative clinical trial design and analysis for rare pediatric diseases. She joined OTAT in 2019 and currently supervises a team of medical officers responsible for regulating cellular and gene therapies for a variety of medical conditions, including serious and life-threatening rare diseases. Dr. Hart has served on multiple committees within CDER, CBER, and as part of FDA-NIH collaborations. She has conducted patient outreach, including participating in Patient Focused Drug Development meetings and Patient Listening Sessions. She has authored and edited multiple FDA Guidances, book chapters, and organized and

presented at Advisory Committee Meetings, FDA hosted meetings and workshops, and spoken at national and international scientific and regulatory conferences.

Kristen Hsu, Executive Director of Clinical Research, Amyloidosis Research Consortium

As Executive Director of Research, Kristen oversees ARC's research efforts through a multi-stakeholder collaborative consortia model. Kristen leads ARC's public private partnership with the FDA, the Amyloidosis Forum, which is dedicated to identifying and bridging scientific gaps that are acting as barriers to drug discovery and development for the treatment of amyloidosis. Kristen brings over ten years of clinical operations experience within the pharmaceutical industry, having designed and executed complex global trials for novel treatments of rare diseases.



CDR Michel Janda, MS, Lead reviewer and Biomedical Engineer, Center for Devices and Radiological Health (CDRH), FDA

CDR Michel Janda is a biomedical engineer that joined the FDA in 2004. As a Public Health Service (PHS) officer he has deployed in support of various national public health needs including most recently the ongoing COVID pandemic. He specializes in the review of stereotaxic systems, computerized medical systems, augmented reality systems, bone growth stimulators, and patient matched guides. He serves as a focal point for the electromagnetic compatibility group and the human factors / usability group. He participates within the safety and performance pathway working group, stereotaxic and robotics technical group, and software pre-cert development advisory group. He also is the primary liaison to ASTM F04.38 Computer Assisted Orthopedic Surgical Systems. Prior to the FDA, he modeled medical linear accelerators within cancer treatment planning software. He received his master's degree in Applied Biomedical Engineering from Johns Hopkins University and bachelor's degrees in Biomedical Engineering and Economics from Washington University in St. Louis.

Naomi Knoble, PhD, Reviewer, Division of Clinical Outcome Assessment, Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER), FDA

Dr. Naomi Knoble is a reviewer in the Division of Clinical Outcome Assessment (DCOA), Office of New Drugs (OND), Center for Drug Evaluation Research (CDER), with the US Food and Drug Administration (FDA) focused on reviewing pediatric rare disease programs. Dr. Knoble is a clinical outcome assessment measurement expert and psychologist specializing in pediatric neuropsychology. She has a MEd in Couples and Family Therapy, MS and PhD in Counseling Psychology from the University of Oregon. Her clinical training included Autism and neurodevelopmental disorders with the Oregon Health & Science University (OHSU) and pediatric neuropsychology with the University of Minnesota Medical School including rare diseases (i.e., MPS disorders), pediatric oncology, organ transplantation, fetal alcohol spectrum disorder, and other neurodevelopmental conditions. Prior to joining FDA, Dr. Knoble was a research scientist and global healthcare consultant specializing in patient-centered outcomes and measurement for Evidera (a subsidiary of PPD) and Mapi (now ICON). She has served as principal investigator of multiple global research studies in COA endpoint development and post-marketing evidence generation across a wide range of indications. She serves as FDA's liaison to C-Path's newly launched Rare Disease Clinical Outcome Assessment Consortium. Dr. Knoble's current regulatory research evaluates score development and interpretation of neurodevelopmental tests for children with disabilities.

Kerry Jo Lee, MD, Associate Director for Rare Diseases, Division of Rare Diseases and Medical Genetics, Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER), FDA

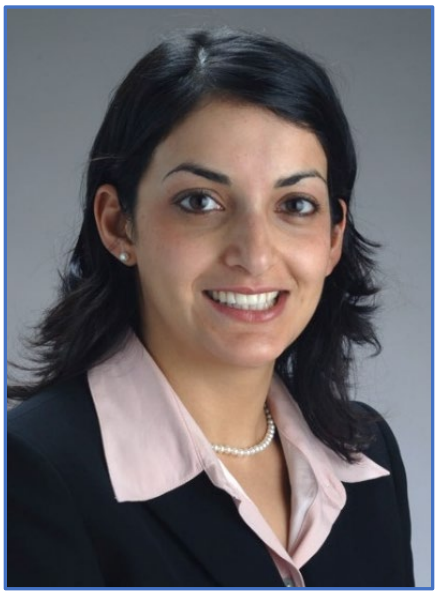
Dr. Kerry Jo Lee is the Associate Director for Rare Diseases in the Division of Rare Diseases and Medical Genetics, Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER). In this role she leads the Rare Diseases Team, a multidisciplinary rare disease programming and policy team that works to promote their mission to facilitate, support, and accelerate the development of drugs and therapeutic biologics for rare diseases. 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. She is 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.

Isabelle Lousada, Founder and CEO, Amyloidosis Research Consortium

Isabelle Lousada has been the driving force behind ARC, building successful collaborations and programs across the sectors to advance the science and understanding of the amyloidosis diseases. Isabelle was diagnosed with AL amyloidosis, and was one of the first patients to successfully undergo a stem cell transplant. For the past twenty years she has been committed to empowering other patients while serving on several boards and committees, speaking at leadership meetings and key events to encourage research, increase access, and support the critical and unmet needs of amyloidosis patients.

Mathew Maurer, MD, Professor of Medicine, Arnold and Arlene Goldstein Professor of Cardiology, Columbia University Irving Medical Center

Dr. Mathew S. Maurer is Director of the Cardiac Amyloidosis Program at New York-Presbyterian Hospital/Columbia University Irving Medical Center. Dr. Maurer is a Professor of Medicine at Columbia University, College of Physicians and Surgeons, where he Directs the Clinical Cardiovascular Research Laboratory for the Elderly (CCRLE). Dr. Maurer is a member of the Advanced Cardiac Care Center at New York-Presbyterian/Columbia. He has extensive expertise in amyloidosis and has served as a co-chair of the steering committee for the ATTR-ACT trial and co-leads the SCAN-MP (Screening for Cardiac Amyloidosis with Nuclear Imaging in Minority Populations) study funded by NHBLI.



Caroline Moazzam, MD, Medical Officer, Center for Devices and Radiological Health (CDRH), FDA

Dr. Caroline Moazzam is an orthopedic surgeon and a Medical Officer at FDA in the Extracolumnar Spinal Devices Team, Office of Orthopedic Devices. As a Medical Officer, Dr. Moazzam reviews orthopedic implants from development through post-marketing performance in an ongoing effort to protect and promote public health. Dr. Moazzam graduated magna cum laude with undergraduate dual degrees in biology and philosophy from Washington University in St Louis. She subsequently earned an MD from Saint Louis University, completed orthopedic surgery residency at Kansas University and completed fellowship at Johns Hopkins University. Prior to joining the Agency 5 years ago, Dr. Moazzam was in clinical practice in Northern Virginia.

Sandra Retzky, DO, JD, MPH, Director, Office of Orphan Product Development (OOPD), FDA

Dr. Sandra "Sandy" Retzky joined the Agency in 2016 and worked in the Center for Tobacco Products as a Medical Reviewer on applications for marketing authority of tobacco products. In 2019, Sandy became a CBER Medical Reviewer and spent several years managing many gene and cell therapy files to treat rare diseases. Sandy initially trained as a pharmacist, receiving her degree from the University of Illinois College of Pharmacy. She graduated from Midwestern University, an osteopathic medical school in Chicago. Sandy's medical credentials include board certification in Obstetrics and Gynecology, fellowship training in Urogynecology, and licensure to practice medicine in Delaware. After practicing medicine for many years, Sandy received an MBA degree from the Wharton School at the University of Pennsylvania and worked in the pharmaceutical and biotech industries for more than a decade evaluating the commercial and clinical potential of externally sourced new medicines and negotiating licensing rights to these assets. During part of this time, she continued to see patients on a pro bono basis at Baylor Women's Correctional Institution in Wilmington, Delaware. In 2010, Sandy transitioned to a career in public health. To make the change, she obtained a Master of Public Health degree from Johns Hopkins Bloomberg School of Public Health in 2011, where she retains a faculty position, and a J.D. degree from the Delaware Law School at Widener University in 2014. Sandy is admitted to practice law in Maryland and New Jersey.

Donna R. Rivera, PharmD, MSc, Associate Director for Pharmacoepidemiology, Oncology Center of Excellence (OCE), FDA

Dr. Donna R. Rivera 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. She is a Scientific Executive Committee member for the COVID-19 and Cancer Consortium (CCC19) and leads Project Post COVIDity, a collaborative RWD effort to assess longitudinal sequalae, outcomes, safety, vaccination, and immunity. 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. She also has previous experience in clinical trials from Stiefel, a GlaxoSmithKline company. Dr. Rivera earned her Doctor of Pharmacy and Master of Science in Pharmaceutical Sciences with a concentration in Pharmaceutical Outcomes and Policy from the University of Florida College of Pharmacy. She completed a postdoctoral fellowship in Pharmacoepidemiology and Pharmacogenomics at the NCI in the Epidemiology and Genomics Research Program.

Aviva Rosenberg, JD, Gaucher Community Alliance

Aviva Rosenberg, JD is the co-founder and co-president of GCA. She is a health care attorney based in Pennsylvania and taught as an adjunct professor at several universities. Aviva was diagnosed with Gaucher disease at age 27, following 20 years of symptoms with no answers. Her son, Eli, also has Type I Gaucher disease. Aviva's passion is educating about genetic diseases and screenings and talking about her personal journey with Gaucher disease, including the need to shorten patients' diagnostic results. After years of dealing with insurance appeals and learning patients' struggles with access and coverage, she was motivated to found Gaucher Community Alliance so that patients can share knowledge and experience to help others. She lives with her husband and three fabulous boys in Pittsburgh, Pennsylvania.

Teresa Rubio, PharmD, Health Science Administrator, Office of Orphan Products Development (OOPD), FDA

Dr. Teresa Rubio is a Health Science Administrator within the Office of Orphan Products Development (OOPD). Before joining OOPD, Teresa worked as a Health Programs Coordinator with the FDA Office of Health and Constituent Affairs, as a senior safety evaluator at CDER Office of Surveillance and Epidemiology, Division of Pharmacovigilance. She has also worked at the American Society of Health-System Pharmacists (ASHP), as a Pharmacy Clinical Coordinator at Marymount Hospital (Cleveland Clinic) and on the pharmacy staff in inpatient and outpatient capacities at the Veterans Affairs Medical Center. Teresa has a B.A. Chemistry from the University of Virginia, a Pharm.D. from the University of Maryland, and completed an ASHP-accredited residency in geriatrics at Veterans Affairs Medical Center in Cleveland.

David L. Scott, MD, PhD, DVM, Medical Officer, Center for Devices and Radiological Health (CDRH), FDA

Dr. David L. Scott received his MD from Yale University and completed an Internal Medicine residency at the Massachusetts General Hospital / Harvard Medical School. After serving as a Clinical Research Fellow in Joint Replacement at the UCLA-affiliated Los Angeles Orthopedic Hospital, he graduated from the Harvard Combined Orthopaedic Residency Program and later returned to Yale as a Fellow in Spine Surgery where he trained in both traditional and minimally invasive methods. David was a member of the Massachusetts General Hospital / Harvard Faculty for over 15 years with medical and surgical experience acquired as a clinician for the Harvard University Health Services and in private practice settings. He received his PhD from the University of Chicago (molecular biology/biological physics) as well as a doctorate in Veterinary Medicine from Cornell while a NIH pre-doctoral trainee. He has undertaken post-doctoral training at Yale, Harvard, and at the Welcome Research Laboratories in the United Kingdom. David is a strong proponent of the One Health Initiative and has a particular interest in comparative orthopedics and oncology.

James Signorovitch, PhD , Managing Principal, The Analysis Group, Inc.

Dr. James Signorovitch advises life sciences organizations on research strategies that bring effective therapeutics to patients through early-phase development, regulatory evaluations, economic appraisals, and real-world monitoring of outcomes. He chairs the statistical working group of the Amyloidosis Forum, and brings experience from treatment evaluations and multistakeholder collaborations across numerous rare diseases.

Wendy Slavit, MPH, CHES, Health Programs Coordinator and Health Scientist, Office of Patient Affairs, Office of Clinical Policy and Programs, FDA

Wendy Slavit is a Health Programs Coordinator and Health Scientist in the Office of Patient Affairs (formerly Patient Affairs Staff), within the Office of Clinical Policy and Programs, Office of the Commissioner. In her role, she collaborates with patient communities, the FDA medical product Centers and other offices to incorporate patient and caregiver perspectives into FDA's work. Ms. Slavit leads and manages FDA's Patient Engagement Collaborative (PEC). The PEC is a small group of patient organizations and individual representatives who discuss how to achieve more meaningful patient engagement in medical product development and other regulatory discussions at the FDA. She also focuses on health education, plain language, and health literacy through communication initiatives like the "For Patients" website and the "Patients Matter" video series. She has over fifteen years of experience in health behavior and health education. Ms. Slavit is passionate about translating science, health research, and policy into easy to comprehend information for patients, caregivers, and the public. She earned a Master of Public Health (MPH) from Emory University with a specialty in Behavioral Sciences and Health Education and a BA from Tufts University in Psychology. Ms. Slavit is also a Certified Health Education Specialist (CHES).

Elizabeth Spehalski, PhD, Pharmacologist, Division of Hematology Oncology Toxicology, Office of Oncologic Diseases (OOD), Office of New Drugs (OND), Center for Drug Evaluation and Research (CDER), FDA

Dr. Elizabeth Spehalski is a nonclinical reviewer in the Division of Hematology and Oncology Toxicology (DHOT) in the Office of Oncologic Diseases (OOD). Dr. Spehalski has extensive experience with basic research in molecular and cellular biology and animal models, which she uses to determine the safety and efficacy of drugs or biologics being considered for clinical trials. Prior to joining the FDA, Dr. Spehalski worked at the NCI, researching ways to target tumor-specific metabolism to increase the radiosensitivity of glioblastoma. Dr. Spehalski earned her undergraduate degree at Villanova University and her Ph.D. at the University of Michigan.

Michelle Tarver, MD, PhD, Deputy Director, Office of Strategic Partnerships and Technology Innovation, Program Director for Patient Science, Digital Health Center of Excellence, Center for Devices and Radiological Health (CDRH), FDA

Dr. Michelle Tarver is the Deputy Director of the Office of Strategic Partnerships and Technology Innovation where she provides co-leadership for all scientific collaborative and emerging technology-related activities at the Center for Devices and Radiological Health (CDRH). This leadership and oversight at CDRH include but are not limited to public health emergency preparedness and response activities, digital health, cybersecurity, conformity standards development and implementation, partnerships, and patient science and engagement. Under her leadership, CDRH is advancing efforts to include underserved populations in the evaluation of medical devices, including people living with rare diseases and with diverse age, gender racial, and ethnic backgrounds. Dr. Tarver received a B.S. in Biochemistry from Spelman College in Atlanta, GA and completed the M.D./Ph.D. program at The Johns Hopkins University School of Medicine and Bloomberg School of Public Health. Following her internal medicine internship, she completed a residency in ophthalmology with fellowship training in ocular inflammation (uveitis) both at the Wilmer Eye Institute (Johns Hopkins). Board-certified in ophthalmology with an epidemiology doctorate, she has worked on laboratory-based and epidemiological studies, clinical trials, registries, developing patient-reported outcome measures as well as surveys to capture patient preferences. As a dedicated clinician, she continues to care for patients with uveitis at Solomon Eye Associates in Bowie, MD.

Weida Tong, PhD, Director, Division of Bioinformatics and Biostatistics, National Center for Toxicological Research (NCTR), FDA

Dr. Weida Tong is Director of the Division of Bioinformatics and Biostatistics at FDA's National Center for Toxicological Research (NCTR). His primary research interest is to apply bioinformatics, Artificial Intelligence (AI), molecular modeling and data analytics for biomarker discovery, drug safety and repurposing, pharmacogenomics, toxicogenomics, and precision medicine. "Repurposing", also known as "drug repositioning", is finding uses for old drugs to treat new diseases—like rare diseases. Currently, Dr. Tong directs five FDA mission-critical projects at NCTR: 1) Developing machine learning and AI for digital health and drug repositioning; 2) Supervising and leading the FDA-led community wide MicroArray and Sequencing Quality Control consortium to analyze technical performance and utility of emerging genomics technologies with an emphasis on regulatory science and precision medicine; 3) Development of Liver Toxicity Knowledge Base to address drug safety concerns related to drug-induced liver injury; 4) Designing and developing computer-based technology to support FDA's effort on bioinformatics and scientific computing; and 5) Conducting molecular modeling and structure-activity relationships on various toxicological endpoints, such as endocrine disruption and carcinogenicity. Dr. Tong has published over 300 peer-reviewed papers and book chapters.

Celia Witten, MD, PhD, Deputy Director, Center for Biologics Evaluation and Research (CBER), FDA

Dr. Celia M. Witten is the Deputy Director of the Center for Biologics Evaluation and Research at the Food and Drug Administration (FDA/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). Before 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.

Janet Woodcock, MD, Principal Deputy Commissioner, FDA

Dr. Janet Woodcock began her long and distinguished FDA career in 1986 with the agency's Center for Biologics Evaluation and Research (CBER) as Director of the Division of Biological Investigational New Drugs. She also served as CBER's Acting Deputy Director, and later as Director of the Office of Therapeutics Research and Review. In 1994, Dr. Woodcock was named Director of the FDA's Center for Drug Evaluation and Research (CDER), overseeing the center's work that is the world's gold standard for drug approval and safety. In that position, she has led many of the FDA's groundbreaking drug initiatives. She has also served in other leadership roles at the FDA, including as Deputy Commissioner and Chief Medical Officer. With the onset of the COVID-19 public health emergency last year, Dr. Woodcock was asked to lend her expertise to "Operation Warp Speed" the initiative to develop therapeutics in response to the pandemic. Dr. Woodcock served as Acting Commissioner of Food and Drugs from January 2021 until February 2022. Dr. Woodcock has received numerous honors during her distinguished public health career, including: a Lifetime Achievement Award in 2015 from the Institute for Safe Medication Practices; the Ellen V. Sigal Advocacy Leadership Award in 2016 from Friends of Cancer Research; the Florence Kelley Consumer Leadership Award in 2017 from the National Consumers League; the 2019 Biotechnology Heritage Award from the Biotechnology Innovation Organization and Science History Institute; and the 2020 Lifetime Achievement Award from NORD. She is also an avid and accomplished gardener.

Marc Yale, International Pemphigus and Pemphigoid Foundation (IPPF) Advocacy & Research Coordinator

Marc Yale was diagnosed in 2007 with Cicatricial Pemphigoid, a rare autoimmune blistering skin disease. Like others with a rare disease, he experienced delays in diagnosis and difficulty finding a knowledgeable physician. Eventually, Marc lost the vision in his left eye from the disease. This inspired him to help others with the disease. In 2008, he joined the International Pemphigus and Pemphigoid Foundation (IPPF) as a Peer Health Coach. He worked with people to improve their quality of life and encouraged them to become self-advocates. In 2009, he helped develop the Pemphigus and Pemphigoid Comprehensive Disease Profile giving experts insight into the patient perspective. In 2016, Marc became the Executive Director of the IPPF. He has recently become the Advocacy & Research Coordinator of the IPPF so that he can focus on research and advocate for all of those affected by pemphigus and pemphigoid. He is a member of the American Academy of Dermatology Drug Transparency Task Force, sits on the Executive Board of Directors for The International Alliance for Dermatological Patient Organizations as their President, serves on the Board of Directors of Haystack Project, and is a committee advisor for Rare Disease Legislative Advocates. Last year, Marc joined Rare Disease International in the establishment of the WHO Collaborative Global Network for Rare Diseases Panel of Experts to ensure a lasting impact on the lives of people living with a rare disease around the globe. Marc currently resides in Ventura, California with his wife of 32 years.