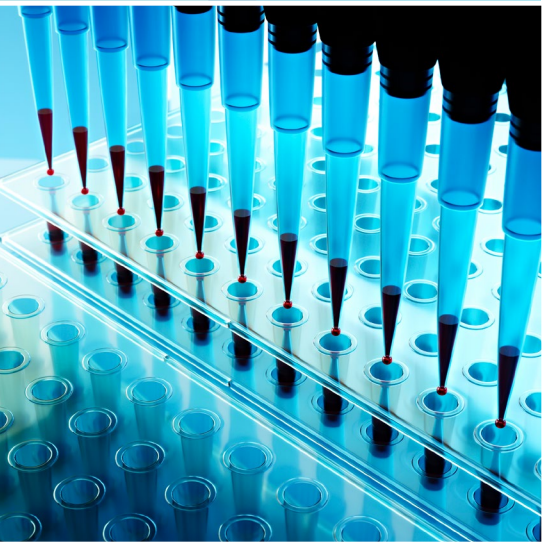
FDA's Rare Disease Day 2024



Virtual Public Meeting

March 1, 2024

Dedicated to patients and providers

U.S. FOOD & DRUG ADMINISTRATION

FDA's Rare Disease Day

Friday, March 1, 2024

Dedicated to patients and providers

Access the Webcast link:
https://youtube.com/live/j6H1jeqdm4I?feature=share

9:00 - 9:10 a.m. Opening remarks:

Robert M. Califf, M.D.
Commissioner of Food and Drugs

Sandra Retzky, D.O., J.D., M.P.H.
Director, Office of Orphan Products Development (OOPD)
Office of the Commissioner

9:10 - 9:15 a.m. Overview of FDA's Rare Disease Day 2024

Lewis Fermaglich, M.D.
Office of Orphan Products Development
Office of the Commissioner

Chanapa Tantibanchachai, M.S.
Press Officer, Office of Media Affairs
Office of the Commissioner

9:15 - 10:45 a.m. Panel 1: Clinical trial foundations and future directions

Session Overview:
FDA experts discuss the legal frameworks governing the use of investigational drugs, biological products, and devices in FDA-regulated research and the standards governing the review and marketing authorization of such products. We will address how FDA incorporates the patient voice in our regulatory decisions. This session will also discuss innovative methods to increase patient access and participation in clinical studies such as decentralized clinical trials (DCTs) and the importance of developing digital health technologies (DHTs) to facilitate them. In the future, we expect more clinical trials to become decentralized where some or all of the trial-related activities occur at locations other than traditional clinical trial sites. These trial-related activities may take place at the homes of trial participants or in local health care facilities that are convenient for trial participants helping to minimize travel burden, improve diversity in study populations, and facilitate research on rare diseases.

Moderators:

Kerry Jo Lee, M.D.
Associate Director for Rare Diseases
Rare Diseases Team, Division of Rare Diseases and Medical Genetics,
Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine
Center for Drug Evaluation and Research

James Myers, J.D.
Associate Director for Policy
Center for Biologics Evaluation and Research

1. ***The legal frameworks for drugs, biological products, and devices in the United States***
   Stefanie Kraus, J.D., M.P.H., Senior Regulatory Counsel in the Office of Regulatory Policy, Center for Drug Evaluation and Research

   Eli Tomar, J.D., M.P.H., Associate Director for Guidance, Legislation & Special Projects, Office of Policy
   Center for Devices and Radiological Health

2. ***What does FDA actually do during review? How do we use the patient voice during review?***
   Kevin Bugin, Ph.D., Deputy Director of Operations, Office of New Drugs, Center for Drug Evaluation and Research

3. ***Decentralization of clinical trials and the need for digital health technologies***
   Leonard Sacks, M.D., Associate Director for Clinical Methodology, Office of Medical Policy, Center for Drug Evaluation and Research

   Charles Viviano M.D., Ph.D., Chief Medical Officer, Office of Clinical Evidence and Analysis, Center for Devices and Radiological Health

4. ***Project Pragmatica—exploring ways to promote efficient, patient-centric drug development for patients with rare cancers***
   Martha Donoghue, M.D., Associate Director for Pediatric Oncology and Rare Cancers, Oncology Center of Excellence, Office of the Commissioner
   Acting Associate Director for Pediatric Oncology in the Office of Oncologic Diseases
   Center for Drug Evaluation and Research

5. ***Q & A***

10:45 - 11:00 a.m. BREAK 1

11:00 a.m. - 12:30 p.m. Panel 2: Navigating information. Empowering patients and providers.

Session Overview:
FDA experts discuss how to find information related to FDA-approved drugs, information on clinical trials, and how to understand informed consent documents for clinical trials.

Moderator: CDR Ryan Presto, Pharm.D., M.B.A.,
Regulatory Officer, Office of Regulatory Operations
Center for Biologics Evaluation and Research

1. ***Labeling for prescription medicines***
   Eric Brodsky, M.D.
   Associate Director of the Labeling Policy Team, Office of New Drugs,
   Center for Drug Evaluation and Research

2. ***Finding important information related to drug development and clinical trials***
   Salvatore Pepe, Pharm.D., M.S.
   Program Coordinator
   Knowledge Management Team, Office of Translational Sciences
   Center for Drug Evaluation and Research

3. ***Understanding informed consent for investigational products***
   Jose Pablo Morales, M.D.
   Senior Medical Advisor and Ethicist
   Office of Clinical Policy
   Office of the Commissioner

   Suzanne Pattee, J.D.
   Regulatory Counsel
   Office of Clinical Policy
   Office of the Commissioner

4. ***Q & A***

12:30 - 1:30 p.m. LUNCH BREAK

1:30 - 3:00 p.m. Panel 3: Patient Engagement at FDA

Session Overview:
Patients provide a unique perspective about their health conditions and are an important part of FDA's public health mission. Through our patient engagement programs and activities, we listen closely to patients and caregivers to help inform medical product development, regulatory decision making, clinical trial design, and patient preferences. In this session, members of a patient committee will discuss their experiences on an FDA-sponsored study to adapt a clinical outcome assessment tool traditionally conducted in the clinic to one for remote-use at home. Having endpoints that can be assessed remotely may decrease travel burden and expense for patients and their families and help remove obstacles for subject recruitment and retention that often lead to underrepresentation of certain groups of otherwise eligible subjects in clinical trials. This panel also includes discussion of patient engagement in public private partnerships and ways patients and care partners can participate and advance clinical research for rare diseases including regenerative medicine, such as gene and cell therapies.

Moderator: Wendy Slavit, M.P.H.
Senior Health Scientist
Patient Affairs Staff
Office of the Commissioner

1. ***Patient Affairs Staff overview of FDA initiatives***
   Ashley Channels, Pharm.D.
   Program Coordinator
   Patient Affairs Staff
   Office of the Commissioner

2. ***Patient experience examples***

   a. Patient committee participants on the FDA-sponsored study to adapt an in-clinic outcome assessment tool for ALS to remote-use at home
   Bruce Virgo
   Angélique van der Lit-van Veldhuizen
   Ashley Lee
   Carla DeMuro, M.S., Vice President, Patient-Centered Outcomes Assessment, RTI Health Solutions

   b. Lysosomal Diseases Consortium—a public private partnership
   Kanwaljit Singh, M.D. M.P.H., Executive Director, International Neonatal Consortium, Critical Path for Lysosomal Diseases, and Critical Path for Alpha-1 Antitrypsin Deficiency
   Director of Pediatric Programs
   Critical Path Institute

   c. RegenMed Ed—Empowering patients and advocates to advance rare disease research
   Anne Rowzee, Ph.D., Associate Director for Policy, Office of Tissues and Products
   Center for Biologics Evaluation and Research

3. ***Q & A***

3:00 - 3:30 p.m. BREAK 2

3:30 - 4:30 p.m. Panel 4: FDA initiatives to advance medical product development for rare diseases

Moderator: Hilary Marston M.D., M.P.H., Chief Medical Officer, FDA

Session Overview:
Experts from each FDA center will spotlight initiatives aimed to improve product development for rare diseases.

1. ***CDER: Update on CDER engagement initiatives for rare diseases***
   Andrea Bell-Vlasov, Ph.D., Science Policy Analyst
   Rare Diseases Team
   Division of Rare Diseases and Medical Genetics
   Office of Rare Diseases, Pediatrics, Urologic, and Reproductive Medicine, Office of New Drugs
   Center for Drug Evaluation and Research

2. ***CDRH: Patient perspectives on home use devices for people with rare diseases***
   Tracy Gray, M.B.A., R.N., M.S., Health Scientist
   Patient Engagement Lead, Patient Science & Engagement
   Center for Devices and Radiological Health

3. ***OCE: Update on Project Catalyst***
   Jeff Summers, M.D., Associate Director for Translational Sciences,
   Office of Oncologic Diseases
   Center for Drug Evaluation and Research

4. ***CBER: Support for clinical trials Advancing Rare disease Therapeutics (START) Pilot Program***
   Wei Liang, Ph.D., Chief, Regulatory Operations Staff
   Office of Therapeutic Products
   Center for Biologics Evaluation and Research

5. ***NCTR: Chimeric Antigen Receptor (CAR)-based therapies: A new vision in treating rare diseases***
   Kelly Mercer, Ph.D., Staff Fellow
   Division of Systems Biology
   National Center for Toxicologic Research

6. ***Q & A***

Speaker and Moderator Biographies (in order of appearance)

Robert M. Califf, M.D.
Commissioner of Food and Drugs

Dr. Robert M. Califf was confirmed 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.

Sandra Retzky, D.O., J.D., M.P.H.

Director, Office of Orphan Products Development (OOPD)
Office of the Commissioner

Dr. Sandra "Sandy" Retzky is the Director of the Office of Orphan Products Development (OOPD) at FDA. Sandy 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 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. She is a Fellow in the American College of Legal Medicine.

Lewis Fermaglich, M.D., M.H.A.

Office of Orphan Products Development,
Office of the Commissioner

Dr. Lewis Fermaglich is a board-certified pediatrician and currently a Medical Officer in the Office of Orphan Products Development (OOPD). In OOPD, he works on orphan drug and rare pediatric disease (RPD) designations, acts 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.

Chanapa Tantibanchachai, M.S.

Press Officer, Office of Media Affairs
Office of the Commissioner

For the past three and a half years, Chanapa has served as a Press Officer for the FDA's Office of Media Affairs, where she helps ensure the FDA remains a trusted authority on public health decisions grounded in data and science by providing timely, accurate and understandable information about the FDA to the media. Prior to joining the FDA, Chanapa worked in the media relations offices for Johns Hopkins Medicine, Johns Hopkins University and the University of Utah, where she wrote about everything from anesthesiology to astrophysics and helped coordinate dozens of news crews in operating rooms. She received her Bachelor and Master of Science degrees in biology from Arizona State University.

At 8 months old, Chanapa was diagnosed with beta thalassemia, a rare blood disorder that requires a lifetime of blood transfusions and medication. Her rare disease experience makes her passionate about equitable access to medical care, new drug forms that can improve patients' quality of life and ways to reduce administrative health care burdens.

Kerry Jo Lee, M.D.

Associate Director for Rare Diseases
Rare Diseases Team, Division of Rare Diseases and Medical Genetics, Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine
Center for Drug Evaluation and Research

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 serves as the Program Management Office for the CDER Accelerating Rare disease Cures Program (ARC) and 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.

James Myers, J.D.

Associate Director for Policy
Center for Biologics Evaluation and Research

James Myers is currently the Associate Director for Policy in the Center for Biologics Evaluation and Research (CBER). In this role, he serves as a principal policy advisor to the CBER Center Director and leads CBER's activities on developing and implementing policies and programs for CBER-regulated products, including working with stakeholders across CBER to support the center's rare disease program. Prior to his current role, James served as the Director of the Division of Regulatory Policy, Office of New Drug Policy, in CDER's Office of New Drugs (OND). He also previously served as a regulatory counsel within CDER's Office of Generic Drugs. Prior to joining FDA in 2014, James worked as an associate at Covington & Burling LLP. James received his BA from Wabash College, MA from Boston College, and JD from Boston College Law School.

Stefanie Kraus, J.D., M.P.H.

Senior Regulatory Counsel in the Office of Regulatory Policy
Center for Drug Evaluation and Research

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

Eli Tomar, J.D., M.P.H.
Associate Director for Guidance, Legislation & Special Projects, Office of Policy
Center for Devices and Radiological Health

Eli Tomar is Associate Director for Guidance, Legislation & Special Projects in the Office of Policy in FDA's Center for Devices and Radiological Health (CDRH). At CDRH, he manages the guidance program, supervises a team of professionals engaged in the development of cross-cutting guidance and policy documents, and oversees the Center's legislative agenda. He also advises the Center on complex regulatory, legal and compliance matters. Prior to joining CDRH in 2020, Mr. Tomar was Counsel in the Washington office of Akin Gump LLP, where he worked on a range of regulatory and policy matters spanning the healthcare and life sciences sectors. Throughout his career in the private sector, he practiced law at several international law firms. Mr. Tomar received a bachelor's degree in political science from the University of Maryland, College Park, and his JD and MPH from the University of California, Los Angeles (UCLA), and is now surprised to find these two schools in the same athletic conference.

Kevin Bugin, Ph.D.
Deputy Director of Operations, Office of New Drugs
Center for Drug Evaluation and Research

Kevin Bugin currently serves as the Deputy Director of Operations in the Office of New Drugs (OND) in FDA's Center for Drug Evaluation and Research (CDER). In this role, Dr. Bugin plays a key leadership role in the management of CDER's evaluation of new drugs and ensures their safety and effectiveness. Prior to his current role, Dr. Bugin served as the Chief of Staff for the Therapeutics Response Efforts as part of the US Government's HHS and DOD operation formerly known as Operation Warp Speed. His leadership during this time played an important role in the successful development and distribution of COVID-19 therapeutics. He is also an adjunct faculty member at the George Washington University in the Clinical Leadership Program, where he focuses on areas of clinical research and medicines development, and the science of team science.

Leonard Sacks, M.D.
Associate Director for Clinical Methodology
Office of Medical Policy
Center for Drug Evaluation and Research

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

Charles Viviano M.D., Ph.D.

Chief Medical Officer, Office of Clinical Evidence and Analysis
Center for Devices and Radiological Health

Dr. Viviano is the Chief Medical Officer in the Office of Clinical Evidence and Analysis in the Center for Devices and Radiological Health at FDA. He previously served as the Clinical Deputy Office Director in the Office of GastroRenal, ObGyn, General Hospital, and Urology Devices (OHT3). Upon joining the FDA in 2015, he was the sole Urology medical officer in CDRH, responsible for the clinical review of all urologic devices, including the first submissions for high intensity focused ultrasound devices for prostate ablation. He was on the NESTcc Data Quality subcommittee and the SPARED (prostate ablation) registry effort. He is also the principal investigator for an FDA-led project identifying patient preferences in prostate cancer treatment.

In OHT3, he was involved in the regulatory review of urologic devices across the total product lifecycle (TPLC), in addition to serving as the senior medical officer participating in challenging gastroenterological, OB-GYN, renal, and general hospital device product reviews. In OCEA, he is primarily involved in clinical study policy, including utilization of real world data across the total product lifecycle.

Prior to joining the FDA, he was an Assistant Professor in the Division of Urology at Duke University where his practice focused on General Urology and Men's Health. Prior to Duke, he was in private practice in Connecticut. He received his medical education at the University of Connecticut and his PhD in Toxicology from the University of North Carolina at Chapel Hill.

Martha Donoghue, M.D.

Associate Director for Pediatric Oncology and Rare Cancers, Oncology Center of Excellence, Office of the Commissioner
Acting Associate Director for Pediatric Oncology in the Office of Oncologic Diseases
Center for Drug Evaluation and Research

Martha Donoghue, MD is a board-certified pediatrician and pediatric hematologist/oncologist. She is the Associate Director for Pediatric Oncology and Rare Cancers in the FDA's Oncology Center of Excellence, Office of the Commissioner and the Acting Associate Director for Pediatric Oncology in the Office of Oncologic Diseases, Center for Drug Evaluation and Research (CDER). In these roles, she oversees the implementation of pediatric regulations designed to facilitate the timely investigation of drugs and biological products for pediatric patients with cancer, supports and promotes consistency of regulatory work relating to pediatric oncology drug development across CDER and the Center for Biologics Evaluation and Research (CBER), and works with stakeholders to address challenges and foster development of drugs to treat pediatric and other rare cancers. Areas of special interest include the use of innovative clinical trial designs and use of real world data to optimize drug development for rare cancers. 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.

CDR Ryan Presto, Pharm.D., M.B.A.

Regulatory Officer, Office of Regulatory Operations
Center for Biologics Evaluation and Research

CDR Ryan Presto has been with CBER since 2019, where he leads the Center's Change Management effort, and the Regulatory Project Management and Artificial Intelligence Seminar Series. He came from the CDER Office of Generic Drugs where he was for 7 years serving in various roles within the Division of Project Management including as a Regulatory Project Manager Team Leader. He has experience leading process development for Center policies & procedures, and the creation of IT platforms.

CDR Presto holds a Doctor of Pharmacy degree from the University of Rhode Island and completed his Master of Business Administration with a focus in Healthcare Administration from the University of Massachusetts Amherst.

Eric Brodsky, M.D.

Associate Director of the Labeling Policy Team, Office of New Drugs
Center for Drug Evaluation and Research

As the Associate Director of the Labeling Policy Team in the Office of New Drugs in the Center for Drug Evaluation and Research at the FDA, Dr. Eric Brodsky oversees OND's implementation of the U.S. Prescribing Information (USPI) regulations and guidances to help promote consistency in labeling practices across CDER; develops labeling resources for CDER staff and industry; provides oversight of labeling quality; provides labeling review training; and assists review teams in review and development of the USPI. Prior to joining the FDA, Dr. Brodsky practiced as an internist with a focus in primary care and hospital medicine in the Washington D.C. area. He received his medical degree from Tufts University School of Medicine, completed an internal medicine residency program at the University of Massachusetts Medical Center, and is board certified in Internal Medicine.

Salvatore Pepe, Pharm.D., M.S.

Program Coordinator
Knowledge Management Team, Office of Translational Sciences
Center for Drug Evaluation and Research

Salvatore Pepe graduated from Northeastern University in Boston, MA in 2010. Subsequently, he attended the University of Florida through the CDER Academic Collaboration Program with the USPHS Commissioned Corps. Upon graduating in 2012 with a MS in Pharmacoepidemiology, he joined the Rare Diseases Program in the Office of New Drugs at FDA. Using his healthcare and data science background, he took a keen interest in regulatory science informatics. In 2015, he joined the Knowledge Management Team in the Office of Translational Sciences, where he continues to use his experience with information systems to inform policy development and support research.

Jose Pablo Morales, M.D.

Senior Medical Advisor and Ethicist
Office of Clinical Policy
Office of the Commissioner

Jose Pablo Morales is a Senior Medical Advisor and Ethicist in the Office of Clinical Policy (OCLiP) within the Office of the Commissioner at FDA. Dr. Morales received his MD from Universidad Pontificia Bolivariana, Colombia, South America. He completed his clinical training in vascular and endovascular surgery at Guy's and St Thomas' Hospital in London, UK. In addition, he completed a 2-year endovascular research fellowship at the Cleveland Clinic, OH.

Dr. Morales joined the FDA in 2009 as a medical officer in CDRH Office of Cardiovascular Devices. He provided clinical and regulatory recommendations for over 1000 clinical consults. These consults included but are not limited to original and panel track PMAs, traditional 510(k), original IDEs, early feasibility studies (EFS), Humanitarian Use Device (HUD), Humanitarian Device Exemption (HDE), breakthrough designations, recall classification and shortages assessments. Dr. Morales later served as the Chief Medical Officer in CDRH Office of Clinical Evidence and Analysis. In addition to supporting CDRH review divisions with topics related to evidence generation and analysis, Dr. Morales also served as the CDRH Center-designee for the FDA IRB and CDRH liaison on several Agency-wide and external consortiums. Finally, under his current role as Senior Medical Advisor/Ethicist, Dr. Morales provides expert opinion and bio-ethical support in regulatory submissions to review divisions across the Agency on a wide variety of complex ethical issues identified during the review of clinical protocols and/or marketing applications. Dr. Morales also participates in several ongoing cross-Agency working groups to develop policies intended to promote and incentivize diversity and inclusion in clinical trials.

Suzanne Pattee, J.D., Regulatory Counsel
Office of Clinical Policy
Office of the Commissioner

Suzanne is a regulatory counsel with the office of Clinical Policy in the Office of the Commissioner. Prior to this position, she worked in the Center for Drug Evaluation and Research (CDER) with the office for Medical Policy and the Office of Policy for Pharmaceutical Quality.

Prior to joining FDA in 2009, Suzanne was a vice president at the Cystic Fibrosis Foundation where she led clinical trial policy initiatives, infection control policies, among others. She spearheaded bioethics issues and patient outreach for a biotechnology trade association. She served as a member of the Secretary's Advisory Committee for Human Research Protections, and as a board member for a research accreditation organization. Suzanne earned her law degree from The George Washington University, and her undergraduate science degree from The College of William and Mary.

Wendy Slavit, M.P.H.
Senior Health Scientist
Patient Affairs Staff
Office of the Commissioner

Wendy Slavit is a Senior Health Scientist in FDA's Patient Affairs, 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 an ongoing, shared setting in which the patient community (PEC members), the FDA, and the Clinical Trials Transformation Initiative discuss many topics for improving communication, education, and patient engagement related to medical product regulation. 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 almost twenty years of experience in public health, 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).

Ashley Channels, Pharm.D.
Program Coordinator
Patient Affairs Staff
Office of the Commissioner

LCDR Ashley Channels is a program coordinator in FDA's Patient Affairs Staff, Office of Clinical Policy and Programs, Office of the Commissioner where she helps organize cross-center patient listening sessions to help amplify the patient voice throughout the agency.

One of LCDR Channels' professional goals is to bring a more representative and equitable patient perspective into FDA's work through accessible patient communication and engagement. LCDR Channels is a member of FDA's Language Access Steering Committee and works with many underserved patient communities regularly in her work.

Prior to working at FDA, LCDR Channels worked in direct patient care for over 11 years with 6 years serving the Eastern Band of Cherokee Indians as a Clinical Pharmacist Practitioner with a focus on chronic disease management and public health initiatives.

LCDR Channels completed her undergraduate work at Texas A&M University followed by Midwestern State University. She completed her graduate degree at Howard University followed by a PGY-1 Residency at Cherokee Indian Hospital.

Bruce Virgo

Bruce Virgo is an Australian living in Edinburgh, Scotland. Married, with 3 adult children, he is a retired international maritime attorney (qualified in Australia, England/Wales & Scotland). Bruce was diagnosed with MND [Motor Neuron Disease]/ALS in late 2014. He was a partner in the London office of an English law firm, working until May 2016, before his condition forced him into sick leave at age 57. Since 2014, Bruce participates in various MND/ALS research projects within the UK and further afield. Additionally, he has been involved in fundraising in the UK, as well as raising disease awareness generally as a Patient Advocate.

He joined the PALS [people with ALS]/CALS [care partners of people with ALS] Advisory Council ("PCAC") of the International Alliance of ALS/MND Associations in March 2020. He has also been on the Patients & Public Advisory Group for the ground-breaking MND-SMART Platform Trial in the UK since November 2018.

Bruce was an International ALS/MND Symposium Patient Fellow in December 2018 and is now on the Selection Committee of the ongoing Patient Fellow Program. He was also the first non-scientific PALS Plenary Panel speaker at the 31st Int. ALS/MND Symposium held virtually in December 2020. In April 2023 he joined the Research Review Committee of UK charity - My Name'5 Doddie Foundation - as a PALS representative.

Angélique van der Lit-van Veldhuizen

Angélique van der Lit-van Veldhuizen was diagnosed with ALS in February 2018 and has been a volunteer for the patient advisory council of ALS Patients in the Netherlands since 2019. She also became a member of Patients and Carers Expert Board of the European Organization for Professionals and with ALS (Eupals) and the Associations PALS [people with ALS] and CALS [care partners of people with ALS] Council of the International Alliance of ALS/MND [Motor Neuron Disease]. To transfer knowledge and raise awareness about ALS, Angélique gives guest lectures and interviews to students. Her motto is "I sit still but my brain needs to be busy and challenged."

Angélique previously served as a manager in the townhall of the local authority of Woerden and Oudewater as well as a civil registrar for the solemnization of marriages. She stopped working in 2021 because of immobility and fatigue due to ALS.

Angélique resides in Montfoort near Utrecht, the Netherlands. She is married to her husband, Gerard, and has two children.

Ashley Lee

Ashley Lee is an active advocate for ALS legislation, research and quality of life. Ashley is obtaining her Ph.D. in psychology and intends to use her experience and education to work with ALS patients and families. She has a passion for improving the VA healthcare system to provide the necessary care and support for people with ALS and their families.

Ashley was 24 years old and a newly married mother to a four-year-old daughter in Florida in the fall of 2009 when her father was diagnosed with ALS. She was told to go home and get his affairs in order, as the average life expectancy is two to five years from symptom onset. Due to the significant delay in diagnosis, he had already been experiencing symptoms for well over two years.

Ashley's Dad was confined to a wheelchair full time by the summer of 2011, requiring assistance with all activities of daily living. In 2016, he went into complete respiratory failure and needed a tracheostomy and ventilator support to survive, subsequently requiring around-the-clock care and attention. Since then, Ashley has been her father's full time care partner.

Carla DeMuro, M.S.

Vice President, Patient-Centered Outcomes Assessment
RTI Health Solutions

Carla (DeMuro) Romano, MS, is a Vice President of Patient-Centered Outcomes Assessment at RTI International, RTI Health Solutions. Carla has extensive experience in the rigorous development and validation of clinical outcome assessment measures for use in clinical trials, as well as large scale epidemiologic and population-based studies. She joined RTI-HS in 2006 after 15 years of working in the pharmaceutical industry and brings her experience with drug development. She has specialized in both the qualitative and quantitative aspects of instrument design and has worked towards creating a clear path from measure development to interpretation. Carla has worked to developed measures across a wide span of therapeutic areas but has special interest and experience in CNS-related disease and has had the great privilege of working with persons living with ALS and their families to support bringing forward their voices in drug development. She is working closely with the FDA to develop and evaluate remote access versions of a widely used clinical outcome assessment in ALS as part of the Accelerating Access to Critical Therapies for Amyotrophic Lateral Sclerosis Act (ACT for ALS) initiative. Additionally, she reports being truly honored to facilitate the Patient Committee as part of this research.

Kanwaljit Singh, M.D., M.P.H.

Executive Director, International Neonatal Consortium, Critical Path for Lysosomal Diseases, and Critical Path for Alpha-1 Antitrypsin Deficiency
Director of Pediatric Programs
Critical Path Institute

Dr. Kanwaljit Singh is the Executive Director of Critical Path Institute's (C-Path) International Neonatal Consortium (INC) and Critical Path for Lysosomal Diseases (CPLD) program. In addition to these roles, he serves as the Director of Pediatric Programs at C-Path. Before joining C-Path, he worked at the University of Massachusetts Medical School (UMass) in Worcester, MA for over seven years as an Instructor of Pediatric Neurology. At UMass, Dr. Singh researched new treatments for Autism Spectrum Disorders (ASD) and pediatric epilepsy and published more than 40 papers on these topics. Before his time at UMass, he did ASD research at the Lurie Center for Autism, which is connected to Massachusetts General Hospital and Harvard Medical School in Boston. Besides his research, Dr. Singh was also a part of institutional review boards at Harvard Medical School.

Anne Rowzee, Ph.D.

Associate Director for Policy, Office of Tissues and Products
Center for Biologics Evaluation and Research

Anne M. Rowzee, Ph.D. joined the Center for Biologics Evaluation and Research, Office of Tissues and Advanced Therapies in October 2018 as an Associate Director for Policy. Dr. Rowzee supports OTAT's ongoing stakeholder engagement efforts and is building a proactive patient outreach program for the Office. Before moving to CBER, Anne was a Science Policy analyst in the Office of Therapeutic Biologics and Biosimilars in the Center for Drug Evaluation and Research where she provided scientific guidance to ensure consistency in agency recommendations across biosimilar development programs. Dr. Rowzee joined FDA in 2011, spearheading communications covering CDER regulatory and research programs for the Office of Communications. During her time in OCOMM, Dr. Rowzee wrote and directed manuscripts for high-visibility peer-reviewed journals and led the OCOMM Editorial Team.

Hilary Marston M.D., M.P.H.
Chief Medical Officer, FDA

Hilary Marston serves as the primary clinical advisor to the Commissioner and oversees the Office of Clinical Policy and Programs. She leads cross-cutting initiatives that support the FDA's centers in making effective, safe, and innovative medical products available for patients.

Dr. Marston previously served as the Senior Advisor for Global COVID-19 Response on the White House COVID-19 Response Team. Her previous roles also include Director for Medical Biopreparedness and Response at the U.S. National Security Council and Medical Officer and Policy Advisor for Pandemic Preparedness at the National Institute of Allergy and Infectious Diseases, National Institutes of Health. Dr. Marston also served in positions with McKinsey & Company and the Bill & Melinda Gates Foundation.

Dr. Marston trained in Internal Medicine and Global Health Equity at Brigham & Women's Hospital. She completed her M.P.H. at the Harvard T.H. Chan School of Public Health.

Andrea Bell-Vlasov, Ph.D.
Science Policy Analyst, Rare Disease Team, Division of Rare Diseases and Medical Genetics, Office of Rare Diseases, Pediatrics, Urologic, and Reproductive Medicine, Office of New Drugs
Center for Drug Evaluation and Research

Andrea Bell-Vlasov joined FDA in 2014 and obtained experience in In Vitro Diagnostics and Rare Diseases Policy. She currently works as a Science Policy Analyst on the Rare Diseases Team in CDER.

Andrea spent 8 years in the Center for Devices and Radiological Health where most of her work was done in the diabetes devices space; mainly for type 1 diabetes. Her time in CDRH provided significant experience in writing regulations, stakeholder outreach, clinical trial design, real world data and evidence, pre- and post-market challenges, and much more. Her work in diabetes earned her a Samuel J. Heyman Service to America Medal in 2017. Andrea currently works in Rare Diseases policy supporting guidance development efforts and leads the 'Learning and Education to Advance and Empower Rare Disease Drug Developers' or otherwise known as the LEADER3D initiative.

Andrea received her Ph.D. in Analytical Chemistry from the University of Michigan.

Tracy Gray, M.B.A., R.N., M.S.
Patient Engagement Lead, Patient Science & Engagement
Center for Devices and Radiological Health

Tracy Gray is the Patient Engagement Lead in FDA's Center for Devices and Radiological Health (CDRH), Office of Strategic Partnerships and Technology Innovation (OST), Patient Science and Engagement Program. She works in collaboration with internal and external stakeholders, to foster a culture of patient engagement in CDRH, through intentional, meaningful patient interactions that provide opportunities of mutual benefit to FDA, patient and caregiver communities. Before joining the FDA, Tracy held leadership positions in the Health Resources and Services Administration (HRSA), and in the non-profit health care sector. Tracy has extensive healthcare experience in the pharmaceutical industry, nonprofit organizations, and direct patient care.

Tracy earned a B.S. in Biological Sciences, from the University of Maryland, College Park, a M.B.A. from Marymount University, and a R.N. and M.S in Clinical Nurse Leadership from the University of Maryland School of Nursing, Baltimore, MD.

Jeff Summers, M.D.
Associate Director for Translational Sciences, Office of Oncologic Diseases
Center for Drug Evaluation and Research

Jeff Summers is a pediatric oncologist and serves as an Associate Office Director in the Office of Oncology Drug Products at the U.S. Food and Drug Administration. Dr. Summers earned his medical degree from the University of Washington School of Medicine. He was a postdoctoral fellow at Fred Hutchinson Cancer Research Center from 1990 to 1995. He completed fellowship training in Pediatric Hematology and Oncology in 2002 at the National Cancer Institute (NCI) in Bethesda, MD.

Wei Liang, Ph.D.
Chief, Regulatory Operations Staff, Office of Therapeutic Products
Center for Biologics Evaluation and Research

Wei Liang serves as the Regulatory Operations Staff Chief in the Office of Therapeutic Products (OTP), Center for Biologics Evaluation and Research (CBER) at the U.S. Food and Drug Administration. Dr. Liang earned her Ph.D. in Molecular Pharmacology at Peking Union Medical College in China. She was a postdoctoral fellow at the NIH from 1998-2002. She joined FDA in 2006 as a pharmacology/toxicology reviewer in CBER OTP (previously known as OTAT). Since 2019, Dr. Liang serves as the Senior Regulatory Advisor as OTAT from 2019 to 2023.

Kelly Mercer, Ph.D.
Staff Fellow, Division of Systems Biology
National Center for Toxicology Research

Kelly Mercer received her doctorate in Biochemistry and Molecular Biology from the University of Arkansas for Medical Sciences in 2001. She was a postdoctoral fellow at St. Jude's Children's Research Hospital from 2001-2003. Since she has conducted scientific research in the fields of cancer, toxicology, and nutrition at the University of Arkansas for Medical Sciences, the USDA-funded Arkansas Children's Nutrition Center, and currently works at the National Center for Toxicological Research (NCTR) within the FDA.