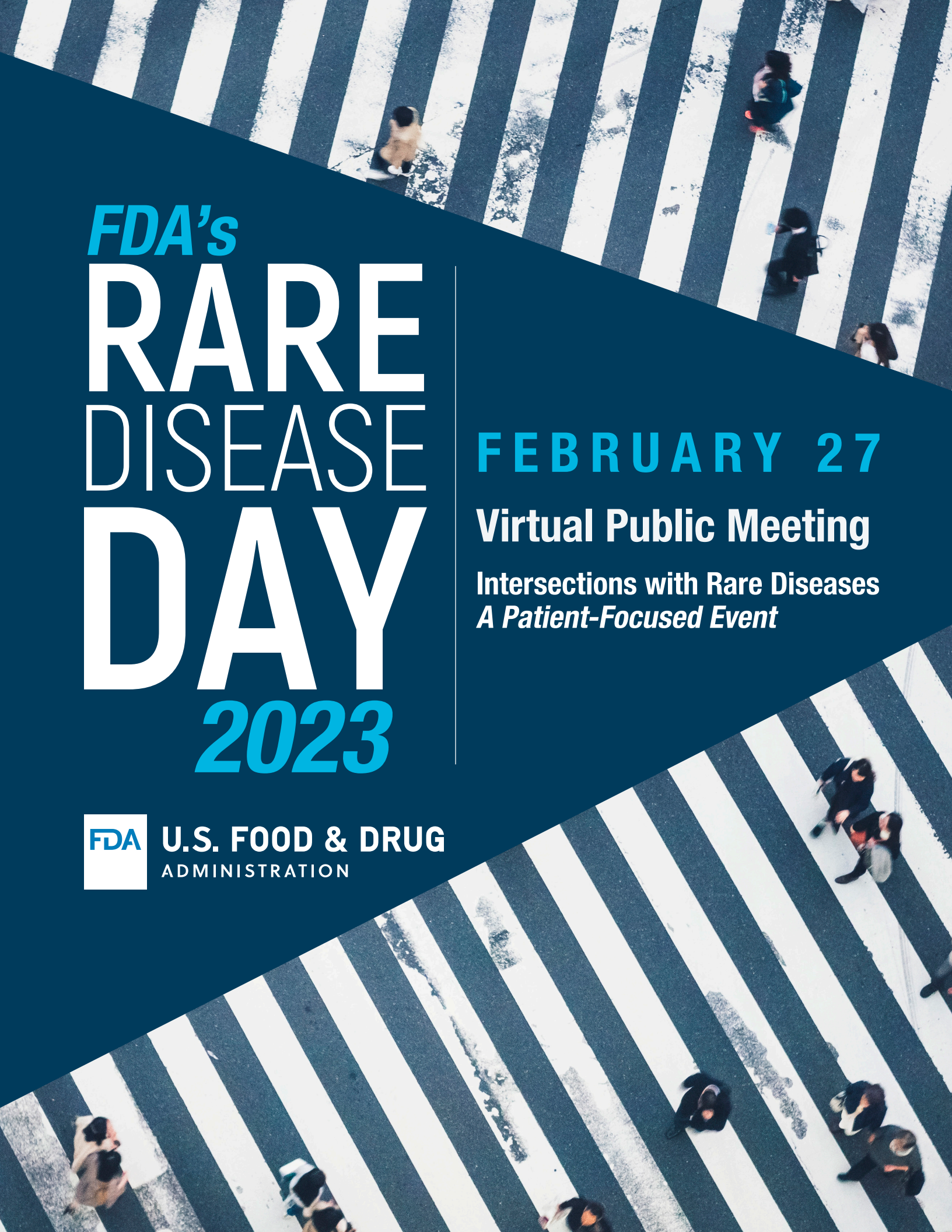


FDA's RARE DISEASE DAY 2023

FEBRUARY 27

Virtual Public Meeting

Intersections with Rare Diseases
A Patient-Focused Event

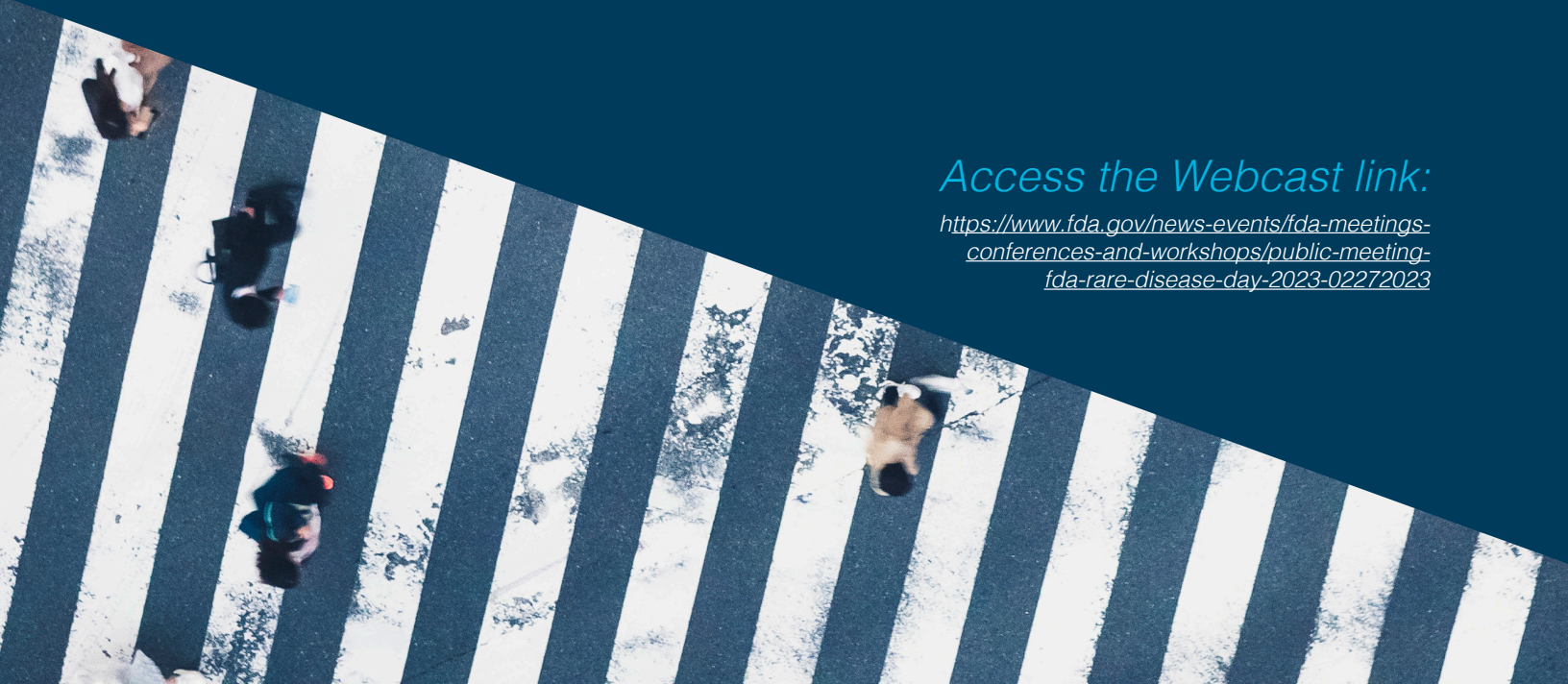
U.S. FOOD & DRUG ADMINISTRATION

Welcome to FDA's Rare Disease 2023 Virtual Public Meeting

Contents

Access the Webcast link:

https://www.fda.gov/news-events/fda-meetings-conferences-and-workshops/public-meeting-fda-rare-disease-day-2023-02272023



Welcome

FDA'S RDD 2023 Theme: "Intersections with Rare Diseases"

Welcome to FDA's Rare Disease Day 2023! Our event is dedicated to rare disease patients, their families, care partners, and health care providers.

This year, our theme is: "Intersections with Rare diseases—a Patient Focused Event." We will share four different topics that "intersect" with rare diseases:

- Clinical trial challenges with small patient populations focusing on children and improving diversity;
- FDA initiatives to advance product development for rare diseases;
- Opportunities for patient engagement with FDA—*Getting connected. Staying Involved;* and
- The role for medical students in shaping rare disease care.

At our lunch break, we are presenting videos from FDA's project called *Stories from the Rare Disease Community.* Our conference concludes with time to hear from audience members during the Open Public Comment Period.

This year's event is also special in another way—it is the 40th anniversary of the Orphan Drug Act. The enactment of the Orphan Drug Act in 1983 was a seminal legislative event—part of a decades long Congressional effort to ensure that everyone in this country with an illness has access to safe and effective medicines—and rare disease drug development has greatly accelerated since its enactment. Core to the Orphan Drug Act is the concept of equity: whether the disease is common or rare, everyone deserves treatment. Today, many countries have embraced this concept and enacted laws to encourage the development of orphan products. The Orphan Drug Act is one of the brightest lights that shine on efforts to improve public health, aspiring to ensure that all patients with rare diseases will one day have the diagnostics and therapies they need.

Meeting Agenda

9:00 a.m. **Welcoming Remarks**

Robert M. Califf, MD, *Commissioner, U.S. Food and Drug Administration*

Sandra Retzky, DO, JD, MPH, *Director, Office of Orphan Products Development, Office of the Commissioner*

9:10 a.m. **RDD at FDA Overview**

Lewis Fermaglich, MD, *Office of Orphan Products Development, Office of the Commissioner*

Shira Strongin, *ORISE Fellow, Office of Medical Policy, Center for Drug Evaluation and Research*

9:15 a.m. **Understanding challenges of clinical trials with small patient populations: important considerations for pediatrics and improving diversity**

Session Overview:
FDA experts discuss two important challenges with clinical trials for rare diseases—including children and ensuring diverse study populations. Enrolling children in clinical trials requires additional safeguards and ethical considerations.

FDA experts will also discuss the importance of having diverse, equitable, and inclusive study populations in clinical trials. As an example of improving inclusiveness, we will describe an FDA-sponsored study to adapt and validate a commonly used endpoint in amyotrophic lateral sclerosis (ALS) clinical studies for remote use. 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.

Moderator:
Kerry Jo Lee, MD, *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*

- **Ethical Considerations for Clinical Investigations of Medical Products Involving Children**

  Donna Snyder, MD, *Senior Pediatric Ethicist and Team Leader in the Office of Pediatric Therapeutics, Office of the Commissioner*

  Elizabeth Hart, MD, *Branch Chief, General Medical Branch 1, Division of Clinical Evaluation and Pharmacology/Toxicology, Office of Therapeutic Products, Center for Biologics Evaluation and Research*

  Martha Donoghue, MD, *Associate Director for Pediatric Oncology and Rare Cancers, Oncology Center of Excellence, Office of the Commissioner*

- **The importance of diversity, equity, and inclusion in clinical trials**

  Lola Fashoyi-Aje, MD, MPH, *Deputy Division Director & Associate Director, Oncology Center of Excellence*

- **Improving diversity in clinical trials: adaptation and validation of the ALS Functional Rating Scale for remote use—an FDA sponsored study**

  Michelle Campbell, PhD, *Associate Director, Stakeholder Engagement and Clinical Outcomes, Office of Neuroscience, CDER*

  Fraser D. Bocell, MEd, PhD, *Clinical Outcome Assessment Reviewer, Patient Science and Engagement Team, Center for Devices and Radiological Health*

- **Q/A 15 min**

10:45 a.m. **FDA initiatives to advance medical product development for rare diseases**

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

Moderator:
Hilary Marston, MD, MPH, *FDA Chief Medical Officer, Office of the Commissioner*

Panelists:

- **Bespoke Gene Therapy Consortium**
  Peter Marks, MD, PhD
  Director, Center for Biologics Evaluation and Research
- **Profound Vision Loss: Expediting Innovation of Bioelectronic Implants for Vision Restoration**
  Malvina Eydelman, MD
  Director, Office of Health Technology 1 / Ophthalmic, Anesthesia, Respiratory, ENT, & Dental Devices, Office of Product Evaluation and Quality, Center for Devices and Radiological Health
- **Accelerating Rare Disease Cures (ARC)**
  Kerry Jo Lee, MD, *Associate Director for Rare Diseases, Rare Diseases Team*
- **Project Catalyst**
  Jeff Summers, MD
  Associate Director for Translational Sciences, Offices of Oncologic Diseases, Center for Drug Evaluation and Research
- **Regulatory Science Research Supporting Rare Diseases**
  Jessica Hawes, PhD
  Deputy Director, Division of Systems Biology
  National Center for Toxicological Research
- **Q/A 15 min**

Meeting Agenda *– continued*

12:00 p.m. **Lunch Break and Fireside Chat with Videos of Patient Stories with Rare Diseases**
Rear Admiral Richardae Araojo, *Associate Commissioner for Minority Health and Director of the Office of Minority Health and Health Equity, and* ***Dr. Sandra Retzky,*** *Director, Office of Orphan Products Development, Office of the Commissioner*

1:00 p.m. **Engaging with FDA— *"Getting connected. Staying involved."***

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 the rare disease patient community will share their experiences engaging with patient-oriented programs and activities at FDA. This panel includes discussion of patient engagement in the design and conduct of device studies. An FDA ORISE Fellow will present research results on shared experiences among patients with rare diseases using data from FDA Patient engagement sessions. Patients will provide their perspectives on engaging with FDA.

Moderator:
Wendy Slavit, MPH, *Senior Health Scientist, Office of Patient Affairs, Office of the Commissioner*

Panelists:

- **Opportunities for people living with rare diseases to engage with FDA Center for Devices and Radiological Health**

  Michelle Tarver, MD, PhD, *Deputy Director, Office of Strategic Partnerships and Technology Innovation, Center for Devices and Radiological Health*

- **Qualitative analysis of shared experiences among patients with rare diseases: Insights from FDA patient engagement sessions**

  Catherine Botescu Mease, PharmD, *ORISE Fellow, Office of Orphan Products Development, Office of the Commissioner*

Patient Community Panelists:

- ***Julie Flygare, JD***
  Patient and Patient Organization leader
  Project Sleep
  Person living with narcolepsy and President & CEO of Project Sleep

- ***Brian Harman***
  Patient Organization leader
  United Mitochondrial Disease Foundation
  President & CEO of United Mitochondrial Disease Foundation

- ***Katie Jackson***
  Caregiver and Patient Organization leader
  Help 4 HD International
  CEO of Help 4 HD International, Huntington's and Juvenile Huntington's Disease
- **Q/A 15 min**

2:30 p.m. **Break**

2:45 p.m. **Spotlighting the Zebras: A Role for Medical Students in Shaping Rare Disease Care**

Session Overview:
A panel of medical students will discuss the gap in medical school education and rare diseases. They advocate for more training in rare diseases during medical school and describe actions taken to increase their own knowledge and experience.

Moderator:
Janet Woodcock, MD, *FDA Principal Deputy Commissioner, Office of the Commissioner*

- ***Vinay Ayyappan,*** *MD/PhD student, Perelman School of Medicine, University of Pennsylvania, Philadelphia, Pennsylvania*
- ***Elizabeth Gonzalez,*** *Medical Scientist Training Program, Perelman School of Medicine at the University of Pennsylvania, Philadelphia, Pennsylvania*
- ***Émilie Pichette,*** *MDCM Program, Faculty of Medicine and Health Sciences, McGill University, Montréal, Quebec, Canada*
- ***Kimberley E. Steele, MD, PhD,*** *FACS, FASMBS, DABOM*
  Surgeon-scientist, Rare Mom Advocate
- ***Denise M. Adams, MD,*** *Director of the Complex Vascular Anomalies Program, Alan R. Cohen Endowed Chair in Pediatrics, Professor of Pediatrics at the University of Pennsylvania Perelman School of Medicine*
- **Q/A 10 min**

3:45 p.m. **Open Public Comment Period**

Public signs up through event registration to speak for three minutes. Maximum number is 18 people to allow some transition between speakers

Moderator:
Catherine Park, MS, *Health Scientist, Office of Orphan Products Development, Office of the Commissioner*

4:45 p.m. **Closing Remarks & Adjournment**

Speaker and Moderator Biographies *(in order of appearance)*

Robert M. Califf, MD, Commissioner, U.S. Food and Drug Administration

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.

Sandra Retzky, DO, JD, MPH, Director, Office of Orphan Products Development, 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, 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.

Lewis Fermaglich, MD, Office of Orphan Products Development, Office of the Commissioner

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.

Shira Strongin, ORISE Fellow, Office of Medical Policy, Center for Drug Evaluation and Research

Shira Strongin received her Bachelor of Arts degree in Political Communications at George Washington University. Currently, she is a first-year graduate student at Georgetown University getting her Master of Science in clinical and translational research with a concentration in biomedical and mechanistic studies. For the past three years, Shira has also been an ORISE Fellow at FDA's Center for Drug Evaluation and Research working on a project collecting real world evidence on drug repurposing—the identification of new uses for "old" drugs that are outside the scope of the original indication. This is a project close to her heart because, as rare disease patient, it was a repurposed drug that saved her life.

Shira is an advocate for patients and families with rare diseases. Her notable keynote addresses include—Raise Hell & Save Yourself—at the Tourette's Association of America Youth Ambassador Dinner and—The Young Adult Voice: Advocacy and Autonomy—at the XP Family Support Conference. Shira's awards include the Global Genes RARE Champion of Hope Teen Advocate, Amelia Moore Sparkle Award for Compassionate Advocacy, and the RAREVoice Award for Teen Federal Advocate.

Kerry Jo Lee, MD, 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 Biologics 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 works to promote their mission to facilitate, support, and accelerate the development of drugs and therapeutic biologics for rare diseases and serves as the program management office for CDER's Accelerating Rare diseases Cures (ARC) Program. Dr. Lee joined the FDA as a medical officer in 2014 with the former Division of Gastroenterology and Inborn Errors Products, OND, CDER. Dr. Lee then moved to a position as a clinical advisor for the Office of New Drug Policy, CDER, where she served as a lead in the areas of benefit-risk assessment, modernization efforts (including the integrated review for marketing applications), and real-world data/evidence programming before serving in her current position.

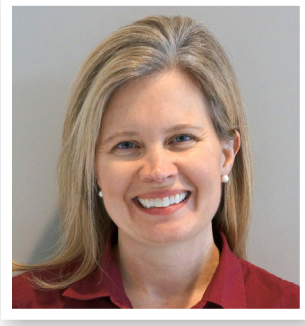
Dr. Lee is a pediatric gastroenterologist/hepatologist and a graduate of Princeton University and the New York University School of Medicine with an honors degree conferred in microbiology. She completed her residency in pediatrics at the Children's Hospital of Los Angeles followed by a post-doctoral clinical fellowship in Pediatric Gastroenterology, Hepatology, and Nutrition at Columbia University College of Physicians and Surgeons in New York. Dr. Lee maintains a steadfast interest in international policy and bioethics and worked for several years at the former National Bioethics Advisory Commission on reports advising the executive branch on ethical and policy issues in both international and domestic clinical trials.

Donna Snyder, MD, Senior Pediatric Ethicist and Team Leader in the Office of Pediatric Therapeutics, Office of the Commissioner

Donna L. Snyder, MD, MBE is Team Leader for the Ethics and International Team and Senior Pediatric Ethicist in the Office of Pediatric Therapeutics (OPT) in the Office of the Commissioner (OC) at the U.S. Food and Drug Administration (FDA). She is a standing member of the FDA Pediatric Review Committee (PeRC) and the FDA Institutional Review Board (IRB). She oversees the Pediatric Ethics consultation service and OPT international outreach activities, such as those related to the Pediatric Cluster and the annual Pharmaceuticals and Medical Devices Agency (PMDA) of Japan and the FDA Pediatric Review Seminar. Dr. Snyder joined FDA in 2012, serving as a medical officer and acting team lead in the Division of Pediatric and Maternal Health within the Office of New Drugs before joining the OPT Pediatric Ethics Team in 2016. Dr. Snyder received her MD from the University of Virginia, completed her internship and residency at the University of Maryland Hospital, her chief residency at Sinai Hospital in Baltimore, a Pediatric Academic Development Fellowship at Johns Hopkins Hospital and a Masters in Bioethics from the University of Pennsylvania. She is board certified in Pediatrics. Prior to joining the FDA in 2012, her wide-ranging experience included general pediatric practice, work in research ethics, for both an independent IRB and the National Institute of Child Health and Human Development (NICHD) IRB, clinical research, and consulting for the pharmaceutical industry.

Elizabeth Hart, MD, Branch Chief, General Medical Branch 1, Division of Clinical Evaluation and Pharmacology/Toxicology, Office of Tissues and Advanced Therapies, Center for Biologics Evaluation and Research

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.



Martha Donoghue, MD, Associate Director for Pediatric Oncology and Rare Cancers, Oncology Center of Excellence, Office of the Commissioner

Martha Donoghue, MD 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.

Lola Fashoyi-Aje, MD, MPH, Deputy Division Director & Associate Director, Oncology Center of Excellence, Office of the Commissioner

Lola A. Fashoyin-Aje, MD, MPH, is a medical oncologist and Deputy Director in the Division of Oncology 3 (DO3) in the Office of Oncologic Diseases (OOD) at the Center for Drug Evaluation and Research- Food and Drug Administration (FDA). In this role, she provides scientific and regulatory policy guidance and oversight to multidisciplinary teams reviewing drugs and biologics under development for the treatment of solid tumor malignancies. Dr. Fashoyin-Aje is also an Associate Director at the FDA Oncology Center of Excellence at the FDA, where she leads scientific and policy initiatives to address clinical and regulatory science and policy issues impacting oncology drug development. Prior to joining the FDA, Dr. Fashoyin-Aje completed her undergraduate and graduate training at Columbia University and Yale University, respectively, and received her M.D. degree from the University of Rochester School of Medicine and Dentistry. She completed postgraduate training in internal medicine and medical oncology at Johns Hopkins.

Michelle Campbell, PhD., Associate Director, Stakeholder Engagement and Clinical Outcomes, Office of Neuroscience, CDER

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

Fraser D. Bocell, MEd, PhD, Clinical Outcome Assessment Reviewer, Patient Science and Engagement Team, Center for Devices and Radiological Health

Fraser D. Bocell, MEd, PhD is a Psychometrician and Clinical Outcome Assessment Reviewer with the Patient Science and Engagement Team in Center for Devices and Radiological Health at the US FDA. At CDRH he provides expertise and training, as well as develops policy on the evaluation and use of COAs in regulatory decision-making. Prior to joining the FDA, he published on the quantitative and qualitative development and evaluation of PROMs, as well as providing statistical expertise to other projects. Dr. Bocell is an expert in psychometric methods and an applied statistician by training, specializing in latent variable models and validity theory. He continues to explore new methods for developing and evaluating COAs and seeks to improve the relevance and utility of COAs in regulatory decision making.

Hilary Marston, MD, MPH
FDA Chief Medical Officer, Office of the Commissioner

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

Peter Marks, MD, PhD
Director, Center for Biologics Evaluation and Research, FDA

Peter Marks received his graduate degree in cell and molecular biology and his medical degree at New York University and completed Internal Medicine residency and Hematology/Medical Oncology training at Brigham and Women's Hospital in Boston. He has worked in academic settings teaching and caring for patients and in industry on drug development. He joined the FDA in 2012 as Deputy Center Director for CBER and became Center Director in 2016.

Malvina B. Eydelman, MD
Director, Office of Health Technology 1 / Ophthalmic, Anesthesia, Respiratory, ENT, & Dental Devices, Office of Product Evaluation and Quality, Center for Devices and Radiological Health

Dr. Eydelman is currently the Director of Office of Ophthalmic, Anesthesia, Respiratory, ENT and Dental Devices.

For over 20 years, as an Expert Medical Officer, Senior Medical Advisor, Director of the Division of Ophthalmic, Neurological and Ear, Nose and Throat (ENT) Devices and Director of the FDA's Pilot Office of Ophthalmic, Dental, Respiratory, ENT, Anesthesia and Sleep Devices, Dr. Eydelman has played a key role in assuring the safety and effectiveness of medical devices.

Dr. Eydelman guided development of more than 50 international and national standards, oversaw development of numerous regulations and guidance; and convened over 30 public meetings of FDA Medical Device Committees. She originated numerous symposia and workshops to facilitate device innovation and has been instrumental in expediting development of novel endpoints for clinical trials of pioneering technologies. Dr. Eydelman has organized multi-stakeholder public- private partnerships and spearheaded many clinical and laboratory studies designed to improve the safety of medical devices.

Dr. Eydelman received her M.D. degree from Harvard Medical School and a Doctorate in Health Sciences and Technology from Massachusetts Institute of Technology (M.I.T.). M.I.T. Sloan School of Management recently awarded Dr. Eydelman the Advanced Certificate for Executives in Management, Innovation and Technology. Dr. Eydelman has been granted a U.S. patent, published more than 100 peer-reviewed articles, book chapters, and monographs and presented over 200 lectures worldwide.

Jeff Summers, MD,
Associate Director for Translational Sciences, Offices 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.

Jessica Hawes, PhD,
Deputy Director, Division of Systems Biology, National Center for Toxicological Research

Dr. Hawes has been with NCTR since 2019, where she serves as the Division of Systems Biology Deputy Director, leading research directions, management, collaborations (internal FDA & external), initiatives, and budgetary allocations and procurement. Dr. Hawes has 10 years of experience conducting drug review and risk assessments for Investigative New Drugs (INDs) and market New Drug Applications (NDAs) within the Office of New Drugs (OND) at the Center for Drug Evaluation and Research (CDER), including safety margin determinations, translation of risk, clinical dose/dosing recommendations, clinical trial safety monitoring, and drug labeling. Dr. Hawes is an active member of numerous scientific subcommittees and working groups across the Agency, and has been invited to chair and/or give dozens of lectures at academic, government, and scientific forums at both national and international levels. She is also a graduate of the competitive FDA Leadership Development Program, with certification in Executive Leadership from the FDA and the American University School of Public Affairs. Prior to joining the FDA, Dr. Hawes received a Ph.D. in Pharmacology from Yale University, conducted brain cancer research as a Postdoctoral Fellow at the National Cancer Institute, and received numerous scientific Young Investigator Awards from multiple organizations, including the National Research Council. She received a Bachelor of Science degree from Weber State University in 1999, majoring in Chemistry with a minor in Physics, where she was recipient of the Graduate of the Year Award.

Rear Admiral Richardae Araojo serves as the Associate Commissioner for Minority Health and Director of the Office of Minority Health and Health Equity at the U.S. Food and Drug Administration (FDA). In this role, RDML Araojo provides leadership, oversight, and direction on minority health and health disparity matters for the Agency. RDML Araojo previously served as the Director of the Office of Medical Policy Initiatives in FDA's Center for Drug Evaluation and Research (CDER), where she led a variety of broad-based medical and clinical policy initiatives to improve the science and efficiency of clinical trials and enhance professional and patient labeling. RDML Araojo joined FDA in 2003, where she held several positions in CDER. RDML Araojo received her Doctor of Pharmacy Degree from Virginia Commonwealth University, completed a Pharmacy Practice Residency at University of Maryland, and earned a Master's degree in Pharmacy Regulation and Policy from the University of Florida.

Wendy Slavit, MPH, Senior Health Scientist, Patient Affairs, 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 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 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).

Michelle E. 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

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.

Catherine Botescu Mease, PharmD, ORISE Fellow, Office of Orphan Products Development, Office of the Commissioner

Catherine Mease, PharmD is an ORISE post-doctoral fellow in the Office of Orphan Products Development, in the Office of the Commissioner at the US Food and Drug Administration. The ORISE fellowship program, administered by the Oak Ridge Institute for Science and Education, offers opportunities for postgraduates to conduct research with federal agencies to advance professional development in government service. Dr. Mease completed her B.S. in Physiology and Neurobiology at the University of Maryland, College Park and her PharmD at the University of Maryland, School of Pharmacy.

During her time as an ORISE fellow, Dr. Mease's research efforts include patient-centered research to identify current unmet needs in rare diseases and understanding the impact of orphan drug designations on medical product development.

Julie Flygare, JD, President & CEO of Project Sleep

Julie Flygare, JD, serves as President & CEO of Project Sleep. Flygare is an internationally recognized patient-perspective leader, an accomplished advocate, and the award-winning author of Wide Awake and Dreaming: A Memoir of Narcolepsy. In March 2022, she delivered the TEDx Talk, "What Can You Learn from a Professional Dreamer?"

Since receiving a diagnosis of narcolepsy with cataplexy in 2007, Flygare advanced her leadership in the sleep and healthcare space through speaking engagements, publications, earned media, collaborations, and advocacy and awareness initiatives. Prior to accepting her current role as President & CEO of Project Sleep, Flygare served as President of Project Sleep's Board of Directors, while also gaining invaluable experience in marketing and philanthropy at the Pancreatic Cancer Action Network and City of Hope. Additionally, she served on the National Institutes of Health's Sleep Disorders Research Advisory Board from 2012 – 2015. Flygare received her B.A. from Brown University in 2005 and her J.D. from Boston College Law School in 2009, focusing on health law, policy, and rare disease drug development.

Brian Harman, President and CEO of the United Mitochondrial Disease Foundation

Brian Harman is President and CEO of the United Mitochondrial Disease Foundation (UMDF). Prior to his arrival at UMDF in July 2018, Mr. Harman joined UMDF from Children's Hospital of Pittsburgh Foundation where he served as senior director of Corporate Engagement and Community Partnerships since 2014. Prior to that position, he spent nearly a decade as director of Annual Giving at Nationwide Children's Hospital in Columbus, Ohio. Mr. Harman also served as executive director as well as other roles within development and grassroots advocacy for the American Cancer Society in Ohio. He is a graduate of Ohio University, from which he has a degree in Interpersonal & Political Communication.

Katie Jackson, CEO of Help 4 HD International

Katie Jackson is the CEO of Help 4 HD International, a leading organization dedicated to supporting individuals impacted by Huntington's disease and Juvenile onset Huntington's disease. She has been with the organization for over ten years, serving as Vice President before taking on the role of CEO in 2017. Katie Jackson was her husband Michael Hinshaw's caregiver throughout his 14-year-long battle with living with Huntington's disease.

In addition to her work with the organization, she has also educated law enforcement, first responders, and other professionals on subjects pertaining to Huntington's disease. Katie is an experienced advocate, having written and contributed to several books, brochures, and publications about Huntington's disease. She also directed a documentary about Juvenile Onset Huntington's disease called "The Warriors," which won several film festival awards.

Janet Woodcock, MD, FDA Principal Deputy Commissioner

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.

Vinay Ayyappan, MD/PhD student, Perelman School of Medicine, University of Pennsylvania, Philadelphia, Pennsylvania

Vinay Ayyappan is an MD/PhD student at the University of Pennsylvania and co-founder of the Penn Medicine Rare Disease Interest Group. His clinical interests include pediatrics and medical genetics, with an interest in improving accessibility of diagnostic resources and clinical knowledge in context of rare disease. His research in the lab of Dr. Arjun Raj focuses on non-genetic heterogeneity in tissues with the goal of bettering our understanding of cell differentiation and organ development, as well as of how this process can be corrupted in congenital disease and cancer. This year, he is pursuing a research fellowship in the Department of Radiology at the University of Cambridge, working to improve our imaging repertoire for cancer.

Elizabeth Marie Gonzalez, Medical Scientist Training Program, Perelman School of Medicine at the University of Pennsylvania, Philadelphia, Pennsylvania

Elizabeth Gonzalez is an MD/PhD student at the University of Pennsylvania and co-founder of Penn Rare Disease Interest Group. Her clinical and research interests involve rare pediatric neurogenetic disorders. She is currently a graduate student at the Children's Hospital of Pennsylvania, working with Dr. Elizabeth Bhoj on TBCK syndrome. As a physician-scientist, she aspires to join drug discovery efforts for rare pediatric diseases. She is also passionate about making advances in rare diseases accessible globally.

Émilie Pichette, MDCM Program, Faculty of Medicine and Health Sciences, McGill University, Montréal, Quebec, Canada

Émilie Pichette is a medical student at McGill University in Montreal, Canada. She previously served as president of the McGill University Rare Disease Interest Group (RareDIG) in 2020-2021 and is currently acting as a senior advisor. She is motivated to raise awareness among medical trainees about the existing gaps within the care of patients with rare disorders. Ultimately, she hopes to translate this into tangible action to improve their experiences within the healthcare system.

Kimberley E. Steele, MD, PhD, FACS, FASMBS, DABOM Surgeon-scientist, Rare Mom Advocate

Kimberley Steele, MD, PhD completed surgical residency at Hershey, Penn State School of Medicine and a Minimally Invasive and Bariatric Surgical Fellowship at Johns Hopkins University School of Medicine. Promoted to rank of Associate Professor and contributing to all facets of academia, she directed the adolescent bariatric surgical program, the bariatric, obesity and metabolic research program and led the surgical simulation and education curriculum.

Dr. Steele is a wife and mom to Michael age 13, Matthew 12. At age 6, after a 14-month diagnostic odyssey, Michael was diagnosed with kaposiform lymphangiomatosis (KLA), an ultra-rare, life-threatening vascular anomaly, with no cure. Having navigated the healthcare system as a rare disease mom through the eyes of a physician, Kimberley took a career detour dedicating her collective academic skills to the rare disease community. Volunteering as an advisor to undergraduate and graduate students, residents and fellows, nursing and medical students, she has encouraged the creation of student Rare Disease Interest Groups (RareDIGs) across North America. Further, Dr. Steele recently founded a 501(c)(3), CaRAVAN - Collaborative Research Advocacy for Vascular Anomalies Network. Communities together on a journey to cures. Filling an unmet need, CaRAVAN is devoted to accelerating research discovery through relentless collaboration of all stakeholders; pooling resources to support researchers and clinicians so that they may find improved therapies for people living with vascular anomalies.

Denise M. Adams, MD
Director of the Complex Vascular Anomalies Program
Alan R. Cohen Endowed Chair in Pediatrics
Professor of Pediatrics at the University of Pennsylvania Perelman School of Medicine

Dr. Adams is a prominent pediatric hematologist-oncologist at the Children's Hospital of Philadelphia (CHOP) and a Professor of Pediatrics at the University of Pennsylvania Perelman School of Medicine. She holds the Alan R. Cohen Endowed Chair in Pediatrics at CHOP.

Dr. Adams is also the Director of the Comprehensive Vascular Anomalies Program (CVAP), a cutting-edge, multidisciplinary program that seeks breakthrough treatments and cures for children, adolescents and young adults with rare, life-threatening tumors and malformations of the vasculature, which includes the arteries, veins, capillaries, lymphatics, and combined lesions.

As a leading expert in the field, Dr. Adams is actively engaged in clinical and translational research on complex vascular anomalies and regularly publishes studies in leading journals on the topic. She has received numerous awards, for her teaching and mentorship and for her research and clinical accomplishments. Hospitals, universities, and conferences around the world have invited her to speak on the topic of vascular anomalies, recognizing her expertise in this area.

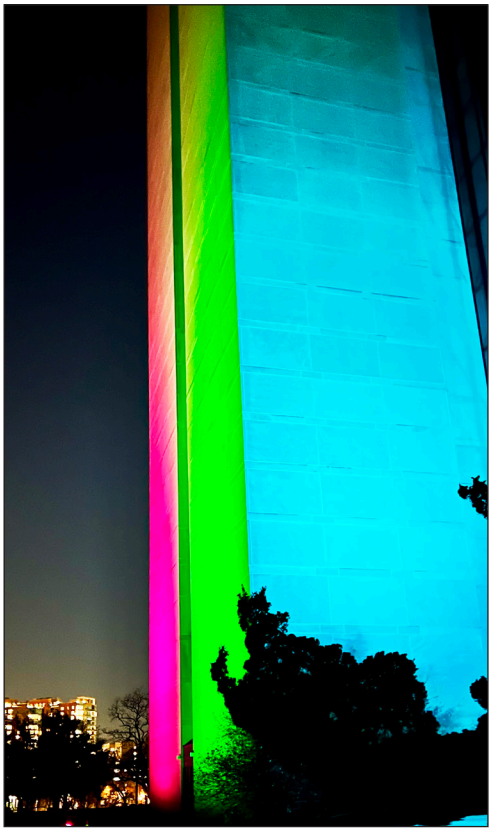
Catherine Park, MS, Health Scientist, Office of Orphan Products Development, Office of the Commissioner

Catherine Park, M.S., is a Health Scientist in FDA's Office of Orphan Products Development in in the Office of the Commissioner. She supports two congressionally mandated designation programs incentivizing product development for rare diseases. These include the Rare Pediatric Disease Designation Program and Humanitarian Use Device Program. Catherine also assists with administration of the Pediatric Device Consortium—a congressionally mandated grant program to incentivize device development specific to the needs of children supporting a critical public health need. Catherine is one of the key planners for FDA's Rare disease Day conferences.

Catherine received her Bachelor of Science degree in Community and Behavioral Health at the University of Maryland School of Public Health: She holds a Master of Science degree in Management of Health Informatics and Analytics from the University of George Washington, Milken Institute of Public Health.

Shining a Light on Rare Diseases

In observance of Rare Disease Week—February 27, 2023 to March 3, 2023—FDA's White Oak Building 1 in Silver Spring Maryland will be illuminated daily from dusk to dawn.



In recognition of Rare Disease Day, NIH lit up Building 1 (left) and Building 38A (right) in Rare Disease Day colors.

Credit: National Center for Advancing Translational Sciences